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INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

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As confidentially submitted to the Securities and Exchange Commission on April 25, 2018 as Amendment No. 2 to the draft registration statement.

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

SCHOLAR ROCK HOLDING CORPORATION
(Exact name of registrant as specified in its charter)

Delaware	**2836**	**82-3750435**
(State or other jurisdiction of incorporation or organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification Number)

620 Memorial Drive, 2nd Floor
Cambridge, MA 02139
(857) 259-3860
(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

Nagesh K. Mahanthappa
President and Chief Executive Officer
620 Memorial Drive, 2nd Floor
Cambridge, MA 02139
(857) 259-3860
(Name, address, including zip code, and telephone number, including area code, of agent for service)

Copies of all communications, including communications sent to agent for service, should be sent to:

Kingsley L. Taft **Laurie A. Burlingame** **Goodwin Procter LLP** **100 Northern Ave.** **Boston, MA 02210** **(617) 570-1000**	**Junlin Ho** **Scholar Rock Holding Corporation** **620 Memorial Drive, 2nd Floor** **Cambridge, MA 02139** **(857) 259-3860**	**Marc A. Recht** **Brent B. Siler** **Nicole C. Brookshire** **Alison A. Haggerty** **Cooley LLP** **500 Boylston St.** **Boston, MA 02116** **(617) 937-2300**

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, as amended, 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, a smaller reporting company or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large Accelerated Filer o	Accelerated Filer o	Non-Accelerated Filer ☒ (Do not check if a smaller reporting company)	Smaller Reporting Company o 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 to Section 7(a)(2)(B) of the Securities Act. ☒

CALCULATION OF REGISTRATION FEE

Title of each Class of Securities to be Registered	Proposed Maximum Aggregate Offering Price[1][2]	Amount of Registration Fee
Common Stock, par value $0.001 per share		

(1) Estimated solely for the purpose of calculating the registration fee pursuant to Rule 457(o) under the Securities Act of 1933, as amended.

(2) Includes the offering price of shares that the underwriters may purchase pursuant to an option to purchase additional shares.

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 that specifically states that this registration statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933, as amended, or until this registration statement shall become effective on such date as the Commission, acting pursuant to said Section 8(a), may determine.

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SUBJECT TO COMPLETION, DATED , 2018

PRELIMINARY PROSPECTUS

Shares

SCHOLAR ROCK

Scholar Rock Holding Corporation

Common Stock

We are offering shares of our common stock. This is our initial public offering and no public market currently exists for our common stock. We anticipate that the initial public offering price will be between $ and $ per share. We have applied to list our common stock on The Nasdaq Global Market under the symbol "SRRK."

Investing in our common stock involves risks. See "Risk Factors" beginning on page 13 of this prospectus.

We are an "emerging growth company" as defined under U.S. federal securities laws and will be subject to reduced public company reporting requirements.

Neither the Securities and Exchange Commission nor any state securities commission 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.

	Per Share	Total
Initial public offering price	$	$
Underwriting discounts and commissions[(1)]	$	$
Proceeds, before expenses, to us	$	$

(1) See "Underwriting" beginning on page 169 of this prospectus for additional information regarding total underwriter compensation.

Delivery of the shares of common stock is expected to be made on or about , 2018. We have granted the underwriters an option for a period of 30 days from the date of this prospectus to purchase additional shares of our common stock. If the underwriters exercise the option in full, the total underwriting discounts and commissions payable by us will be $, and the total proceeds to us, before expenses, will be $.

Joint Book-Running Managers

Jefferies **Cowen** **BMO Capital Markets**

Co-Manager

Wedbush PacGrow

Prospectus date , 2018

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We and the underwriters have not authorized anyone to provide any information or to make any representations other than those contained in this prospectus or in any free writing prospectuses we have prepared. We and the underwriters take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may provide you. We are offering to sell, and seeking offers to buy, shares of common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or of any sale of the common stock.

PROSPECTUS SUMMARY

This summary highlights information contained elsewhere in this prospectus. Before investing in our common stock, you should carefully read this entire prospectus, including our consolidated financial statements and the related notes included elsewhere in this prospectus and the section titled "Risk Factors." As used in this prospectus, unless the context otherwise requires, references to the "company," "we," "us" and "our" refer to Scholar Rock Holding Corporation together with its consolidated subsidiaries.

Overview

We are a biopharmaceutical company focused on the discovery and development of innovative medicines for the treatment of serious diseases in which signaling by protein growth factors plays a fundamental role. Our newly elucidated understanding of the molecular mechanisms of growth factor activation enabled us to develop a proprietary platform for the discovery and development of monoclonal antibodies that locally and selectively target these signaling proteins at the cellular level. We believe this approach, acting in the disease microenvironment, avoids the historical challenges associated with inhibiting growth factors for therapeutic effect. We believe our focus on biologically validated growth factors may facilitate a more efficient development path. We are advancing our lead product candidate, SRK-015, a selective first-in-class inhibitor of the activation of the growth factor myostatin in skeletal muscle, into clinical development for the treatment of spinal muscular atrophy, or SMA. We expect to initiate a Phase 1 clinical trial in the second quarter of 2018. Utilizing our proprietary platform, we are also creating a pipeline of novel product candidates with the potential to transform the lives of patients suffering from a wide range of serious diseases, including other neuromuscular disorders, cancer, fibrosis and anemia.

Our Approach and Proprietary Platform

Our proprietary platform is designed to discover and develop monoclonal antibodies that have a high degree of specificity to achieve selective modulation of growth factor signaling. Growth factors are naturally occurring proteins that typically act as signaling molecules between cells and play a fundamental role in regulating a variety of normal cellular processes, including cell growth and differentiation. Current therapeutic approaches to treating diseases in which growth factors play a fundamental role involve directly targeting an active growth factor or its receptor systemically throughout the body and have suffered from a variety of shortcomings:

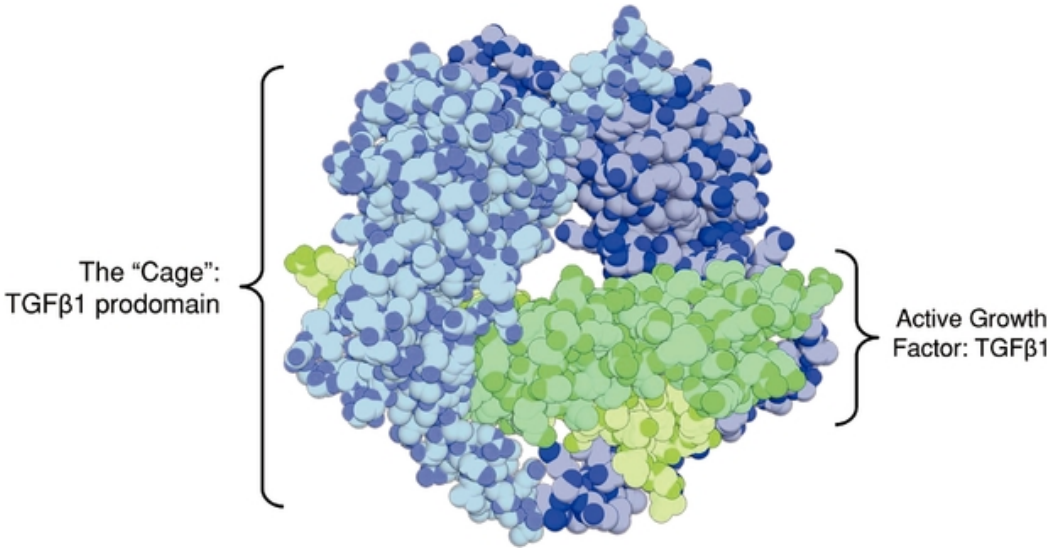
- multiple growth factors often signal through the same or overlapping sets of related receptors, making it difficult to specifically modulate one pathway over another;
- members of the same growth factor superfamily share considerable structural similarities, making it difficult to achieve specific inhibition of the targeted growth factor; this can result in broad systemic inhibition that can cause undesirable, and in many cases toxic, side effects; and
- systemic and nonselective inhibition of a growth factor can block the growth factor's role in the disease process, but can also simultaneously interfere with its normal physiological roles.

Our innovative approach is rooted in our structural biology insights into the mechanism by which certain growth factors are activated in close proximity to the cell surface, which we refer to as "supracellular activation." We integrate these insights with sophisticated protein expression, assay development and monoclonal antibody discovery capabilities. We believe our proprietary platform can address the challenges of current therapeutic approaches to treating diseases in which growth factors play a fundamental role by:

- targeting the natural activation mechanism to prevent activation of the growth factor rather than attempting to inhibit the growth factor after activation;
- achieving heightened specificity for the targeted growth factor while minimizing interactions with structurally similar and related growth factors, thereby reducing the risk of unintended systemic adverse events; and

- § targeting the disease microenvironment, where we believe we can interfere with the disease process while minimizing the effects on the normal physiological processes mediated by the same growth factors.

Unlike many other proteins that are produced and secreted by cells in a mature, or active, form, many growth factors are expressed by cells in a precursor, or latent, form. For example, TGFb1 is produced by cells as a single protein which is then enzymatically processed by the cells into two distinct and physically separated domains — the mature growth factor and the remaining portion of the original protein, referred to as the prodomain — which remain associated as part of a complex. This secreted complex is latent, or inactive, and must first be activated to carry out its normal function in a highly localized tissue or disease microenvironment. In a seminal peer-reviewed publication in 2011, Timothy A. Springer, Ph.D., one of our co-founders, elucidated a new understanding of the mechanism of supracellular activation as it applies to members of the TGFb superfamily, by solving a high resolution x-ray crystal structure of this latent form of TGFb1, as illustrated in the graphic below.



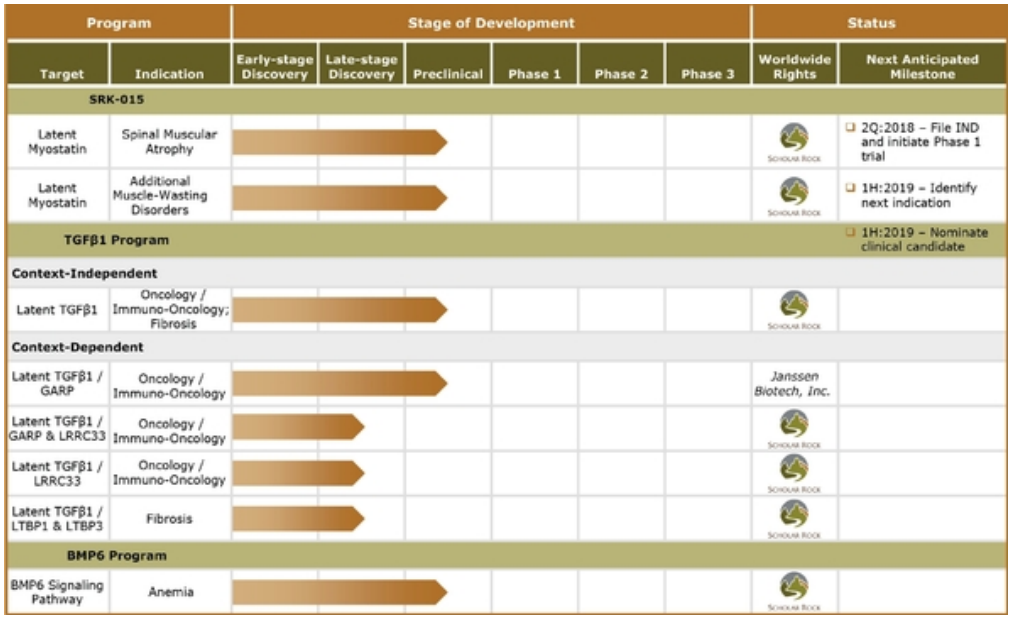
Structural representation of the latent form of TGFb1 wherein the prodomain wraps around the active growth factor.

This research explained at a molecular level why the secreted form of TGFb1 is inactive. The prodomain, though physically separated from the mature growth factor domain, forms a "cage" around the active form of TGFb1, blocking the growth factor from signaling through its receptor. Only when the cage is "unlocked" by a supracellular activation event can the growth factor be released and mediate its effects in the local microenvironment. Dr. Springer further hypothesized that this phenomenon likely holds true for most members of the TGFb superfamily, though the exact nature of the activation event, such as integrin binding or enzymatic cleavage, may differ among members of the superfamily. Importantly, while many growth factors are structurally very similar, their cages are structurally diverse, and this provides the basis for our approach to improved selectivity.

Our Pipeline Programs

Using our innovative approach and proprietary platform, we are creating a pipeline of novel product candidates that selectively inhibit the supracellular activation of growth factors believed to be important drivers in a variety of diseases, including neuromuscular disorders, cancer, fibrosis and anemia. Our proprietary platform includes (i) our know-how expressing and purifying latent protein growth factor

complexes in quantity and quality sufficient to enable antibody discovery; (ii) strategies to identify rare antibodies that selectively bind targeted latent protein growth factor complexes; and (iii) assays developed by us in which to test the highly selective antibodies' ability to modulate the activation of specific latent growth factors. In addition to such know-how, our proprietary platform is covered by two patent families, with issued patents projected to expire in 2034. We have worldwide rights to our proprietary platform and all of our product candidates, with the exception of certain early-stage antibodies that specifically inhibit the activation of TGFb1 in the context of regulatory T cells, which we licensed to Janssen Biotech, Inc., or Janssen, a subsidiary of Johnson & Johnson.



Our Lead Product Candidate and Additional Programs

SRK-015

We are advancing our lead antibody product candidate, SRK-015, a first-in-class inhibitor of the activation of myostatin, into clinical development for the treatment of SMA. Myostatin is a negative regulator of muscle mass expressed primarily in skeletal muscle tissue and a member of the transforming growth factor beta, or TGFb, superfamily, a group of more than 30 related growth factors that mediate diverse biological processes. Vertebrate animals that lack the myostatin gene display increased muscle mass and strength relative to their normal counterparts, but are otherwise healthy. We believe inhibition of the activation of myostatin may promote a clinically meaningful increase in muscle mass and strength. As a result, we have focused our initial development efforts for SRK-015 on the treatment of SMA. SMA is a rare, and often fatal, genetic disorder that typically manifests in young children, characterized by atrophy of the voluntary muscles of the limbs and trunk and dramatically reduced normal neuromuscular function. An estimated 30,000 to 35,000 patients suffer from SMA in the United States and Europe. In preclinical studies, we observed that SRK-015 promoted increased muscle mass and strength, while selectively avoiding interaction with other closely related growth factors that play distinctly different physiological roles. We believe that SRK-015 has the potential to be the first muscle-directed therapy to reverse or prevent muscle atrophy in SMA patients and could be used both as a monotherapy or in conjunction with the current standard of care. In March 2018, we filed an Investigational New Drug application, or IND, with the U.S. Food and Drug Administration, or FDA, for SRK-015, and in April, 2018, the FDA notified us that our Phase 1 first-in-human clinical trial of SRK-015 may proceed. We plan to commence our Phase 1 clinical

trial in the second quarter of 2018. The FDA has granted orphan drug designation for SRK-015 for the treatment of SMA.

TGFb1

Our second antibody program is focused on the discovery and development of highly specific inhibitors of the activation of TGFb1. TGFb1 is also a member of the TGFb superfamily, and increased signaling by TGFb1 is a key driver of a number of disease-relevant processes, including tissue and organ fibrosis, immune system evasion by cancer cells, and bone marrow fibrosis associated with hematological disorders. Historically, selectively targeting TGFb1 has been challenging due to off-target inhibition of other, closely related growth factors, TGFb2 and TGFb3. Pan-TGFb inhibition has been associated with a range of toxicities, most notably cardiac toxicity. In preclinical studies of our antibodies, we have observed inhibition of TGFb1 activation *in vitro* and immunomodulatory and antifibrotic activity in multiple disease models *in vivo*. In addition, we have completed a 28-day pilot toxicology study of our leading antibody and, to date, we have not observed any drug-related toxicity. In the same study, we tested pan-TGFb inhibitors and observed the toxicities, including cardiac toxicity that have been observed by others. We are actively evaluating a limited number of our selective inhibitors of the activation of TGFb1 in multiple disease models, and we intend to nominate a clinical candidate to initially pursue in one or more of our currently targeted indications of oncology, immuno-oncology and fibrosis, by the first half of 2019.

BMP6

Our third antibody program targets the signaling of bone morphogenetic protein 6, or BMP6, another member of the TGFb superfamily, which is involved in a diverse set of biological processes in various parts of the body. For example, in the liver, BMP6 signaling is a key controller of the body's ability to regulate iron levels. Given BMP6's important role in iron metabolism, we believe that targeting BMP6 signaling in a liver-selective fashion presents the potential to address both iron-restricted anemias and iron overload conditions. In preclinical studies of our antibodies targeting BMP6 signaling in the liver, we have observed increased iron levels in the bloodstream of healthy animals and we are now evaluating a limited number of these antibodies in disease models of iron restriction.

Our Strategy

Using our proprietary platform to unlock the therapeutic potential of targeting growth factor signaling in the disease microenvironment, our goal is to deliver novel therapies to underserved patients suffering from a wide range of serious diseases, including neuromuscular disorders, cancer, fibrosis and anemia. To achieve this goal we plan to:

- rapidly advance our lead product candidate, SRK-015, through clinical proof-of-concept;
- advance our TGFb1 program into clinical development;
- explore additional indications for our existing and emerging product candidates;
- continue to leverage our proprietary platform to expand our pipeline beyond current lead programs;
- selectively seek strategic collaborations to maximize the value of our proprietary platform and pipeline; and
- attract and retain people that share our commitment to scientific excellence and a focus on patients.

We have worldwide rights to our proprietary platform and all of our product candidates and antibodies, with the exception of certain early-stage antibodies that specifically inhibit the activation of TGFb1 in the context of regulatory T cells, which we licensed to Janssen.

We have assembled an experienced management team, board of directors, scientific founders and advisory board who bring extensive industry experience to our company. The members of our team have deep experience in discovering, developing and commercializing therapeutics with a particular focus on rare diseases, having worked at companies such as Alnylam Pharmaceuticals, Inc., Avila Therapeutics, Inc.,

Biogen, Inc. and Dyax Corp. We were founded by internationally respected scientists, Drs. Timothy A. Springer and Leonard I. Zon of Harvard Medical School and Boston Children's Hospital.

Since our inception in 2012, we have raised over $100 million through convertible preferred stock financings. Our investors include ARCH Venture Partners, Cormorant Asset Management, EcoR1 Capital, Fidelity Management and Research Company, Invus, The Kraft Group, Polaris Partners, Redmile Group and Timothy A. Springer, Ph.D.

Risks Affecting Our Business

Our business is subject to a number of risks, including risks that may prevent us from achieving our business objectives or may adversely affect our business, financial condition, results of operations, cash flows and prospects that you should consider before making a decision to invest in our common stock. These risks are discussed more fully in the section titled "Risk Factors" beginning on page 13 of this prospectus, and include the following:

- We have limited operating history, have incurred net losses in every year since our inception and anticipate that we will continue to incur net losses in the future.
- We will require additional capital to fund our operations and if we fail to obtain necessary capital, we will not be able to complete the development and commercialization of SRK-015 and any future product candidates.
- Our business is highly dependent on the success of our lead product candidate, SRK-015, as well as any future clinical candidates that are generated from our other preclinical programs. All of our product candidates will require significant additional preclinical and clinical development before we may be able to seek regulatory approval for and launch a product commercially.
- Our approach to the discovery and development of innovative medicines for the treatment of serious diseases in which signaling by protein growth factors plays a fundamental role is based on our proprietary platform, which is unproven and may not result in marketable products.
- Clinical development involves a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of any product candidates.
- We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.
- If we lose key management personnel, or if we fail to recruit additional highly skilled personnel, our ability to identify and develop new or next generation product candidates will be impaired, could result in loss of markets or market share and could make us less competitive.
- Our success depends in part on our ability to protect our intellectual property. It is difficult and costly to protect our proprietary rights and technology, and we may not be able to ensure their protection.
- We rely on third parties to conduct certain aspects of our preclinical studies and will rely on third parties to conduct our clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines or comply with regulatory requirements, we may not be able to obtain regulatory approval of or commercialize any potential product candidates.
- Our future collaborations will be important to our business. If we are unable to enter into new collaborations, or if these collaborations are not successful, our business could be adversely affected.

Corporate History

We were incorporated in 2017 under the laws of the state of Delaware as the holding company for Scholar Rock, Inc., a wholly owned subsidiary of Scholar Rock, LLC. Pursuant to the terms of a corporate reorganization that was completed on December 22, 2017, all of the equity interests in Scholar Rock, LLC were exchanged for the same number and class of newly issued securities of Scholar Rock Holding

Corporation and, as a result, Scholar Rock, LLC became a wholly owned subsidiary of Scholar Rock Holding Corporation. See the section titled "Restructuring" for additional information. Our principal executive offices are located at 620 Memorial Drive, 2nd Floor, Cambridge, MA 02139, and our phone number is (857) 259-3860. Our website address is http://www.scholarrock.com. The information contained in or accessible from our website is not incorporated into this prospectus, and you should not consider it part of this prospectus.

We own various U.S. federal trademark applications and unregistered trademarks, including our company name and our logo. All other trademarks or trade names referred to in this prospectus are the property of their respective owners. Solely for convenience, the trademarks and trade names in this prospectus are referred to without the symbols ® and ™, but such references should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto.

Implications of Being an Emerging Growth Company

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act, or the JOBS Act, enacted in April 2012. For so long as we remain an emerging growth company, we are permitted and intend to rely on certain exemptions from various public company reporting requirements, including not being required to have our internal control over financial reporting audited by our independent registered public accounting firm pursuant to Section 404(b) of the Sarbanes-Oxley Act of 2002, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and any golden parachute payments not previously approved. In particular, in this prospectus, we have provided only two years of audited financial statements and have not included all of the executive compensation related information that would be required if we were not an emerging growth company. Accordingly, the information contained herein may be different than the information you receive from other public companies in which you hold stock.

We will remain an emerging growth company until the earlier to occur of (1) the last day of 2023, (2) the last day of the fiscal year in which we have total annual gross revenues of at least $1.07 billion, (3) the last day of the fiscal year in which we are deemed to be a "large accelerated filer," under the rules of the U.S. Securities and Exchange Commission, or SEC, which means the market value of our equity securities that is held by non-affiliates exceeds $700 million as of the prior June 30th, and (4) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

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

THE OFFERING

Common stock offered by us	shares
Common stock to be outstanding immediately after this offering	shares
Option to purchase additional shares offered by us	shares
Use of proceeds	We estimate that we will receive net proceeds from the sale of shares of our common stock in this offering of approximately $ million, or $ million if the underwriters exercise their option to purchase additional shares in full, 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. We intend to use the net proceeds from this offering, together with our existing cash, cash equivalents and marketable securities, to fund research and development activities for SRK-015 through ; and to fund TGFb1, BMP6 and other preclinical research and development activities. We intend to use the remaining proceeds from this offering for working capital and other general corporate purposes. For a more complete description of our intended use of the proceeds from this offering, see "Use of Proceeds."
Risk factors	You should carefully read the "Risk Factors" section of this prospectus for a discussion of factors that you should consider before deciding to invest in our common stock.
Proposed Nasdaq Global Market symbol	"SRRK"

The number of shares of our common stock to be outstanding after this offering is based on 54,471,355 shares of our common stock (which includes 3,483,237 shares of restricted common stock) outstanding as of December 31, 2017, giving effect to the conversion of all outstanding shares of our convertible preferred stock into an aggregate of 43,135,911 shares of our common stock upon the completion of this offering, and excludes:

- 21,739 shares of common stock issuable upon the exercise of a warrant outstanding as of December 31, 2017, at an exercise price of $1.38 per share;
- 4,120,333 shares of common stock (including 1,885,156 and 1,015,000 shares of common stock issuable upon the exercise of stock options granted subsequent to December 31, 2017 at exercise prices of $2.02 and $2.51 per share, respectively) reserved for future issuance as of December 31, 2017 under our 2017 Stock Option and Incentive Plan, any unissued shares of which will cease to be available for issuance upon the completion of this offering;
- shares of our common stock that will become available for future issuance under our 2018 Stock Option and Incentive Plan upon the effectiveness of the registration statement of which this prospectus forms a part; and

- shares of our common stock that will become available for future issuance under our 2018 Employee Stock Purchase Plan upon the effectiveness of the registration statement of which this prospectus forms a part.

Unless otherwise indicated, all information in this prospectus reflects or assumes the following:

- the filing of our amended and restated certificate of incorporation and the effectiveness of our amended and restated by-laws upon the completion of this offering;
- the conversion of all outstanding shares of convertible preferred stock into an aggregate of 43,135,911 shares of common stock upon the completion of this offering;
- the conversion of the outstanding warrant to purchase 21,739 shares of convertible preferred stock into a warrant to purchase 21,739 shares of common stock;
- no exercise of outstanding options or warrants after December 31, 2017;
- a one-for- reverse split of our common stock effected on , 2018; and
- no exercise by the underwriters of their option to purchase up to additional shares of common stock in this offering.

Summary Consolidated Financial Data

The summary consolidated financial data set forth below should be read together with the consolidated financial statements and the related notes to those statements, as well as the section entitled "Management's Discussion and Analysis of Financial Condition and Results of Operations." We have derived the summary consolidated statement of operations data for the years ended December 31, 2016 and 2017 and the summary consolidated balance sheet data as of December 31, 2017 from our audited consolidated financial statements included elsewhere in this prospectus. Our historical results are not necessarily indicative of results that may be expected in the future.

	Year Ended December 31,	
	2016	2017
	(in thousands, except share, unit, per-share and per-unit data)	
Consolidated Statement of Operations Data:		
Collaboration revenue	$ 379	$ —
Operating expenses:		
Research and development	12,477	19,944
General and administrative	4,112	5,085
Total operating expenses	16,589	25,029
Loss from operations	(16,210)	(25,029)
Other income (expense):		
Interest income (expense), net	(19)	44
Other income (expense), net	22	(10)
Total other income	3	34
Net loss	$ (16,207)	$ (24,995)
Net loss per common unit, basic and diluted	$ (3.54)	
Net loss per share, basic and diluted		$ (5.36)
Weighted average common units outstanding, basic and diluted	4,576,500	
Weighted average common shares outstanding, basic and diluted		4,665,036
Pro forma net loss per share, basic and diluted (unaudited)[1]		$ (0.72)
Pro forma weighted average common shares outstanding, basic and diluted (unaudited)[1]		34,567,011

(1) Basic and diluted pro forma net loss per share give effect to the automatic conversion of all shares of convertible preferred stock into shares of common stock upon completion of this offering, assuming such conversion occurred on the later of January 1, 2017 or the original issuance dates of the convertible preferred units or convertible preferred stock.

	As of December 31, 2017		
	Actual	Pro Forma(1)	Pro Forma As Adjusted(2)
		(in thousands)	
Consolidated Balance Sheet Data:			
Cash, cash equivalents and marketable securities	$ 57,959	$ 57,959	$
Working capital(3)	54,177	54,177	
Total assets	61,637	61,637	
Convertible preferred stock	109,232	—	
Accumulated deficit	(57,525)	(57,525)	
Total stockholders' (deficit) equity	(53,522)	55,747	

(1) Pro forma amounts give effect to the automatic conversion of all of our outstanding shares of convertible preferred stock into an aggregate of 43,135,911 shares of common stock upon completion of this offering and the automatic conversion of the outstanding warrant to purchase 21,739 shares of convertible preferred stock into a warrant to purchase 21,739 shares of common stock, and the resulting reclassification of the warrant liability to additional paid-in capital.

(2) Pro forma as adjusted amounts reflect pro forma adjustments described in footnote (1) as well as the sale of shares of our common stock in this offering at the 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 estimated underwriting discounts and commissions and estimated offering expenses payable by us. A $1.00 increase (decrease) in the assumed intial 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, cash equivalents and marketable securities, working capital, total assets and total stockholders' equity by $ 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 estimated underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, each increase (decrease) of 1.0 million in the number of shares offered by us in this offering would increase (decrease) the pro forma as adjusted amount of each of cash, cash equivalents and marketable securities, working capital, total assets and total stockholders' equity by $ million, assuming the assumed initial offering price remains the same and after deducting estimated underwriting discounts commissions and estimated offering expenses payable by us.

(3) We define working capital as current assets less current liabilities. See our consolidated financial statements and related notes appearing at the end of this prospectus for further details regarding our current assets and current liabilities.

REORGANIZATION

Reorganization and Convertible Preferred Stock

On December 22, 2017, we completed a series of transactions pursuant to which Scholar Rock Merger Sub, LLC, a wholly owned subsidiary of Scholar Rock Holding Corporation, was merged with and into Scholar Rock, LLC, or the Reorganization. In connection with the Reorganization:

- Holders of Scholar Rock, LLC Series B convertible preferred units received one share of Scholar Rock Holding Corporation Series B convertible preferred stock for each outstanding Series B convertible preferred unit held immediately prior to the Reorganization, with an aggregate of 13,526,994 shares of Scholar Rock Holding Corporation Series B convertible preferred stock issued in the Reorganization;

- Holders of Scholar Rock, LLC Series A-4 convertible preferred units received one share of Scholar Rock Holding Corporation Series A-4 convertible preferred stock for each outstanding Series A-4 convertible preferred unit held immediately prior to the Reorganization, with an aggregate of 3,906,738 shares of Scholar Rock Holding Corporation Series A-4 convertible preferred stock issued in the Reorganization;

- Holders of Scholar Rock, LLC Series A-3 convertible preferred units received one share of Scholar Rock Holding Corporation Series A-3 convertible preferred stock for each outstanding Series A-3 convertible preferred unit held immediately prior to the Reorganization, with an aggregate of 5,579,709 shares of Scholar Rock Holding Corporation Series A-3 convertible preferred stock issued in the Reorganization;

- Holders of Scholar Rock, LLC Series A-2 convertible preferred units received one share of Scholar Rock Holding Corporation Series A-2 convertible preferred stock for each outstanding Series A-2 convertible preferred unit held immediately prior to the Reorganization, with an aggregate of 5,066,915 shares of Scholar Rock Holding Corporation Series A-2 convertible preferred stock issued in the Reorganization;

- Holders of Scholar Rock, LLC Series A-1 convertible preferred units received one share of Scholar Rock Holding Corporation Series A-1 convertible preferred stock for each outstanding Series A-1 convertible preferred unit held immediately prior to the Reorganization, with an aggregate of 2,000,000 shares of Scholar Rock Holding Corporation Series A-1 convertible preferred stock issued in the Reorganization;

- Holders of Scholar Rock, LLC common units received one share of Scholar Rock Holding Corporation common stock for each outstanding common unit held immediately prior to the Reorganization, with an aggregate of 4,576,500 shares of common stock issued in the Reorganization;

- Holders of Scholar Rock, LLC vested and unvested incentive units, irrespective of any strike price or voting rights on any such outstanding incentive units, exchanged such incentive units for an equal number of shares of common stock or restricted common stock, respectively. The restricted common stock was issued with the same vesting terms as the unvested incentive units held immediately prior to the Reorganization. An aggregate of 6,758,945 shares of common stock and restricted common stock were issued to the prior holders of incentive units in the Reorganization; and

- The outstanding warrant to purchase 21,739 Series A-3 convertible preferred units at an exercise price of $1.38 per unit was converted to a warrant to purchase 21,739 shares of Series A-3 convertible preferred stock at the same exercise price per share.

We issued 13,055,555 shares of Series C convertible preferred stock on December 22, 2017.

Our Series C convertible preferred stock, Series B convertible preferred stock, Series A-4 convertible preferred stock, Series A-3 convertible preferred stock, Series A-2 convertible preferred stock and Series A-1 convertible preferred stock are designated as convertible preferred stock under our amended and restated certificate of incorporation. All outstanding shares of our convertible preferred stock are convertible into shares of common stock at the then-effective conversion ratios. In connection with the Reorganization,

by operation of law, we acquired all assets of Scholar Rock, LLC and assumed all of its liabilities and obligations. The purpose of the Reorganization was to reorganize our corporate structure so that Scholar Rock Holding Corporation would continue as a corporation and so that our existing investors would own our capital stock rather than equity interests in a limited liability company. For the convenience of the reader, except as context otherwise requires, all information included in this prospectus is presented giving effect to the Reorganization.

RISK FACTORS

Investing in our common stock involves a high degree of risk. You should carefully consider the risks described below, as well as the other information in this prospectus, including our consolidated financial statements and the related notes and "Management's Discussion and Analysis of Financial Condition and Results of Operations," before deciding whether to invest in our common stock. The occurrence of any of the events or developments described below could harm our business, financial condition, results of operations and growth prospects. In such an event, the market price of our common stock could decline and you may lose all or part of your investment. Additional risks and uncertainties not presently known to us or that we currently deem immaterial also may impair our business operations.

Risks Related to Our Business and Operations

We have limited operating history, incurred net losses in every year since our inception and anticipate that we will continue to incur net losses in the future.

We are a biopharmaceutical company with a limited operating history. We were formed in 2012 and our operations to date have been focused on organizing and staffing our company, business planning, raising capital, establishing our intellectual property portfolio and performing research and development of monoclonal antibodies that selectively inhibit activation of growth factors for therapeutic effect. Consequently, we have no meaningful operations upon which to evaluate our business and predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing drug products. Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect or an acceptable safety profile, gain regulatory approval and become commercially viable. We have not yet demonstrated the ability to progress any product candidate through clinical trials, we have no products approved for commercial sale and we have not generated any revenue from product sales to date. We continue to incur significant research and development and other expenses related to our ongoing operations. As a result, we are not profitable and have incurred losses in each period since our inception. For the years ended December 31, 2016 and 2017, we reported a net loss of $16.2 million and $25.0 million, respectively. As of December 31, 2017, we had an accumulated deficit of $57.5 million. We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our research and development of, and seek regulatory approvals for, our lead product candidate, SRK-015, and any future product candidates.

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

- advance the development of our lead product candidate, SRK-015, into Phase 1 clinical development, and, if successful, later-stage clinical trials;
- advance our other preclinical development programs into clinical development;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- increase the amount of research and development activities to identify and develop product candidates using our proprietary platform technology;
- hire additional clinical, quality control and scientific personnel;
- expand our operational, financial and management systems and increase personnel, including personnel to support our clinical development, manufacturing and commercialization efforts and our operations as a public company;
- maintain, expand and protect our intellectual property portfolio;
- establish a sales, marketing, medical affairs and distribution infrastructure to commercialize any products for which we may obtain marketing approval and intend to commercialize on our own or jointly with third parties; and
- invest in or in-license other technologies.

To become and remain profitable, we or any potential future collaborators must develop and eventually commercialize products with significant market potential. This will require us to be successful in a range of challenging activities, including completing preclinical studies and clinical trials, obtaining marketing approval for product candidates, manufacturing, marketing and selling products for which we may obtain marketing approval and satisfying any post-marketing requirements. We may never succeed in any or all of these activities and, even if we do, we may never generate revenue that is 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.

Even if we succeed in commercializing one or more of our product candidates, we will continue to incur substantial research and development and other expenditures to develop and market additional product candidates. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

We will require additional capital to fund our operations and if we fail to obtain necessary capital, we will not be able to complete the development and commercialization of SRK-015 and any future product candidates.

Our operations have consumed substantial amounts of cash since inception. We expect to continue to spend substantial amounts of cash to conduct further research and development and preclinical studies and clinical trials of SRK-015 and any future product candidates, to seek regulatory approvals for our product candidates and to launch and commercialize any products for which we receive regulatory approval. As of December 31, 2017, we had approximately $58.0 million in cash, cash equivalents and marketable securities. Based on our current operating plan, we believe that the net proceeds from this offering, together with existing cash, cash equivalents and marketable securities, will be sufficient to fund our operating expenses and capital expenditure requirements . However, our future capital requirements and the period for which our existing resources will support our operations may vary significantly from what we expect, and we will in any event require additional capital in order to complete clinical development of any of our current programs. Our monthly spending levels will vary based on new and ongoing development and corporate activities. Because the length of time and activities associated with development of our product candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and any approved marketing and commercialization activities. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the initiation, progress, timing, costs and results of preclinical studies and clinical trials for our product candidates;
- the clinical development plans we establish for these product candidates;
- the number and characteristics of product candidates that we develop;
- the terms of any collaboration agreements we may choose to enter into;
- the outcome, timing and cost of meeting regulatory requirements established by the U.S. Food and Drug Administration, or FDA, the European Medicines Agency, or EMA, and other comparable foreign regulatory authorities;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights;
- the cost of defending intellectual property disputes, including patent infringement actions brought by third parties against us or our product candidates;

- the effect of competing technological and market developments;
- the cost and timing of completion of commercial-scale outsourced manufacturing activities; and
- the cost of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval in regions where we choose to commercialize our products on our own.

We do not have any committed external source of funds or other support for our development efforts and we cannot be certain that additional funding will be available on acceptable terms, or at all. Until we can generate sufficient product or royalty revenue to finance our cash requirements, which we may never do, we expect to finance our future cash needs through a combination of public or private equity offerings, debt financings, collaborations, strategic alliances, licensing arrangements and other marketing or distribution arrangements. If we raise additional funds through public or private equity offerings, the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights. Further, to the extent that we raise additional capital through the sale of common stock or securities convertible or exchangeable into common stock, your ownership interest will be diluted. In addition, any debt financing may subject us to fixed payment obligations and covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, including, for example, the covenants included in our existing loan and security agreement with Silicon Valley Bank. If we raise additional capital through marketing and distribution arrangements or other collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish certain valuable rights to our product candidates, technologies, future revenue streams or research programs or grant licenses on terms that may not be favorable to us. We also could be required to seek collaborators for SRK-015 or any future product candidate at an earlier stage than otherwise would be desirable or relinquish our rights to product candidates or technologies that we otherwise would seek to develop or commercialize ourselves. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of one or more of our product candidates or one or more of our other research and development initiatives. Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

Our business is highly dependent on the success of our lead product candidate, SRK-015, as well as any future clinical product candidates that are generated from our other preclinical programs. All of our product candidates will require significant additional preclinical and clinical development before we may be able to seek regulatory approval for and launch a product commercially.

We are very early in our development efforts. Because SRK-015 is our lead product candidate, if SRK-015 encounters safety or efficacy problems, development delays or regulatory issues or other problems, our development plans and business would be significantly harmed. SRK-015 is currently being advanced into a Phase 1 clinical development program for the treatment of spinal muscular atrophy, or SMA, which we expect to initiate in the second quarter of 2018. All of our other programs are in preclinical development, and we have yet to nominate a clinical candidate from these programs.

SRK-015 and any future clinical product candidates will require additional preclinical and clinical development, regulatory review and approval in one or more jurisdictions, substantial investment, and access to sufficient commercial manufacturing capacity and significant marketing efforts before we can generate any revenue from product sales. We may not have the financial resources to continue development of, or to modify existing or enter into new collaborations for, a product candidate if we experience any issues that delay or prevent regulatory approval of, or our ability to commercialize, our product candidates, including:

- negative or inconclusive results from our preclinical studies or clinical trials or the clinical trials of others for product candidates similar to ours, leading to a decision or requirement to conduct additional preclinical testing or clinical trials or abandon a program;

- product-related side effects experienced by subjects in our clinical trials or by individuals using drugs or therapeutic biologics similar to our product candidates;
- delays in submitting Investigational New Drug applications, or INDs, or comparable foreign applications or delays or failure in obtaining the necessary approvals from regulators to commence a clinical trial, or a suspension or termination of a clinical trial once commenced;
- conditions imposed by the FDA, EMA or comparable foreign authorities regarding the scope or design of our clinical trials;
- delays in enrolling subjects in clinical trials;
- high drop-out rates of subjects from clinical trials;
- inadequate supply or quality of product candidates or other materials necessary for the conduct of our clinical trials;
- greater than anticipated clinical trial costs;
- poor effectiveness of our product candidates during clinical trials;
- unfavorable FDA, EMA or other regulatory agency inspection and review of a clinical trial site;
- failure of our third-party contractors or investigators to comply with regulatory requirements or otherwise meet their contractual obligations in a timely manner, or at all;
- delays and changes in regulatory requirements, policy and guidelines, including the imposition of additional regulatory oversight around clinical testing generally or with respect to our technology in particular; or
- varying interpretations of data by the FDA, EMA and similar foreign regulatory agencies.

Our approach to the discovery and development of innovative medicines for the treatment of serious diseases in which signaling by protein growth factors plays a fundamental role is based on our proprietary platform, which is unproven and may not result in marketable products.

Our proprietary platform is designed to discover and develop monoclonal antibodies that have a high degree of specificity to achieve selective modulation of growth factor signaling. Our approach is rooted in our structural biology insights into the mechanism by which certain growth factors are activated in close proximity to the cell surface, which we refer to as "supracellular activation." We integrate these insights with sophisticated protein expression, assay development and monoclonal antibody discovery capabilities. However, the scientific research that forms the basis of our efforts to develop product candidates utilizing our proprietary platform is ongoing. We have not yet tested any monoclonal antibodies discovered through use of our proprietary platform in humans. Therefore, we may ultimately discover that our proprietary platform and any product candidates resulting therefrom do not possess properties required for therapeutic effectiveness. As a result, we may never succeed in developing a marketable product. If our product candidates discovered utilizing our proprietary platform prove to be ineffective, unsafe or commercially unviable, our entire proprietary platform and pipeline would have little, if any, value, which would have a material and adverse effect on our business, financial condition, results of operations and prospects.

We will need to grow the size of our organization, and we may experience difficulties in managing this growth.

As of April 25, 2018, we had 49 full-time employees. As our clinical development and commercialization plans and strategies develop, and as we transition into operating as a public company, we expect we will need additional managerial, clinical, regulatory, sales, marketing, financial, legal and other personnel. Future growth would impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, maintaining and motivating additional employees;

- managing our development efforts effectively, including the clinical and FDA review process for SRK-015 and any future product candidates, while complying with our contractual obligations to contractors and other third parties; and

- improving our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to commercialize our product candidates, if approved, will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert a disproportionate amount of its attention away from day-to-day activities in order to devote a substantial amount of time to managing these growth activities.

We currently rely, and for the foreseeable future will continue to rely, in substantial part on certain independent organizations, advisors and consultants to provide certain services, including contract manufacturers and companies focused on antibody development and discovery activities. There can be no assurance that the services of independent organizations, advisors and consultants will continue to be available to us on a timely basis when needed, or that we can find qualified replacements. In addition, if we are unable to effectively manage our outsourced activities or if the quality, accuracy or quantity of the services provided is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain, or may be substantially delayed in obtaining, regulatory approval of our product candidates or otherwise advance our business. There can be no assurance that we will be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all.

If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop and commercialize SRK-015 or any future product candidates and, accordingly, may not achieve our research, development and commercialization goals.

If we lose key management personnel, or if we fail to recruit additional highly skilled personnel, our ability to identify and develop new or next generation product candidates will be impaired, could result in loss of markets or market share and could make us less competitive.

Our ability to compete in the highly competitive biotechnology and pharmaceutical industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. We are highly dependent on our management, scientific and medical personnel, including Nagesh K. Mahanthappa, Ph.D., our Chief Executive Officer and President, Rhonda M. Chicko, C.P.A., our Chief Financial Officer, and Yung H. Chyung, M.D., our Chief Medical Officer. The loss of the services of any of our executive officers, other key employees, and other scientific and medical advisors, and our inability to find suitable replacements could result in delays in product development and harm our business.

We conduct our operations at our facility in Cambridge, Massachusetts. This region is headquarters to many other biopharmaceutical companies and many academic and research institutions. Competition for skilled personnel in our market is intense and may limit our ability to hire and retain highly qualified personnel on acceptable terms or at all.

To induce valuable employees to remain at our company, in addition to salary and cash incentives, we have provided restricted stock awards and stock options that vest over time. The value to employees of restricted stock awards and stock options that vest over time may be significantly affected by movements in our stock price that are beyond our control, and may at any time be insufficient to counteract more lucrative offers from other companies. Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us on short notice. Although we have employment agreements with our key employees, these employment agreements provide for at-will employment, which means that any of our employees could leave our employment at any time, with or without notice. We do not maintain "key man" insurance policies on the lives of these individuals or the

lives of any of our other employees. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level and senior scientific and medical personnel.

Our internal computer systems, or those used by our contract research organizations, or other contractors or consultants, may fail or suffer security breaches.

Despite the implementation of security measures, our internal computer systems and those of our future contract research organizations, or CROs, and other contractors and consultants are vulnerable to damage from computer viruses and unauthorized access. While we have not experienced any such material system failure or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations. For example, the loss of preclinical or clinical data could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Likewise, we may rely on third parties for the manufacture of our product candidates and to conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our product candidates could be delayed.

Our employees, independent contractors, consultants, commercial partners and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk of employee fraud or other illegal activity by our employees, independent contractors, consultants, commercial partners and vendors. Misconduct by these parties could include intentional, reckless and/or negligent conduct that fails to comply with the laws of the FDA, EMA and other similar foreign regulatory bodies, provide true, complete and accurate information to the FDA, EMA and other similar foreign regulatory bodies, comply with manufacturing standards we have established, comply with healthcare fraud and abuse laws in the United States and similar foreign fraudulent misconduct laws, or report financial information or data accurately or to disclose unauthorized activities to us. If we obtain FDA approval of any of our product candidates and begin commercializing those products in the United States, our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase. These laws may impact, among other things, our current activities with principal investigators and research patients, as well as proposed and future sales, marketing and education programs. In connection with this offering, we will adopt a code of business conduct and ethics, but it is not always possible to identify and deter misconduct by our employees, independent contractors, consultants, commercial partners and vendors, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any actions are instituted against us and we are not successful in defending ourselves or asserting our rights, those actions could result in the imposition of civil, criminal and administrative penalties, damages, monetary fines, individual imprisonment, disgorgement, possible exclusion from participation in government healthcare programs, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, contractual damages, reputational harm, diminished profits and future earnings and the curtailment of our operations.

We have disclosed that there is substantial doubt about our ability to continue as a going concern.

In Note 1 to our consolidated financial statements, we disclose that there is substantial doubt about our ability to continue as a going concern. If we are unable to obtain sufficient funding, we could be forced to delay, reduce or eliminate all of our research and development programs, product portfolio expansion or commercialization efforts, and our financial condition and results of operations will be materially and

adversely affected and we may be unable to continue as a going concern. After the completion of this offering, future financial statements may disclose substantial doubt about our ability to continue as a going concern. If we seek additional financing to fund our business activities in the future and there remains substantial doubt about our ability to continue as a going concern, investors or other financing sources may be unwilling to provide additional funding to us on commercially reasonable terms or at all.

Risks Related to Research and Development and the Biopharmaceutical Industry

Preclinical development is uncertain. Our preclinical programs may experience delays or may never advance to clinical trials, which would adversely affect our ability to obtain regulatory approvals or commercialize these programs on a timely basis or at all, which would have an adverse effect on our business.

We have yet to nominate a clinical candidate for all of our programs, other than SRK-015. Before we can commence clinical trials for any product candidate in these programs, we must complete extensive preclinical studies that support our planned INDs in the United States, or similar applications in other jurisdictions. We cannot be certain of the timely completion or outcome of our preclinical studies and cannot predict if the FDA, EMA or other regulatory authorities will accept our proposed clinical programs or if the outcome of our preclinical studies will ultimately support the further development of our programs. As a result, we cannot be sure that we will be able to submit INDs or similar applications for our preclinical programs on the timelines we expect, if at all, and we cannot be sure that submission of INDs or similar applications will result in the FDA, the EMA or other regulatory authorities allowing clinical trials to begin.

Due to our limited resources and access to capital, we must prioritize development of certain programs and product candidates; these decisions may prove to be wrong and may adversely affect our business.

We may fail to identify viable new product candidates for clinical development from our current or future research programs for a number of reasons. If we fail to identify additional potential product candidates, our business could be materially harmed.

Research programs to pursue the development of our existing and planned product candidates for additional indications and to identify new product candidates and disease targets require substantial technical, financial and human resources whether or not they are ultimately successful. Our research programs may initially show promise in identifying potential indications and/or product candidates, yet fail to yield results for clinical development for a number of reasons, including:

- the research methodology used may not be successful in identifying potential indications and/or product candidates;
- potential product candidates may, after further study, be shown to have harmful adverse effects or other characteristics that indicate they are unlikely to be effective products; or
- it may take greater human and financial resources than we will possess to identify additional therapeutic opportunities for our product candidates or to develop suitable potential product candidates through internal research programs, thereby limiting our ability to develop, diversify and expand our product portfolio.

Because we have limited financial and human resources, we intend to initially focus on research programs and product candidates for a limited set of indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential or a greater likelihood of success. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities.

Accordingly, there can be no assurance that we will ever be able to identify additional therapeutic opportunities for our product candidates or to develop suitable potential product candidates through internal research programs, which could materially adversely affect our future growth and prospects. We may focus our efforts and resources on potential product candidates or other potential programs that ultimately prove to be unsuccessful.

The successful development of biopharmaceuticals is highly uncertain.

Successful development of biopharmaceuticals is highly uncertain and is dependent on numerous factors, many of which are beyond our control. Product candidates that appear promising in the early phases of development may fail to reach the market for several reasons including:

- preclinical study results may show the product candidate to be less effective than desired or to have harmful or problematic side effects;
- clinical trial results may show the product candidates to be less effective than expected (e.g., a clinical trial could fail to meet its primary endpoint(s)) or to have unacceptable side effects or toxicities;
- failure to receive the necessary regulatory approvals or a delay in receiving such approvals. Among other things, such delays may be caused by slow enrollment in clinical trials, patients dropping out of trials, length of time to achieve trial endpoints, additional time requirements for data analysis, or biologics license application, or BLA, preparation, discussions with the FDA, an FDA request for additional preclinical or clinical data, or unexpected safety or manufacturing issues;
- manufacturing costs, formulation issues, pricing or reimbursement issues, or other factors that make a product candidate uneconomical; and
- the proprietary rights of others and their competing products and technologies that may prevent one of our product candidates from being commercialized.

The length of time necessary to complete clinical trials and to submit an application for marketing approval for a final decision by a regulatory authority varies significantly from one product candidate to the next, and may be difficult to predict.

Even if we are successful in getting market approval, commercial success of any approved products will also depend in large part on the availability of coverage and adequate reimbursement from third-party payors, including government payors such as the Medicare and Medicaid programs and managed care organizations, which may be affected by existing and future health care reform measures designed to reduce the cost of health care. Third-party payors could require us to conduct additional studies, including post-marketing studies related to the cost effectiveness of a product, to qualify for reimbursement, which could be costly and divert our resources. If government and other health care payors were not to provide coverage and adequate reimbursement levels for one any of our products once approved, market acceptance and commercial success would be reduced.

In addition, if any of our product candidates are approved for marketing, we will be subject to significant regulatory obligations regarding the submission of safety and other post-marketing information and reports and registration, and will need to continue to comply (or ensure that our third-party providers) comply with current good manufacturing practices, or cGMPs, and good clinical practices, or GCPs, for any clinical trials that we conduct post-approval. In addition, there is always the risk that we or a regulatory authority might identify previously unknown problems with a product post-approval, such as adverse events of unanticipated severity or frequency. Compliance with these requirements is costly, and any failure to comply or other issues with our product candidates post-approval could adversely affect our business, financial condition and results of operations.

Clinical development involves a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of SRK-015 or any future product candidates.

To obtain the requisite regulatory approvals to commercialize any product candidates, we must demonstrate through extensive preclinical studies and clinical trials that our product candidates are safe, pure and potent or effective in humans. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. We may be unable to establish clinical endpoints that applicable regulatory authorities would consider clinically meaningful, and a clinical trial can fail at any stage of testing.

Differences in trial design between early-stage clinical trials and later-stage clinical trials make it difficult to extrapolate the results of earlier clinical trials to later clinical trials. Moreover, clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in clinical trials have nonetheless failed to obtain marketing approval of their products.

Successful completion of clinical trials is a prerequisite to submitting a BLA to the FDA, a Marketing Authorization Application, or MAA, to the EMA, and similar marketing applications to comparable foreign regulatory authorities, for each product candidate and, consequently, the ultimate approval and commercial marketing of any product candidates. We do not know whether any of our clinical trials will begin or be completed on schedule, if at all.

We may experience delays in initiating or completing clinical trials. We also may experience numerous unforeseen events during, or as a result of, any future clinical trials that we could conduct that could delay or prevent our ability to receive marketing approval or commercialize SRK-015 or any future product candidates, including:

- regulators or institutional review boards, or IRBs, or ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may experience delays in reaching, or fail to reach, agreement on acceptable terms with prospective trial sites and prospective CROs the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- clinical trials of any product candidates may fail to show safety, purity or potency, or produce negative or inconclusive results and we may decide, or regulators may require us, to conduct additional preclinical studies or clinical trials or we may decide to abandon product development programs;
- the number of subjects required for clinical trials of any product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or subjects may drop out of these clinical trials or fail to return for post-treatment follow-up at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all, or may deviate from the clinical trial protocol or drop out of the trial, which may require that we add new clinical trial sites or investigators;
- we may elect to, or regulators, IRBs or ethics committees may require that we or our investigators, suspend or terminate clinical research or trials for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of any product candidates may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate to initiate or complete a given clinical trial;
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators, IRBs or ethics committees to suspend or terminate the trials, or reports from clinical testing of other therapies may raise safety or efficacy concerns about our product candidates; and
- the FDA, EMA or other regulatory authorities may require us to submit additional data such as long-term toxicology studies, or impose other requirements before permitting us to initiate a clinical trial.

We could also encounter delays if a clinical trial is suspended or terminated by us, the IRBs of the institutions in which such trials are being conducted, or the FDA, EMA or other regulatory authorities, or

recommended for suspension or termination by the Data Safety Monitoring Board, or DSMB, for such trial. A suspension or termination may be imposed due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA, EMA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product or treatment, failure to establish or achieve clinically meaningful trial endpoints, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. Many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates. Further, the FDA, EMA or other regulatory authorities may disagree with our clinical trial design and our interpretation of data from clinical trials, or may change the requirements for approval even after they have reviewed and commented on the design for our clinical trials.

Our product development costs will increase if we experience delays in clinical testing or marketing approvals. We do not know whether any of our clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates and may allow our competitors to bring products to market before we do, potentially impairing our ability to successfully commercialize our product candidates and harming our business and results of operations. Any delays in our clinical development programs may harm our business, financial condition and results of operations significantly.

Our future clinical trials or those of our future collaborators may reveal significant adverse events not seen in our preclinical studies and may result in a safety profile that could inhibit regulatory approval or market acceptance of any of our product candidates.

Before obtaining regulatory approvals for the commercial sale of any products, we must demonstrate through lengthy, complex and expensive preclinical studies and clinical trials that SRK-015 and any future product candidate is both safe and effective for use in its target indication. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. In addition, initial success in clinical trials may not be indicative of results obtained when such trials are completed. There is typically an extremely high rate of attrition from the failure of product candidates proceeding through clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy profile despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or unacceptable safety issues, notwithstanding promising results in earlier trials. Most product candidates that commence clinical trials are never approved as products and there can be no assurance that any of our future clinical trials will ultimately be successful or support further clinical development of SRK-015 or any of our future product candidates.

If significant adverse events or other side effects are observed in any of our clinical trials, we may have difficulty recruiting patients to our clinical trials, patients may drop out of our trials, or we may be required to abandon the trials or our development efforts of one or more product candidates altogether. We, the FDA, EMA or other applicable regulatory authorities, or an IRB may suspend clinical trials of a product candidate at any time for various reasons, including a belief that subjects in such trials are being exposed to unacceptable health risks or adverse side effects. Some potential therapeutics developed in the biotechnology industry that initially showed therapeutic promise in early-stage trials have later been found to cause side effects that prevented their further development. Even if the side effects do not preclude the product candidate from obtaining or maintaining marketing approval, undesirable side effects may inhibit

market acceptance of the approved product due to its tolerability versus other therapies. Any of these developments could materially harm our business, financial condition and prospects.

If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

We may experience difficulties in patient enrollment in our clinical trials for a variety of reasons. The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the trial until its conclusion. The enrollment of patients depends on many factors, including:

- the patient eligibility and exclusion criteria defined in the protocol;
- the size of the patient population required for analysis of the trial's primary endpoints;
- the proximity of patients to trial sites;
- the design of the trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- our ability to obtain and maintain patient consents; and
- the risk that patients enrolled in clinical trials will drop out of the trials before completion.

For example, we are initially developing SRK-015 for the treatment of SMA, which is a rare disease, affecting only an estimated 30,000 to 35,000 patients in the United States and Europe. As a result, we may encounter difficulties enrolling subjects in our clinical trials for SRK-015 due, in part, to the small size of this patient population. In addition, our clinical trials will compete with other clinical trials for product candidates that are in the same therapeutic areas as our product candidates, and this competition will reduce the number and types of patients available to us, because some patients who might have opted to enroll in our trials may instead opt to enroll in a trial being conducted by one of our competitors. Since the number of qualified clinical investigators is limited, we expect to conduct some of our clinical trials at the same clinical trial sites that some of our competitors use, which will reduce the number of patients who are available for our clinical trials in such clinical trial site.

Delays in patient enrollment may result in increased costs or may affect the timing or outcome of our future clinical trials, which could prevent completion of these trials and adversely affect our ability to advance the development of our product candidates.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our product candidates.

We face an inherent risk of product liability as a result of testing SRK-015 and any of our future product candidates in clinical trials and will face an even greater risk if we commercialize any products, if approved. For example, we may be sued if our product candidates cause or are perceived to cause injury or are found to be otherwise unsuitable during clinical trials, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- inability to bring a product candidate to the market;
- decreased demand for our products;
- injury to our reputation;
- withdrawal of clinical trial participants and inability to continue clinical trials;
- initiation of investigations by regulators;

- costs to defend the related litigation;
- diversion of management's time and our resources;
- substantial monetary awards to trial participants;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenue;
- exhaustion of any available insurance and our capital resources;
- the inability to commercialize any product candidate, if approved; and
- decline in our share price.

Our inability to obtain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop, alone or with collaborators. We need to obtain additional insurance for clinical trials as our lead product candidate SRK-015 enters the clinical development phase. However, we may be unable to obtain, or may obtain on unfavorable terms, clinical trial insurance in amounts adequate to cover any liabilities from any of our clinical trials. Our insurance policies may also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts. Even if our agreements with any future corporate collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.

The biopharmaceutical industry is characterized by intense competition and rapid innovation. Our competitors may be able to develop other compounds or drugs that are able to achieve similar or better results. Our potential competitors include major multinational pharmaceutical companies, established biotechnology companies, specialty pharmaceutical companies and universities and other research institutions. Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations and well-established sales forces. Smaller or early-stage companies may also prove to be significant competitors, particularly as they develop novel approaches to treating disease indications that our product candidates are also focused on treating. Established pharmaceutical companies may also invest heavily to accelerate discovery and development of novel therapeutics or to in-license novel therapeutics that could make the product candidates that we develop obsolete. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors, either alone or with collaborative partners, may succeed in developing, acquiring or licensing on an exclusive basis drug or biologic products that are more effective, safer, more easily commercialized or less costly than our product candidates or may develop proprietary technologies or secure patent protection that we may need for the development of our technologies and products. We believe the key competitive factors that will affect the development and commercial success of our product candidates are efficacy, safety, tolerability, reliability, convenience of use, price and reimbursement.

We anticipate competing with other companies that are focused on treating disease indications that our product candidates are also focused on treating. A competitor may develop technologies focused on the same disease pathway as our technology or may focus on treating the targeted disease in a completely different manner. To the extent a new drug is developed that is more efficacious than any product candidate developed by us, this could reduce or negate the need for our product candidate. In addition, while we believe our product candidates may be used in conjunction with existing or emerging standard of care in

certain disease indications, including SMA, as companies continue to improve upon existing standard of care, more efficacious drug therapies could become available, reducing or completely negating the benefit of our product candidates. Our competitors may also include companies that are or will be developing therapies for the same therapeutic areas that we are targeting within our early pipeline, including neuromuscular disorders, cancer, fibrosis and anemia.

Even if we obtain regulatory approval of our product candidates, the availability and price of our competitors' products could limit the demand and the price we are able to charge for our product candidates. We may not be able to implement our business plan if the acceptance of our product candidates is inhibited by price competition or the reluctance of physicians to switch from existing methods of treatment to our product candidates, or if physicians switch to other new drug or biologic products or choose to reserve our product candidates for use in limited circumstances. For additional information regarding our competition, see "Business — Competition."

Even if a product candidate we develop receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

If SRK-015 or any future product candidate we develop receives marketing approval, whether as a single agent or in combination with other therapies, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors, and others in the medical community. For example, doctors may deem it sufficient to treat patients with SMA with an SMN upregulator such as nusinersen, and therefore will not be willing to utilize SRK-015 in conjunction with such SMN upregulator. If the product candidates we develop do not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of any product candidate, if approved for commercial sale, will depend on a number of factors, including:

- efficacy and potential advantages compared to alternative treatments;
- the ability to offer our products, if approved, for sale at competitive prices;
- convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- the ability to obtain sufficient third-party coverage and adequate reimbursement; and
- the prevalence and severity of any side effects.

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 handling, use, storage, treatment and disposal of hazardous materials and wastes. Our research and development activities involve the use of biological and hazardous materials and 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, which could cause an interruption of our commercialization efforts, research and development efforts and business operations, environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling and disposal of these materials and specified waste products. Although we believe that the safety procedures utilized by our third-party manufacturers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources and state or federal or other applicable authorities may curtail our use of certain materials and/or interrupt our business operations. Furthermore, environmental laws and

regulations are complex, change frequently and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. 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.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. We do not carry specific biological waste or hazardous waste insurance coverage, workers compensation or property and casualty and general liability insurance policies that include coverage for damages and fines arising from biological or hazardous waste exposure or contamination.

Comprehensive Tax Reform Legislation Could Adversely Affect Our Business And Financial Condition.

On December 22, 2017, President Trump signed into law the "Tax Cuts and Jobs Act," or the TCJA, that significantly reforms the Internal Revenue Code of 1986, as amended, or the Code. The TCJA, among other things, contains significant changes to corporate taxation, including reduction of the corporate tax rate, limitation of the tax deduction for interest expense, limitation of the deduction for net operating losses and elimination of net operating loss carrybacks and modifying or repealing many business deductions and credits (including reducing the business tax credit for certain clinical testing expenses incurred in the testing of certain drugs for rare diseases or conditions generally referred to as "orphan drugs"). Our net deferred tax assets and liabilities were revalued at the newly enacted U.S. corporate rate. We continue to examine the impact this tax reform legislation may have on our business. The overall impact of the TCJA is uncertain and our business and financial condition could be adversely affected.

Our ability to use our net operating loss carryforwards and certain tax credit carryforwards may be subject to limitation.

As of December 31, 2017, we had net operating loss carryforwards for federal and state income tax purposes of $50.4 million and $50.0 million, respectively, which begin to expire in 2034. As of December 31, 2017, we also had available tax credit carryforwards for federal and state income tax purposes of $1.1 million and $0.7 million, respectively, which begin to expire in 2034 and 2020, respectively. Under Section 382 of the Code, changes in our ownership may limit the amount of our net operating loss carryforwards and tax credit carryforwards that could be utilized annually to offset our future taxable income, if any. This limitation would generally apply in the event of a cumulative change in ownership of our company of more than 50% within a three-year period. Any such limitation may significantly reduce our ability to utilize our net operating loss carryforwards and tax credit carryforwards before they expire. Private placements and other transactions that have occurred since our inception, as well as this offering, may trigger such an ownership change pursuant to Section 382. Any such limitation, whether as the result of this offering, prior private placements, sales of our common stock by our existing stockholders or additional sales of our common stock by us, could have a material adverse effect on our results of operations in future years. The reduction of the corporate tax rate under TCJA may cause a reduction in the economic benefit of our net operating loss carryforwards and other deferred tax assets available to us. Under the TCJA, net operating losses generated after December 31, 2017 will not be subject to expiration.

Our current operations are concentrated in one location, and we or the third parties upon whom we depend may be adversely affected by earthquakes or other natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Our current operations are located in our facilities in Cambridge, Massachusetts. Any unplanned event, such as flood, fire, explosion, earthquake, extreme weather condition, medical epidemics, power shortage, telecommunication failure or other natural or manmade accidents or incidents that result in us being unable to fully utilize our facilities, or the manufacturing facilities of our third-party contract manufacturers, may have a material and adverse effect on our ability

to operate our business, particularly on a daily basis, and have significant negative consequences on our financial and operating conditions. Loss of access to these facilities may result in increased costs, delays in the development of our product candidates or interruption of our business operations. Earthquakes or other natural disasters could further disrupt our operations, and have a material and 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 our research facilities or the manufacturing facilities of our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible, for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which, could have a material adverse effect on our business. As part of our risk management policy, we maintain insurance coverage at levels that we believe are appropriate for our business. However, in the event of an accident or incident at these facilities, we cannot assure you that the amounts of insurance will be sufficient to satisfy any damages and losses. If our facilities, or the manufacturing facilities of our third-party contract manufacturers, are unable to operate because of an accident or incident or for any other reason, even for a short period of time, any or all of our research and development programs may be harmed. Any business interruption may have a material and adverse effect on our business, financial condition, results of operations and prospects.

Risks Related to Government Regulation

The regulatory approval process for our product candidates in the United States, EU and other jurisdictions is currently uncertain and will be lengthy, time-consuming and inherently unpredictable and we may experience significant delays in the clinical development and regulatory approval, if any, of our product candidates.

The research, testing, manufacturing, labeling, approval, selling, import, export, marketing and distribution of drug products, including biologics, are subject to extensive regulation by the FDA in the United States and other regulatory authorities. We are not permitted to market any biological product in the United States until we receive a biologics license from the FDA. We have not previously submitted a BLA to the FDA, or similar marketing application to comparable foreign authorities. A BLA must include extensive preclinical and clinical data and supporting information to establish that the product candidate is safe, pure and potent for each desired indication. A BLA must also include significant information regarding the chemistry, manufacturing and controls for the product, and the manufacturing facilities must complete a successful pre-license inspection.

The FDA may also require a panel of experts, referred to as an Advisory Committee, to deliberate on the adequacy of the safety and efficacy data to support approval. The opinion of the Advisory Committee, although not binding, may have a significant impact on our ability to obtain approval of any product candidates that we develop based on the completed clinical trials.

In addition, clinical trials can be delayed or terminated for a variety of reasons, including delays or failures related to:

- obtaining regulatory authorization to begin a clinical trial, if applicable;
- the availability of financial resources to begin and complete the planned trials;
- reaching agreement on acceptable terms with prospective CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- obtaining approval at each clinical trial site by an independent IRB or ethics committee;
- recruiting suitable patients to participate in a trial in a timely manner;
- having patients complete a trial or return for post-treatment follow-up;
- clinical trial sites deviating from trial protocol, not complying with GCP requirements or dropping out of a trial;
- addressing any patient safety concerns that arise during the course of a clinical trial;
- addressing any conflicts with new or existing laws or regulations;
- adding new clinical trial sites; or
- manufacturing qualified materials under cGMP regulations for use in clinical trials.

Patient enrollment is a significant factor in the timing of clinical trials and is affected by many factors. Further, a clinical trial may be suspended or terminated by us, the IRBs for the institutions in which such trials are being conducted, or the FDA, EMA or other regulatory authorities, or recommended for suspension or termination by the DSMB for such trial, due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA, EMA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. If we experience termination of, or delays in the completion of, any clinical trial of our product candidates, the commercial prospects for our product candidates will be harmed, and our ability to generate product revenue will be delayed. In addition, any delays in completing any clinical trials will increase our costs, slow down our product development and approval process and jeopardize our ability to commence product sales and generate revenue.

The FDA may disagree with our regulatory plan and we may fail to obtain regulatory approval of our product candidates.

The general approach for FDA approval of a new biologic or drug is dispositive data from two well-controlled, Phase 3 clinical trials of the relevant biologic or drug in the relevant patient population. Phase 3 clinical trials typically involve hundreds of patients, have significant costs and take years to complete.

Our clinical trials results may also not support approval. In addition, our product candidates could fail to receive regulatory approval for many reasons, including the following:

- the FDA, EMA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA, EMA or comparable foreign regulatory authorities that our product candidates are safe and effective for any of their proposed indications;
- the results of clinical trials may not meet the level of statistical significance required by the FDA, EMA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that our product candidates' clinical and other benefits outweigh their safety risks;

- the FDA, EMA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to the satisfaction of the FDA, EMA or comparable foreign regulatory authorities to support the submission of a BLA or other comparable submission in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere;
- the FDA, EMA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA, EMA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

We have received Orphan Drug Designation for SRK-015 for the treatment of SMA and we may seek Orphan Drug Designation for our future product candidates, and we may be unable to maintain the benefits associated with Orphan Drug Designation, including the potential for market exclusivity.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs and therapeutic biologics for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug or therapeutic biologic as an orphan drug if it is a drug or therapeutic biologic intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug or therapeutic biologic will be recovered from sales in the United States. In the United States, Orphan Drug Designation entitles a party to financial incentives such as opportunities for grant funding toward clinical trial costs, tax advantages and user-fee waivers. In addition, if a product that has Orphan Drug Designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications, including a full NDA or BLA, to market the same product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or where the manufacturer is unable to assure sufficient product quantity.

Even though we obtained Orphan Drug Designation for SRK-015 for the treatment of SMA, or if we obtain Orphan Drug Designation for any of our future product candidates in specific indications, we may not be the first to obtain marketing approval of these product candidates for the orphan-designated indication due to the uncertainties associated with developing pharmaceutical products. In addition, exclusive marketing rights in the United States may be limited if we seek approval for an indication broader than the orphan-designated indication or may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs or therapeutic biologics with different active moieties can be approved for the same condition. Even after an orphan product is approved, the FDA can subsequently approve the same drug or therapeutic biologic with the same active moiety for the same condition if the FDA concludes that the later drug or therapeutic biologic is safer, more effective or makes a major contribution to patient care. Orphan Drug Designation neither shortens the development time or regulatory review time of a drug or therapeutic biologic nor gives the drug or therapeutic biologic any advantage in the regulatory review or approval process. In addition, while we may seek Orphan Drug Designation for our future product candidates, we may never receive such designations.

We may seek Breakthrough Therapy Designation or Fast Track Designation from the FDA, for certain of our product candidates, but receipt of either such designation may not actually lead to a faster development or regulatory review or approval process.

We may seek Breakthrough Therapy Designation or Fast Track Designation for certain of our product candidates.

A breakthrough therapy is defined as a product that is intended, alone or in combination with one or more other products, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For products that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Products designated as breakthrough therapies by the FDA can also be eligible for accelerated approval.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a Breakthrough Therapy Designation 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. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the products no longer meet the conditions for qualification and rescind the breakthrough designation.

If a product is intended for the treatment of a serious or life-threatening condition and the product demonstrates the potential to address unmet medical needs for this condition, the product sponsor may apply for Fast Track Designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. Even if we do receive Fast Track Designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development program.

Our relationships with healthcare providers and physicians and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors in the United States and elsewhere play a primary role in the recommendation and prescription of pharmaceutical products. Arrangements with third-party payors and customers can expose pharmaceutical manufacturers to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti-Kickback Statute and the federal False Claims Act, which may constrain the business or financial arrangements and relationships through which such companies sell, market and distribute pharmaceutical products. In particular, the research of our product candidates, as well as the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient

recruitment for clinical trials. The applicable federal, state and foreign healthcare laws and regulations laws that may affect our ability to operate include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, 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, to induce, or in return for, either the referral of an individual, or the purchase, lease, order or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs. A person or entity can be found guilty of violating the statute without actual knowledge of the statute or specific intent to violate it. In addition, 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 federal False Claims Act, or FCA. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers, and formulary managers on the other. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution;

- the federal civil and criminal false claims laws and civil monetary penalty laws, including the FCA, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, false or fraudulent claims for payment to, or approval by Medicare, Medicaid, or other federal healthcare programs, knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim or an obligation to pay or transmit money to the federal government, or knowingly concealing or knowingly and improperly avoiding or decreasing or concealing an obligation to pay money to the federal government. Manufacturers can be held liable under the FCA even when they do not submit claims directly to government payors if they are deemed to "cause" the submission of false or fraudulent claims. The FCA also permits a private individual acting as a "whistleblower" to bring actions on behalf of the federal government alleging violations of the FCA and to share in any monetary recovery;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity can be found guilty of violating HIPAA without actual knowledge of the statute or specific intent to violate it;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose, among other things, requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses , known as covered entities, as well as their respective business associates, independent contractors that perform services for covered entities them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions;

- the federal Physician Payments Sunshine Act, created under the Patient Protection and Affordable Care Act, and its implementing regulations, which require some manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to the United States Department of Health and Human Services, or HHS, information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members;

- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers; and

- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, and may be broader in scope than their federal equivalents; state and foreign 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; state and foreign 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; state and local laws that require the registration of pharmaceutical sales representatives; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. Ensuring business arrangements comply with applicable healthcare laws, as well as responding to possible investigations by government authorities, can be time- and resource-consuming and can divert a company's attention from the business.

It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and 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, including the imposition of civil, criminal and administrative penalties, damages, fines, disgorgement, individual imprisonment, possible exclusion from participation in federal and state funded healthcare programs, contractual damages and the curtailment or restricting of our operations, as well as additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws. Any action for violation of these laws, even if successfully defended, could cause a pharmaceutical manufacturer to incur significant legal expenses and divert management's attention from the operation of the business. Prohibitions or restrictions on sales or withdrawal of future marketed products could materially affect business in an adverse way.

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not mean that we will be successful in obtaining regulatory approval of our product candidates in other jurisdictions.

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction, while a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. For example, even if the FDA grants marketing approval of a product candidate, the EMA or comparable regulatory authorities in foreign jurisdictions must also approve the manufacturing, marketing and promotion of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the United States, including additional preclinical studies or clinical trials as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to approval.

We may also submit marketing applications in other countries. Regulatory authorities in jurisdictions outside of the United States have requirements for approval of product candidates with which we must comply prior to marketing in those jurisdictions. 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 products in certain countries. If we fail to comply with the regulatory requirements in international markets and/or receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed.

Even if we receive regulatory approval of any product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our product candidates.

If any of our product candidates are approved, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing studies and submission of safety, efficacy and other post-market information, including both federal and state requirements in the United States and requirements of comparable foreign regulatory authorities. In addition, we will be subject to continued compliance with cGMP and GCP requirements for any clinical trials that we conduct post-approval.

Manufacturers and manufacturers' facilities are required to comply with extensive FDA, EMA and comparable foreign regulatory authority requirements, including ensuring that quality control and manufacturing procedures conform to cGMP regulations. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMP and adherence to commitments made in any BLA, other marketing application, and previous responses to inspection observations. Accordingly, we and others with whom we work must continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production and quality control.

Any regulatory approvals that we receive for our product candidates may be subject to 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 safety and efficacy of the product candidate. The FDA may also require a risk evaluation and mitigation strategies, or REMS, program as a condition of approval of our product candidates, which could entail requirements for long-term patient follow-up, a medication guide, physician communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. In addition, if the FDA, EMA or a comparable foreign regulatory authority approves

our product candidates, we will have to comply with requirements including submissions of safety and other post-marketing information and reports and registration.

The FDA may impose consent decrees or withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with our product candidates, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of our products, withdrawal of the product from the market or voluntary or mandatory product recalls;
- fines, warning letters or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or suspension or revocation of license approvals;
- product seizure or detention or refusal to permit the import or export of our product candidates; and
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising, and promotion of products that are placed on the market. Products may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses and a company that is found to have improperly promoted off-label uses may be subject to significant liability. The policies of the FDA, EMA and of other regulatory authorities may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability.

Coverage and reimbursement may be limited or unavailable in certain market segments for our product candidates, if approved, which could make it difficult for us to sell any product candidates profitably.

The success of our product candidates, if approved, depends on the availability of coverage and adequate reimbursement from third-party payors. We cannot be sure that coverage and reimbursement will be available for, or accurately estimate the potential revenue from, our product candidates or assure that coverage and reimbursement will be available for any product that we may develop.

Patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Coverage and adequate reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance.

Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which drugs and treatments they will cover and the amount of reimbursement. Coverage and reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and

- neither experimental nor investigational.

In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors. As a result, obtaining coverage and reimbursement approval of a product from a government or other third-party payor is a time-consuming and costly process that could require us to provide to each payor supporting scientific, clinical and cost-effectiveness data for the use of our products on a payor-by-payor basis, with no assurance that coverage and adequate reimbursement will be obtained. Even if we obtain coverage for a given product, the resulting reimbursement payment rates might not be adequate for us to achieve or sustain profitability or may require co-payments that patients find unacceptably high. Additionally, third-party payors may not cover, or provide adequate reimbursement for, long-term follow-up evaluations required following the use of product candidates. Patients are unlikely to use our product candidates unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our product candidates. There is significant uncertainty related to insurance coverage and reimbursement of newly approved products. It is difficult to predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

Payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, the Middle Class Tax Relief and Job Creation Act of 2012 required that the Centers for Medicare & Medicaid Services, the agency responsible for administering the Medicare program, or CMS, reduce the Medicare clinical laboratory fee schedule by 2% in 2013, which served as a base for 2014 and subsequent years. In addition, effective January 1, 2014, CMS also began bundling the Medicare payments for certain laboratory tests ordered while a patient received services in a hospital outpatient setting. Additional state and federal healthcare reform measures are expected to be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for certain pharmaceutical products or additional pricing pressures.

Moreover, increasing efforts by governmental and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our product candidates. There has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed and enacted federal and state legislation 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. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations, cost containment initiatives and additional legislative changes. At the federal level, the Trump administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. While any proposed measures will require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Ongoing healthcare legislative and regulatory reform measures may have a material adverse effect on our business and results of operations.

Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the ACA, was passed, which substantially changes the way health care is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. The ACA, among other things, subjects biological products to potential competition by lower-cost biosimilars, 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, 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, establishes annual fees and taxes on manufacturers of certain branded prescription drugs, and creates a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% (and 70% commencing January 1, 2019) 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.

Some of the provisions of the ACA have yet to be fully implemented, while certain provisions have been subject to judicial and Congressional challenges, as well as efforts by the Trump administration to repeal or replace certain aspects of the ACA. Since January 2017, President Trump has signed two Executive Orders and other directives designed to delay the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. For example, the Trump administration has concluded that cost-sharing reduction, or CSR, payments to insurance companies required under the ACA have not received necessary appropriations from Congress and announced that it will discontinue these payments immediately until such appropriations are made. The loss of the CSR payments is expected to increase premiums on certain policies issued by qualified health plans under the ACA. A bipartisan bill to appropriate funds for CSR payments was introduced in the Senate, but the future of that bill is uncertain. Several state Attorneys General have filed lawsuits to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the ACA. While Congress has not passed comprehensive repeal legislation, two bills affecting the implementation of certain taxes under the ACA have been signed into law. The TCJA includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." Additionally, on January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain ACA-mandated fees, including the so-called "Cadillac" tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. Further, the Bipartisan Budget Act of 2018, or the BBA, among other things, amends the ACA, effective January 1, 2019, to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole." Moreover, CMS has recently proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

Other legislative changes have been proposed and adopted in the United States since the ACA was enacted. On August 2, 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 2013 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 2% per fiscal year. These reductions went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, including the BBA, will remain in effect through 2027 unless additional congressional action is taken. On January 2, 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several types of providers.

These laws, and future state and federal healthcare reform measures may be adopted in the future, any of which may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

EU drug marketing and reimbursement regulations may make materially affect our ability to market and receive coverage for our products in the European member states.

We intend to seek approval to market our product candidates in both the United States and in selected foreign jurisdictions. If we obtain approval in one or more foreign jurisdictions for our product candidates, we will be subject to rules and regulations in those jurisdictions. In some foreign countries, particularly those in the EU, the pricing of biologics is subject to governmental control and other market regulations which could put pressure on the pricing and usage of our product candidates. In these countries, pricing negotiations with governmental authorities can take considerable time after obtaining marketing approval of a product candidate. In addition, market acceptance and sales of our product candidates will depend significantly on the availability of adequate coverage and reimbursement from third-party payors for our product candidates and may be affected by existing and future health care reform measures.

Much like the federal Anti-Kickback Statute prohibition in the United States, the provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in the EU. The provision of benefits or advantages to physicians is governed by the national anti-bribery laws of EU Member States, such as the UK Bribery Act 2010. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain EU Member States must be publically disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual EU Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the EU Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

In addition, in most foreign countries, including the European Economic Area, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing and reimbursement vary widely from country to country. For example, the EU provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. Reference pricing used by various EU member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. In some countries, we may be required to conduct a clinical study or other studies that compare the cost-effectiveness of any of our product candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval. There can be no

assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products. Historically, products launched in the EU do not follow price structures of the United States and generally prices tend to be significantly lower. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If pricing is set at unsatisfactory levels or if reimbursement of our products is unavailable or limited in scope or amount, our revenues from sales by us or our strategic partners and the potential profitability of any of our product candidates in those countries would be negatively affected.

European data collection is governed by restrictive regulations governing the use, processing, and cross-border transfer of personal information.

The collection and use of personal health data in the EU is governed by the provisions of the Data Protection Directive, and as of May 2018 the General Data Protection Regulation, or GDPR. These directives impose several requirements relating to the consent of the individuals to whom the personal data relates, the information provided to the individuals, notification of data processing obligations to the competent national data protection authorities and the security and confidentiality of the personal data. The Data Protection Directive and GDPR also impose strict rules on the transfer of personal data out of the EU to the United States. Failure to comply with the requirements of the Data Protection Directive, the GDPR, and the related national data protection laws of the EU Member States may result in fines and other administrative penalties. The GDPR introduces new data protection requirements in the EU and substantial fines for breaches of the data protection rules. The GDPR regulations may impose additional responsibility and liability in relation to personal data that we process and we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules. This may be onerous and adversely affect our business, financial condition, results of operations and prospects.

Additional laws and regulations governing international operations

If we further expand our operations outside of the United States, we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The FCPA prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The Securities and Exchange Commission, or SEC, also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

We are subject to certain U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions, and other trade laws and regulations. We can face serious consequences for violations.

Among other matters, U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions, and other trade laws and regulations, which are collectively referred to as Trade Laws, prohibit companies and their employees, agents, clinical research organizations, legal counsel, accountants, consultants, contractors, and other partners from authorizing, promising, offering, providing, soliciting, or receiving directly or indirectly, corrupt or improper payments or anything else of value to or from recipients in the public or private sector. Violations of Trade Laws can result in substantial criminal fines and civil penalties, imprisonment, the loss of trade privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We also expect our non-U.S. activities to increase in time. We plan to engage third parties for clinical trials and/or to obtain necessary permits, licenses, patent registrations, and other regulatory approvals and we can be held liable for the corrupt or other illegal activities of our personnel, agents, or partners, even if we do not explicitly authorize or have prior knowledge of such activities.

Risks Related to Our Intellectual Property

Our success depends in part on our ability to protect our intellectual property. It is difficult and costly to protect our proprietary rights and technology, and we may not be able to ensure their protection.

Our commercial success will depend in large part on obtaining and maintaining patent, trademark and trade secret protection of our proprietary technologies and our product candidates, their respective components, formulations, combination therapies, methods used to manufacture them and methods of treatment, as well as successfully defending these patents against third-party challenges. Our ability to stop unauthorized third parties from making, using, selling, offering to sell or importing our product candidates is dependent upon the extent to which we have rights under valid and enforceable patents that cover these activities. If we are unable to secure and maintain patent protection for any product or technology we develop, or if the scope of the patent protection secured is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to commercialize any product candidates we may develop may be adversely affected.

The patenting process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, we may not pursue or obtain patent protection in all relevant markets. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Moreover, in some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from or license to third parties and are reliant on our licensors or licensees.

The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates or uses thereof in the United States or in other foreign countries. Even if the patents do successfully issue, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property or prevent others from designing around our claims. If the breadth or strength of protection provided by the patent applications we hold with respect to our product

candidates is threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize, our product candidates. Further, if we encounter delays in our clinical trials, the period of time during which we could market our product candidates under patent protection would be reduced.

Since patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that we were the first to file any patent application related to our product candidates. Furthermore, for United States applications in which all claims are entitled to a priority date before March 16, 2013, an interference proceeding can be provoked by a third-party or instituted by the United States patent office, or USPTO, to determine who was the first to invent any of the subject matter covered by the patent claims of our applications.

We cannot be certain that we are the first to invent the inventions covered by pending patent applications and, if we are not, we may be subject to priority disputes. We may be required to disclaim part or all of the term of certain patents or all of the term of certain patent applications. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. No assurance can be given that if challenged, our patents would be declared by a court to be valid or enforceable or that even if found valid and enforceable, a competitor's technology or product would be found by a court to infringe our patents. We may analyze patents or patent applications of our competitors that we believe are relevant to our activities, and consider that we are free to operate in relation to our product candidates, but our competitors may achieve issued claims, including in patents we consider to be unrelated, which block our efforts or may potentially result in our product candidates or our activities infringing such claims. The possibility exists that others will develop products which have the same effect as our products on an independent basis which do not infringe our patents or other intellectual property rights, or will design around the claims of patents that we have had issued that cover our products.

Recent or future patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. Under the enacted Leahy-Smith America Invents Act, or America Invents Act, enacted in 2013, the United States moved from a "first to invent" to a "first-to-file" system. Under a "first-to-file" system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to a patent on the invention regardless of whether another inventor had made the invention earlier. The America Invents Act includes a number of other significant changes to U.S. patent law, including provisions that affect the way patent applications are prosecuted, redefine prior art and establish a new post-grant review system. The effects of these changes are currently unclear as the USPTO only recently developed new regulations and procedures in connection with the America Invents Act and many of the substantive changes to patent law, including the "first-to-file" provisions, only became effective in March 2013. In addition, the courts have yet to address many of these provisions and the applicability of the act and new regulations on specific patents discussed herein have not been determined and would need to be reviewed. However, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. For example:

- § others may be able to make or use compounds or cells that are similar to the biological compositions of our product candidates but that are not covered by the claims of our patents;

- the active biological ingredients in our current product candidates will eventually become commercially available in biosimilar drug products, and no patent protection may be available with regard to formulation or method of use;
- we or our licensors, as the case may be, may fail to meet our obligations to the U.S. government in regards to any in-licensed patents and patent applications funded by U.S. government grants, leading to the loss of patent rights;
- we or our licensors, as the case may be, might not have been the first to file patent applications for these inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- it is possible that our pending patent applications will not result in issued patents;
- it is possible that there are prior public disclosures that could invalidate our or our licensors' patents, as the case may be, or parts of our or their patents;
- it is possible that others may circumvent our owned or in-licensed patents;
- it is possible that there are unpublished applications or patent applications maintained in secrecy that may later issue with claims covering our products or technology similar to ours;
- the laws of foreign countries may not protect our or our licensors', as the case may be, proprietary rights to the same extent as the laws of the United States;
- the claims of our owned or in-licensed issued patents or patent applications, if and when issued, may not cover our product candidates;
- our owned or in-licensed issued patents may not provide us with any competitive advantages, may be narrowed in scope, or be held invalid or unenforceable as a result of legal challenges by third parties;
- the inventors of our owned or in-licensed patents or patent applications may become involved with competitors, develop products or processes which design around our patents, or become hostile to us or the patents or patent applications on which they are named as inventors;
- it is possible that our owned or in-licensed patents or patent applications omit individual(s) that should be listed as inventor(s) or include individual(s) that should not be listed as inventor(s), which may cause these patents or patents issuing from these patent applications to be held invalid or unenforceable;
- we have engaged in scientific collaborations in the past, and will continue to do so in the future. Such collaborators may develop adjacent or competing products to ours that are outside the scope of our patents;
- we may not develop additional proprietary technologies for which we can obtain patent protection;
- it is possible that product candidates or diagnostic tests we develop may be covered by third parties' patents or other exclusive rights; or
- the patents of others may have an adverse effect on our business.

We depend on intellectual property licensed from third parties and termination of any of these licenses could result in the loss of significant rights, which would harm our business.

We are dependent on patents, know-how and proprietary technology, both our own and licensed from others. Any termination of these licenses could result in the loss of significant rights and could harm our ability to commercialize our product candidates. See "Business — License Agreements" for additional information regarding our license agreements.

Disputes may also arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;

- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

We are generally also subject to all of the same risks with respect to protection of intellectual property that we license, as we are for intellectual property that we own, which are described below. If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize products could suffer.

If we fail to comply with our obligations under our patent licenses with third parties, we could lose license rights that are important to our business.

We are a party to license agreements pursuant to which we in-license key patent and patent applications for our product candidates. These existing licenses impose various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with these obligations, our licensors may have the right to terminate the license, in which event we would not be able to develop or market the products covered by such licensed intellectual property.

We may have limited control over the maintenance and prosecution of these in-licensed patents and patent applications, activities or any other intellectual property that may be related to our in-licensed intellectual property. For example, we cannot be certain that such activities by these licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents and other intellectual property rights. We have limited control over the manner in which our licensors initiate an infringement proceeding against a third-party infringer of the intellectual property rights, or defend certain of the intellectual property that is licensed to us. It is possible that the licensors' infringement proceeding or defense activities may be less vigorous than had we conducted them ourselves.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to patent protection, we rely heavily upon know-how and trade secret protection, as well as non-disclosure agreements and invention assignment agreements with our employees, consultants and third-parties, to protect our confidential and proprietary information, especially where we do not believe patent protection is appropriate or obtainable. In addition to contractual measures, we try to protect the confidential nature of our proprietary information using physical and technological security measures. Such measures may not, for example, in the case of misappropriation of a trade secret by an employee or third-party with authorized access, provide adequate protection for our proprietary information. Our security measures may not prevent an employee or consultant from misappropriating our trade secrets and providing them to a competitor, and recourse we take against such misconduct may not provide an adequate remedy to protect our interests fully. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret can be difficult, expensive, and time-consuming, and the outcome is unpredictable. In addition, trade secrets may be independently developed by others in a manner that could prevent legal recourse by us. If any of our confidential or proprietary information, such as our trade secrets, were to be disclosed or

misappropriated, or if any such information was independently developed by a competitor, our competitive position could be harmed.

In addition, courts outside the United States are sometimes less willing to protect trade secrets. If we choose to go to court to stop a third-party from using any of our trade secrets, we may incur substantial costs. These lawsuits may consume our time and other resources even if we are successful. Although we take steps to protect our proprietary information and trade secrets, including through contractual means with our employees and consultants, third parties may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or disclose our technology.

Thus, we may not be able to meaningfully protect our trade secrets. It is our policy to require our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to execute confidentiality agreements upon the commencement of employment or consulting relationships with us. These agreements provide that all confidential information concerning our business or financial affairs developed or made known to the individual or entity during the course of the party's relationship with us is to be kept confidential and not disclosed to third parties except in specific circumstances. In the case of employees, the agreements provide that all inventions conceived by the individual, and which are related to our current or planned business or research and development or made during normal working hours, on our premises or using our equipment or proprietary information, are our exclusive property. In addition, we take other appropriate precautions, such as physical and technological security measures, to guard against misappropriation of our proprietary technology by third parties. We have also adopted policies and conduct training that provides guidance on our expectations, and our advice for best practices, in protecting our trade secrets.

Third-party claims of intellectual property infringement may prevent or delay our product discovery and development efforts.

Our commercial success depends in part on our ability to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the proprietary rights of third parties. There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including interference, derivation, *inter partes* review, post grant review, and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. We may be exposed to, or threatened with, future litigation by third parties having patent or other intellectual property rights alleging that our product candidates and/or proprietary technologies infringe their intellectual property rights. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may give rise to claims of infringement of the patent rights of others. Moreover, it is not always clear to industry participants, including us, which patents cover various types of drugs, products or their methods of use or manufacture. Thus, because of the large number of patents issued and patent applications filed in our fields, there may be a risk that third parties may allege they have patent rights encompassing our product candidates, technologies or methods.

If a third-party claims that we infringe its intellectual property rights, we may face a number of issues, including, but not limited to:

- infringement and other intellectual property claims which, regardless of merit, may be expensive and time-consuming to litigate and may divert our management's attention from our core business;
- substantial damages for infringement, which we may have to pay if a court decides that the product candidate or technology at issue infringes on or violates the third-party's rights, and, if the court finds

that the infringement was willful, we could be ordered to pay treble damages and the patent owner's attorneys' fees;

- a court prohibiting us from developing, manufacturing, marketing or selling our product candidates, or from using our proprietary technologies, unless the third-party licenses its product rights to us, which it is not required to do;
- if a license is available from a third-party, we may have to pay substantial royalties, upfront fees and other amounts, and/or grant cross-licenses to intellectual property rights for our products; and
- redesigning our product candidates or processes so they do not infringe, which may not be possible or may require substantial monetary expenditures and time.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations or could otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

Third parties may assert that we are employing their proprietary technology without authorization. Generally, conducting clinical trials and other development activities in the United States is protected under the Safe Harbor exemption as set forth in 35 U.S.C. § 271. If and when SRK-015 or another one of our product candidates is approved by the FDA, that certain third-party may then seek to enforce its patent by filing a patent infringement lawsuit against us. While we do not believe that any claims of such patent that could otherwise materially adversely affect commercialization of our antibody candidates, if approved, are valid and enforceable, we may be incorrect in this belief, or we may not be able to prove it in a litigation. In this regard, patents issued in the U.S. by law enjoy a presumption of validity that can be rebutted only with evidence that is "clear and convincing," a heightened standard of proof. There may be third-party patents of which we are currently unaware with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of our product candidates, constructs or molecules used in or formed during the manufacturing process, or any final product itself, the holders of any such patents may be able to block our ability to commercialize the product candidate unless we obtained a license under the applicable patents, or until such patents expire or they are finally determined to be held invalid or unenforceable. Similarly, if any third-party patent were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, the holders of any such patent may be able to block our ability to develop and commercialize the product candidate unless we obtained a license or until such patent expires or is finally determined to be held invalid or unenforceable. In either case, such a license may not be available on commercially reasonable terms or at all. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, or at all, our ability to commercialize our product candidates may be impaired or delayed, which could in turn significantly harm our business. Even if we obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for

willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize our product candidates, which could harm our business significantly.

Third parties may assert that our employees or consultants have wrongfully used or disclosed confidential information or misappropriated trade secrets.

As is common in the biotechnology and pharmaceutical industries, we employ individuals who were previously employed at universities or other biopharmaceutical or pharmaceutical companies, including our competitors or potential competitors. Although no claims against us are currently pending, and although we try to ensure that our employees and consultants do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of a former employer or other third parties. 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. Even if we are successful in defending against such claims, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. 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. This type of litigation or proceeding could substantially increase our operating losses and reduce our resources available for development 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 substantially greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other intellectual property related proceedings could adversely affect our ability to compete in the marketplace.

We may not be successful in obtaining or maintaining necessary rights to develop any future product candidates on acceptable terms.

Because our programs may involve additional product candidates that may require the use of proprietary rights held by third parties, the growth of our business may depend in part on our ability to acquire, in-license or use these proprietary rights.

Our product candidates may also require specific formulations to work effectively and efficiently and these rights may be held by others. We may develop products containing our compounds and pre-existing pharmaceutical compounds. We may be required by the FDA or comparable foreign regulatory authorities to provide a companion diagnostic test or tests with our product candidates. These diagnostic test or tests may be covered by intellectual property rights held by others. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify as necessary or important to our business operations. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all, which would harm our business. We may need to cease use of the compositions or methods covered by such third-party intellectual property rights, and may need to seek to develop alternative approaches that do not infringe on such intellectual property rights which may entail additional costs and development delays, even if we were able to develop such alternatives, which may not be feasible. Even if we are able to obtain a license, it may be non-exclusive,

thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology.

Additionally, we sometimes collaborate with academic institutions to accelerate our preclinical research or development under written agreements with these institutions. In certain cases, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to others, potentially blocking our ability to pursue our program. If we are unable to successfully obtain rights to required third-party intellectual property or to maintain the existing intellectual property rights we have, we may have to abandon development of such program and our business and financial condition could suffer.

The licensing and acquisition of third-party intellectual property rights is a competitive area, and companies, which may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates. More established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. There can be no assurance that we will be able to successfully complete such negotiations and ultimately acquire the rights to the intellectual property surrounding the additional product candidates that we may seek to acquire.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that one or more of our patents is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated, held unenforceable, or interpreted narrowly and could put our patent applications at risk of not issuing. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business.

We may choose to challenge the patentability of claims in a third-party's U.S. patent by requesting that the USPTO review the patent claims in an *ex-parte* re-exam, *inter partes* review or post-grant review proceedings. These proceedings are expensive and may consume our time or other resources. We may choose to challenge a third-party's patent in patent opposition proceedings in the European Patent Office, or EPO, or other foreign patent office. The costs of these opposition proceedings could be substantial, and may consume our time or other resources. If we fail to obtain a favorable result at the USPTO, EPO or other patent office then we may be exposed to litigation by a third-party alleging that the patent may be infringed by our product candidates or proprietary technologies.

In addition, because some patent applications in the United States may be maintained in secrecy until the patents are issued, patent applications in the United States and many foreign jurisdictions are typically not published until 18 months after filing, and publications in the scientific literature often lag behind actual discoveries, we cannot be certain that others have not filed patent applications for technology covered by our owned and in-licensed issued patents or our pending applications, or that we or, if applicable, a licensor were the first to invent the technology. Our competitors may have filed, and may in the future file, patent applications covering our products or technology similar to ours. Any such patent application may have priority over our owned and in-licensed patent applications or patents, which could require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on

inventions similar to those owned by or in-licensed to us, we or, in the case of in-licensed technology, the licensor may have to participate in an interference proceeding declared by the USPTO to determine priority of invention in the United States. If we or one of our licensors is a party to an interference proceeding involving a U.S. patent application on inventions owned by or in-licensed to us, we may incur substantial costs, divert management's time and expend other resources, even if we are successful.

Interference proceedings provoked by third parties or brought by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could result in a loss of our current patent rights and could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Litigation or interference proceedings may result in a decision adverse to our interests and, even if we are successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our trade secrets or confidential information, particularly in countries where the laws may not protect those rights as fully as in the United States.

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. 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.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent application process and following the issuance of a patent. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Noncompliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. In such an event, our competitors might be able to enter the market, which would have a material adverse effect on our business.

Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court or the USPTO.

If we or one of our licensing partners initiate legal proceedings against a third-party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate, as applicable, is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace, and there are numerous grounds upon which a third-party can assert invalidity or unenforceability of a patent. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we,

our patent counsel and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, or if we are otherwise unable to adequately protect our rights, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection could have a material adverse impact on our business and our ability to commercialize or license our technology and product candidates.

Likewise, our current owned patents covering our proprietary technologies and our product candidates are expected to expire in 2034, without taking into account any possible patent term adjustments or extensions. Our earliest patents may expire before, or soon after, our first product achieves marketing approval in the United States or foreign jurisdictions. Upon the expiration of our current patents, we may lose the right to exclude others from practicing these inventions. The expiration of these patents could also have a similar material adverse effect on our business, results of operations, financial condition and prospects. We own pending patent applications covering our proprietary technologies or our product candidates that if issued as patents are expected to expire from 2033 through 2038, without taking into account any possible patent term adjustments or extensions. However, we cannot be assured that the USPTO or relevant foreign patent offices will grant any of these patent applications.

Changes in patent law in the U.S. and in ex-U.S. jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, in the case *Amgen Inc. v. Sanofi,* the Federal Circuit held that a well characterized antigen is insufficient to satisfy the written description requirement of certain claims directed to a genus of antibodies that are solely defined by function; and in the case of *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.,* the U.S. Supreme Court held that certain claims to DNA molecules are not patentable. We cannot predict how these decisions or any future decisions by the courts, the U.S. Congress or the USPTO may impact the value of our patents. Similarly, any adverse changes in the patent laws of other jurisdictions could have a material adverse effect on our business and financial condition.

We have limited foreign intellectual property rights and may not be able to protect our intellectual property rights throughout the world.

We have limited intellectual property rights outside the United States. Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in 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, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection but where enforcement is not as strong as that in the United States. These products may compete with our products in jurisdictions where we do not have any issued patents and our patent claims 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 biopharmaceutical products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products against third parties in violation of our proprietary rights generally. The initiation of proceedings by third parties to challenge the scope or validity of our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

We may incur substantial costs as a result of litigation or other proceedings relating to patents, and we may be unable to protect our rights to our products and technology.

If we or our licensors choose to go to court to stop a third-party from using the inventions claimed in our owned or in-licensed patents, that third-party may ask the court to rule that the patents are invalid and/or should not be enforced against that third-party. These lawsuits are expensive and would consume time and other resources even if we or they, as the case may be, were successful in stopping the infringement of these patents. In addition, there is a risk that the court will decide that these patents are not valid and that we or they, as the case may be, do not have the right to stop others from using the inventions.

There is also the risk that, even if the validity of these patents is upheld, the court will refuse to stop the third-party on the ground that such third-party's activities do not infringe our owned or in-licensed patents. In addition, the U.S. Supreme Court has recently changed some legal principles that affect patent applications, granted patents and assessment of the eligibility or validity of these patents. As a consequence, issued patents may be found to contain invalid claims according to the newly revised eligibility and validity standards. Some of our owned or in-licensed patents may be subject to challenge and subsequent invalidation or significant narrowing of claim scope in proceedings before the USPTO, or during litigation, under the revised criteria which could also make it more difficult to obtain patents.

We, or our licensors, may not be able to detect infringement against our owned or in-licensed patents, as the case may be, which may be especially difficult for manufacturing processes or formulation patents. Even if we or our licensors detect infringement by a third-party of our owned or in-licensed patents, we or our licensors, as the case may be, may choose not to pursue litigation against or settlement with the third-party. If we, or our licensors, later sue such third-party for patent infringement, the third-party may have certain legal defenses available to it, which otherwise would not be available except for the delay between when the infringement was first detected and when the suit was brought. Such legal defenses may make it impossible for us or our licensors to enforce our owned or in-licensed patents, as the case may be, against such third-party.

If another party questions the patentability of any of our claims in our owned or in-licensed U.S. patents, the third-party can request that the USPTO review the patent claims such as in an *inter partes* review, *ex parte* re-exam or post-grant review proceedings. These proceedings are expensive and may result in a loss of scope of some claims or a loss of the entire patent. In addition to potential USPTO review proceedings, we may become a party to patent opposition proceedings at the EPO or similar proceedings in other foreign patent offices, where either our owned or in-licensed foreign patents are challenged. One of our in-licensed European patents is involved in a multi-party European opposition proceeding at the EPO. While we believe that the granted claims will ultimately be found to be valid, there is a risk that one or more of the grounds

raised by the opponents will invalidate one or more of the granted claims. This may prevent us from asserting this patent against our competitors marketing otherwise infringing products in relevant European countries where this patent has been granted.

In the future, we may be involved in similar proceedings challenging the patent rights of others, and the outcome of such proceedings is highly uncertain. An adverse determination in any such proceeding could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. The costs of these opposition or similar proceedings could be substantial, and may result in a loss of scope of some claims or a loss of the entire patent. An unfavorable result at the USPTO, EPO or other patent office may result in the loss of our right to exclude others from practicing one or more of our inventions in the relevant country or jurisdiction, which could have a material adverse effect on our business.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions such as patent term adjustments and/or extensions, may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including biosimilars. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If we do not obtain patent term extension and data exclusivity for any product candidates we may develop, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Action of 1984 Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents, or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our business, financial condition, results of operations, and prospects could be materially harmed.

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.

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 or may be forced to stop using these names, which we need for name recognition by potential partners or customers in our markets of interest. If we are unable to establish name recognition

based on our trademarks and trade names, we may not be able to compete effectively and our business may be adversely affected.

Risks Related to Our Reliance On Third Parties

We rely on third parties to conduct certain aspects of our preclinical studies and will rely on third parties to conduct our clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines or comply with regulatory requirements, we may not be able to obtain regulatory approval of or commercialize any potential product candidates.

We depend upon third parties to conduct certain aspects of our preclinical studies and will depend on third parties, including independent investigators, to conduct our clinical trials, under agreements with universities, medical institutions, CROs, strategic partners and others. We expect to have to negotiate budgets and contracts with such third parties, which may result in delays to our development timelines and increased costs.

We will rely especially heavily on third parties over the course of our clinical trials, and, as a result, will have limited control over the clinical investigators and limited visibility into their day-to-day activities, including with respect to their compliance with the approved clinical protocol. Nevertheless, we are responsible for ensuring that each of our trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards, and our reliance on third parties does not relieve us of our regulatory responsibilities. We and these third parties are required to comply with GCP requirements, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for product candidates in clinical development. Regulatory authorities enforce these GCP requirements through periodic inspections of trial sponsors, clinical investigators and trial sites. If we or any of these third parties fail to comply with applicable GCP requirements, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to suspend or terminate these trials or perform additional preclinical studies or clinical trials before approving our marketing applications. We cannot be certain that, upon inspection, such regulatory authorities will determine that any of our clinical trials comply with the GCP requirements. In addition, our clinical trials must be conducted with biologic product produced under cGMP, requirements and may require a large number of patients.

Our failure or any failure by these third parties to comply with these regulations or to recruit a sufficient number of patients may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be implicated if any of these third parties violates federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

Any third parties conducting aspects of our preclinical studies or our future clinical trials will not be our employees and, except for remedies that may be available to us under our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our preclinical studies and clinical programs. These third parties may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other product development activities, which could affect their performance on our behalf. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the preclinical or clinical data they obtain is compromised due to the failure to adhere to our protocols or regulatory requirements or for other reasons, our development timelines, including clinical development timelines, may be extended, delayed or terminated and we may not be able to complete development of, obtain regulatory approval of or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed.

If any of our relationships with these third-party CROs or others terminate, we may not be able to enter into arrangements with alternative CROs or other third parties or to do so on commercially reasonable terms.

Switching or adding additional CROs involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new CRO begins work. As a result, delays may occur, which can materially impact our ability to meet our desired development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

Because we rely on third-party manufacturing and supply partners, our supply of research and development, preclinical and clinical development materials may become limited or interrupted or may not be of satisfactory quantity or quality.

We rely on third-party contract manufacturers to manufacture some of our preclinical product candidate supplies and will rely on third-party contract manufacturers to manufacture all of our clinical trial product supplies. We do not own manufacturing facilities for producing any clinical trial product supplies. There can be no assurance that our preclinical and clinical development product supplies will not be limited, interrupted, or of satisfactory quality or continue to be available at acceptable prices. In particular, any replacement of our manufacturer could require significant effort and expertise because there may be a limited number of qualified replacements; this could be particularly problematic where we rely on a single-source supplier, as is currently the case for the manufacture of SRK-015.

The manufacturing process for a product candidate is subject to FDA and foreign regulatory authority review. Suppliers and manufacturers must meet applicable manufacturing requirements and undergo rigorous facility and process validation tests required by regulatory authorities in order to comply with regulatory standards, such as cGMPs. In the event that any of our manufacturers fails to comply with such requirements or to perform its obligations to us in relation to quality, timing or otherwise, or if our supply of components or other materials becomes limited or interrupted for other reasons, we may be forced to manufacture the materials ourselves, for which we currently do not have the capabilities or resources, or enter into an agreement with another third-party, which we may not be able to do on reasonable terms, if at all. In some cases, the technical skills or technology required to manufacture our product candidates may be unique or proprietary to the original manufacturer and we may have difficulty transferring such skills or technology to another third-party and a feasible alternative may not exist. These factors would increase our reliance on such manufacturer or require us to obtain a license from such manufacturer in order to have another third-party manufacture our product candidates. If we are required to change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations and guidelines. The delays associated with the verification of a new manufacturer could negatively affect our ability to develop product candidates in a timely manner or within budget.

We expect to continue to rely on third-party manufacturers if we receive regulatory approval for SRK-015 or any future product candidate. To the extent that we have existing, or enter into future, manufacturing arrangements with third parties, we will depend on these third parties to perform their obligations in a timely manner consistent with contractual and regulatory requirements, including those related to quality control and assurance. If we are unable to obtain or maintain third-party manufacturing for product candidates, or to do so on commercially reasonable terms, we may not be able to develop and commercialize our product candidates successfully. Our or a third-party's failure to execute on our manufacturing requirements and comply with cGMP could adversely affect our business in a number of ways, including:

- an inability to initiate or continue clinical trials of product candidates under development;
- delay in submitting regulatory applications, or receiving regulatory approvals, for product candidates;
- loss of the cooperation of an existing or future collaborator;
- subjecting third-party manufacturing facilities or our manufacturing facilities to additional inspections by regulatory authorities;

- requirements to cease distribution or to recall batches of our product candidates; and
- in the event of approval to market and commercialize a product candidate, an inability to meet commercial demands for our products.

In addition, we contract with fill and finishing providers with the appropriate expertise, facilities and scale to meet our needs. Failure to maintain cGMP can result in a contractor receiving FDA sanctions, which can impact our ability to operate or lead to delays in any clinical development programs. We believe that our current fill and finish contractor is operating in accordance with cGMP, but we can give no assurance that FDA, EMA or other regulatory agencies will not conclude that a lack of compliance exists. In addition, any delay in contracting for fill and finish services, or failure of the contract manufacturer to perform the services as needed, may delay any clinical trials, registration and launches, which could negatively affect our business.

Our reliance on third parties, such as manufacturers and antibody discovery vendors, may subject us to risks relating to manufacturing scale-up and may cause us to undertake substantial obligations, including financial obligations.

For example, in order to conduct clinical trials of our product candidates, we will need to manufacture them in large quantities. We, or any manufacturing partners, may be unable to successfully increase the manufacturing capacity for any of our product candidates in a timely or cost effective manner, or at all. In addition, quality issues may arise during scale-up activities. If we, or any manufacturing partners, are unable to successfully scale up the manufacture of our product candidates in sufficient quality and quantity, the development, testing, and clinical trials of that product candidate may be delayed or infeasible, and regulatory approval or commercial launch of any resulting product may be delayed or not obtained, which could significantly harm our business.

In addition, we rely, and intend to continue to rely, on third party entities to conduct antibody discovery based on criteria and specifications provided by us. Certain antibody discovery vendors may require us to enter into a license agreement with them for the right to use antibodies discovered by them in humans or for commercial purposes. While we have not executed such an agreement to date, there can be no assurance that we will not be required to execute such an agreement in the future if we select a clinical candidate that includes such an antibody and advance that clinical candidate into clinical trials. Such license agreements could include substantial milestone payments and royalties to the extent we choose to use an antibody discovered by such vendors. In addition, if we do not meet our obligations under such license agreements, the counterparties may have the ability to terminate the license agreements and we could lose the right to use the discovered antibodies, which could significantly and adversely impact our business.

Our future collaborations will be important to our business. If we are unable to enter into new collaborations, or if these collaborations are not successful, our business could be adversely affected.

A part of our strategy is to strategically evaluate and, as deemed appropriate, enter into additional partnerships in the future when strategically attractive, including potentially with major biotechnology or pharmaceutical companies. We have limited capabilities for product development and do not yet have any capability for commercialization. Accordingly, we may enter into collaborations with other companies to provide us with important technologies and funding for our programs and technology.

Any future collaborations we enter into may pose a number of risks, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs or license arrangements based on clinical trial results, changes in the collaborators'

strategic focus or available funding, or external factors, such as a strategic transaction that may divert resources or create competing priorities;

- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products and product candidates if the collaborators believe that the competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- collaborators may fail to comply with applicable regulatory requirements regarding the development, manufacture, distribution or marketing of a product candidate or product;
- collaborators with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or terminations of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- if a collaborator of ours is involved in a business combination, the collaborator might deemphasize or terminate the development or commercialization of any product candidate licensed to it by us; and
- collaborations may be terminated by the collaborator, and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

If our collaborations do not result in the successful discovery, development and commercialization of product candidates or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under such collaboration. All of the risks relating to product development, regulatory approval and commercialization described in this prospectus also apply to the activities of our therapeutic collaborators.

Additionally, if one of our collaborators terminates its agreement with us, we may find it more difficult to attract new collaborators and our perception in the business and financial communities could be adversely affected.

We face significant competition in seeking appropriate partners for our product candidates, and the negotiation process is time-consuming and complex. In order for us to successfully partner our product candidates, potential partners must view these product candidates as economically valuable in markets they determine to be attractive in light of the terms that we are seeking and other available products for licensing by other companies. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. Our

ability to reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. If we are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms, or at all, we may have to curtail the development of a product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms, or at all. If we fail to enter into collaborations or do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our product candidates, bring them to market and generate revenue from sales of drugs or continue to develop our technology, and our business may be materially and adversely affected. Even if we are successful in our efforts to establish new strategic partnerships, the terms that we agree upon may not be favorable to us, and we may not be able to maintain such strategic partnerships if, for example, development or approval of a product candidate is delayed or sales of an approved product are disappointing. Any delay in entering into new strategic partnership agreements related to our product candidates could delay the development and commercialization of our product candidates and reduce their competitiveness even if they reach the market.

Risks Related to Our Common Stock and this Offering

No public market for our common stock currently exists, and we do not know whether an active, liquid and orderly trading market will develop for our common stock, or what the market price of our common stock will be, and as a result it may be difficult for you to sell your shares of our common stock.

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 Global Market, an active trading market for our shares may never develop or be sustained following this offering. You may not be able to sell your shares quickly or at the market price if trading in shares of our common stock is not active. The initial public offering price for our common stock will be determined through negotiations with the underwriters, and the negotiated price may not be indicative of the market price of the common stock after the offering. As a result of these and other factors, you may be unable to resell your shares of our common stock at or above the initial public offering price.

Further, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into strategic partnerships or acquire companies or products by using our shares of common stock as consideration.

The price of our stock may be volatile, and you could lose all or part of your investment.

The trading price of our common stock following this offering is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. In addition to the factors discussed in this "Risk Factors" section and elsewhere in this prospectus, these factors include:

- the commencement, enrollment or results of our planned Phase 1 clinical trial for SRK-015;
- any delay in identifying a clinical candidate for our other development programs;
- any delay in our regulatory filings for SRK-015 and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings, including without limitation the FDA's issuance of a "refusal to file" letter or a request for additional information;

- adverse results or delays in future clinical trials;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- adverse regulatory decisions, including failure to receive regulatory approval of SRK-015 or any future product candidate;
- changes in laws or regulations applicable to SRK-015 or any future product candidate, including but not limited to clinical trial requirements for approvals;
- adverse developments concerning our manufacturers;
- our inability to obtain adequate product supply for any approved product or inability to do so at acceptable prices;
- our inability to establish collaborations, if needed;
- our failure to commercialize our product candidates, if approved;
- additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to the use of SRK-015 or any future product candidate;
- introduction of new products or services offered by us or our competitors;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- our ability to effectively manage our growth;
- actual or anticipated variations in quarterly operating results;
- our cash position;
- our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;
- publication of research reports about us or our industry, or product candidates in particular, or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- changes in the market valuations of similar companies;
- overall performance of the equity markets;
- sales of our common stock by us or our stockholders in the future;
- trading volume of our common stock;
- changes in accounting practices;
- ineffectiveness of our internal controls;
- 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;
- general political and economic conditions; and
- other events or factors, many of which are beyond our control.

In addition, the stock market in general, and the market for biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. If the market price of our common stock after this offering does not exceed the initial public offering price, you may not realize any return on your investment in us and may lose some or all of your investment. In the past, securities class action litigation has often been instituted against companies following periods of volatility in the market price of a company's securities. This type of litigation, if instituted, could result in substantial costs and a diversion of management's attention and resources, which would harm our business, operating results or financial condition.

We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.

We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Furthermore, our ability to pay cash dividends is currently restricted by the terms of our credit facility with Silicon Valley Bank, and future debt or other financing arrangements may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock. Any return to stockholders will therefore be limited to the appreciation of their stock.

Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

Immediately following the completion of this offering, our executive officers, directors and their affiliates will beneficially hold, in the aggregate, approximately % of our outstanding voting stock. Therefore, even after this offering, these stockholders will have the ability to influence us through this ownership position. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

If you purchase our common stock in this offering, you will incur immediate and substantial dilution in the book value of your shares.

The initial public offering price will be substantially higher than the pro forma as adjusted net tangible book value per share of our common stock after this offering. Investors purchasing common stock in this offering will pay a price per share that substantially exceeds the pro forma as adjusted net tangible book value per share after this offering. As a result, investors purchasing common stock in this offering will incur immediate dilution of $ per share, 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, representing the difference between our pro forma as adjusted net tangible book value per share after giving effect to this offering and the assumed initial public offering price. Further, investors purchasing common stock in this offering will contribute approximately % of the total amount invested by stockholders since our inception, but will own only approximately % of the shares of common stock outstanding after this offering.

This dilution is due to our investors who purchased shares prior to this offering having paid substantially less when they purchased their shares than the price offered to the public in this offering. To the extent outstanding options are exercised, there will be further dilution to new investors. As a result of the dilution to investors purchasing shares in this offering, investors may receive significantly less than the purchase price paid in this offering, if anything, in the event of our liquidation. For a further description of the dilution that you will experience immediately after this offering, see "Dilution."

We are an emerging growth company, and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act, or the JOBS Act, enacted in April 2012. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements and exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not previously

approved. We could be an emerging growth company for up to five years following the year in which we complete this offering, although circumstances could cause us to lose that status earlier. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the completion of this offering, (b) in which we have total annual gross revenue of at least $1.07 billion or (c) in which we are deemed to be a large accelerated filer, which requires the market value of our common stock that is held by non-affiliates to exceed $700 million as of the prior June 30th, and (2) the date on which we have issued more than $1 billion in non-convertible debt during the prior three-year period. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of delayed adoption of new or revised accounting standards and, therefore, we will be subject to the same requirements to adopt new or revised accounting standards as other public companies that are not emerging growth companies.

We will incur significant increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. We will be subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, which will require, among other things, that we file with the SEC, annual, quarterly and current reports with respect to our business and financial condition. In addition, the Sarbanes-Oxley Act, as well as rules subsequently adopted by the SEC and the Nasdaq Global Market to implement provisions of the Sarbanes-Oxley Act, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices. Further, in July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as "say on pay" and proxy access. Recent legislation permits emerging growth companies to implement many of these requirements over a longer period and up to five years from the pricing of this offering. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to public companies to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. If these requirements divert the attention of our management and personnel from other business concerns, they could have an adverse effect on our business. The increased costs will decrease our net income or increase our net loss, and may require us to reduce costs in other areas of our business or increase the prices of our products or services. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the same or similar coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

We may not be able to meet the internal control reporting requirements imposed by the SEC resulting in a possible decline in the price of our common stock and our inability to obtain future financing.

As directed by Section 404 of the Sarbanes-Oxley Act, the SEC adopted rules requiring each public company to include a report of management on the company's internal controls over financial reporting in

its annual reports. Although the Dodd-Frank Wall Street Reform and Consumer Protection Act exempts companies with a public float of less than $75 million from the requirement that our independent registered public accounting firm attest to our financial controls, this exemption does not affect the requirement that we include a report of management on our internal control over financial reporting and does not affect the requirement to include the independent registered public accounting firm's attestation if our public float exceeds $75 million.

While we expect to expend significant resources in developing the necessary documentation and testing procedures required by Section 404 of the Sarbanes-Oxley Act, there is a risk that we may not be able to comply timely with all of the requirements imposed by this rule. Regardless of whether we are required to receive a positive attestation from our independent registered public accounting firm with respect to our internal controls, if we are unable to do so, investors and others may lose confidence in the reliability of our financial statements and our stock price and ability to obtain equity or debt financing as needed could suffer.

In addition, in the event that our independent registered public accounting firm is unable to rely on our internal controls in connection with its audit of our financial statements, and in the further event that it is unable to devise alternative procedures in order to satisfy itself as to the material accuracy of our financial statements and related disclosures, it is possible that we would be unable to file our Annual Report on Form 10-K with the SEC, which could also adversely affect the market for and the market price of our common stock and our ability to secure additional financing as needed.

Sales of a substantial number of shares of our common stock by our existing stockholders in the public market could cause our stock price to fall.

If our existing stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after the lock-up and other legal restrictions on resale discussed in this prospectus lapse, the trading price of our common stock could decline. Based on shares of common stock outstanding as of December 31, 2017, upon the completion of this offering we will have outstanding a total of shares of common stock. Of these shares, only the shares of common stock sold in this offering by us, plus any shares sold upon exercise of the underwriters' option to purchase additional shares, will be freely tradable without restriction in the public market immediately following this offering.

The lock-up agreements pertaining to this offering will expire 180 days from the date of this prospectus, subject to earlier release of all or a portion of the shares subject to such agreements by Jefferies LLC and Cowen and Company, LLC in their sole discretion. After the lock-up agreements expire, based upon the number of shares of common stock, on an as-converted basis, outstanding as of December 31, 2017, up to an additional shares of common stock will be eligible for sale in the public market. Approximately % of these additional shares are held by directors, executive officers and other affiliates and will be subject to certain limitations of Rule 144 under the Securities Act of 1933, as amended, or the Securities Act.

In addition, shares of common stock that are either subject to outstanding options or reserved for future issuance under our existing equity compensation plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, the lock-up agreements and Rule 144 and Rule 701 under the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline. Additionally, the number of shares of our common stock reserved for issuance under 2018 Stock Option and Incentive Plan will automatically increase on January 1 of each year, beginning on January 1, 2019 and continuing through and including January 1, 2029, by % of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by our board of directors. Unless our board of directors elects not to increase the number of shares available for future grant each year, our stockholders may experience additional dilution.

After this offering, the holders of shares of our common stock as of December 31, 2017 will be entitled to rights with respect to the registration of their shares under the Securities Act, subject to the 180-day lock-up agreements described above. See "Description of Capital Stock — Registration Rights." Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares held by affiliates, as defined in Rule 144 under the Securities Act. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

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

Our management will have broad discretion in the application of our existing cash, cash equivalents and marketable securities and the net proceeds from this offering, including for any of the purposes described in the section titled "Use of Proceeds," and you will not have the opportunity as part of your investment decision to assess whether such proceeds are being used appropriately. Because of the number and variability of factors that will determine our use of our existing cash and cash equivalents and the net proceeds from this offering, their ultimate use may vary substantially from their currently intended use. Our management might not apply our existing cash and cash equivalents and the net proceeds from this offering in ways that ultimately increase the value of your investment. The failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest the net proceeds from this offering in short-term, investment-grade, interest-bearing securities. These investments may not yield a favorable return to our stockholders. If we do not invest or apply the net proceeds from this offering in ways that enhance stockholder value, we may fail to achieve expected financial results, which could cause our stock price to decline.

Anti-takeover provisions under our charter documents and Delaware law could delay or prevent a change of control which could limit the market price of our common stock and may prevent or frustrate attempts by our stockholders to replace or remove our current management.

Our amended and restated certificate of incorporation and amended and restated bylaws, which are to become effective upon the completion of this offering, contain provisions that could delay or prevent a change of control of our company or changes in our board of directors that our stockholders might consider favorable. Some of these provisions include:

- a board of directors divided into three classes serving staggered three-year terms, such that not all members of the board will be elected at one time;
- a prohibition on stockholder action through written consent, which requires that all stockholder actions be taken at a meeting of our stockholders;
- a requirement that special meetings of stockholders be called only by the chairman of the board of directors, the chief executive officer, or by a majority of the total number of authorized directors;
- advance notice requirements for stockholder proposals and nominations for election to our board of directors;
- a requirement that no member of our board of directors may be removed from office by our stockholders except for cause and, in addition to any other vote required by law, upon the approval of not less than two-thirds of all outstanding shares of our voting stock then entitled to vote in the election of directors;
- a requirement of approval of not less than two-thirds of all outstanding shares of our voting stock to amend any bylaws by stockholder action or to amend specific provisions of our certificate of incorporation; and
- the authority of the board of directors to issue convertible preferred stock on terms determined by the board of directors without stockholder approval and which convertible preferred stock may include rights superior to the rights of the holders of common stock.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporate Law, which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These anti-takeover provisions and other provisions in our amended and restated certificate of incorporation and amended and restated bylaws could make it more difficult for stockholders or potential acquirers to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors and could also delay or impede a merger, tender offer or proxy contest involving our company. These provisions could also discourage proxy contests and make it more difficult for you and other stockholders to elect directors of your choosing or cause us to take other corporate actions you desire. Any delay or prevention of a change of control transaction or changes in our board of directors could cause the market price of our common stock to decline.

If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. Securities and industry analysts do not currently, and may never, publish research on our company. If no securities or industry analysts commence coverage of our company, the trading price for our stock would likely be negatively impacted. In the event securities or industry analysts initiate coverage, if one or more of the analysts who covers us downgrades our stock or publishes inaccurate or unfavorable research about our business, our stock price may decline. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

Our amended and restated certificate of incorporation will contain certain exclusive forum provisions requiring that substantially all disputes between us and our stockholders be resolved in certain judicial forums, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

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 certificate of incorporation or our bylaws, any action to interpret, apply, enforce, or determine the validity of our certificate of incorporation or bylaws, or any action asserting a claim against us that is governed by the internal affairs doctrine. The choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage such lawsuits against us and our directors, officers and other employees. Alternatively, if a court were to find the choice of forum provision contained in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could adversely affect our business and financial condition. In addition, our amended and restated certificate of incorporation will contain a provision by virtue of which, unless we consent in writing to the selection of an alternative forum, the United States District Court for the District of Massachusetts will be the exclusive forum for any complaint asserting a cause of action arising under the Securities Act.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements that are based on management's beliefs and assumptions and on information currently available to management. Some of the statements in the sections titled "Prospectus Summary," "Risk Factors," "Management's Discussion and Analysis of Financial Condition and Results of Operations," "Business" and elsewhere in this prospectus contain forward-looking statements. In some cases, you can identify forward-looking statements by the following words: "may," "will," "could," "would," "should," "expect," "intend," "plan," "anticipate," "believe," "estimate," "predict," "project," "potential," "continue," "ongoing" or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words.

These statements involve risks, uncertainties and other factors that may cause actual results, levels of activity, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements. Although we believe that we have a reasonable basis for each forward-looking statement contained in this prospectus, we caution you that these statements are based on a combination of facts and factors currently known by us and our projections of the future, about which we cannot be certain. Forward-looking statements in this prospectus include, but are not limited to, statements about:

- the timing of initiation and completion of our Phase 1 clinical trial and future clinical trials for our lead product candidate, SRK-015, and the results from these trials;
- the success, cost and timing of our other product development activities, preclinical studies and clinical trials, including statements regarding our ability to identity a clinical candidate and lead indication in our TGFb1 program, the timing of initiation and completion of preclinical studies or clinical trials and related preparatory work, and the timing of the availability of the results of these studies and trials;
- our success in identifying and executing a development program for additional indications for SRK-015 and our TGFb1 program;
- our ability to obtain funding for our operations, including funding necessary to complete further development and, upon successful development, if approved, commercialization of SRK-015 or any of our future product candidates;
- the potential for our identified research priorities to advance our proprietary platform, development programs or product candidates;
- our ability to obtain and maintain regulatory approval from the U.S. Food and Drug Administration, European Medicines Agency and other regulatory authorities for SRK-015 and any future product candidates, and any related restrictions, limitations or warnings in the label of any approved product candidate;
- our expectations regarding our ability to obtain and maintain intellectual property protection for our product candidates and the duration of such protection;
- our ability and the potential to successfully manufacture our product candidates for clinical trials and for commercial use, if approved;
- the size and growth potential of the markets for our product candidates, and our ability to serve those markets, either alone or in combination with others;
- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing; and
- our use of the proceeds from this offering.

In addition, you should refer to the section titled "Risk Factors" of this prospectus for a discussion of other important factors that may cause actual results to differ materially from those expressed or implied by the forward-looking statements. As a result of these factors, we cannot assure you that the forward-looking

statements in this prospectus will prove to be accurate. Furthermore, if the forward-looking statements prove to be inaccurate, the inaccuracy may be material. In light of the significant uncertainties in these forward-looking statements, you should not regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified time frame, or at all. The forward-looking statements in this prospectus represent our views as of the date of this prospectus. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we have no current intention of doing so except to the extent required by applicable law. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this prospectus.

The market data and certain other statistical information used throughout this prospectus are based on independent industry publications, governmental publications, reports by market research firms or other independent sources that we believe to be reliable sources. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. We are responsible for all of the disclosure contained in this prospectus, and we believe these industry publications and third-party research, surveys and studies are reliable. While we are not aware of any misstatements regarding any third-party information presented in this prospectus, their estimates, in particular, as they relate to projections, involve numerous assumptions, are subject to risks and uncertainties and are subject to change based on various factors, including those discussed under the section titled "Risk Factors" and elsewhere in this prospectus. Some data are also based on our good faith estimates.

USE OF PROCEEDS

We estimate that the net proceeds to us from the sale of the shares of our common stock in this offering will be approximately $ million, or approximately $ million if the underwriters exercise their option to purchase additional shares in full, based upon 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 estimated underwriting discounts and commissions and estimated offering expenses payable by us.

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) the net proceeds to us from this offering by approximately $ million, assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same. Similarly, an increase (decrease) of 1,000,000 shares in the number of shares offered by us would increase (decrease) the net proceeds to us from this offering by approximately $ million, assuming that the assumed initial public offering price remains the same. We do not expect that a change in the initial public offering price or the number of shares by these amounts would have a material effect on our uses of the proceeds from this offering, although it may accelerate the time at which we will need to seek additional capital.

We currently expect to use the net proceeds from this offering, together with our cash, cash equivalents and marketable securities, as follows:

- $ to fund research and development activities for SRK-015 through ;
- $ to fund TGFb1, BMP6 and other preclinical research and development activities; and
- the remainder for working capital and other general corporate purposes.

Based on our current plans, we believe our existing cash, cash equivalents and marketable securities, together with the net proceeds from this offering, will be sufficient to fund our operating expenses and capital expenditure requirements through .

We cannot specify with certainty all of the particular uses for the net proceeds to be received upon the completion of this offering. Due to uncertainties inherent in the product development process, it is difficult to estimate the exact amounts of the net proceeds that will be used for any particular purpose. We may use our existing cash, cash equivalents and marketable securities and the future payments, if any, generated from any future collaboration agreements to fund our operations, either of which may alter the amount of net proceeds used for a particular purpose. In addition, the amount, allocation and timing of our actual expenditures will depend upon numerous factors, including the results of our research and development efforts, the timing and success of clinical trials and the timing of regulatory submissions. Accordingly, we will have broad discretion in using these proceeds.

Pending their uses, we plan to invest the net proceeds of this offering in short-term, interest-bearing, investment-grade instruments, certificates of deposit or direct or guaranteed obligations of the U.S. government.

DIVIDEND POLICY

We have never declared or paid any cash dividends on our common stock or any other securities. We anticipate that we will retain all available funds and any future earnings, if any, for use in the operation of our business and do not anticipate paying cash dividends in the foreseeable future. In addition, our ability to pay cash dividends is currently restricted by the terms of our credit facility with Silicon Valley Bank, and future debt or other financing arrangements may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock.

CAPITALIZATION

The following table sets forth our cash, cash equivalents and marketable securities and our capitalization as of December 31, 2017:

- on an actual basis;
- on a pro forma basis to give effect to (1) the automatic conversion of all outstanding shares of our convertible preferred stock into an aggregate of 43,135,911 shares of common stock upon the completion of this offering, (2) the automatic conversion of the outstanding warrant to purchase 21,739 shares of convertible preferred stock into a warrant to purchase 21,739 shares of common stock, resulting in the reclassification of the warrant liability to additional paid-in capital, and (3) the filing and effectiveness of our amended and restated certificate of incorporation upon the completion of this offering; and
- on a pro forma as adjusted basis to give further effect to our issuance and sale of shares of our 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, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

You should read the information in this table together with our consolidated financial statements and the related notes appearing elsewhere in this prospectus, as well as the sections of this prospectus captioned "Selected Consolidated Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations."

	As of December 31, 2017		
	Actual	Pro Forma	Pro Forma As Adjusted
	(in thousands, except share and per-share data)		
Cash, cash equivalents and marketable securities	$ 57,959	$ 57,959	$
Convertible preferred stock, $0.001 par value; 43,157,651 shares authorized, 43,135,911 shares issued and outstanding and aggregate liquidation preference of $109,561, actual; no shares issued or outstanding, pro forma or pro forma as adjusted	$ 109,232	$ —	$ —
Stockholders' equity (deficit):			
Preferred stock, $0.001 par value; no shares issued or outstanding, actual; shares authorized and no shares issued or outstanding, pro forma and pro forma as adjusted	—	—	
Common stock, $0.001 par value; 60,000,000 shares authorized, 11,335,445 shares issued and outstanding, actual; shares authorized, pro forma and pro forma as adjusted; 54,471,356 shares issued and outstanding, pro forma; shares issued and outstanding, pro forma as adjusted	11	54	
Additional paid-in capital	3,994	113,220	
Accumulated other comprehensive loss	(2)	(2)	
Accumulated deficit	(57,525)	(57,525)	
Total stockholders' (deficit) equity	(53,522)	55,747	
Total capitalization	$ 55,710	$ 55,747	$

The pro forma as adjusted information is illustrative only, and our capitalization following the completion of this offering will depend on the actual initial public offering price and other terms of this offering determined at pricing. 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) the pro forma as adjusted amount of each of cash, cash equivalents and marketable securities, additional paid-in capital, total stockholders' equity and total capitalization by $ million, assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, each increase (decrease) of 1,000,000 shares in the number of shares offered by us in this offering, as set forth of the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of cash and cash equivalents, additional paid-in capital, total stockholders' equity and total capitalization by $ million, assuming no change in the assumed initial public offering price per share, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The actual, pro forma and pro forma as adjusted information set forth in the table excludes:

- 21,739 shares of common stock issuable upon the exercise of a warrant outstanding as of December 31, 2017, at an exercise price of $1.38 per share;
- 4,120,333 shares of common stock (including 1,885,156 and 1,015,000 shares of common stock issuable upon the exercise of stock options granted subsequent to December 31, 2017, at exercise prices of $2.02 and $2.51 per share, respectively) reserved for future issuance as of December 31, 2017 under our 2017 Stock Option and Incentive Plan, any unissued shares of which will cease to be available for issuance upon the completion of this offering;
- shares of our common stock that will become available for future issuance under our 2018 Stock Option and Incentive Plan upon the effectiveness of the registration statement of which this prospectus forms a part; and
- shares of our common stock that will become available for future issuance under our 2018 Employee Stock Purchase Plan upon the effectiveness of the registration statement of which this prospectus forms a part.

DILUTION

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

Our historical net tangible book value (deficit) as of December 31, 2017 was $(53.5) million, or $(4.72) per share of common stock. Our historical net tangible book value (deficit) is the amount of our total tangible assets less our total liabilities and the carrying value of our convertible preferred stock. Historical net tangible book value (deficit) per share represents historical net tangible book value (deficit) divided by the 11,335,445 shares of common stock outstanding as of December 31, 2017.

Our pro forma net tangible book value as of December 31, 2017 was $55.7 million, or $1.02 per share of our common stock. Pro forma net tangible book value represents the amount of our total tangible assets less our total liabilities, after giving effect to the automatic conversion immediately prior to the completion of this offering of all outstanding shares of our convertible preferred stock into an aggregate of 43,135,911 shares of common stock and the automatic conversion of the outstanding warrant to purchase 21,739 shares of convertible preferred stock into a warrant to purchase 21,739 shares of common stock, resulting in the reclassification of the warrant liability to additional paid-in capital. Pro forma net tangible book value per share represents pro forma net tangible book value divided by the total number of shares outstanding as of December 31, 2017, after giving effect to the pro forma adjustments described above.

After giving further effect to the sale and issuance of shares of our 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 of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of December 31, 2017 would have been $ million, or $ per share. This represents an immediate increase in pro forma as adjusted net tangible book value per share of $ to existing stockholders and immediate dilution of $ in pro forma as adjusted net tangible book value per share to new investors participating in this offering. Dilution per share to new investors is determined by subtracting pro forma as adjusted net tangible book value per share after this offering from the assumed initial public offering price per share paid by new investors.

The following table illustrates this dilution on a per-share basis to new investors:

Assumed initial public offering price per share		$
Historical net tangible book value (deficit) per share as of December 31, 2017	$ (4.71)	
Increase in net tangible book value per share attributable to the automatic conversion of all outstanding shares of convertible preferred stock and the warrant upon completion of this offering		
Pro forma net tangible book value (deficit) per share as of December 31, 2017		
Increase in pro forma as adjusted net tangible book value per share attributable to new investors participating in this offering		
Pro forma as adjusted net tangible book value per share after this offering		
Dilution per share to new investors participating in this offering		$

The pro forma as adjusted information discussed above is illustrative only and will depend on the actual initial price to public and other terms of this offering determined at pricing. 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) our pro forma as adjusted net tangible book value by $, or $ per share, and increase (decrease) the dilution per share to 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, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. An increase of 1.0 million in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase our pro forma as adjusted net tangible book value by $, or $ per share, and decrease the dilution per share to investors participating in this offering by $ per share, assuming that the assumed initial public offering price remains the same, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. A decrease of 1.0 million in the number of shares offered by us, as set forth on the cover page of this prospectus, would decrease our pro forma as adjusted net tangible book value by $, or $ per share, and increase the dilution per share to investors participating in this offering by $ per share, assuming that the assumed initial public offering price remains the same, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

If the underwriters exercise their option in full to purchase additional shares of common stock in this offering, our pro forma as adjusted net tangible book value per share after this offering would be $, representing an immediate increase in pro forma as adjusted net tangible book value per share of $ to existing stockholders and immediate dilution in pro forma as adjusted net tangible book value per share of $ to investors participating in this 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 following table summarizes, on the pro forma as adjusted basis described above as of December 31, 2017, the total number of shares of common stock purchased from us on an as converted to common stock basis, the total consideration paid or to be paid, and the average price per share paid or to be paid by existing stockholders and by investors participating 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	Percentage	Amount (in thousands)	Percentage	
Existing stockholders		%	$	%	$
Investors participating in this offering					
Total	—	%	$	%	

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) the total consideration paid by new investors by $ million and, in the case of an increase, would increase the percentage of total consideration paid by new investors by percentage points and, in the case of a decrease, would decrease the percentage of total consideration paid by new investors by percentage points, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same. An increase (decrease) of 1.0 million in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase (decrease) the total consideration paid by new investors by $ million and, in the case of an increase, would increase the

percentage of total consideration paid by new investors by percentage points and, in the case of a decrease, would decrease the percentage of total consideration paid by new investors by percentage points, assuming no change in the assumed initial public offering price per share.

The table assumes no exercise of the underwriters' option to purchase additional shares in this offering. If the underwriters' option to purchase additional shares is exercised in full, the number of shares of our common stock held by existing stockholders would be reduced to % of the total number of shares of our common stock outstanding after this offering, and the number of shares of common stock held by new investors participating in the offering would be increased to % of the total number of shares outstanding after this offering.

The above discussion and tables are based on shares of common stock issued and outstanding as of December 31, 2017 and excludes:

- 21,739 shares of common stock issuable upon the exercise of a warrant outstanding as of December 31, 2017, at an exercise price of $1.38 per share;
- 4,120,333 shares of common stock (including 1,885,156 and 1,015,000 shares of common stock issuable upon the exercise of stock options granted subsequent to December 31, 2017 at exercise prices of $2.02 and $2.51 per share, respectively) reserved for future issuance as of December 31, 2017 under our 2017 Stock Option and Incentive Plan, any unissued shares of which will cease to be available for issuance upon the completion of this offering;
- shares of our common stock that will become available for future issuance under our 2018 Stock Option and Incentive Plan upon the effectiveness of the registration statement of which this prospectus forms a part; and
- shares of our common stock that will become available for future issuance under our 2018 Employee Stock Purchase Plan upon the effectiveness of the registration statement of which this prospectus forms a part.

New investors will experience further dilution if our outstanding warrant is exercised, new options or warrants are issued under our equity incentive plans or we issue additional shares of common stock, other equity securities or convertible debt securities in the future.

SELECTED CONSOLIDATED FINANCIAL DATA

We have derived the consolidated statement of operations data for the years ended December 31, 2016 and 2017 and the consolidated balance sheet data as of December 31, 2016 and 2017 from our audited consolidated financial statements appearing elsewhere in this prospectus. You should read the following selected consolidated financial data together with our consolidated financial statements and the related notes appearing elsewhere in this prospectus and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of this prospectus. The selected consolidated financial data contained in this section are not intended to replace our consolidated financial statements and the related notes. Our historical results are not necessarily indicative of the results that may be expected in the future.

	Year Ended December 31,	
	2016	2017
	(in thousands, except share, unit, per-share and per-unit data)	
Consolidated Statement of Operations Data:		
Collaboration revenue	$ 379	$ —
Operating expenses:		
Research and development	12,477	19,944
General and administrative	4,112	5,085
Total operating expenses	16,589	25,029
Loss from operations	(16,210)	(25,029)
Other income (expense):		
Interest income (expense), net	(19)	44
Other income (expense), net	22	(10)
Total other income	3	34
Net loss	$ (16,207)	$ (24,995)
Net loss per common unit, basic and diluted	$ (3.54)	
Net loss per share, basic and diluted		$ (5.36)
Weighted average common units outstanding, basic and diluted	4,576,500	
Weighted average common shares outstanding, basic and diluted		4,665,036
Pro forma net loss per share, basic and diluted (unaudited)[1]		$ (0.72)
Pro forma weighted average common shares outstanding, basic and diluted (unaudited)[1]		34,567,011

(1) See Note 17 to our consolidated financial statements appearing at the end of this prospectus for further details on the calculation of basic and diluted net loss per common unit and share and pro forma basic and diluted net loss per share.

	As of December 31,	
	2016	2017
	(in thousands)	
Consolidated Balance Sheet Data:		
Cash, cash equivalents and marketable securities	$ 29,531	$ 57,959
Total assets	32,782	61,637
Convertible preferred units	58,057	—
Convertible preferred stock	—	109,232
Accumulated deficit	(32,530)	(57,525)
Total stockholders' deficit	(30,027)	(53,522)

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 "Selected Consolidated Financial Data" section of this prospectus and our consolidated financial statements and related notes appearing elsewhere 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 biopharmaceutical company focused on the discovery and development of innovative medicines for the treatment of serious diseases in which signaling by protein growth factors plays a fundamental role. Our newly elucidated understanding of the molecular mechanisms of growth factor activation enabled us to develop a proprietary platform for the discovery and development of monoclonal antibodies that locally and selectively target these signaling proteins at the cellular level. We believe this approach, acting in the disease microenvironment, avoids the historical challenges associated with inhibiting growth factors for therapeutic effect. We believe our focus on biologically validated growth factors may facilitate a more efficient development path. We are advancing our lead product candidate, SRK-015, a selective first-in-class inhibitor of the activation of the growth factor myostatin in skeletal muscle, into clinical development for the treatment of spinal muscular atrophy, or SMA. We expect to initiate a Phase 1 clinical trial in the second quarter of 2018. Utilizing our proprietary platform, we are also creating a pipeline of novel product candidates with the potential to transform the lives of patients suffering from a wide range of serious diseases, including other neuromuscular disorders, cancer, fibrosis and anemia.

As more fully described in the section of this prospectus titled "Reorganization," on December 22, 2017, we completed a series of transactions pursuant to which Scholar Rock Merger Sub, LLC, a wholly owned subsidiary of Scholar Rock Holding Corporation, was merged with and into Scholar Rock LLC. As part of the transactions, all convertible preferred units and common units of Scholar Rock, LLC issued and outstanding immediately prior to the Reorganization were exchanged for shares of Scholar Rock Holding Corporation capital stock of the same class or series on a one-for-one basis. Previously outstanding vested and unvested incentive units, irrespective of any strike price or voting rights, were exchanged for an equal number of shares of common stock or restricted common stock, respectively. The restricted common stock was issued with the same vesting terms as the unvested incentive units held immediately prior to the Reorganization. Upon consummation of the Reorganization, the historical consolidated financial statements of Scholar Rock, LLC became the historical consolidated financial statements of Scholar Rock Holding Corporation, the entity whose shares are being offered in this offering. Except as otherwise indicated or the context otherwise requires, all information included in this prospectus is presented giving effect to the Reorganization.

Since inception, our operations have focused on organizing and staffing our company, business planning, raising capital, establishing our intellectual property portfolio and performing research and development of monoclonal antibodies that selectively inhibit activation of growth factors for therapeutic effect. Revenue generation activities have been limited to research services and the issuance of a license, in each case, pursuant to an option and license agreement with Janssen Biotech, Inc., or Janssen, a subsidiary of Johnson & Johnson. We do not have any products approved for sale and have not generated any revenue from product sales. We have funded our operations primarily through private placements of our convertible preferred stock and borrowings under a loan and security agreement, or the credit facility, with Silicon Valley Bank, or SVB. From inception through December 31, 2017, we have raised an aggregate of

$111.2 million of gross proceeds through the issuance of equity and debt to fund our operations, of which $109.2 million was from the issuance of convertible preferred stock and $2.0 million was from borrowings under the credit facility.

Since inception, we have incurred significant operating losses. Our net losses were $16.2 million and $25.0 million for the years ended December 31, 2016 and 2017, respectively. As of December 31, 2017, we had an accumulated deficit of $57.5 million. We expect to continue to incur significant expenses and operating losses for the foreseeable future. In addition, we anticipate that our expenses will increase significantly in connection with our ongoing activities, as we:

- continue activities in support of our Investigational New Drug application, or IND, filed with the U.S. Food and Drug Administration, or FDA, in March 2018, and commence our Phase 1 first-in-human clinical trial for SRK-015, our lead product candidate;
- continue to discover, validate and develop additional product candidates including from our program focused on inhibitors of the activation of transforming growth factor beta 1, or TGFb1;
- maintain, expand and protect our intellectual property portfolio;
- hire additional research, development and business personnel; and
- prepare and begin to operate as a public company upon the completion of this offering.

We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for SRK-015 or any of our future product candidates. In addition, if we obtain regulatory approval for SRK-015 or any of our future product candidates and do not enter into a third-party commercialization partnership, we expect to incur significant expenses related to developing our commercialization capability to support product sales, marketing and distribution activities.

As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of public or private equity offerings and debt financings, government funding arrangements, collaborations, strategic alliances and marketing, distribution or licensing arrangements. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements as, and when, needed, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more of our product candidates.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when or if we will be able to achieve or maintain profitability. Even if we are able to generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

As of December 31, 2017, we had cash, cash equivalents and marketable securities of $58.0 million. We believe that the net proceeds from this offering, together with our existing cash, cash equivalents and marketable securities, will enable us to fund our operating expenses and capital expenditure requirements .

Financial Operations Overview

Collaboration Revenue

We do not have any products approved for sale, and as a result, we have not generated any revenue from product sales and do not expect to generate any revenue from the sale of products in the foreseeable future. We cannot predict if, when, or to what extent we will generate revenue from the commercialization and sale

of SRK-015 or any of our future product candidates. We may never succeed in obtaining regulatory approval for SRK-015 or any of our future product candidates.

To date, all of our revenue has been derived from our option and license agreement with Janssen. We expect that our revenue for the next several years will be derived primarily from payments under our option and license agreement with Janssen or other collaboration and license agreements that we may enter into in the future, if any.

Expenses

Research and Development

Research and development expenses consist primarily of costs incurred for our research and development activities, including our product candidate discovery efforts, and preclinical studies and clinical trials under our research programs, which include:

- employee-related expenses, including salaries, benefits and equity-based compensation expense for our research and development personnel;
- costs of funding research performed by third parties that conduct research and development and preclinical activities on our behalf;
- cost of manufacturing clinical supply related to SRK-015 and any of our future product candidates;
- cost of conducting clinical trials of SRK-015 and any of our future product candidates;
- consulting and professional fees related to research and development activities, including equity-based compensation to non-employees;
- costs of purchasing laboratory supplies and non-capital equipment used in our preclinical studies;
- costs related to compliance with clinical regulatory requirements;
- facility costs and other allocated expenses, which include expenses for rent and maintenance of facilities, insurance, depreciation and other supplies; and
- fees for maintaining license and other amounts due under our third-party licensing agreements.

Research and development costs are expensed as incurred. Costs for certain activities are recognized based on an evaluation of the progress to completion of specific tasks. Nonrefundable advance payments for research and development services to be received in the future from third parties are deferred and capitalized. The capitalized amounts are expensed as the related services are performed.

The successful development of SRK-015 and any future product candidates is highly uncertain. As such, at this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the remainder of the development of SRK-015 and any future product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from the sale of our product candidates, if approved. This is due to the numerous risks and uncertainties associated with developing product candidates, including the uncertainty of:

- establishing an appropriate safety profile;
- successful enrollment in and completion of clinical trials;
- whether our product candidates show safety and efficacy in our clinical trials;
- receipt of marketing approvals from applicable regulatory authorities, if any;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our product candidates;
- commercializing the product candidates, if and when approved, whether alone or in collaboration with others; and
- continued acceptable safety profile of the products following any regulatory approval.

A change in the outcome of any of these variables with respect to the development of SRK-015 or any of our future product candidates would significantly change the costs and timing associated with the development of that product candidate.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect research and development costs to increase significantly for the foreseeable future as our product candidate development programs progress. However, we do not believe that it is possible at this time to accurately project total program-specific expenses through commercialization. There are numerous factors associated with the successful commercialization of any of our product candidates, including future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time based on our stage of development. Additionally, future commercial and regulatory factors beyond our control will impact our clinical development programs and plans.

General and Administrative

General and administrative expenses consist primarily of employee-related expenses, including salaries, benefits and equity-based compensation expenses for personnel in executive, finance, accounting, business development, legal and human resources functions. Other significant general and administrative expenses include facility costs not otherwise included in research and development expenses, legal fees relating to patent and corporate matters and fees for accounting and consulting services.

We anticipate that our general and administrative expenses will increase in the future as our business expands to support expected growth in research and development activities, including the initiation of the planned Phase 1 clinical program from SRK-015 and any future clinical programs. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants, among other expenses. We also anticipate increased expenses associated with being a public company, including costs for audit, legal, regulatory and tax-related services, director and officer insurance premiums and investor relations costs. In addition, if we obtain regulatory approval for any of our product candidates and do not enter into a third-party commercialization collaboration, we expect to incur significant expenses related to building a sales and marketing team to support product sales, marketing and distribution activities.

Interest Income (expense), net

Interest income (expense), net consists primarily of interest expense incurred on our credit facility, including amortization of debt discount and debt issuance costs, and interest income earned on our cash, cash equivalents and marketable securities.

Other Income (expense)

Other income (expense), net consists primarily of non-cash changes in the fair value of warrants issued in connection with our credit facility and a gain recorded on the sale of fixed assets which was recorded in 2016.

Results of Operations

Comparison of the Years Ended December 31, 2016 and 2017

The following table summarizes our results of operations for the years ended December 31, 2016 and 2017 (in thousands except percentages):

	Year Ended December 31,		Change	
	2016	2017	$	%
Collaboration revenue	$ 379	$ —	$ (379)	(100)%
Operating expenses:				
Research and development	12,477	19,944	7,467	60%
General and administrative	4,112	5,085	973	24%
Total operating expenses	16,589	25,029	8,440	51%
Loss from operations	(16,210)	(25,029)	(8,819)	54%
Other income (expense):				
Interest income (expense), net	(19)	44	63	332%
Other income (expense), net	22	(10)	(32)	(145)%
Total other income	3	34	31	NM*
Net loss	$ (16,207)	$ (24,995)	$ (8,788)	54%

* NM means not meaningful.

Collaboration Revenue

Collaboration revenue decreased by $0.4 million from $0.4 million for the year ended December 31, 2016 to $0 for the year ended December 31, 2017. We completed our performance obligations related to conducting research services to identify molecules with either one of two pharmacological profiles under the option and license agreement with Janssen in 2016, and recorded $0.4 million of collaboration revenue related to these activities during that period. No milestones were achieved in 2017.

Research and Development

Research and development expense increased by $7.5 million from $12.5 million for the year ended December 31, 2016 to $19.9 million for the year ended December 31, 2017, an increase of 60%. The

following table summarizes our research and development expense for the years ended December 31, 2016 and 2017 (in thousands except percentages):

	Year Ended December 31,		Change	
	2016	2017	$	%
External costs by program:				
SRK-015	$ 3,113	$ 6,513	$ 3,400	109%
Other early development candidates and unallocated costs	1,361	3,025	1,664	122%
Total external costs	4,474	9,538	5,064	113%
Internal costs:				
Employee compensation and benefits	4,760	6,409	1,649	35%
Facility and other	3,243	3,997	754	23%
Total internal costs	8,003	10,406	2,403	30%
Total research and development expense	$ 12,477	$ 19,944	$ 7,467	60%

The increase in research and development expense was primarily attributable to the following:

- The $5.1 million increase in external costs primarily related to increased research and preclinical development and manufacturing cost associated with our lead product candidate, SRK-015 and other external research costs associated with our other early development candidates.

- The $1.6 million increase in employee compensation and benefits costs related to increased headcount in our research and development function.

A significant portion of our research and development costs have been external costs, which we track on a program-by-program basis after a clinical product candidate has been identified. Our internal research and development costs are primarily personnel-related costs, depreciation and other indirect costs. We do not track our internal research and development expenses on a program-by-program basis as they are deployed across multiple projects under development.

General and Administrative

General and administrative expense increased by $1.0 million from $4.1 million for the year ended December 31, 2016 to $5.1 million for the year ended December 31, 2017.

The increase in general and administrative expense was primarily attributable to an increase of $0.6 million in employee compensation and benefits due to increased headcount and and an increase of $0.3 million in professional and consulting fees primarily due to increases in legal fees related to business development, regulatory and patent costs, accounting and audit fees and public and investor relations fees due to ongoing business activities.

Interest Income (expense), Net

The increase in interest income (expense), net was attributable to increased income earned on our investment portfolio, which increased significantly year-over-year.

Liquidity and Capital Resources

Sources of Liquidity

We have funded our operations from inception through December 31, 2017 with the gross proceeds of $109.2 million from sales of our convertible preferred stock and borrowings of $2.0 million under our credit

facility with SVB. The following table provides information regarding our total cash, cash equivalents and marketable securities at December 31, 2016 and 2017 (in thousands):

	December 31,	
	2016	2017
Cash and cash equivalents	$ 10,033	$ 56,461
Marketable securities	19,498	1,498
Total cash, cash equivalents and marketable securities	$ 29,531	$ 57,959

In August 2015, we entered into the credit facility with SVB for an equipment line of credit of up to $2.0 million to finance the purchase of eligible equipment. Pursuant to the credit facility, SVB was obligated to make up to five equipment advances, each in an amount of at least $100,000 during the draw period. In August 2016, we amended the credit facility to extend the draw period to December 31, 2016. We borrowed $0.7 million against the line of credit in 2015 and $1.3 million in 2016, which fulfilled the maximum credit line of $2.0 million at December 31, 2016. Amounts borrowed bear interest at an annual prime rate less 0.25%. In the event of a default, and during such an event, the annual interest rate will increase by 5%. For each advance, interest-only payments were due and paid through June 2016. Principal and interest payments commenced on July 1, 2016 for a period of 36 months. A final payment fee equal to 4% of the aggregate advances is also due on June 1, 2019. We have the option to prepay the outstanding balance of the loan in full subject to a prepayment fee of 0.5% to 1.0%, depending on when the prepayment occurs. All borrowings under the credit facility mature on July 1, 2019. The loan balance at December 31, 2017 was $1.0 million.

We granted SVB a security interest in all equipment financed under the credit facility. The credit facility contains negative covenants restricting our activities, including limitations on dispositions, change in business ownership or location, mergers or acquisitions, incurring indebtedness or liens, paying dividends or making investments and certain other business transactions.

We also issued a warrant to SVB to purchase 21,739 Series A-3 convertible preferred units at a purchase price of $1.38 per unit, which became exercisable for 21,739 shares of Series A-3 convertible preferred stock at a purchase price of $1.38 per share in connection with the Reorganization. The SVB warrant is exercisable immediately and expires on August 10, 2025. Following the completion of this offering, the warrant will be exercisable for shares of our common stock.

Cash Flows

The following table provides information regarding our cash flows for the years ended December 31, 2016 and 2017 (in thousands):

	Year Ended December 31,	
	2016	2017
Net cash used in operations	$ (15,141)	$ (21,737)
Net cash (used in) provided by investing activities	(20,319)	17,665
Net cash provided by financing activities	1,002	50,500
Net (decrease) increase in cash and cash equivalents	$ (34,458)	$ 46,428

Net Cash Used in Operating Activities

The use of cash in all periods resulted primarily from our net losses adjusted for non-cash charges and changes in components of working capital. Net cash used in operating activities was $21.7 million for the year ended December 31, 2017 compared to $15.1 million for the year ended December 31, 2016. The increase in cash used in operating activities was due to an increase in net loss of $8.8 million for the year ended December 31, 2017 as compared to the year ended December 31, 2016 partially offset by $1.1 million of cash provided by operating assets and liabilities.

Net Cash Used in Investing Activities

Net cash provided by investing activities was $17.7 million for the year ended December 31, 2017 compared to net cash used in investing activities of $20.3 million for the year ended December 31, 2016. Net cash provided by investing activities for the year ended December 31, 2017 consisted of the maturity of marketable securities. Net cash used in investing activities for the year ended December 31, 2016 consisted of the purchase of marketable securities.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $50.5 million during the year ended December 31, 2017 compared to $1.0 million during the year ended December 31, 2016. The cash provided by financing activities for the year ended December 31, 2017 was primarily the result of $51.2 million of net proceeds received from private placements of our convertible preferred stock. The cash provided by financing activities for the year ended December 31, 2016 was primarily the result of borrowings under the loan and security agreement.

Funding Requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development for, initiate later stage clinical trials for, and seek marketing approval for, SRK-015 and any of our future product candidates. In addition, if we obtain marketing approval for SRK-015 or any of our future product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution, which costs we might offset through entry into collaboration agreements with third parties. Furthermore, upon the completion of this offering, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

We expect that the net proceeds from this offering, together with our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses and capital expenditure requirements . We have based this estimate on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect. Our future capital requirements will depend on many factors, including:

- the costs of conducting future clinical trials;
- the costs of future manufacturing;
- the scope, progress, results and costs of discovery, preclinical development, laboratory testing and clinical trials for other potential product candidates we may develop, if any;
- the costs, timing and outcome of regulatory review of our product candidates;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the achievement of milestones or occurrence of other developments that trigger payments under any collaboration agreements we might have at such time;
- the costs and timing of future commercialization activities, including product sales, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;
- the amount of revenue, if any, received from commercial sales of our product candidates, should any of our product candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, obtaining, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- our headcount growth and associated costs as we expand our business operations and research and development activities; and
- the cost of operating as a public company.

Identifying potential product candidates and conducting preclinical studies 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 commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interests may be diluted, and the terms of these securities may include liquidation or other preferences that could adversely affect your rights as a common stockholder. Additional debt financing, if available, 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 impact our ability to conduct our business.

If we raise funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Critical Accounting Policies and Use of Estimates

This management's discussion and analysis is based on our consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these consolidated financial statements requires us to make judgments and estimates that affect the reported amounts of assets, liabilities, revenues and expenses and the disclosure of contingent assets and liabilities in our consolidated financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe to be reasonable under the circumstances. Actual results may differ from these estimates under different assumptions or conditions. On an ongoing basis, we evaluate our judgments and estimates in light of changes in circumstances, facts and experience. The effects of material revisions in estimates, if any, will be reflected in the consolidated financial statements prospectively from the date of change in estimates.

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

Accrued Research and Development Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued expenses as of each balance sheet date. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf 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 expenses as of each balance sheet date based on facts and circumstances known to us at that time. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary. The significant estimates in our accrued research and development expenses include the costs incurred for services performed by our vendors in connection with research and development activities for which we have not yet been invoiced. In certain instances, we prepay for services to be provided in the future. These amounts are expensed as the services are performed.

We base our expenses related to research and development activities on our estimates of the services received and efforts expended pursuant to quotes and contracts with vendors that conduct research and development 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. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the research and development expense. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or prepaid balance accordingly. Nonrefundable advance payments for goods and services that will be used in future research and development activities are expensed when the activity has been performed or when the goods have been received rather than when the payment is made.

Although we do not expect our estimates to be materially different from amounts incurred, if our estimates of the status and timing of services performed differ from the actual status and timing of services performed, it could result in us reporting amounts that are too high or too low in any particular period. To date, there have been no material differences between our estimates of such expenses and the amounts incurred.

Equity-Based Compensation

Prior to the Reorganization, our former parent company, Scholar Rock, LLC, granted incentive units, which we accounted for as equity-classified awards. As part of the Reorganization, the incentive units were exchanged for shares of our common stock.

We measure employee equity-based compensation based on the grant date fair value of the equity-based awards and recognize equity-based compensation expense on a straight-line basis over the requisite service period of the awards, which is generally the vesting period of the respective award. For awards subject to performance conditions, we recognize equity-based compensation expense using an accelerated recognition method over the remaining period when we determine that achievement of the milestone is probable. As of January 1, 2016, we made an accounting policy election to recognize forfeitures as they occur upon adoption of guidance per Accounting Standard Update, or ASU, No. 2016-09, Compensation — Stock Compensation, or ASU 2016-09. The adoption of ASU 2016-09 did not have a material impact on our consolidated financial statements. The term "forfeitures" is distinct from "cancellations" or "expirations" and represents only the unvested portion of the surrendered equity-based award.

We recognize compensation expense for equity-based awards granted to non-employees over the related service period of the award. The fair value of the non-employee equity-based awards are subject to re-measurement at each reporting period prior to vesting, using the then-current fair value of the common or incentive securities.

We classify equity-based compensation expense in our consolidated statement of operations in the same manner in which the award recipient's salary and related costs are classified or in which the award recipient's service payments are classified.

Determination of the Fair Value of Equity-Based Awards

As there has been no public market for our common units or incentive units to date, the estimated fair value of our common units and incentive units has been approved by our board of directors, with input from management, as of the date of each award grant, considering our most recently available independent third-party valuations of common units and incentive units and our board of directors assessment, with input from management, of additional objective and subjective factors that we believed were relevant and which may have changed from the date of the most recent valuation through the date of the grant. In addition, there has been no public market for our common stock to date. The estimated fair value of our common stock has been determined by our board of directors as of the date of each award grant considering our most recently available independent third-party valuations of common stock 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. These independent third-party valuations were performed in accordance with the guidance outlined in the American Institute of Certified Public Accountants' Accounting and Valuation Guide, Valuation of Privately-Held-Company Equity Securities Issued as Compensation. We estimated the value of our equity using the market approach, including the guideline public company method and a precedent transaction method which "backsolves" to a preferred price. We allocated equity value to our common units, incentive units and convertible preferred units or to our shares of common stock and shares of our convertible preferred stock, as the case may be, using either an option-pricing method, or OPM, or a hybrid method, which is a hybrid between the OPM and the probability-weighted expected return method. The OPM treats common securities and preferred securities as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under this method, the common units and incentive units and common stock have value only if the funds available for distribution to members exceed the value of the preferred security liquidation preference at the time of the liquidity event, such as a strategic sale or a merger. The hybrid method estimates the probability-weighted value across multiple scenarios but uses the OPM to estimate the allocation of value within at least one of the scenarios. In addition to the OPM, the hybrid method considers an initial public offering, or IPO, scenario in which the shares of convertible preferred stock are assumed to convert to common stock. The future value of the common units, incentive units and common stock in the IPO scenario is discounted back to the valuation date at an appropriate risk adjusted discount rate. In the hybrid method, the present value

indicated for each scenario is probability weighted to arrive at an indication of value for the common units, incentive units and common stock.

As of December 31, 2016, our third-party valuation report estimated a valuation of our common units of $1.15 per unit, and our incentive units (with a strike price of $0.78) of $0.97 per unit. As of May 18, 2017, our third-party valuation report estimated a valuation of our common units of $1.24 per unit, and our incentive units (with a strike price of $0.78) of $1.05 per unit. As of September 27, 2017, our third-party valuation report estimated a valuation of our common units of $1.71 per unit, and our incentive units (with a strike price of $0.97) of $1.38 per unit. As of December 22, 2017, our third-party valuation report estimated a value of our common stock of $2.02 per share. As of March 5, 2018, our third-party valuation report estimated a value of our common stock of $2.51 per share.

In addition to considering the results of these third-party valuations, management considered various objective and subjective factors to determine the fair value of our common units, incentive units and common stock as of each grant date, which may be a date later than the most recent third-party valuation date, including:

- the prices of our preferred securities sold to or exchanged between outside investors in arm's length transactions, and the rights, preferences and privileges of our preferred securities as compared to those of our common units, incentive units or common stock, including the liquidation preferences of our preferred securities;
- the progress of our research and development efforts, including the status of preclinical studies and planned clinical trials for our product candidates;
- the lack of liquidity of our equity as a private company;
- our stage of development and business strategy and the material risks related to our business and industry;
- the achievement of enterprise milestones, including entering into collaboration and license agreements;
- the valuation of publicly traded companies in the life sciences and biotechnology sectors, as well as recently completed mergers and acquisitions of peer companies;
- any external market conditions affecting the biotechnology industry, and trends within the biotechnology industry;
- the likelihood of achieving a liquidity event for the holders of our common units, incentive units and common stock, such as an IPO, or a sale of our company, given prevailing market conditions; and
- the analysis of IPOs and the market performance of similar companies in the biopharmaceutical industry.

The assumptions underlying these valuations represent management's best estimates, which involve inherent uncertainties and the application of management judgment. As a result, if factors or expected outcomes change and we use significantly different assumptions or estimates, our equity-based compensation expense could be materially different. Following the completion of this offering, the fair value of our common stock will be determined based on the quoted market price of our common stock.

The following table sets forth by grant date and type of award, the number of incentive units or stock options granted; the per unit strike price of incentive units or the per share exercise price of stock options granted between January 1, 2016 and the date of this prospectus.

Date of Issuance	Type of Award	Number of Units or Shares Subject to Awards Granted	Per Unit Strike Price or Per Share Exercise Price	Fair Value per Common Unit on Grant Date	Per Unit or Share Estimated Fair Value of Awards on Grant Date[1]
April 25, 2016	Incentive unit	800,000	$ 0.78	$ 1.20	$0.98
June 3, 2016	Incentive unit	5,000	$ 0.78	$ 1.20	$0.98
August 12, 2016	Incentive unit	652,600	$ 0.78	$ 1.19	$0.98
November 30, 2016	Incentive unit	692,000	$ 0.78	$ 1.16	$0.97
February 14, 2017	Incentive unit	403,160	$ 0.78	$ 1.15	$0.97
February 21, 2017	Incentive unit	42,000	$ 0.78	$ 1.15	$0.97
June 2, 2017	Incentive unit	56,000	$ 0.78	$ 1.24	$1.05
September 27, 2017	Incentive unit	1,043,302	$ 0.97	$ 1.71	$1.38
October 26, 2017	Incentive unit	230,000	$ 0.97	$ 1.71	$1.38
February 20, 2018[2]	Stock option	1,885,156	$ 2.02	$ 2.02	$1.47 - $1.70
April 3, 2018	Stock option	925,000	$ 2.51	$ 2.51	$1.79
April 15, 2018	Stock option	90,000	$ 2.51	$ 2.51	$1.79

(1) For the purposes of recording stock-based compensation for grants of incentive units and common stock to non-employees, we measure the fair value of the award on the service completion date (vesting date). At the end of each reporting period prior to completion of the services, we re-measure the value of any unvested portion of the award based on the then-current fair value of the award and adjust expense accordingly.

(2) Includes 1,734,856 and 95,300 stock options for employee service-based awards with a per share estimated grant date fair value of $1.47 and $1.49, respectively, and 55,000 stock options for employee performance-based awards and non-employee awards with a per share estimated grant date fair value of $1.70.

Revenue Recognition

As of December 31, 2017, all of our revenue to date had been generated exclusively from our option and license agreement with Janssen. We recognize revenue in accordance with Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC, Topic 605, *Revenue Recognition*, or ASC 605, for each unit of accounting when all of the following criteria are met: persuasive evidence of an arrangement exists; delivery has occurred or services have been rendered; the seller's price to the buyer is fixed or determinable; and collectability is reasonably assured. Amounts received prior to satisfying the revenue recognition criteria are recorded as deferred revenue on our consolidated balance sheets.

Multiple Element Arrangements

The terms of our option and license agreement contain multiple deliverables, which we account for based on the guidance in ASC Topic 605-25, *Revenue Recognition — Multiple Element Arrangements,* or ASC 605-25. We evaluate multiple-element arrangements to determine (i) the deliverables included in the arrangement and (ii) whether the individual deliverables represent separate units of accounting or whether they must be accounted for as a combined unit of accounting. When deliverables are separable, consideration received is allocated to the separate units of accounting based on the relative selling price method and the appropriate revenue recognition principles are applied to each unit. When we determine that an arrangement should be accounted for as a single unit of accounting, we must determine the period over which the performance obligations will be performed and revenue will be recognized. This evaluation

requires us to make judgments about the individual deliverables and whether such deliverables are separable from the other aspects of the contractual relationship. Deliverables are considered separate units of accounting provided that (i) the delivered item has value to the customer on a standalone basis and (ii) if the arrangement includes a general right of return with respect to the delivered item, delivery or performance of the undelivered item is considered probable and substantially in our control. In assessing whether an item has standalone value, we consider factors such as the research, development, manufacturing and commercialization capabilities of the collaboration partner and the availability of the associated expertise in the general marketplace. In addition, we consider whether the collaboration partner can use any other deliverable for its intended purpose without the receipt of the remaining deliverable, whether the value of the deliverable is dependent on the undelivered item, and whether there are other vendors that can provide the undelivered items.

The consideration received under an arrangement that is fixed or determinable is then allocated among the separate units of accounting based on the relative selling prices of the separate units of accounting. We determine the selling price of a unit of accounting within each arrangement using vendor-specific objective evidence of selling price, if available; third-party evidence of selling price if vendor-specific objective evidence is not available; or best estimate of selling price, if neither vendor-specific objective evidence nor third-party evidence is available. Determining the best estimate of selling price for a unit of accounting requires significant judgment. In developing the best estimate of selling price for a unit of accounting, we consider applicable market conditions and relevant entity-specific factors, including factors that were contemplated in negotiating the agreement with the customer and estimated costs. We validate the best estimate of selling price for units of accounting by evaluating whether changes in the key assumptions used to determine the best estimate of selling price will have a significant effect on the allocation of arrangement consideration between multiple units of accounting.

We recognize arrangement consideration allocated to each unit of accounting when all of the revenue recognition criteria are satisfied for that particular unit of accounting. In the event that a deliverable does not represent a separate unit of accounting, we recognize revenue from the combined unit of accounting over the contractual or estimated performance period for the undelivered items, which is typically the term of our research and development obligations. If there is no discernible pattern of performance or objectively measurable performance measures do not exist, then we recognize revenue under the arrangement on a straight-line basis over the period we are expected to complete our performance obligations. Conversely, if the pattern of performance over which the service is provided to the customer can be determined and objectively measurable performance measures exist, then we recognize revenue under the arrangement using the proportional performance method. Revenue recognized is limited to the lesser of the cumulative amount of payments received or the cumulative amount of revenue earned, as of the period ending date.

Options in an arrangement are considered substantive if, at the inception of the arrangement, we are at risk as to whether the collaboration partner will choose to exercise the option. Factors that we consider in evaluating whether an option is substantive include the cost to exercise the option, the overall objective of the arrangement, the benefit the counterparty might obtain from the arrangement without exercising the option, and the likelihood the option will be exercised. When an option is considered substantive, we would not consider the option or item underlying the option to be a deliverable at the inception of the arrangement and the associated option fees are not included in allocable consideration, assuming the option is not priced at a significant and incremental discount. We recognize consideration related to the exercise of a substantive option, that is not priced at a significant and incremental discount, upon exercise of the option, assuming there are no remaining deliverables associated with the option and all other revenue recognition criteria are met.

Recognition of Milestones and Royalties

At the inception of an arrangement that includes milestone payments, we evaluate whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. This

evaluation includes an assessment of whether: (1) the consideration is commensurate with either our performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from our performance to achieve the milestone, (2) the consideration relates solely to past performance, and (3) the consideration is reasonably relative to all of the deliverables and payment terms within the arrangement. We evaluate factors such as clinical, regulatory, commercial and other risks that must be overcome to achieve the respective milestones and the level of effort and investment required to achieve the respective milestones in making this assessment. There is considerable judgment involved in determining whether a milestone satisfies all of the criteria required to conclude that a milestone is substantive. In accordance with ASC Topic 605-28, *Revenue Recognition — Milestone Method*, or ASC 605-28, a clinical or regulatory milestone that is considered substantive will be recognized as revenue in its entirety upon successful accomplishment of the milestone, assuming all other revenue recognition criteria are met. Milestones that are not considered substantive would be recognized as revenue as earned if there are no remaining performance obligations or over the remaining period of performance, assuming all other revenue recognition criteria are met. Revenue from a commercial milestone payment will be accounted for as royalties and recorded as revenue upon achievement of the milestone, assuming all other revenue recognition criteria are met.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under applicable SEC rules.

Contractual Obligations, Commitments and Contingencies

The following table summarizes our significant contractual obligations as of payment due date by period at December 31, 2017 (thousands):

	Total	Less than 1 Year	1 to 3 Years	3 to 5 Years	More than 5 Years
Credit facility[1]	$ 1,141	$ 692	$ 449	$ —	$ —
Operating lease obligations[2]	8,761	7,449	1,312	—	—
Purchase obligation with a third party contract manufacturer[3]	638	638	—	—	—
Total	$ 10,540	$ 8,779	$ 1,761	$ —	$ —

(1) Consists of repayment obligation under the credit facility with SVB, including interest.

(2) Represents future minimum repayments under our non-cancellable operating leases that expire five years after the landlord delivers the expansion space to us, which is expected to occur in the second quarter of 2018.

(3) We are required to make certain minimum payments to a third party contract manufacturer. The amounts included in the table above represent the minimum contractual payments in excess of payments made by us as of December 31, 2017.

Under various licensing and related agreements with third parties, we have agreed to make milestone payments and pay royalties to third parties. Pursuant to an exclusive license agreement with Children's Medical Center Corporation, or CMCC, a holder of our common stock, we paid CMCC an annual license maintenance fee of $5,000 in each of 2015 and 2016. Beginning in 2017, this obligation increased to $10,000 per year, and continues until the agreement is terminated. We will also be responsible for up to $1.3 million of development milestone payments through the first regulatory approval of a licensed product, tiered royalty payments of low single-digit percentages on net sales of licensed products in the event that we realize sales from products covered by the license agreement, and between 10% and 20% of non-royalty

income attributable to a sublicense of the CMCC rights. Such products include products developed using our proprietary platform that are covered by a valid claim contained in any patent under the license agreement. Amounts paid to CMCC are recorded as research and development expense in the statements of operations.

We enter into agreements in the normal course of business with vendors for preclinical studies, preclinical and clinical supply and manufacturing services, professional consultants for expert advice and other vendors for other services for operating purposes. We have not included these payments in the table of contractual obligations above since the contracts do not contain any minimum purchase commitments and are cancelable at any time by us, generally upon 30 days prior written notice, and therefore we believe that our non-cancelable obligations under these agreements are not material.

Quantitative and Qualitative Disclosures About Market Risks

We are exposed to market risk related to changes in interest rates. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our cash equivalents are in the form of a money market fund, which is primarily invested in short-term U.S. Treasury obligations, and our marketable securities consist of U.S. Treasury obligations that have contractual maturities of less than one year.

Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term nature of the investments in our portfolio, an immediate one percentage point change in market interest rates would not have a material impact on the fair market value of our investment portfolio or on our financial position or results of operations.

We are not currently exposed to significant market risk related to changes in foreign currency exchange rates; however, we have contracted with and may continue to contract with foreign vendors that are located in Europe. Our operations may be subject to fluctuations in foreign currency exchange rates in the future.

Inflation generally affects us by increasing our cost of labor. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the years ended December 31, 2016 or December 31, 2017.

Emerging Growth Company Status

In April 2012, the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, was enacted. Section 107 of the JOBS Act provides that an "emerging growth company," or EGC, can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act, for complying with new or revised accounting standards. Thus, an EGC 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 delayed adoption of new or revised accounting standards and, therefore, we will be subject to the same requirements to adopt new or revised accounting standards as other public companies that are not emerging growth companies.

We are in the process of evaluating the benefits of relying on other exemptions and reduced reporting requirements under the JOBS Act. Subject to certain conditions, as an EGC, we expect that:

- we will present in this prospectus only two years of audited financial statements, in addition to any required unaudited financial statements, with correspondingly reduced Management's Discussion and Analysis of Financial Condition and Results of Operations disclosure;
- we will avail ourselves of the exemption from providing an auditor's attestation report on our system of controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act;
- we will avail ourselves of the exemption from complying with any requirement that may be adopted by the Public Company Accounting Oversight Board, or PCAOB, regarding mandatory audit firm

rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements, known as the auditor discussion and analysis; and

- we will provide less extensive disclosure about our executive compensation arrangements

We will remain an EGC until the earlier of (i) the last day of the fiscal year in which we have total annual gross revenues of $1.07 billion or more; (ii) the last day of 2023; (iii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission.

Recent Accounting Pronouncements

We have reviewed all recently issued standards and have determined that, other than as disclosed in Note 2 to our consolidated financial statements appearing elsewhere in this prospectus, such standards will not have a material impact on our financial statements or do not otherwise apply to our operations.

BUSINESS

Overview

We are a biopharmaceutical company focused on the discovery and development of innovative medicines for the treatment of serious diseases in which signaling by protein growth factors plays a fundamental role. Our newly elucidated understanding of the molecular mechanisms of growth factor activation enabled us to develop a proprietary platform for the discovery and development of monoclonal antibodies that locally and selectively target these signaling proteins at the cellular level. We believe this approach, acting in the disease microenvironment, avoids the historical challenges associated with inhibiting growth factors for therapeutic effect. We believe our focus on biologically validated growth factors may facilitate a more efficient development path. We are advancing our lead product candidate, SRK-015, a selective first-in-class inhibitor of the activation of the growth factor myostatin in skeletal muscle, into clinical development for the treatment of spinal muscular atrophy, or SMA. We expect to initiate a Phase 1 clinical trial in the second quarter of 2018. Utilizing our proprietary platform, we are also creating a pipeline of novel product candidates with the potential to transform the lives of patients suffering from a wide range of serious diseases, including other neuromuscular disorders, cancer, fibrosis and anemia.

Our proprietary platform is designed to discover and develop monoclonal antibodies that have a high degree of specificity to achieve selective modulation of growth factor signaling. Growth factors are naturally occurring proteins that typically act as signaling molecules between cells and play a fundamental role in regulating a variety of normal cellular processes, including cell growth and differentiation. Current therapeutic approaches to treating diseases in which growth factors play a fundamental role involve directly targeting an active growth factor or its receptor systemically throughout the body and have suffered from a variety of shortcomings:

- multiple growth factors often signal through the same or overlapping sets of related receptors, making it difficult to specifically modulate one pathway over another;
- members of the same growth factor superfamily share considerable structural similarities, making it difficult to achieve specific inhibition of the targeted growth factor; this can result in broad systemic inhibition that can cause undesirable, and in many cases toxic, side effects; and
- systemic and non-selective inhibition of a growth factor can block the growth factor's role in the disease process, but can also simultaneously interfere with its normal physiological roles.

Our innovative approach is rooted in our structural biology insights into the mechanism by which certain growth factors are activated in close proximity to the cell surface, which we refer to as "supracellular activation." We integrate these insights with sophisticated protein expression, assay development and monoclonal antibody discovery capabilities. We believe our proprietary platform can address the challenges of current therapeutic approaches to treating diseases in which growth factors play a fundamental role by:

- targeting the natural activation mechanism to prevent activation of the growth factor rather than attempting to inhibit the growth factor after activation;
- achieving heightened specificity for the targeted growth factor while minimizing interactions with structurally similar and related growth factors, thereby reducing the risk of unintended systemic adverse events; and
- targeting the disease microenvironment, where we believe we can interfere with the disease process while minimizing the effects on the normal physiological processes mediated by the same growth factors.

We are advancing our lead antibody product candidate, SRK-015, a first-in-class inhibitor of the activation of myostatin, into clinical development for the treatment of SMA. Myostatin is a negative regulator of muscle mass expressed primarily in skeletal muscle tissue, and a member of the transforming growth factor beta, or TGFb, superfamily, a group of more than 30 related growth factors that mediate diverse biological

processes. Vertebrate animals that lack the myostatin gene display increased muscle mass and strength relative to their normal counterparts, but are otherwise healthy. We believe inhibition of the activation of myostatin may promote a clinically meaningful increase in muscle mass and strength. As a result, we have focused our initial development efforts for SRK-015 on the treatment of SMA. SMA is a rare, and often fatal, genetic disorder arising from a deficiency of a protein known as "survival of motor neuron," or SMN. This disease typically manifests in young children and is characterized by atrophy of the voluntary muscles of the limbs and trunk and dramatically reduced normal neuromuscular function. An estimated 30,000 to 35,000 patients suffer from SMA in the United States and Europe. In preclinical studies, we observed that SRK-015 promoted increased muscle mass and strength, and *in vitro* studies have shown that the antibody selectively avoids interaction with other closely related growth factors that play distinctly different physiological roles. We believe that SRK-015 has the potential to be the first muscle-directed therapy to reverse or prevent muscle atrophy in SMA patients and could be used both as a monotherapy or in conjunction with therapies that upregulate the expression of SMN. In March 2018, we filed an Investigational New Drug application, or IND, with the U.S. Food and Drug Administration, or FDA for SRK-015. In April 2018, the FDA notified us that our Phase 1 first-in-human clinical trial of SRK-015 may proceed, and we plan to commence our Phase 1 clinical trial in the second quarter of 2018.

Our second antibody program is focused on the discovery and development of highly specific inhibitors of the activation of TGFb1. TGFb1 is also a member of the TGFb superfamily, and increased signaling by TGFb1 is a key driver of a number of disease-relevant processes, including tissue and organ fibrosis, immune system evasion by cancer cells, and bone marrow fibrosis associated with hematological disorders. Historically, selectively targeting TGFb1 signaling has been challenging due to the inability of both small molecule inhibitors and antibodies to avoid off-target inhibition of other, closely related growth factors, TGFb2 and TGFb3. Treatment of animals with these pan-TGFb inhibitors has been associated with a range of toxicities, most notably cardiac toxicity. In preclinical studies of our antibodies, we have observed specific inhibition of TGFb1 activation *in vitro* and immunomodulatory and antifibrotic activity in multiple disease models *in vivo*. In addition, we have completed a 28-day pilot toxicology study of our leading antibody and, to date, we have not observed any drug-related toxicity up to the highest doses tested in the study. In the same study, we tested pan-TGFb inhibitors and observed the toxicities, including cardiac toxicity, that have been observed by others. We are actively evaluating a limited number of our selective inhibitors of the activation of TGFb1 in multiple disease models, and we intend to nominate a clinical candidate to initially pursue in one or more of our currently targeted indications of oncology, immuno-oncology and fibrosis by the first half of 2019.

Our third antibody program targets the signaling of bone morphogenetic protein 6, or BMP6, another member of the TGFb superfamily, which is involved in a diverse set of biological processes in various parts of the body. For example, in the liver, BMP6 signaling is a key controller of the body's ability to regulate iron levels. Given BMP6's important role in iron metabolism, we believe that targeting BMP6 signaling in a liver-selective fashion presents the potential to address both iron-restricted anemias and iron overload conditions. In preclinical studies of our antibodies that target BMP6 signaling in the liver, we have observed increased iron levels in the bloodstream of healthy animals and we are now evaluating a limited number of these antibodies in disease models of iron restricted anemia.

We have worldwide rights to our proprietary platform and all of our product candidates and antibodies with the exception of certain early-stage antibodies that specifically inhibit the activation of TGFb1 in the context of regulatory T cells, which we licensed to Janssen Biotech, Inc., or Janssen, a subsidiary of Johnson & Johnson.

We have assembled an experienced management team, board of directors, scientific founders and advisory board who bring extensive industry experience to our company. The members of our team have deep experience in discovering, developing and commercializing therapeutics with a particular focus on rare diseases, having worked at companies such as Alnylam Pharmaceuticals, Inc., Avila Therapeutics, Inc.,

Biogen, Inc. and Dyax Corp. We were founded by internationally respected scientists, Drs. Timothy A. Springer and Leonard I. Zon of Harvard Medical School and Boston Children's Hospital.

Since our inception in 2012, we have raised over $100 million through convertible preferred stock financings. Our investors include ARCH Venture Partners, Cormorant Asset Management, EcoR1 Capital, Fidelity Management and Research Company, Invus, The Kraft Group, Polaris Partners, Redmile Group and Timothy A. Springer, Ph.D.

Our Approach and Proprietary Platform

Our innovative approach is rooted in our newly elucidated understanding of the molecular mechanisms of growth factor activation and signaling and is designed to discover and develop monoclonal antibody product candidates that can inhibit the activation of a growth factor with an unprecedented degree of selectivity. Our proprietary platform is designed to generate product candidates that target the growth factor's latent precursor form prior to its activation within the disease microenvironment, or tissue where it is localized, and would normally signal upon activation.

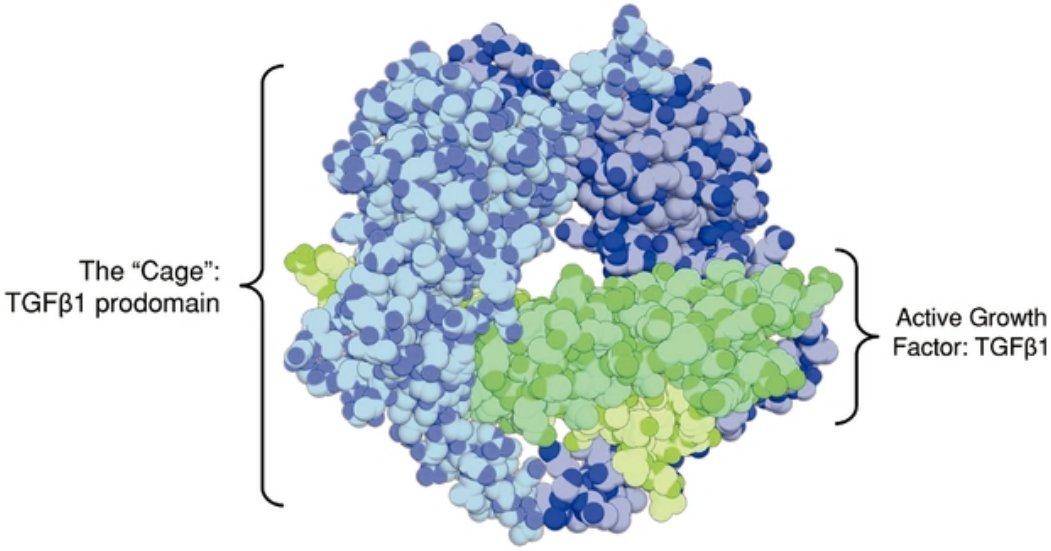
Growth factors are naturally occurring proteins that typically act as signaling molecules between cells and play a fundamental role in regulating a variety of normal cellular processes. Members of the TGFb superfamily of growth factors, for example, can mediate diverse biological functions, including cell growth and differentiation, tissue homeostasis, immune modulation and extracellular matrix remodeling. Growth factors, including members of the TGFb superfamily, such as myostatin, TGFb1 and BMP6, have also been shown to play a fundamental role in a variety of disease processes, including neuromuscular disorders, cancer, fibrosis and anemia. Because of the importance of growth factors in multiple diseases, the pharmaceutical industry has made many attempts to inhibit growth factors in a variety of therapeutic settings. However, products utilizing conventional approaches have seen only limited success. Current therapeutic approaches to treating diseases in which growth factors play a fundamental role involve directly targeting an activated growth factor or its receptor systemically throughout the body and have suffered from a variety of shortcomings:

- multiple growth factors often signal through the same or overlapping sets of related receptors, making it difficult to specifically modulate one pathway over another;
- members of the same growth factor superfamily share considerable similarities (for example, myostatin and GDF11 are approximately 90% identical in the growth factor domains) making it difficult to achieve selective inhibition of the targeted growth factor. Inhibiting both the intended growth factor target and other closely related targets can result in unintentionally broad systemic inhibition that can cause undesirable, and in many cases toxic, side effects; and
- systemic and nonselective inhibition of a growth factor can block the growth factor's role in the disease process, but can also simultaneously interfere with its normal physiological roles.

Our approach to the discovery and development of growth factor-targeted drugs is fundamentally new and different from traditional approaches. Our approach is based on the breakthrough discovery by the laboratory of our co-founder, Timothy A. Springer Ph.D. of Harvard Medical School and Boston Children's Hospital, of the supracellular activation mechanism by which growth factors in the TGFb superfamily are locally activated by a variety of specific stimuli in close proximity to the cell surface.

Unlike many other proteins that are produced and secreted by cells in a mature, or active, form, many growth factors are expressed by cells in a precursor, or latent, form. For example, TGFb1 is produced by cells as a single protein which is then enzymatically processed by the cells into two distinct and physically separated domains — the mature growth factor and the remaining portion of the original protein, referred to as the prodomain — which remain associated as part of a complex. This secreted complex is latent, or inactive, and must first be activated to carry out its normal function in a highly localized tissue or disease microenvironment. In a seminal peer-reviewed publication in 2011, Dr. Springer elucidated a new understanding of the mechanism of supracellular activation as it applies to members of the TGFb

superfamily, by solving a high resolution x-ray crystal structure of this latent form of TGFb1, as illustrated in the graphic below.



Structural representation of the latent form of TGFb1 wherein the prodomain wraps around the active growth factor.

This research explained at a molecular level why the secreted form of TGFb1 is inactive. The prodomain, though physically separated from the mature growth factor domain, forms a "cage" around the active form of TGFb1, blocking the growth factor from signaling through its receptor. Only when the cage is "unlocked" by a supracellular activation event can the growth factor be released and mediate its effects in the local microenvironment. Dr. Springer further hypothesized that this phenomenon likely holds true for most members of the TGFb superfamily, though the exact nature of the activation event, such as integrin binding or enzymatic cleavage, may differ among members of the superfamily. Importantly, while many growth factors are structurally very similar, their cages are structurally diverse, and this provides the basis for our approach to improved selectivity.

To enable our novel approach, we have built a proprietary platform that is rooted in our structural biology insights into supracellular activation. We integrate these insights with sophisticated protein expression, assay development and monoclonal antibody discovery capabilities. In addition to such know-how, our proprietary platform is covered by two patent families, with issued patents projected to expire in 2034. The key elements of our proprietary platform include the following:

- focusing on growth factor targets with a high degree of evidence implicating them in a disease process or processes;
- generating recombinant versions of the latent forms of targeted growth factors, as well as versions of closely related growth factors utilizing proprietary technology and in-house expertise;
- developing proprietary assays in which we are able to recapitulate the natural supracellular activation mechanism that these growth factors undergo in the human body;
- designing sophisticated selection strategies utilizing recombinant antibody libraries such as phage and yeast display that allow us to identify monoclonal antibodies, a well-established therapeutic modality, that can modulate the supracellular activation of these growth factors without having an effect on the activation of other closely related growth factors; and

- optimizing the output of such selections to ensure that our product candidates have the appropriate characteristics for manufacturability and further development.

Using our innovative approach and proprietary platform, we are creating a pipeline of novel product candidates that selectively modulate the activation of growth factors implicated in a variety of serious diseases.

We believe there are several important advantages to our approach over conventional therapeutic approaches, which inhibit the growth factors or their receptors systemically throughout the body:

- targeting the latent precursor allows intervention at the site of action, within the microenvironment of the diseased tissue. Because our antibodies specifically bind the latent forms of the growth factors, we can prevent the activation of the growth factors. Given that many growth factors act primarily within the microenvironment where they are activated, as opposed to exerting their effects systemically, we believe that prevention of activation is a preferred mode of action for achieving improved outcomes. In contrast, traditional approaches to targeting growth factor signaling are focused on inhibiting the growth factor after it has been activated and released systemically;

- targeting the latent precursor allows heightened selectivity among structurally related growth factors, which we believe could limit off-target effects. For example, two members of the TGFb superfamily, myostatin and GDF11, are 90% identical in their growth factor domains. Therefore, many of the traditional inhibitors that target myostatin also inadvertently inhibit GDF11. Similarly, most of the known inhibitors of TGFb are pan-inhibitors, meaning they do not distinguish among the three isoforms of TGFb, namely, TGFb1, TGFb2 and TGFb3. Despite the sequence similarities of the active forms of these growth factors, their cages are structurally diverse. We have been able to harness this diversity to generate antibodies that specifically bind the inactive growth factor precursors and inhibit activation of a particular growth factor of interest, but not others that are closely related; and

- targeting these precursor forms in the disease microenvironment, we believe we can interfere with the disease process while minimizing the effects on the normal physiological processes mediated by growth factors.

Our Strategy

Using our proprietary platform to unlock the therapeutic potential of targeting growth factor signaling in the disease microenvironment, our goal is to deliver novel therapies to underserved patients suffering from a wide range of serious diseases, including neuromuscular disorders, cancer, fibrosis and anemia. To achieve this goal we plan to:

- ***Rapidly advance our lead product candidate, SRK-015, through clinical proof-of-concept.*** We are currently developing our lead product candidate, SRK-015, for the treatment of patients with SMA. By targeting the latent form of myostatin and specifically inhibiting its activation in muscle, we believe SRK-015 holds considerable promise in addressing the atrophy of skeletal muscle in patients with SMA. In March 2018, we filed an IND for SRK-015 and, in April 2018, the FDA notified us that our Phase 1 first-in-human clinical trial of SRK-015 may proceed. We plan to commence our Phase 1 clinical trial in the second quarter of 2018. Assuming successful results and subject to regulatory feedback, we intend to conduct a Phase 2 clinical proof-of-concept trial to evaluate the efficacy and safety of SRK-015 in patients with later-onset SMA, including those patients who are being treated with a currently approved SMA therapy. We plan to commence our Phase 2 trial in the first quarter of 2019 and expect to report top-line results in the second half of 2019.

- ***Advance our TGFb1 program into clinical development.*** Our second antibody program is focused on the discovery and development of highly specific inhibitors of the activation of TGFb1. We believe that the selectivity of our antibodies is a significant differentiator in our efforts to address the historical challenges with inhibiting the TGFb signaling pathway. In preclinical studies of our antibodies, we have observed inhibition of TGFb1 activation *in vitro*, and immunomodulatory and antifibrotic activity in multiple *in vivo* disease models. We intend to nominate a clinical candidate in one or more of our currently targeted indications of oncology, immuno-oncology and fibrosis in our TGFb1 program by the first half of 2019.

- ***Explore additional indications for our existing and emerging product candidates.*** Given the multiple physiological roles played by the distinct targets in our lead programs, we believe that there is potential for us to address multiple additional indications beyond those already selected. For example, we believe that SRK-015 may have a role in treating other muscle-wasting diseases and we believe that our TGFb1 program has the potential to address multiple disorders associated with increased TGFb1 signaling, such as tissue and organ fibrosis, immune system evasion by cancer cells, and bone marrow fibrosis associated with hematological disorders. Our goal is to maximize the value of our existing programs by exploring their potential in additional indications.

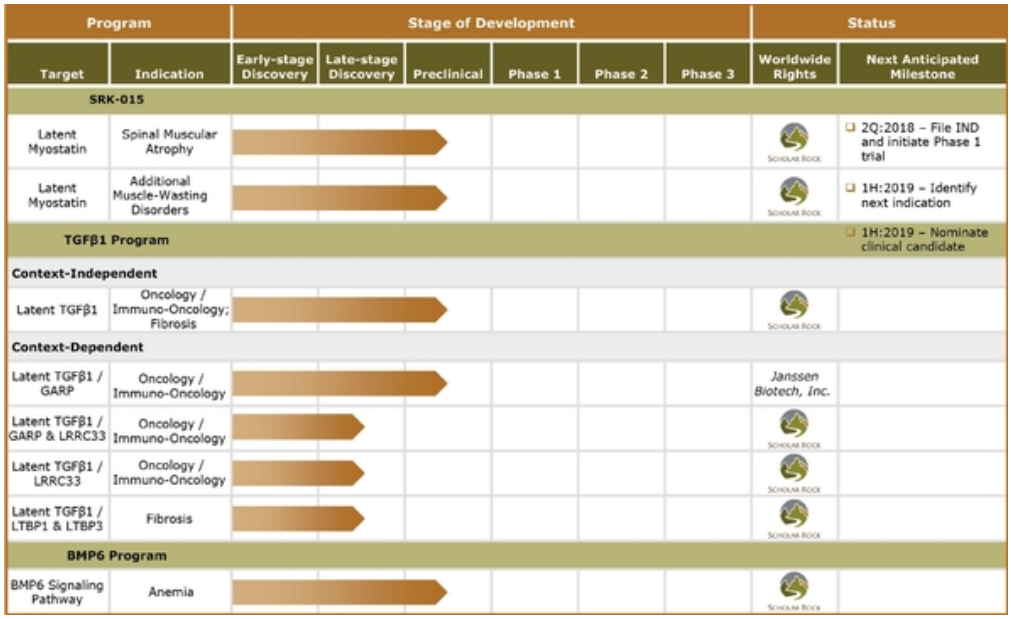
- ***Continue to leverage our proprietary platform to expand our pipeline beyond current lead programs.*** We will continue to leverage our proprietary platform to selectively target the activation of additional growth factors, both within and beyond the TGFb superfamily. Given the established role of signaling by protein growth factors in numerous diseases, we believe that these efforts could result in multiple new opportunities to treat diseases with high unmet medical need. In order to support our pipeline expansion and intention to be the leader in the field of growth factor-targeted drug development, we are investing in the technologies supporting our proprietary platform, including a focus on tools and assays to enhance and accelerate our drug discovery process.

- ***Selectively seek strategic collaborations to maximize the value of our proprietary platform and pipeline.*** Given the potential of our proprietary platform to generate novel product candidates that could treat a wide variety of diseases, we believe that we can maintain in-house discipline with respect to our key development and commercialization efforts, while at the same time maximizing the full potential of our proprietary platform for other disease areas and indications. As a result, we may seek to form strategic collaborations around certain targets, product candidates or disease areas that we believe could benefit from the resources of either larger biopharmaceutical companies or those specialized in a particular area of relevance.

- ***Attract and retain people that share our commitment to scientific excellence and a focus on patients.*** We are focused on developing novel medicines that make a significant difference in the lives of patients suffering from devastating and life-threatening diseases. In addition to building a team of people with deep experience in biology, protein sciences, antibody drug discovery, and development and operations, we believe our focus on a patient-centric collaborative and passionate workplace culture is critical to the success of our mission. We will continue to emphasize this focus as we grow and build our company.

Our Pipeline Programs

Using our innovative approach and proprietary platform, we are creating a pipeline of novel product candidates that selectively inhibit the supracellular activation of growth factors believed to be important drivers in a variety of diseases, including neuromuscular disorders, cancer, fibrosis and anemia. Our proprietary platform includes (i) our know-how expressing and purifying latent protein growth factor complexes in quantity and quality sufficient to enable antibody discovery; (ii) strategies to identify rare antibodies that selectively bind targeted latent protein growth factor complexes; and (iii) assays developed by us in which to test the highly selective antibodies' ability to modulate the activation of specific latent growth factors. We have worldwide rights to our proprietary platform and all of our product candidates, with the exception of certain early-stage antibodies that specifically inhibit the activation of TGFb1 in the context of regulatory T cells, which we licensed to Janssen. The following summarizes our pipeline programs:



Our Lead Product Candidate and Additional Programs

SRK-015 — Our Inhibitor of Myostatin Activation

We are developing SRK-015, a selective first-in-class inhibitor of the activation of the growth factor myostatin in skeletal muscle, for the treatment of SMA. Myostatin, a member of the TGFb superfamily of growth factors, is expressed primarily in skeletal muscle cells and the absence of its gene is associated with an increase in muscle mass and strength in multiple animal species. We believe that inhibition of the activation of myostatin may promote a clinically meaningful increase in muscle mass and strength. In preclinical studies, treatment with SRK-015 resulted in an increase in muscle mass and strength in healthy animals as well as maintenance of muscle in multiple models of muscle atrophy. In March 2018, we filed an IND for SRK-015 and, in April 2018, the FDA notified us that our Phase 1 first-in-human clinical trial of SRK-015 may proceed. We plan to commence our Phase 1 clinical trial in the second quarter of 2018.

Background on SMA

SMA is a rare, and often fatal, genetic disorder that typically manifests in young children. It is characterized by the loss of motor neurons, atrophy of the voluntary muscles of the limbs and trunk and progressive muscle weakness. Disease severity in SMA can range from patients who die soon after birth to patients who live into adulthood with varying degrees of morbidity. The underlying pathology of SMA is caused by insufficient production of a protein known as "survival of motor neuron," or SMN. The SMN protein, essential for the survival of motor neurons, is encoded by two genes, SMN1 and SMN2.

- SMN1 genes produce the majority of functional SMN protein; healthy individuals have one or two functional copies of SMN1, while patients with SMA have mutations in or deletions of both copies of the gene.

- SMN2 genes produce only 10% to 20% of functional SMN protein and an individual's copy number of the SMN2 gene can range from zero to eight. In SMA patients, the number of SMN2 genes present in their genome is correlated with disease onset and severity; patients who have a lower number of SMN2 gene copies generally develop earlier and more severe SMA, because they produce less SMN protein.

SMA Natural History and Epidemiology

SMA, the most common monogenic cause of death in infants, is a rare neuromuscular disorder. An estimated 30,000 to 35,000 patients suffer from SMA in the United States and Europe. Patients with SMA can be categorized as one of four types, Type 1 through Type 4. More than 80% of SMA patients currently living are categorized as having Type 2 or Type 3 disease, sometimes referred to as later-onset SMA, and represent our initially targeted patient population.

- Type 1 disease is the most severe form, with clinical signs emerging at or shortly following birth. Patients with Type 1 SMA suffer from respiratory compromise and often require mechanical ventilation shortly after birth. Type 1 infants are never able to sit without support. Type 1 SMA is the most common form of the disease, and accounts for 58% of patients born with SMA. Historically, only 1% of patients with Type 1 disease survive beyond two years of age without mechanical respiratory support. Type 1 SMA represents only 14% of patients with SMA, although recent therapies may extend patient lifespans. Type 1 patients begin to lose motor neurons and muscle mass before birth.

- Type 2 disease manifests in early childhood and is less severe than Type 1 disease, although patients exhibit profound deficits in motor function. Patients with Type 2 disease may be able to sit independently but they will never walk without aid. While only 29% of the incident population, patients with Type 2 disease account for 51% of the patients living with SMA today.

- Type 3 disease manifests usually in childhood and accounts for about 13% of patients born with SMA, although patients in this category account for 35% of all patients with SMA. While Type 3 SMA patients usually learn to walk unaided, the majority lose that ability over time. Ambulatory Type 3 SMA patients commonly suffer from substantial motor functional impairment, as evidenced by Hammersmith Functional Motor Scale Expanded, or HFMSE, scores and Six-Minute Walk Test distances, two commonly used measures of motor function.

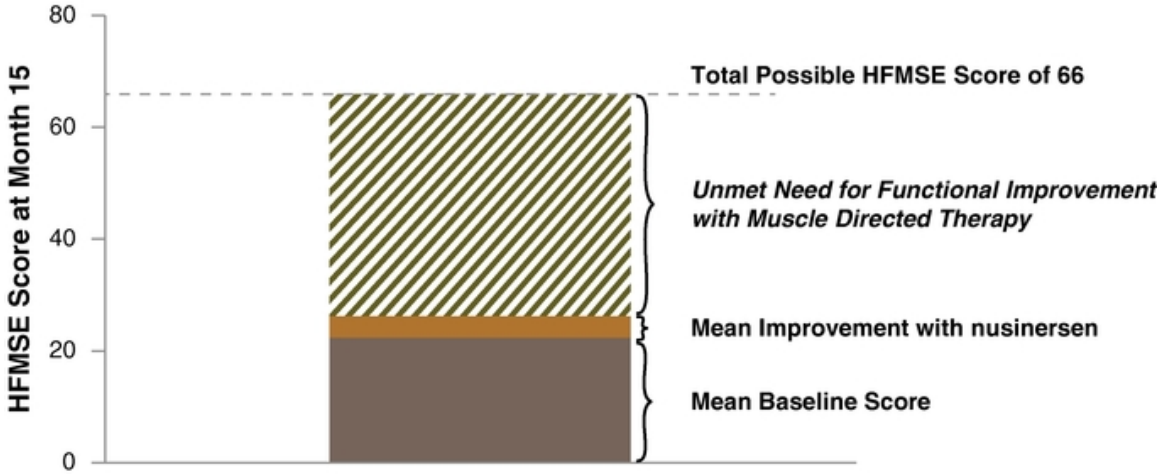
- Type 4 disease is the mildest form of SMA, and its population is not well characterized. After symptom onset, which is most commonly reported between 20 and 30 years of age, patients experience mild to moderate muscle weakness and increasing disabilities. Patients are ambulatory and their life expectancy is normal.

Unmet Medical Need in SMA

Patients with SMA continue to have a high unmet medical need despite recent approval of nusinersen, an SMN upregulator. Nusinersen was approved by the FDA in December 2016 and the European Medicines Agency, or EMA, in June 2017 for the treatment of patients with SMA. Nusinersen is an antisense

oligonucleotide directed against SMN2 that aims to increase functional SMN protein expression. SMN upregulator therapies act primarily to preserve motor neurons. While this approach may improve motor function, it does not act directly on the muscle to reverse or prevent atrophy. We believe that SRK-015 has the potential to be the first muscle-directed therapy to reverse or prevent muscle atrophy in patients with all types of SMA and further improve patient outcomes as a monotherapy or when used in conjunction with SMN upregulator therapies such as nusinersen.

The CHERISH pivotal trial of nusinersen in later-onset SMA patients made use of the HFMSE, a validated outcome measure specifically designed for evaluation of Type 2 and 3 SMA patients that is often used in clinical practice and studies. This examination assesses 33 individual items of motor activity, each scored from 0 to 2 points (lower score indicates worse motor function), with a maximum possible score of 66. The HFMSE evaluates a patient's ability to perform basic tasks such as sitting, reaching one's hand to one's head, changing body positions (e.g. sitting to lying position), crawling, standing, kneeling, squatting, jumping and ascending/descending stairs. These tasks are viewed by SMA patients and caregivers as meaningful and relevant to conducting activities of daily living. In this trial, as illustrated in the figure below, treatment with nusinersen improved motor function, but the HFSME scores of treated patients remained well below those of healthy children. Patients who received nusinersen achieved a 3.9-point mean improvement at Month 15 from a mean baseline of 22.4. Compared to control patients, there was a statistically significant difference of 4.9 points in the mean change from baseline to Month 15 in the HFMSE score. The percentage of nusinersen-treated patients achieving a > 3-point increase was 57%. Although this trial met its primary endpoint and demonstrated a clinically meaningful benefit overall, these results also indicate that most of the gap in attaining normal HFMSE performance has not been adequately addressed by nusinersen therapy and significant unmet need remains.



Mean improvement in HFMSE score experienced by patients with later-onset SMA in the Phase 3 CHERISH clinical trial of nusinersen.

While nusinersen has shown improvement in motor function in patients with SMA, there remains a significant unmet need for an effective muscle-directed therapy that can reverse or prevent muscle atrophy, thereby improving muscle strength and motor function in patients with SMA. Given the novel mechanism of SRK-015, we believe this therapy has the potential to provide a clinically meaningful improvement in motor function in a broad population of SMA patients who may or may not be on background SMN upregulator therapies, such as nusinersen. Accordingly, we believe SRK-015 has the potential to fulfill a significant unmet medical need for SMA patients both as a monotherapy and in conjunction with standard of care.

Myostatin in SMA and Challenges with Traditional Approaches

Our lead product candidate, SRK-015, is a selective inhibitor of the activation of latent myostatin that acts locally within skeletal muscle. Myostatin, also known as growth differentiation factor 8, or GDF8, is a member of the TGFb superfamily and is produced by skeletal muscle cells. As with other tissues and organs in the human body, healthy muscle homeostasis is maintained by a proper balance of growth signals, or anabolic stimuli, and breakdown signals, or catabolic stimuli. In humans, the anabolic stimuli that drive muscle growth are proteins such as human growth hormone and insulin-like growth factor 1. In contrast, myostatin is a catabolic agent that functions as a negative regulator of muscle mass.

Skeletal muscle fibers are generally classified as fast-twitch or slow-twitch. Fast-twitch fibers play a key role in motor activities such as those involving quick bursts of strength, sprinting or eccentric contraction. In contrast, slow-twitch fibers are important for endurance activities. Animals lacking functional myostatin genes, or its receptor, have larger muscles and increased strength compared to normal animals. While the absence of myostatin does lead to overall increases in muscle mass, a preferential effect on muscles enriched for fast-twitch muscle fibers has been observed in animals. Such animals are otherwise healthy and live a normal life-span.

Because of its role in regulating muscle mass, myostatin has been a popular target for a variety of drug development programs. There have been two general approaches to trying to inhibit the signaling of myostatin in humans. The first is to develop an antibody, or an antibody-like molecule, that binds to mature myostatin in circulation and prevents its ability to signal through its receptor, the ActRIIb receptor. The second is to develop an antibody to the ActRIIb receptor itself, or a soluble decoy of the ActRIIb receptor, with a goal of preventing myostatin signaling through its receptor. Both of these approaches, however, have significant limitations.

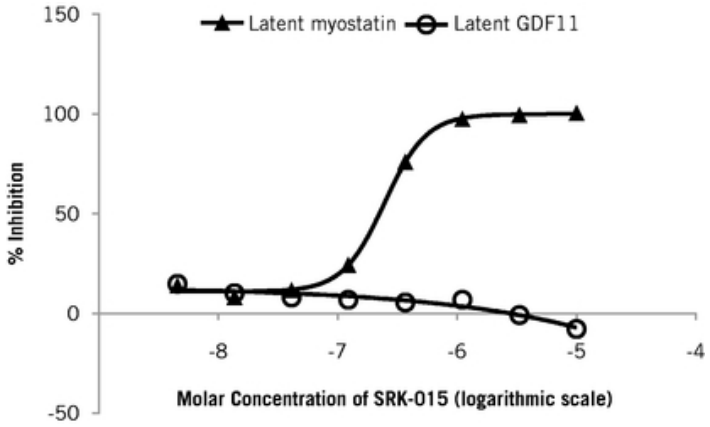
As a member of the TGFb superfamily, mature myostatin shares considerable structural similarity with other family members. For example, the active form of myostatin and its most closely related family member, GDF11, are 90% identical in the growth factor domains, making it extremely challenging to identify antibodies that are truly specific for myostatin and do not interfere with other targets. Moreover, attempts to interrupt myostatin signaling through its receptor are complicated by the fact that the ActRIIb receptor, in addition to being the receptor for myostatin, is also the receptor for a number of related family members, including GDF11, activin and other growth factors. Attempts to block the signaling of myostatin by targeting its receptor therefor inevitably interfere with the signaling of these other growth factors, many of which are involved in normal biological processes unrelated to muscle.

There are multiple examples of clinical trials demonstrating the risk of non-selective inhibition of myostatin. For example, in a Phase 2 trial in Duchenne Muscular Dystrophy reported in 2017, a soluble decoy of the ActRIIb receptor resulted in bleeding side effects believed by the sponsor to be unrelated to inhibition of myostatin signaling, but instead related to the inhibition of signaling by certain other members of the TGFb superfamily known to be important in the maintenance of vascular integrity. These side effects resulted in termination of the clinical program. More recently, results from a clinical trial were reported showing that treatment of patients with an antibody to the ActRIIb receptor resulted in suppression of the levels of follicle stimulating hormone, an important reproductive hormone. In this trial, the sponsor believed that these effects were likely related to inhibition of signaling through the ActRIIb receptor.

Our Solution

Utilizing our proprietary platform, we targeted the precursor form of myostatin and generated SRK-015, a selective first-in-class inhibitor of the activation of myostatin from its inactive precursor in skeletal muscle where myostatin resides and signals upon activation. While mature myostatin is 90% identical in the growth factor domain to its most closely related TGFb superfamily member, GDF11, the prodomain that cages mature myostatin and keeps it in its latent precursor form is only 52% identical to the GDF11 prodomain.

As a result, in preclinical studies, we observed that SRK-015 bound to latent myostatin with a high level of selectivity, while having no binding to, and no effect on, the activation of related TGFb family members.



SRK-015 showed dose-dependent inhibition of the activation of latent myostatin in an in vitro activation assay and had no effect on latent GDF11 activation.

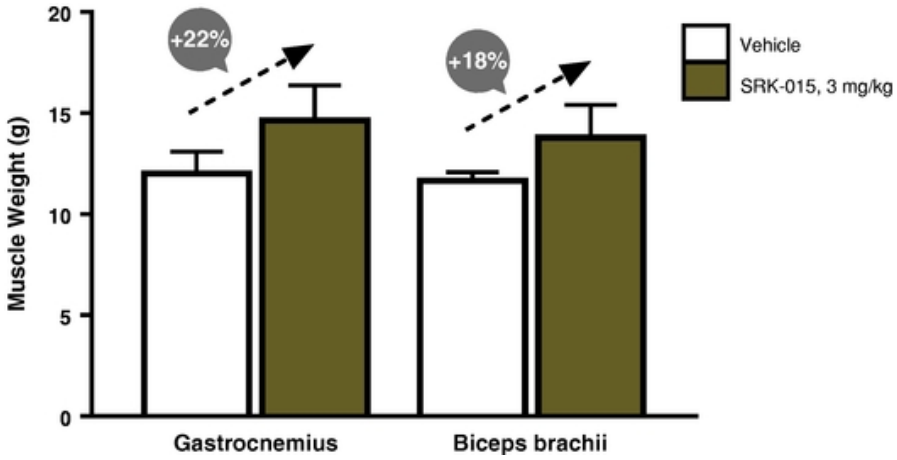
We believe that the pathophysiologic and clinical characteristics of SMA are well-aligned with the optimal setting for observing therapeutic benefit from inhibition of myostatin activation. These characteristics are summarized in the figure below. Since myostatin regulates muscle catabolism rather than anabolism, we believe that having a background of anabolic capacity is important to drive muscle growth in the setting of myostatin inhibition. Anabolic capacity is most robust in younger individuals and diminishes as one ages. Furthermore, in SMA, there is a significant but incomplete loss of motor neurons, ensuring at least some intact connectivity between muscle and nerve, also known as innervation. This partial loss of motor neurons causes substantial atrophy of fast-twitch muscle fibers that in turn leads to many of the motor function impairments. Validated outcome measures are available for SMA clinical trials that are relevant to fast-twitch fiber activity. These outcome measures, such as the HFMSE, assess a large number of motor activities that involve short-term bursts of strength, which are driven by fast-twitch muscle fibers. These endpoints therefore measure an outcome that may be more likely to be directly affected by SRK-015.

Optimal Setting for Myostatin Inhibition		Key Characteristics of SMA
Younger population	▶	Genetic disorder with onset in childhood
Muscle disease with at least partially intact innervation	▶	Incomplete loss of motor neurons
Need for increase in fast-twitch muscle fibers	▶	Substantial deficit in fast-twitch fibers
Clinical trial endpoint driven by fast-twitch fiber function	▶	Fast-twitch fiber function: prominent role in SMA outcome measures

Table summarizing why the pathophysiologic and clinical characteristics of SMA are well aligned with the optimal setting for observing therapeutic benefit from inhibition of myostatin activation.

SRK-015 Preclinical Results

In our earliest pharmacology work, we observed that treatment with SRK-015 robustly increased muscle mass and strength in healthy mice and rats. In addition to increasing muscle mass, we also observed that treatment with SRK-015 resulted in gains in muscle function. The increase in muscle mass was replicated in non-human primates. As shown in the figure below, in this study the gastrocnemius (a calf muscle) and biceps brachii (an arm muscle), two muscles containing a higher proportion of fast-twitch fibers than slow-twitch fibers, increased in size by 18% and 22%, respectively, in cynomolgus monkeys treated with SRK-015 as compared to the vehicle control group.

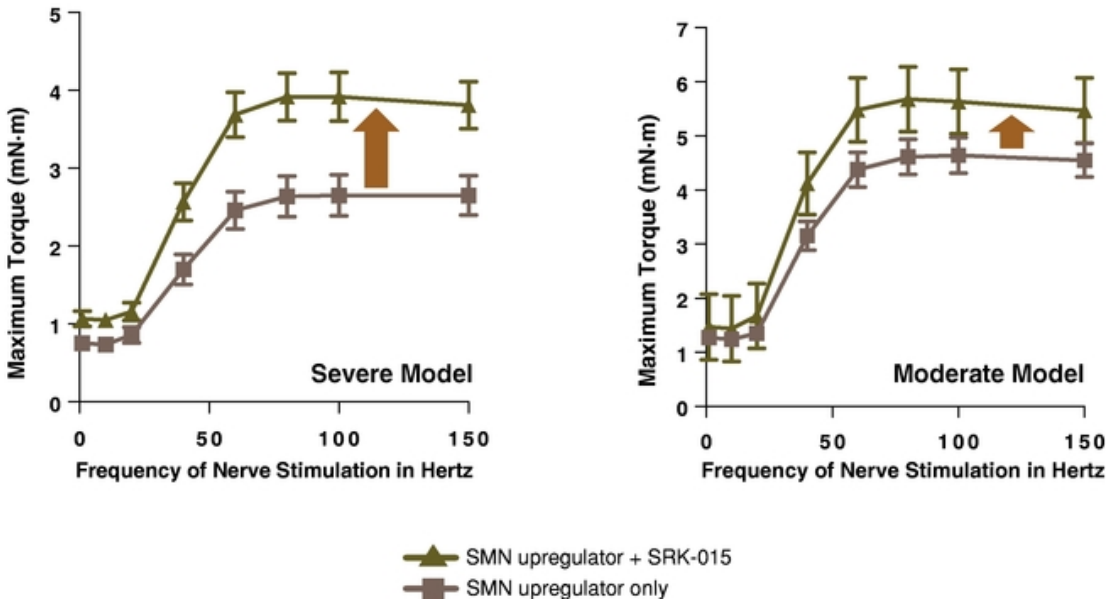


SRK-015 treatment increased muscle mass, as measured by muscle weight in the gastrocnemius (a calf muscle) and biceps brachii (an arm muscle) of cynomolgus monkeys as compared to monkeys treated with the vehicle only.

We next assessed the ability of SRK-015 to improve muscle function in the D7 mouse model, a genetic model of SMA wherein the SMN1 gene has been deleted and copies of the human SMN2 gene have been introduced, thus mimicking the genetics of the human disease. SMN D7 mice are extremely fragile if not treated with a drug that upregulates the underlying deficiency in SMN. Accordingly, this model is best suited for determining the effect of a product candidate such as SRK-015 when administered in conjunction with an SMN upregulator. In this study, we used a small molecule SMN2 splice modulator, SMN-C1, as the SMN upregulator.

We evaluated the ability of SRK-015 to improve muscle force generation in two versions of the D7 mouse model: one designed to emulate a more severe form of SMA and the other a more moderate form of SMA. As shown in the figure below, in both models animals treated with SRK-015 in conjunction with the SMN

upregulator experienced a significant increase in maximum muscle torque generation in the leg compared to animals treated with the SMN upregulator alone.



SRK-015, in combination with an SMN upregulator, improved in vivo muscle force generation in versions of the D7 mouse model designed to emulate either a severe (left side) or moderate (right side) form of SMA, as compared to SMN upregulator therapy alone. Muscle force was assessed by maximum torque generation following nerve stimulation, at a range of frequencies, in the plantarflexor muscle group in the leg. The arrows indicate the increase in muscle force generation due to SRK-015 treatment.

We have filed patent applications seeking to cover SRK-015 as well as other antibodies with the same mechanism. In September 2017, we announced the issuance of U.S. Patent 9,758,576, which covers monoclonal antibodies that selectively inhibit myostatin signaling by blocking the proteolytic activation of latent myostatin, providing protection for our lead antibody SRK-015, as well as any other monoclonal antibodies that work by this unique mechanism of action. This patent expires in May 2034, not including any potential patent term extension.

Clinical Development Overview

We are currently advancing SRK-015 into a clinical development program for the treatment of SMA. Our IND-enabling toxicology studies in rat and cynomolgus monkeys have been completed and, in March 2018, we filed an IND for SRK-015. In April 2018, the FDA notified us that our Phase 1 first-in-human clinical trial of SRK-015 may proceed. We plan to commence our Phase 1 clinical trial in the second quarter of 2018. Our Phase 1 trial is designed to assess the safety, tolerability, pharmacokinetics, immunogenicity and pharmacodynamics of single- and multiple-ascending doses of intravenous SRK-015 in healthy adult volunteers.

Assuming the successful completion of our Phase 1 trial, we plan to conduct a Phase 2 proof-of-concept trial to evaluate the efficacy and safety of SRK-015 in patients with later-onset SMA. This includes Type 2 and non-ambulatory Type 3 SMA patients who may already be receiving an approved SMN upregulator therapy like nusinersen as background standard of care, as well as ambulatory Type 3 patients who will be

administered SRK-015 as a monotherapy. We expect top-line results from the Phase 2 proof-of-concept trial to be available in the second half of 2019.

Beyond the initial proof-of-concept trials in Type 2 and Type 3 SMA patients, we believe that SRK-015 has the potential to contribute an important therapeutic benefit to patients with both more and less severe forms of SMA.

On March 22, 2018, the FDA granted orphan drug designation for SRK-015 for the treatment of SMA.

Other Myostatin Indications

We believe that SRK-015 has therapeutic potential to improve muscle function in multiple other muscle-wasting disorders, including muscle atrophy due to partial denervation, incomplete spinal cord injury, amyotrophic lateral sclerosis, glucocorticoid-induced muscle-wasting and Duchenne muscular dystrophy. These disorders bear many of the characteristics relevant to the optimal setting in which we believe that therapeutic benefit from myostatin inhibition may be observed. In addition to conducting a proof of concept study in SMA, we are actively considering the investigation of SRK-015 in multiple other indications.

Inhibitor of TGFb1 Activation Programs

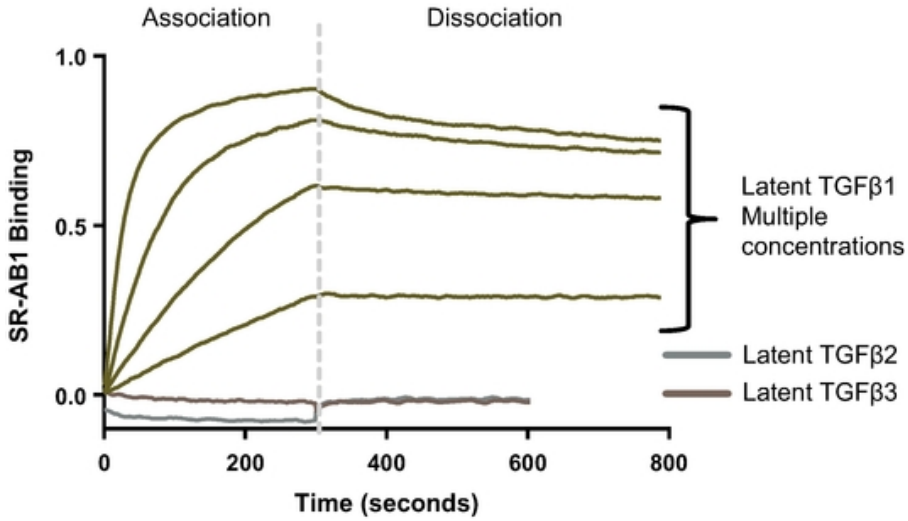
TGFb1 is also a member of the TGFb superfamily, and increased signaling by TGFb1 is a key driver of a number of disease-relevant processes, including tissue and organ fibrosis, immune system evasion by cancer cells and bone marrow fibrosis associated with hematological disorders. Historically, selectively targeting TGFb1 signaling has been challenging due to the inability of both small molecule inhibitors and antibodies to avoid off-target inhibition of other, closely related growth factors, TGFb2 and TGFb3. Treatment of animals with these pan-TGFb inhibitors has been associated with a range of toxicities, most notably cardiac toxicity. Furthermore, since each of these growth factors signals through the same TGFb receptor, ALK5, inhibitors of the TGFb receptor suffer from similar dose-limiting toxicities. Using our proprietary platform, we have generated highly specific and local inhibitors of the activation of TGFb1 that, in our preclinical studies, showed no detectable inhibition of the activation of TGFb2 or TGFb3.

Identification of Selective Inhibitors of TGFb1 Activation

TGFb1 is produced by cells as a single protein and is then enzymatically processed by the cells into two distinct and physically separated domains — the mature, active growth factor and the remaining portion of the original protein, referred to as the prodomain — which remain associated and inactive. This complex also includes one of a number of "presenting molecules" which when secreted serve to tether the latent precursor in specific locations in the body. TGFb1 is produced by a variety of cell types, including fibroblasts, which deposit latent TGFb1 in connective tissue, as well as regulatory T cells and macrophages, which display latent TGFb1 on their cell surfaces.

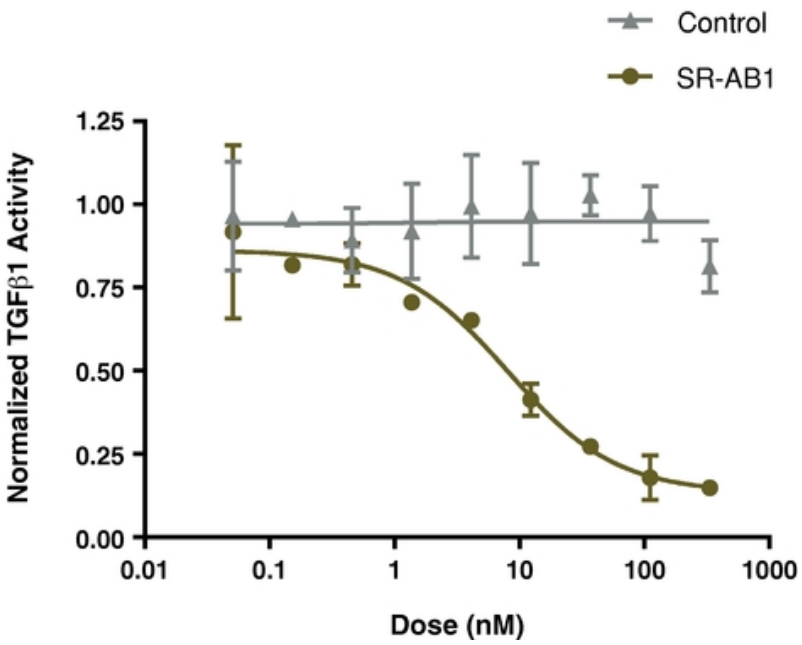
In a seminal peer-reviewed publication in 2011, Dr. Springer elucidated a new understanding of the mechanism of supracellular activation as it applies to members of the TGFb superfamily, by solving a high resolution x-ray crystal structure of the latent form of TGFb1. This research explained at a molecular level why the secreted form of TGFb1 is inactive. The prodomain, though physically separated from the mature growth factor domain, forms a "cage" around the active form of TGFb1, blocking the ability of the growth factor to signal through its receptor. Integrin proteins are able to unlock the "cage" by binding to the prodomain of the latent TGFb1 complex and applying force to pull the complex open, allowing the mature growth factor to be released and signal in its microenvironment. While mature TGFb1 shares a high degree of structural similarity with its closely related family members, TGFb2 and TGFb3, their respective cages are structurally diverse. By taking advantage of the differences among the prodomains, together with our understanding of the activation mechanism and ability to recapitulate the activation mechanism *in vitro*, we were able to identify multiple highly selective inhibitors of the activation of latent TGFb1.

We have conducted *in vitro* and *in vivo* studies to characterize our selective TGFb1 activation inhibitors. An example of the selectivity and *in vitro* inhibitory activity observed for one of our selective TGFb1 activation inhibitors, SR-AB1, is shown in the figure below. This inhibitor bound to latent TGFb1 with high affinity and showed no detectable binding to latent TGFb2 or latent TGFb3. Given the *in vitro* and *in vivo* activity observed with SR-AB1 and at least three additional TGFb1 activation inhibitors, we believe that one or more of these may be advanced as a clinical candidate, and upcoming comparative *in vivo* studies may enable selection of the best candidate. We intend to nominate a clinical candidate in one or more of our currently targeted indications of oncology, immuno-oncology and Fibrosis in our TGFb1 program by the first half of 2019.



SR-AB1, one of our selective TGFb1 activation inhibitors, showed dose-dependent binding of latent TGFb1 with no detectable binding to latent TGFb2 or latent TGFb3 in vitro.

We have also observed potent inhibitory activity for this inhibitor in an *in vitro* latent TGFb1 activation assay, as shown in the figure below.



SR-AB1 showed dose-dependent inhibition of TGFb1 activity in an in vitro cell based assay of latent TGFb1 activation.

We have also completed 7- and 28-day pilot toxicology studies for our leading selective inhibitor of the activation of latent TGFb1, and have identified no drug-related toxicities up to 100 mg/kg dosed weekly, the highest dose tested. This is in contrast to the cardiac pathology we observed after up to one week of dosing with an ALK5 inhibitor or an inhibitor of all three forms of mature TGFb.

TGFb1 in Fibrosis

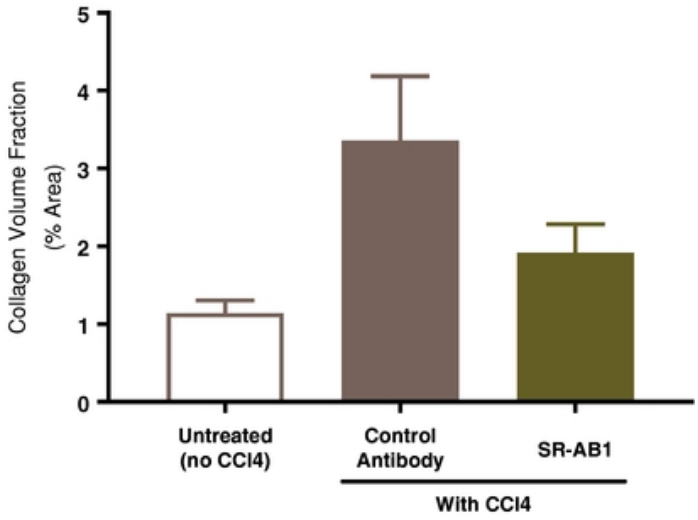
Based on our preclinical results, we believe that specific inhibition of TGFb1 alone may be sufficient to suppress profibrotic signaling in multiple organs, and holds the promise of better tolerated and more effective therapies for a variety of fibrotic diseases than historical approaches. We are currently evaluating our selective inhibitors of latent TGFb1 activation in a variety of translational models of organ fibrosis.

Fibrosis is a pathological feature of disease in virtually all organs, characterized by excessive accumulation of extracellular matrix in the affected tissue, and accounts for substantial morbidity and mortality. Multiple peer-reviewed studies have implicated TGFb signaling as a central regulator of fibrosis. TGFb is upregulated in many animal models of fibrosis, and overexpression of TGFb *in vivo* induces fibrotic changes. Furthermore, TGFb inhibition in animal models has been shown to reduce fibrosis in models of hepatic, renal and cardiac fibrosis. In humans, in an open-label trial of fresolimumab, an inhibitor of all three forms of TGFb, in systemic sclerosis, a fibrotic connective tissue disease, improved clinical skin disease as measured by the modified Rodnan skin score, a commonly used measure of skin thickness, was observed, although bleeding episodes were also reported in this trial. These data suggest that novel approaches to targeting TGFb signaling may have broad applicability to the treatment of fibrotic disease.

We observed that our selective inhibitors of the activation of latent TGFb1 resulted in the inhibition of TGFb signaling, measured by the phosphorylation of SMAD2/3, a direct downstream target of TGFb1, in a

progressive, genetic mouse model of kidney fibrosis known as the Alport Syndrome model. In this model, we observed that our selective inhibitor of the activation of latent TGFb1 completely suppressed phosphorylation of SMAD 2/3, and was as effective as 1D11, an inhibitor of all three mature forms of TGFb. Based on these observations, we believe that TGFb1 is the primary driver of TGFb signaling in this disease model.

We have also observed in the unilateral ureteral obstruction model, a well-characterized model of renal fibrosis, that a number of our selective TGFb1 activation inhibitors resulted in robust suppression of TGFb1 target genes and downstream fibrotic markers. Furthermore, in the CCl4 model of liver fibrosis, we have observed that a number of our selective antibodies inhibited fibrotic progression in the liver, reducing collagen content both as assessed by a pathologist and, as shown in the figure below for one of our antibodies, SR-AB1, by quantitative histopathological staining.



SR-AB1, a selective inhibitor of TGFb1 activation, inhibited carbon tetrachloride (CCl4) induced liver fibrosis, as compared to a negative control antibody. Fibrosis was identified by increased collagen deposition, as assessed by quantitative histopathological staining.

TGFb1 in Cancer Immunotherapy

We believe that our preclinical and safety data suggest that specific inhibition of the activation of latent TGFb1 in combination with checkpoint inhibitors may have a significant impact in treating innate resistance to checkpoint immunotherapies. We are continuing to evaluate combinations of checkpoint inhibitors and our selective inhibitors of the activation of latent TGFb1 in preclinical models of cancer immunotherapy.

Immune checkpoints are cellular mechanisms that act as a brake on the immune system, and tumors express these proteins in the tumor microenvironment to create an immunosuppressive environment to evade being killed by the immune system. Immune checkpoint proteins, such as PD-1/PD-L1, have therefore become key therapeutic targets in the tumor microenvironment. By inhibiting these proteins, the brakes on the immune system are released, allowing the T cells to kill the cancer cells. There are currently multiple approved immunotherapies that target the PD-1/PD-L1 pathway, including pembrolizumab, marketed as Keytruda, nivolumab, marketed as Opdivo, and atezolizumab, marketed as Tecentriq. A significant proportion of patients fail to respond to checkpoint inhibition because they have an innate resistance to immunotherapy or initially respond but subsequently progress.

Multiple peer-reviewed studies have implicated TGFb signaling in innate resistance to checkpoint inhibition. Whole-exome sequencing of pre-treatment melanoma tumors identified multiple TGFb-related signaling signatures associated with innate resistance to anti-PD-1 therapy. It has also been reported that retrospective pathway analysis of the atezolizumab bladder cancer trial identified the TGFb pathway as a major determinant of resistance to atezolizumab. The combination of atezolizumab with an anti-TGFb antibody in a tumor model, known as the EMT6 syngeneic tumor model, increased the number of complete responses to 70%, from 10% and 0% with treatment by atezolizumab or the anti-TGFb antibody alone, respectively. Our analysis of publicly available human tumor data has identified TGFb1 as the predominant TGFb isoform in many human tumors, in particular for those cancers, such as bladder, lung and melanoma, where checkpoint therapies are already approved.

We have conducted both *in vitro* and *in vivo* mechanistic studies with our antibodies in order to evaluate whether our inhibitors of the activation of latent TGFb1 may be effective in cancer immunotherapy. *In vitro*, we have observed that, by inhibiting the activation of latent TGFb1, our antibodies suppressed the effect that human regulatory T cells have on the proliferation of human effector T cells. Moreover, in an *in vivo* model of colitis, we have observed that treatment with our antibodies increased immune system activity, a desired outcome in cancer immunotherapy.

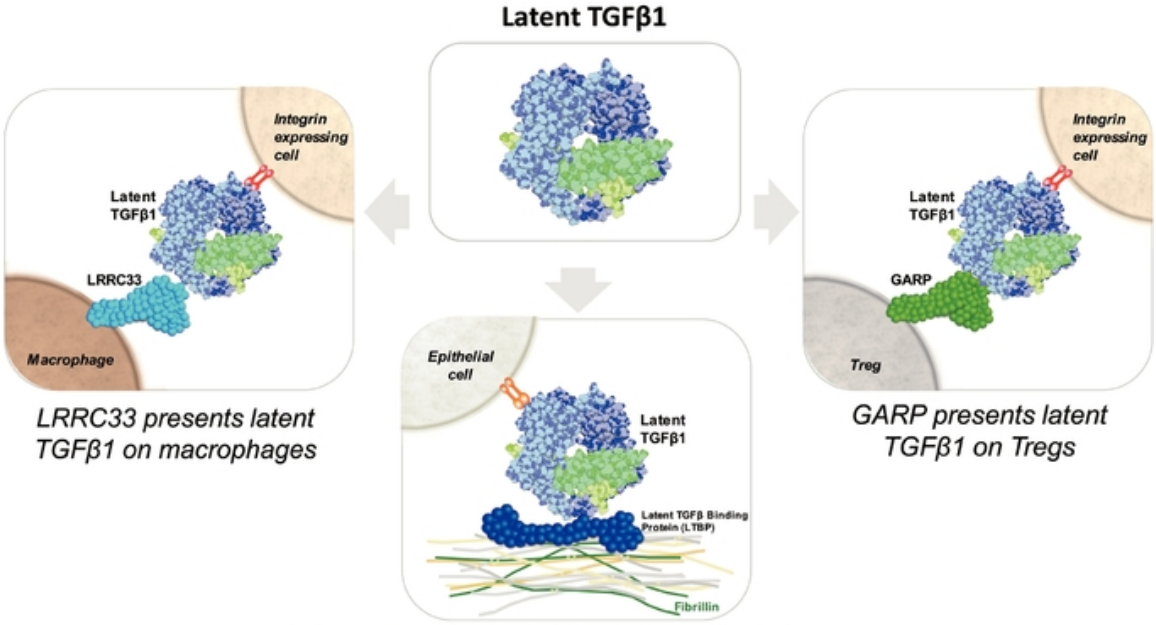
TGFb1 in Myelofibrosis

Multiple peer-reviewed studies implicate TGFb1 as a driver of fibrotic progression in myelofibrosis. We are currently evaluating our selective inhibitors of the activation of TGFb1 in models of myelofibrosis, a hematological disorder characterized by fibrosis of the bone marrow. Myelofibrosis affects between 17,000 and 18,000 patients in the United States with significant morbidity and mortality. The only currently approved treatment for myelofibrosis, a JAK2 inhibitor, provides symptomatic benefit, but only modest reductions in bone marrow fibrosis. Therefore, we believe that significant unmet need remains for new therapeutic options.

TGFb1 is produced by multiple cell types in the bone marrow microenvironment, including myofibroblasts, megakaryocytes and myeloid cells, and it has been shown to be upregulated in both human patient samples and preclinical mouse models of myelofibrosis. Inhibition of TGFb signaling with an ALK5 inhibitor reduced splenomegaly, collagen deposition and bone marrow fibrosis in a preclinical model of myelofibrosis. Furthermore, reconstitution of bone marrow with TGFb1 knockout bone marrow stem cells in a model of hematological disease protected animals from bone marrow fibrosis, suggesting that TGFb1 expression is necessary for disease pathogenesis.

Context-dependent inhibition of TGFb1

When latent TGFb1 is secreted from cells, it is further associated with a third protein, referred to as a presenting molecule. The presenting molecules are covalently bound to the prodomain, and serve to tether the latent TGFb1 complex in a particular microenvironment. Unlike TGFb1, a given presenting molecule's expression pattern is restricted to particular cellular and tissue environments. For example, the presenting molecule GARP is found primarily on regulatory T cells, or Tregs, the presenting molecules LTBP1 and LTBP3 are localized to the connective tissue in the extracellular matrix, and the presenting molecule LRRC33 is found primarily on certain myeloid lineage cells such as macrophages.



Latent TGFb1 is associated with distinct presenting molecules in particular cellular and tissue environments.

Using our proprietary platform, we are able to identify antibodies that selectively inhibit the activation of latent TGFb1 in the context of specific presenting molecules, which we refer to as context-dependent inhibition. For example, we have identified antibodies that specifically bind to and inhibit the activation of GARP-presented latent TGFb1 on regulatory T cells with no detectable binding to latent TGFb1 associated with other presenting molecules. These antibodies are the subject of our license agreement with Janssen.

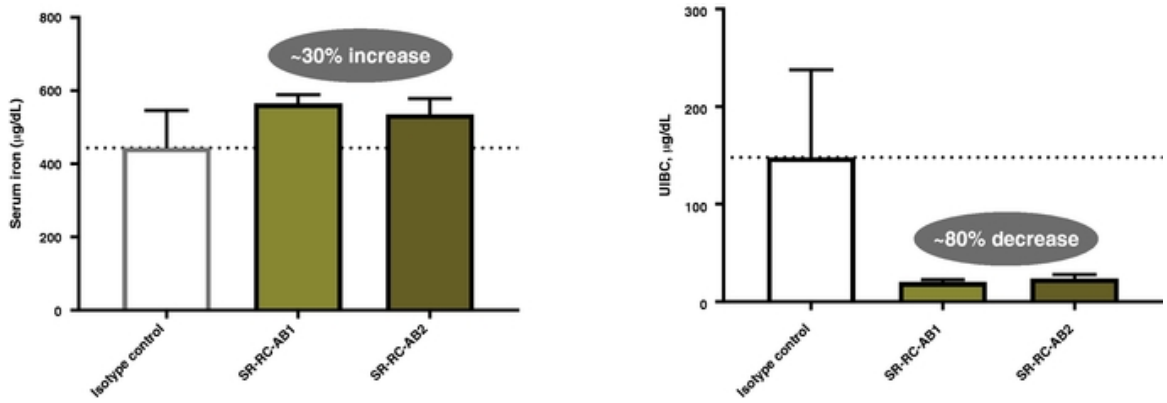
We have an active discovery program to identify antibodies that specifically bind to and inhibit the activation of LTBP1- and LTBP3-presented latent TGFb1 with no cross-reactivity to GARP- or LRRC33-presented latent TGFb1. We believe that such antibodies may have therapeutic potential for the treatment of organ fibrosis by inhibiting TGFb1 function in connective tissue while having no impact on the activation or signaling of TGFb1 in the immune system. We have identified antibodies with the desired binding specificity and *in vitro* inhibitory activity, and these are currently undergoing further optimization and characterization.

We also have an active discovery program to identify antibodies that specifically bind to and inhibit the activation of LRRC33-presented latent TGFb1 with no cross-reactivity to LTBP1-, LTBP3- or GARP- presented latent TGFb1. We believe that such antibodies may have therapeutic potential for specific oncology and cancer immunotherapy applications where selective modulation of myeloid lineage cells is desirable, for example inhibition of tumor-associated macrophages. We also have a related program to identify antibodies that specifically inhibit the activation of both LRRC33- and GARP-presented latent TGFb1, with no cross-reactivity to LTBP1- or LTBP3-presented latent TGFb1. We believe such antibodies could have broad inhibitory activity against TGFb1 in the immune system for cancer immunotherapy, while avoiding inhibition of TGFb1 in other tissues. We have identified antibodies that potentially meet the desired binding specificities, and these are currently undergoing characterization and further optimization.

BMP6 Signaling Program

We believe that liver-selective inhibition of BMP6 signaling could provide a way to target a variety of iron-restricted anemias, including anemia of chronic kidney disease, anemia of cancer and anemia of chronic inflammation. We are currently evaluating a limited number of our liver-selective inhibitors of BMP6 signaling in preclinical disease models of iron-restricted anemia.

BMPs are a broad subfamily of growth factors in the TGFb superfamily originally discovered by their ability to induce the formation of bone and cartilage. Beyond their association with bone, like many other growth factors, the BMPs are involved in a diverse set of biological processes. For example, while BMP6 plays roles in many different biologies, including fat metabolism and ovarian physiology, in the liver it functions as a critical control point in iron modulation in humans via regulation of hepcidin, a central regulator of iron homeostasis. Traditional approaches to inhibiting the signaling of BMP6 systemically would likely perturb the numerous different physiological processes in which BMP6 is involved. While the details of BMP6 activation are different from myostatin and TGFb1, activation of BMP6 is a localized phenomenon, driven by a co-receptor molecule, RGMc, also known as hemojuvelin, which is required for BMP6 signaling upon binding to its receptor. RGMc is a member of a small family of proteins that include RGMa and RGMb. While each of these family members shares significant structural homology, particularly across their BMP binding domains, their physiological roles are quite different. RGMa and RGMb are reported to have roles in nervous system biology, immunity, inflammation, angiogenesis, and growth. Unlike RGMa and RGMb, RGMc's known function is localized to hepatocytes. As such identification of RGMc selective-antibodies that do not bind to RGMa or RGMb could provide the potential for liver-specific modulation of BMP6 biology. Utilizing our structural biology insights into BMP6 and its co-receptors, we have identified highly specific inhibitors of RGMc's interaction with BMP6 and, as shown in the figure below, in a preclinical study in rats we have shown proof-of-principle that our antibodies can modulate iron levels *in vivo*.



SR-RC-AB1 and SR-RC-AB2, two of our selective BMP6 signaling inhibitors, increased serum iron in rats as compared to control (left side) and reduced unsaturated iron binding capacity, or UIBC, in healthy rats (right side).

License Agreements

License Agreement with Janssen

On December 17, 2013 we entered into an option and license agreement with Janssen, or the Janssen Agreement. Pursuant to the Janssen Agreement, Janssen funded our drug discovery research to identify molecules with either one or two pharmacological profiles, over a two-year period beginning on December 17, 2013, or the collaboration period. During the collaboration period, we granted Janssen a non-exclusive license to research, develop, and use the collaboration molecule(s) and/or lead molecule(s). Janssen was not granted a license to commercialize any collaboration molecule, lead molecule or a product

that is derived from an optioned molecule, or a licensed product, unless and until Janssen exercised its license option in accordance with the Janssen Agreement. We received funding from Janssen based on a set rate per annual full-time equivalent personnel working on the research plus actual external costs incurred by us up to a maximum dollar amount as specified in the Janssen Agreement, with costs approximating the funding provided. During the collaboration period, we billed Janssen quarterly, in arrears, based on time and actual costs incurred, and Janssen was not entitled to any refunds.

The activities under the Janssen Agreement were governed by a program committee, consisting of three members from each of our company and Janssen, with all decisions being by unanimous vote or written consent, subject to an escalating dispute resolution procedure in the event any disputes could be not resolved by the program committee.

We also granted Janssen an option to exclusively license molecules identified during the collaboration period that meet either one or both pharmacological profiles by providing us with written notice and paying an option exercise fee of $1.0 million per option exercised (up to two). If Janssen failed to exercise its license option by the end of the collaboration period, the term could be extended for up to one additional year by mutual written agreement of the parties. Once Janssen exercised its option, our obligations under the program plan for the molecule and related pharmacological profile ceased and Janssen assumed full responsibility for further development of the molecules at its sole cost, and we were obligated to transfer any and all manufacturing related activities for such molecule to Janssen at Janssen's sole cost. In December 2015, Janssen exercised its option for collaboration molecules for one pharmacological profile, the selective inhibition of TGFb1 in the context of regulatory T cells. In addition, the parties agreed to extend the collaboration period for the second pharmacological profile through March 31, 2016. The option exercise period for this profile expired unexercised on March 31, 2016, and all rights with respect to molecules generated during the collaboration period with respect to this second pharmacological profile were retained by us.

After Janssen exercised its option, it became obligated to pay us up to $25 million upon the achievement of specified development milestones and up to $97 million upon the achievement of specified regulatory milestones. In addition for any licensed product, Janssen is required to pay to us up to $130 million upon the achievement of specified annual net sales thresholds. For a period commencing on the first commercial sale of a product, on a product-by-product and country-by-country basis, until the latest to occur of (i) the expiration date of the last valid claim within the licensed patent rights covering the licensed product, (ii) the tenth anniversary date of the first commercial sale of a licensed product, or (iii) the termination or expiration of regulatory exclusivity for a licensed product, such period the royalty period, Janssen is required to pay to us, single digit percentage tiered royalties based on annual net sales thresholds.

The Janssen Agreement will expire on a country-by-country basis on the expiration of the last royalty period for a licensed product within such country. Janssen has the right to terminate the Janssen Agreement, in whole or in part, without cause upon 90 days written notice to us. In addition, either we, or Janssen may terminate the Janssen Agreement if the other party commits a material breach of the agreement and fails to cure such breach within 60 days (or 30 days in the case of a failure to make any payment) after written notice is provided, or, upon the other party's bankruptcy, insolvency, dissolution or winding up. Upon termination, any licensed product reverts to us and if Janssen has commenced clinical trials for such licensed product, upon commercialization of such licensed product, we will be required to pay Janssen single digit percentage tiered royalties on such licensed product based on annual net sales thresholds.

License Agreement with Children's Medical Corporation

On December 17, 2013, we entered into an exclusive license agreement with Children's Medical Center Corporation, or CMCC, or the CMCC Agreement, to gain exclusive control over co-owned patent rights related to our platform technology. Under the CMCC Agreement, we received an exclusive worldwide license to CMCC's rights in certain patent rights jointly owned by us and CMCC, to develop and commercialize any product or process that but for the licenses granted to us under the CMCC Agreement would infringe such

patent rights, a licensed product and licensed process, respectively, for any use. We are entitled to sublicense the rights granted to us under the CMCC Agreement. These licenses and rights are subject to certain limitations and retained rights, including retained rights to practice and use the patent rights for research, educational, clinical and charitable purposes. In addition, the CMCC Agreement obligates us to meeting certain diligence milestones, including obligations to raise funds, seek collaborations and initiate discovery efforts.

As consideration for the license, we paid CMCC a non-refundable license fee of $5,000 and issued to CMCC 76,500 common units, which were exchanged for 76,500 shares of common stock in connection with the Reorganization. We must pay CMCC annual license maintenance fees, which were $5,000 through 2016 and increased to $10,000 for 2017 and each year thereafter. We will also be responsible for up to $1.3 million of development and regulatory milestone payments through the first regulatory approval of a licensed product, tiered royalty payments of low single-digit percentages on net sales of licensed products in the event that we realize sales from products covered by the license agreement, and between 10% and 20% of non-royalty income attributable to a sublicense of the CMCC rights. Such products include products developed using our proprietary platform that are covered by a valid claim contained in any patent under the license agreement. Amounts paid to CMCC are recorded as research and development expense in the statement of operations. The royalty term will terminate on the expiration date of the last valid claim within the licensed patent rights.

CMCC may terminate the CMCC Agreement if we commit a breach of the agreement, and fail to cure such breach within 60 days (or 30 days in the case of our failure to make any payment) after written notice is provided, or immediately upon our bankruptcy, insolvency, dissolution or winding up, or upon 30 days' notice if we bring patent challenges relating to any patent families licensed by us under the CMCC Agreement. In addition, we may terminate the CMCC Agreement for convenience upon three months prior written notice to CMCC. Upon expiration of the CMCC Agreement, we will have a worldwide, perpetual, irrevocable, sublicensable license to the intellectual property previously covered by the CMCC Agreement.

Intellectual Property

Our commercial success depends in part on our ability to protect intellectual property for our product candidates, including our lead product candidate SRK-015, and related methods, as well as our novel approach and proprietary platform for generating monoclonal antibodies; to secure freedom to operate to enable commercialization of our product candidates, if approved; and to prevent others from infringing upon our patent rights. Our policy is to seek to protect our intellectual property position by filing patent applications in key jurisdictions, including the United States, Europe, Canada, Japan and Australia, covering our proprietary technology, inventions and improvements that are important to innovate, develop, sustain and implement our business.

We file patent applications directed to compositions comprising our antibodies, classes of antibodies covering our product candidates, use of such antibodies for treating diseases, as well as related manufacturing methods. We have no issued patents or pending applications directed to our BMP6 program at this time. As of April 25, 2018, we have 13 international patent families (PCT filings) pending. Among the pending families, six have been nationalized, in which five applications have matured into U.S. issued patents, two granted in Australia and one in South Africa. Collectively, there are 37 national utility applications pending. In addition, there are two patent filings which are in the priority year. We continue to review new inventions for new patent filings.

We have no contested proceedings or third-party claims relating to any patents at this time, but we can not provide any assurances that we will not have such proceedings or third-party claims at a later date.

Ownership and IP Rights

Our earliest patent family, PCT/US2013/068613 (published as WO 2014/074532), is jointly owned by us and CMCC. CMCC is the assignee of the intellectual property rights transferred from two of our co-founders,

Drs. Timothy A. Springer and Leonard I. Zon. The portion of rights owned by CMCC is exclusively licensed to us. We are the sole legal owner of all subsequent patent families we have to date.

As described, a portion of our TGFb technology is out-licensed to Janssen. This is carved out as PCT/US2017/042162 (published as WO 2018/013939). The licensee takes lead in the prosecution of this patent family. The licensee also has a non-exclusive license to our platform technology to enable their development in the licensed field.

Brief descriptions of our patent families are provided below, with projected patent terms excluding any possible patent term adjustments or extensions.

Platform

Our novel approach to generating selective modulators of supracellular activation of growth factors is broadly embodied in our two earliest patent families, PCT/US2013/068613 (published as WO 2014/074532) and PCT/US2014/036933 (published as WO 2014/182676). These patent families are directed to methods for modulating the activation of the TGFb superfamily of growth factors by using a monoclonal antibody that specifically targets an inactive form of the growth factor, thereby preventing release of mature growth factor from its latent complex. The TGFb superfamily is a group of more than 30 related growth factors that mediate diverse biological processes and includes TGFb1 and myostatin (also known as GDF-8). Issued U.S. patents include: U.S. Patents Nos. 9,573,995 (issued 02/21/2017); 9,758,576 (issued 09/12/2017); 9,580,500 (issued 02/28/2017); 9,399,676 (issued 07/26/2016) and 9,758,577 (issued 09/12/2017). These patents are projected to expire in 2034.

Specifically, U.S. Patent No. 9,573,995 has issued composition of matter claims directed to an antibody that specifically binds to GARP associated with a human TGFb1 LAP complex.

U.S. Patent No. 9,758,576 has issued composition of matter claims directed to an isolated monoclonal antibody, or a fragment thereof, that specifically binds the prodomain of a pro/latent GDF-8 complex, thereby preventing proteolytic cleavage between residues Arg 75 and Asp 76 of GDF-8 prodomain, so as to inhibit the release of mature GDF-8 growth factor from the complex.

U.S. Patent No. 9,580,500 has issued claims directed to phage display library-based antibody production methods for identifying an antibody that binds a GARP/proTGFb1 complex.

U.S. Patent No. 9,399,676 has issued claims directed to phage display library-based antibody production methods for identifying an antibody that binds a pro/latent GDF-8 complex that has been subjected to enzymatic cleavage. Related product-by-process claims are included in issued U.S. Patent No. 9,758,577.

Myostatin Activation Inhibitors

Five patent families have been filed to date to cover proprietary myostatin inhibitors and their use in the treatment of various muscle diseases. Patent prosecution of these five pending patent families is in the early stages, and no patents have issued to date.

Two families are directed to composition of matter claims that cover our proprietary antibodies. PCT/US2015/059468 filed November 6, 2015, broadly covers a class of monoclonal antibodies that specifically bind inactive precursors thereby preventing activation of myostatin. This patent family is projected to expire in November 2035. A second family, PCT/US2016/052014 filed September 15, 2016, discloses the specific amino acid sequence of SRK-015 and is projected to expire in September 2036.

In addition, the following three patent families are directed to therapeutic use/methods. PCT/US2017/012606 (published as WO 2017/120523) broadly covers treatment methods for a number of muscle and neuromuscular disease and disorders with the use of an antibody that specifically blocks the activation step of myostatin. This patent family is projected to expire in January 2037. PCT/US2017/037332 (published as WO 2017/218592) is directed to methods for treating neuromuscular diseases and selecting patient populations that are likely to respond to myostatin inhibition. This filing

includes the treatment of SMA in patients who are on an SMN upregulator therapy. This patent family is projected to expire in June 2037. Finally, PCT/US2018/012686 (expected to publish in July 2018) relates to the treatment of metabolic diseases with the use of a myostatin activation inhibitor and is projected to expire in January 2038.

In addition to the five pending patent families listed above, the issued claims of U.S. Patent 9,758,576 from the platform patents discussed in detail above cover monoclonal antibodies that selectively inhibit myostatin signaling by blocking the proteolytic activation of latent myostatin. These issued composition of matter claims provide protection for our lead antibody SRK-015, as well as any other monoclonal antibodies that work by this unique mechanism of action. This patent expires in May 2034, not including any potential patent term extension.

TGFb1 Activation Inhibitors

Five patent families have been filed to date, covering various aspects of our TGFb program. Patent prosecution of these five pending patent families is in the early stages, and no patents have issued to date. Isoform-specific inhibitors of TGFb1 and related methods are described in PCT/US2017/021972 (published as WO 2017/156500). This family is projected to expire in March 2037. Among TGFb1 inhibitors, one of our leading context-independent antibodies is separately claimed and related preclinical data are described in PCT/US2018/012601. This patent application is expected to publish in July 2018 and is projected to expire in January 2038.

PCT/US2017/042162 (published as WO 2018/013939) is a collaboration patent family exclusively licensed to Janssen. This patent family covers antibodies that specifically inhibit GARP-associated TGFb1, and is projected to expire in July 2037. Janssen takes prosecution lead in this case.

Two additional patent families related to the TGFb program have been filed and are still in the priority year. These provisional applications will be converted to international patent applications (PCT) in May and July 2018, respectively.

Intellectual Property Protection

We cannot predict whether the patent applications we pursue will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide any proprietary protection from competitors. Even if our pending patent applications are granted as issued patents, those patents, as well as any patents we license from third parties, may be challenged, circumvented or invalidated by third parties. While there are currently no contested proceedings or third-party claims relating to any of the patents described above, we cannot provide any assurances that we will not have such proceedings or third-party claims at a later date.

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the earliest date of filing a non-provisional patent application. In the United States, the patent term of a patent that covers an FDA-approved drug or biologic may also be eligible for patent term extension, which permits patent term restoration as compensation for the patent term lost during FDA regulatory review process. The Hatch-Waxman Amendments permit a patent term extension of up to five years beyond the expiration of the patent. The length of the patent term extension is related to the length of time the drug or biologic is under regulatory review. Patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug or biologic may be extended. Similar provisions are available in Europe and other foreign jurisdictions to extend the term of a patent that covers an approved drug or biologic or provide an additional period of protection for the approved pharmaceutical product following expiry of the patent. In the future, if our products receive FDA approval, we expect to apply for patent term extensions on patents covering those products. We plan to seek patent term extensions to any of our issued patents in any jurisdiction where these are available, however there is no guarantee that the applicable authorities, including the U.S. Patent and Trademark

Office in the United States and the national patent offices in Europe, will agree with our assessment of whether such extensions should be granted, and if granted, the length of such extensions.

In addition to our reliance on patent protection for our inventions, product candidates and research programs, we also rely on trade secret protection for our confidential and proprietary information. For example, certain elements of our proprietary platform may be based on unpatented trade secrets that are not publicly disclosed. Although we take steps to protect our proprietary information and trade secrets, including through contractual means with our employees and consultants, third parties may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or disclose our technology. Thus, we may not be able to meaningfully protect our trade secrets. It is our policy to require our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to execute confidentiality agreements upon the commencement of employment or consulting relationships with us. These agreements provide that all confidential information concerning our business or financial affairs developed or made known to the individual or entity during the course of the party's relationship with us is to be kept confidential and not disclosed to third parties except in specific circumstances. In the case of employees, the agreements provide that all inventions conceived by the individual, and which are related to our current or planned business or research and development or made during normal working hours, on our premises or using our equipment or proprietary information, are our exclusive property. In addition, we take other appropriate precautions, such as physical and technological security measures, to guard against misappropriation of our proprietary technology by third parties. We have also adopted policies and conduct training that provides guidance on our expectations, and our advice for best practices, in protecting our trade secrets.

Manufacturing

We do not own or operate facilities for clinical drug manufacturing, storage, distribution or quality testing. Currently, all of our clinical manufacturing is outsourced to third-party manufacturers. As our development programs expand and we build new process efficiencies, we expect to continually evaluate this strategy with the objective of satisfying demand for registration trials and, if approved, the manufacture, sale and distribution of commercial products.

Antibody Discovery

We rely on third party entities to conduct antibody discovery based on criteria and specifications provided by us. Certain antibody discovery vendors require us to enter into a license agreement with them for the right to use antibodies discovered by them in humans or for commercial purposes. Such license agreement could include substantial milestone payments and royalties to the extent we choose to use an antibody discovered by such vendor. While we have not executed such an agreement to date, there can be no assurance that we will not be required to execute such an agreement at a later date if we select a clinical candidate that includes such an antibody and advance that clinical candidate into clinical trials.

Competition

The biotechnology and pharmaceutical industries are characterized by rapid evolution of technologies, fierce competition and strong defense of intellectual property. While we believe that our product candidates, discovery programs, technology, knowledge, experience and scientific resources provide us with competitive advantages, we face competition from major pharmaceutical and biotechnology companies, academic institutions, governmental agencies and public and private research institutions, among others.

Any product candidates that we successfully develop and commercialize will compete with currently approved therapies and new therapies that may become available in the future. Key product features that would affect our ability to effectively compete with other therapeutics include the efficacy, safety and convenience of our products.

At this time, there are no FDA- or EMA-approved muscle-directed treatments for SMA. We believe SRK-015 may be used in conjunction with SMN upregulators. Biogen markets nusinersen, the only currently marketed SMN upregulator. AveXis, Inc., Généthon, Novartis and Roche have SMN upregulators in various stages of preclinical or clinical development. In addition, Catalyst Pharmaceuticals, Inc., Cytokinetics Incorporated and Roche are developing investigational agents with other mechanisms of action for the treatment of SMA.

Acceleron Pharma, Inc., Novartis, Pfizer, Regeneron Pharmaceuticals, Inc. and Roche are developing therapies for muscle-wasting diseases, other than SMA, that are intended to work, at least in part, through inhibition of the myostatin signaling pathway.

Our competitors may also include companies that are or will be developing therapies for the same therapeutic areas that we are targeting within our early pipeline, including other neuromuscular disorders, cancer, fibrosis and anemia.

Many of the companies against which we may compete have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

The availability of reimbursement from government and other third-party payors will also significantly affect the pricing and competitiveness of our products. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market.

Government Regulation

Government authorities in the United States at the federal, state and local level and in other countries regulate, among other things, the research, development, testing, manufacture, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of drug and biological products, such as SRK-015 and any future product candidates. Generally, before a new drug or biologic can be marketed, considerable data demonstrating its quality, safety and efficacy must be obtained, organized into a format specific for each regulatory authority, submitted for review and approved by the regulatory authority.

U.S. Biological Product Development

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and its implementing regulations and biologics under the FDCA, the Public Health Service Act, or PHSA, and their implementing regulations. Both drugs and biologics also are subject to other federal, state and local statutes and regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state and local statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or post-market may subject an applicant to administrative or judicial sanctions. These sanctions could include, among other actions, the FDA's refusal to approve pending applications, withdrawal of an approval, a clinical hold, untitled or warning letters, product recalls or market withdrawals, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement and civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

SRK-015 and any future product candidates must be approved by the FDA through a Biologics License Application, or BLA, process before they may be legally marketed in the United States. The process generally involves the following:

- Completion of extensive preclinical studies in accordance with applicable regulations, including studies conducted in accordance with good laboratory practice, or GLP, requirements;
- Submission to the FDA of an IND application, which must become effective before human clinical trials may begin;
- Approval by an institutional review board, or IRB, or independent ethics committee at each clinical trial site before each trial may be initiated;
- Performance of adequate and well-controlled human clinical trials in accordance with applicable IND regulations, good clinical practice, or GCP, requirements and other clinical trial-related regulations to establish the safety and efficacy of the investigational product for each proposed indication;
- Submission to the FDA of a BLA;
- A determination by the FDA within 60 days of its receipt of a BLA to accept the filing for review;
- Satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities where the biologic will be produced to assess compliance with cGMP requirements to assure that the facilities, methods and controls are adequate to preserve the biologic's identity, strength, quality and purity;
- Potential FDA audit of the clinical trial sites that generated the data in support of the BLA; and
- FDA review and approval of the BLA, including consideration of the views of any FDA advisory committee, prior to any commercial marketing or sale of the biologic in the United States.

The preclinical and clinical testing and approval process requires substantial time, effort and financial resources, and we cannot be certain that any approvals for SRK-015 and any future product candidates will be granted on a timely basis, or at all.

Preclinical Studies and IND

Preclinical studies include laboratory evaluation of product chemistry and formulation, as well as *in vitro* and animal studies to assess the potential for adverse events and in some cases to establish a rationale for therapeutic use. The conduct of preclinical studies is subject to federal regulations and requirements, including GLP regulations for safety/toxicology studies.

An IND sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical studies, among other things, to the FDA as part of an IND. An IND is a request for authorization from the FDA to administer an investigational product to humans, and must become effective before human clinical trials may begin. Some long-term preclinical testing may continue after the IND is submitted. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time, the FDA raises concerns or questions related to one or more proposed clinical trials and places the trial on clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. As a result, submission of an IND may not result in the FDA allowing clinical trials to commence.

Clinical Trials

The clinical stage of development involves the administration of the investigational product to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the trial sponsor's control, in accordance with GCP requirements, which include the requirement that all patients provide their informed consent for their participation in any clinical trial. Clinical trials are conducted under 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 and assess efficacy. Each protocol, and any subsequent amendments to the protocol, must be submitted to

the FDA as part of the IND. Furthermore, each clinical trial must be reviewed and approved by an IRB for each institution at which the clinical trial will be conducted to ensure that the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the informed consent form that must be provided to each clinical trial subject or his or her legal representative, and must monitor the clinical trial until completed. There also are requirements governing the reporting of ongoing clinical trials and completed clinical trial results to public registries.

A sponsor who wishes to conduct a clinical trial outside of the United States may, but need not, obtain FDA authorization to conduct the clinical trial under an IND. If a foreign clinical trial is not conducted under an IND, the sponsor may submit data from the clinical trial to the FDA in support of a BLA. The FDA will accept a well-designed and well-conducted foreign clinical study not conducted under an IND if the study was conducted in accordance with GCP requirements, and the FDA is able to validate the data through an onsite inspection if deemed necessary.

Clinical trials generally are conducted in three sequential phases, known as Phase 1, Phase 2 and Phase 3, and may overlap.

- Phase 1 clinical trials generally involve a small number of healthy volunteers or disease-affected patients who are initially exposed to a single dose and then multiple doses of the product candidate. The primary purpose of these clinical trials is to assess the metabolism, pharmacologic action, side effect tolerability and safety of the product candidate.

- Phase 2 clinical trials generally involve studies in disease-affected patients to evaluate proof of concept and/or determine the dosing regimen(s) for subsequent investigations. At the same time, safety and further pharmacokinetic and pharmacodynamic information is collected, possible adverse effects and safety risks are identified and a preliminary evaluation of efficacy is conducted.

- Phase 3 clinical trials generally involve a large number of patients at multiple sites and are designed to provide the data necessary to demonstrate the effectiveness of the product for its intended use, its safety in use and to establish the overall benefit/risk relationship of the product and provide an adequate basis for product labeling.

Post-approval trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval of a BLA.

Progress reports detailing the results of the clinical trials, among other information, must be submitted at least annually to the FDA and written IND safety reports must be submitted to the FDA and the investigators for serious and unexpected suspected adverse events, findings from other studies or animal or *in vitro* testing that suggest a significant risk for human subjects and any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure.

Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. The FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug or biologic has been associated with unexpected serious harm to patients. Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether a trial may move forward at designated check points based on access to certain data from the trial. Concurrent with clinical trials, companies usually complete additional animal studies and also must develop additional information about the chemistry and physical characteristics of the drug or biologic as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable

of consistently producing quality batches of the product and, among other things, companies must develop methods for testing the identity, strength, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidates do not undergo unacceptable deterioration over their shelf life.

FDA Review Process

Following completion of the clinical trials, data are analyzed to assess whether the investigational product is safe and effective for the proposed indicated use or uses. The results of preclinical studies and clinical trials are then submitted to the FDA as part of a BLA, along with proposed labeling, chemistry and manufacturing information to ensure product quality and other relevant data. The BLA is a request for approval to market the biologic for one or more specified indications and must contain proof of safety and efficacy for a drug or safety, purity and potency for a biologic. The application may include both negative and ambiguous results of preclinical studies and clinical trials, as well as positive findings. Data may come from company-sponsored clinical trials intended to test the safety and efficacy of a product's use or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and efficacy of the investigational product to the satisfaction of the FDA. FDA approval of a BLA must be obtained before a biologic may be marketed in the United States.

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each BLA must be accompanied by a user fee. The FDA adjusts the PDUFA user fees on an annual basis. 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 products designated as orphan drugs, unless the product also includes a non-orphan indication.

The FDA reviews all submitted BLAs before it accepts them for filing, and may request additional information rather than accepting the BLA for filing. The FDA must make a decision on accepting a BLA for filing within 60 days of receipt, and such decision could include a refusal to file (RTF) by the FDA. Once the submission is accepted for filing, the FDA begins an in-depth review of the BLA. Under the goals and policies agreed to by the FDA under PDUFA, the FDA has 10 months, from the filing date, in which to complete its initial review of an original BLA and respond to the applicant, and six months from the filing date of an original BLA designated for priority review. The FDA does not always meet its PDUFA goal dates for standard and priority BLAs, and the review process is often extended by FDA requests for additional information or clarification.

Before approving a BLA, the FDA will conduct a pre-approval inspection of the manufacturing facilities for the new product to determine whether they comply with cGMP requirements. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. The FDA also may audit data from clinical trials to ensure compliance with GCP requirements. Additionally, the FDA may refer applications for novel products or products which 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, if any. The FDA is not bound by recommendations of an advisory committee, but it considers such recommendations when making decisions on approval. The FDA likely will reanalyze the clinical trial data, which could result in extensive discussions between the FDA and the applicant during the review process. After the FDA evaluates a BLA, it will issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the biologic with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete and the application will not be approved in its present form. A Complete Response Letter usually describes all of the specific deficiencies in the BLA identified by the FDA. The Complete Response Letter may require additional clinical data, additional pivotal Phase 3 clinical trial(s) and/or other significant and time-consuming requirements related to clinical trials, preclinical studies or manufacturing. If a Complete

Response Letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application. Even if such data and information are submitted, the FDA may decide that the BLA does not satisfy the criteria for approval. Data obtained from clinical trials are not always conclusive and the FDA may interpret data differently than we interpret the same data.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biological product intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making the product available in the United States for this type of disease or condition will be recovered from sales of the product. On March 22, 2018, the FDA granted orphan drug designation for SRK-015 for the treatment of SMA.

Orphan drug designation must be requested before submitting a BLA. After the FDA grants orphan drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications to market the same drug for the same indication for seven years from the date of such approval, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity by means of greater effectiveness, greater safety or providing a major contribution to patient care or in instances of drug supply issues. Competitors, however, may receive approval of either a different product for the same indication or the same product for a different indication but that could be used off-label in the orphan indication. Orphan drug exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval before we do for the same product, as defined by the FDA, for the same indication we are seeking approval, or if our product is determined to be contained within the scope of the competitor's product for the same indication or disease. If one of our products designated as an orphan drug receives marketing approval for an indication broader than that which is designated, it may not be entitled to orphan drug exclusivity. Orphan drug status in the European Union has similar, but not identical, requirements and benefits.

Expedited Development and Review Programs

The FDA has a fast track program that is intended to expedite or facilitate the process for reviewing new drugs and biologics that meet certain criteria. Specifically, new drugs and biologics are eligible for fast track designation if they are intended to treat a serious or life threatening condition and preclinical or clinical data demonstrate the potential to address unmet medical needs for the condition. Fast track designation applies to both the product and the specific indication for which it is being studied. The sponsor can request the FDA to designate the product for fast track status any time before receiving BLA approval, but ideally no later than the pre-BLA meeting. Any product submitted to the 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 priority review and accelerated approval. Any product is eligible for priority review if it treats a serious or life-threatening condition and, if approved, would provide a significant improvement in safety and effectiveness compared to available therapies. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug or biologic designated for priority review in an effort to facilitate the review.

A product may also be eligible for accelerated approval, if it treats a serious or life-threatening condition and generally provides a meaningful advantage over available therapies. In addition, it must demonstrate an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit. As a condition of approval, the FDA may require that a

sponsor of a drug or biologic receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. If the FDA concludes that a drug or biologic shown to be effective can be safely used only if distribution or use is restricted, it will require such post-marketing restrictions, as it deems necessary to assure safe use of the product. If the FDA determines that the conditions of approval are not being met, the FDA can withdraw its accelerated approval for such drug or biologic.

Additionally, a drug or biologic may be eligible for designation as a breakthrough therapy if the product is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over currently approved therapies on one or more clinically significant endpoints. The benefits of breakthrough therapy designation include the same benefits as fast track designation, plus intensive guidance from the FDA to ensure an efficient drug development program.

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

Pediatric Information

Under the Pediatric Research Equity Act, or PREA, a BLA or supplement to a BLA must contain data to assess the safety and efficacy of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of pediatric data or full or partial waivers. A sponsor who is planning to submit a marketing application for a drug that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration submit an initial Pediatric Study Plan, or PSP, within 60 days of an end-of-Phase 2 meeting or, if there is no such meeting, as early as practicable before the initiation of the Phase 3 or Phase 2/3 study. The initial PSP must include an outline of the pediatric study or studies that the sponsor plans to conduct, including study objectives and design, age groups, relevant endpoints and statistical approach, or a justification for not including such detailed information, and any request for a deferral of pediatric assessments or a full or partial waiver of the requirement to provide data from pediatric studies along with supporting information. The FDA and the sponsor must reach an agreement on the PSP. A sponsor can submit amendments to an agreed-upon initial PSP at any time if changes to the pediatric plan need to be considered based on data collected from preclinical studies, early phase clinical trials and/or other clinical development programs.

Post-marketing Requirements

Following approval of a new product, the manufacturer and the approved product are subject to continuing regulation by the FDA, including, among other things, monitoring and record-keeping activities, reporting of adverse experiences, complying with promotion and advertising requirements, which include restrictions on promoting products for unapproved uses or patient populations (known as "off-label use") and limitations on industry-sponsored scientific and educational activities. Although physicians may prescribe legally available products for off-label uses, manufacturers may not market or promote such uses. Prescription drug and biologic promotional materials must be submitted to the FDA in conjunction with their first use. Further, if there are any modifications to the drug or biologic, including changes in indications, labeling or manufacturing processes or facilities, the applicant may be required to submit and obtain FDA approval of a new BLA or BLA supplement, which may require the development of additional data or preclinical studies and clinical trials.

The FDA may also place other conditions on approvals including the requirement for a Risk Evaluation and Mitigation Strategy, or REMS, to assure the safe use of the product. 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 an approved REMS, if required. A REMS could include medication guides, physician communication plans or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. Any of these limitations on approval or marketing could restrict the commercial

promotion, distribution, prescription or dispensing of products. Product approvals may be withdrawn for non-compliance with regulatory standards or if problems occur following initial marketing.

FDA regulations require that products be manufactured in specific approved facilities and in accordance with cGMP regulations. We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our products in accordance with cGMP regulations. These manufacturers must comply with cGMP regulations that require, among other things, quality control and quality assurance, the maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. Manufacturers and other entities involved in the manufacture and distribution of approved drugs or biologics are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP requirements and other laws. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance. The discovery of violative conditions, including failure to conform to cGMP regulations, could result in enforcement actions, and the discovery of problems with a product after approval may result in restrictions on a product, manufacturer or holder of an approved BLA, including recall.

Other Regulatory Matters

Manufacturing, sales, promotion and other activities following product approval are also subject to regulation by numerous regulatory authorities in the United States in addition to the FDA, including the Centers for Medicare & Medicaid Services, or CMS, other divisions of the Department of Health and Human Services, or DHHS, the Department of Justice, the Drug Enforcement Administration, the Consumer Product Safety Commission, the Federal Trade Commission, the Occupational Safety & Health Administration, the Environmental Protection Agency and state and local governments.

Other Healthcare Laws

Healthcare providers, physicians, and third-party payors will play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. Our future arrangements with third-party payors, healthcare providers and physicians may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any drugs for which we obtain marketing approval. In particular, the research of our product candidates, as well as the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials. In the United States, these laws include, without limitation, state and federal anti-kickback, false claims, physician transparency, and patient data privacy and security laws and regulations, including but not limited to those described below.

- The Anti Kickback Statute, or AKS, which makes it illegal for among other things, any person or entity, including a prescription drug manufacturer (or a party acting on its behalf), to knowingly and willfully solicit, receive, offer or pay any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, that is intended to induce or reward referrals, including the purchase, recommendation, order or prescription of a particular drug, for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. Violations of this law are punishable by up to five years in prison, criminal fines, administrative civil money penalties and exclusion from participation in federal healthcare programs. In addition, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it to have committed a violation.

- The federal civil and criminal false claims laws, including the False Claims Act, which prohibits individuals or entities (including prescription drug manufacturers) from knowingly presenting, or causing to be presented false or fraudulent claims for payment by a federal healthcare program or making a false statement or record material to payment of a false claim or avoiding, decreasing or concealing an obligation to pay money to the federal government. The government may deem manufacturers to have "caused" the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers or promoting a product off label. Claims which include items or services resulting from a violation of the federal Anti Kickback Statute are false or fraudulent claims for purposes of the False Claims Act. Our future marketing and activities relating to the reporting of wholesaler or estimated retail prices for our products, the reporting of prices used to calculate Medicaid rebate information and other information affecting federal, state and third-party reimbursement for our products, and the sale and marketing of our product and any future product candidates, are subject to scrutiny under this these laws.

- The Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes that prohibit among other things, knowingly and willfully executing a scheme, or attempting to execute a scheme, to defraud any healthcare benefit program, including private payors, or falsifying, concealing or covering up a material fact or making any materially false statements in connection with the delivery of or payment for healthcare benefits, items or services.

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose, among other things, specified requirements on covered entities, which include certain healthcare providers, health plans and healthcare clearinghouses, and their business associates, which include individuals or entities that perform services for covered entities involving the creation, use, maintenance or disclosure of, individually identifiable health information, relating to the privacy and security of individually identifiable health information including mandatory contractual terms and required implementation of technical safeguards of such information. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions.

- The Physician Payments Sunshine Act, enacted as part of the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, the ACA, which impose new annual reporting requirements for certain manufacturers of drugs, devices, biologics, and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, for certain payments and "transfers of value" provided to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members.

- Analogous state and foreign fraud and abuse laws and regulations, such as state anti kickback and false claims laws, which may be broader in scope and apply regardless of payor. Such laws are enforced by various state agencies and through private actions. Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant federal government compliance guidance, require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers, and restrict marketing practices or require disclosure of marketing expenditures. Some state and local laws require the registration of pharmaceutical sales representatives. State and foreign laws also govern the privacy and security of health information in some circumstances. Such data privacy and security laws may differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. It is possible that governmental authorities will conclude that our business practices do 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 related governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, disgorgement, exclusion of drugs from participation in state and federal healthcare programs, such as Medicare and Medicaid, reputational harm, additional oversight and reporting obligations if we become subject to a corporate integrity agreement or similar settlement to resolve allegations of non compliance with these laws and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to similar actions, penalties and sanctions. Ensuring business arrangements comply with applicable healthcare laws, as well as responding to possible investigations by government authorities, can be time and resource consuming and can divert a company's attention from the business.

Current and Future Healthcare Reform Legislation

In the United States and foreign jurisdictions, there have been a number of 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. We expect that current laws, 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, or any collaborators, may receive for any approved products.

The ACA, for example, contains provisions that subject biological products to potential competition by lower cost biosimilars and may reduce the profitability of drug products through increased rebates for drugs reimbursed by Medicaid programs, extension of Medicaid rebates to Medicaid managed care plans, mandatory discounts for certain Medicare Part D beneficiaries and annual fees based on pharmaceutical companies' share of sales to federal health care programs. The Trump Administration and Congress have taken steps to make administrative or legislative changes, including modification, repeal, or replacement of all, or certain provisions of, the ACA, which may impact reimbursement for drugs and biologics. On January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the ACA 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. On October 13, 2017, President Trump signed an Executive Order terminating the cost sharing subsidies that reimburse insurers under the ACA. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. In addition, the Centers for Medicare & Medicaid Services, or CMS has recently proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces. Further, each chamber of Congress has put forth multiple bills designed to repeal or repeal and replace portions of the ACA. While Congress has not passed repeal legislation, two bills affecting the implementation of certain taxes under the ACA have been signed into law. The Tax Reform Act includes a provision repealing, effective January 1, 2019, the tax based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." Additionally, on January 22, 2018, President Trump signed a continuing resolution on appropriations for

fiscal year 2018 that delayed the implementation of certain ACA-mandated fees, including the "Cadillac" tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. Further, the Bipartisan Budget Act of 2018, or the BBA, among other things, amends the ACA, effective January 1, 2019, to increase from 50 percent to 70 percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole." Congress may consider addtional legislation to repeal or repeal and replace other elements of the ACA. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

Additionally, other federal health reform measures have been proposed and adopted in the United States since the ACA was enacted:

- 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 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and, due to subsequent legislative amendments, including the BBA, will remain in effect through 2025 unless additional Congressional action is taken.

- The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

- The Middle Class Tax Relief and Job Creation Act of 2012 required that the CMS, reduce the Medicare clinical laboratory fee schedule by 2% in 2013, which served as a base for 2014 and subsequent years. In addition, effective January 1, 2014, CMS also began bundling the Medicare payments for certain laboratory tests ordered while a patient received services in a hospital outpatient setting.

Further, there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which have resulted in several recent Congressional inquiries and proposed bills and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. In addition, the United States government, state legislatures, and foreign governments have shown significant interest in implementing cost containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs to limit the growth of government paid health care costs. For example, the United States government has passed legislation requiring pharmaceutical manufacturers to provide rebates and discounts to certain entities and governmental payors to participate in federal healthcare programs. Additionally, the Trump Administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. While any proposed measures will require authorization through additional legislation to become effective, Congress and the Trump Administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. Individual states in the United States have also become increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Packaging and Distribution in the United States

If our products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. Products must meet applicable child resistant packaging requirements under the U.S. Poison Prevention Packaging Act. Manufacturing, sales, promotion and other activities also are potentially subject to federal and state consumer protection and unfair competition laws.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products.

The failure to comply with any of these laws or regulatory requirements subjects firms to possible legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in criminal prosecution, fines or other penalties, injunctions, exclusion from federal healthcare programs, requests for recall, seizure of products, total or partial suspension of production, denial or withdrawal of product approvals, or refusal to allow a firm to enter into supply contracts, including government contracts. 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. Prohibitions or restrictions on sales or withdrawal of future products marketed by us could materially affect our business in an adverse way.

Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

Other U.S. Environmental, Health and Safety Laws and Regulations

We may be subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our 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 for failure to comply with such laws and regulations.

We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Current or future environmental laws and regulations may impair our research, development or production efforts. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions.

U.S. Patent Term Restoration and Marketing Exclusivity

Depending upon the timing, duration and specifics of FDA approval of SRK-015 and any future product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch Waxman Amendments. The Hatch Waxman Amendments permit restoration of the patent term of up to five years as

compensation for patent term lost during product development and FDA regulatory review process. Patent term restoration, however, 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 is generally 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, except that the review period is reduced by any time during which the applicant failed to exercise due diligence. Only one patent applicable to an approved drug is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The U.S. PTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may apply for restoration of patent term for our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant BLA.

An abbreviated approval pathway for biological products shown to be similar to, or interchangeable with, an FDA licensed reference biological product was created by the Biologics Price Competition and Innovation Act of 2009, or BPCI Act. This amendment to the PHSA, in part, attempts to minimize duplicative testing. Biosimilarity, which requires that the biological product be highly similar to the reference product notwithstanding minor differences in clinically inactive components and that there be no clinically meaningful differences between the product and the reference product in terms of safety, purity and potency, can be shown through analytical studies, animal studies and a clinical trial or trials. Interchangeability requires that a biological product be biosimilar to the reference product and that the product can be expected to produce the same clinical results as the reference product in any given patient and, for products administered multiple times to an individual, that the product and the reference product may be alternated or switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biological product without such alternation or switch.

A reference biological product is granted 12 years of data exclusivity from the time of first licensure of the product, and the FDA will not accept an application for a biosimilar or interchangeable product based on the reference biological product until four years after the date of first licensure of the reference product. "First licensure" typically means the initial date the particular product at issue was licensed in the United States. Date of first licensure does not include the date of licensure of (and a new period of exclusivity is not available for) a biological product if the licensure is for a supplement for the biological product or for a subsequent application by the same sponsor or manufacturer of the biological product (or licensor, predecessor in interest, or other related entity) for a change (not including a modification to the structure of the biological product) that results in a new indication, route of administration, dosing schedule, dosage form, delivery system, delivery device or strength, or for a modification to the structure of the biological product that does not result in a change in safety, purity, or potency.

Pediatric exclusivity is another type of regulatory market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing regulatory exclusivity periods. This six month exclusivity may be granted based on the voluntary completion of a pediatric trial in accordance with an FDA issued "Written Request" for such a trial.

European Union Drug Development

In the European Union, or EU, our future products also may be subject to extensive regulatory requirements. As in the United States, medicinal products can be marketed only if a marketing authorization from the competent regulatory agencies has been obtained.

Similar to the United States, the various phases of preclinical and clinical research in the European Union are subject to significant regulatory controls. Although the EU Clinical Trials Directive 2001/20/EC has sought to harmonize the EU clinical trials regulatory framework, setting out common rules for the control and authorization of clinical trials in the EU, the EU Member States have transposed and applied the

provisions of the Directive differently. This has led to significant variations in the member state regimes. Under the current regime, before a clinical trial can be initiated it must be approved in each of the EU countries where the trial is to be conducted by two distinct bodies: the National Competent Authority, or NCA, and one or more Ethics Committees, or ECs. Under the current regime all suspected unexpected serious adverse reactions to the investigated drug that occur during the clinical trial have to be reported to the NCA and ECs of the Member State where they occurred.

The EU clinical trials legislation currently is undergoing a transition process mainly aimed at harmonizing and streamlining clinical trial authorization, simplifying adverse event reporting procedures, improving the supervision of clinical trials and increasing their transparency. Recently enacted Clinical Trials Regulation EU No 536/2014 ensures that the rules for conducting clinical trials in the EU will be identical.

European Union Drug Marketing

Much like the Anti Kickback Statute prohibition in the United States, the provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in the EU. The provision of benefits or advantages to physicians is governed by the national anti bribery laws of European Union Member States, such as the U.K. Bribery Act 2010. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain EU Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual EU Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the EU Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

European Union Drug Review and Approval

In the European Economic Area, or EEA, which is comprised of the 27 Member States of the EU (including Norway and excluding Croatia), Iceland and Liechtenstein, medicinal products can only be commercialized after obtaining a Marketing Authorization, or MA. There are two types of marketing authorizations.

The Community MA is issued by the European Commission through the Centralized Procedure, based on the opinion of the Committee for Medicinal Products for Human Use, or CHMP, of the European Medicines Agency, or EMA, and is valid throughout the entire territory of the EEA. The Centralized Procedure is mandatory for certain types of products, such as biotechnology medicinal products, orphan medicinal products, advanced therapy medicines such as gene therapy, somatic cell therapy or tissue engineered medicines and medicinal products containing a new active substance indicated for the treatment of HIV, AIDS, cancer, neurodegenerative disorders, diabetes, autoimmune and other immune dysfunctions and viral diseases. The Centralized Procedure is optional for products containing a new active substance not yet authorized in the EEA, or for products that constitute a significant therapeutic, scientific or technical innovation or which are in the interest of public health in the EU.

National MAs, which are issued by the competent authorities of the Member States of the EEA and only cover their respective territory, are available for products not falling within the mandatory scope of the Centralized Procedure. Where a product has already been authorized for marketing in a Member State of the EEA, this National MA can be recognized in another Member States through the Mutual Recognition Procedure. If the product has not received a National MA in any Member State at the time of application, it can be approved simultaneously in various Member States through the Decentralized Procedure. Under the Decentralized Procedure an identical dossier is submitted to the competent authorities of each of the Member States in which the MA is sought, one of which is selected by the applicant as the Reference Member State, or RMS. The competent authority of the RMS prepares a draft assessment report, a draft summary of the product characteristics, or SPC, and a draft of the labeling and package leaflet, which are

sent to the other Member States (referred to as the Member States Concerned) for their approval. If the Member States Concerned raise no objections, based on a potential serious risk to public health, to the assessment, SPC, labeling, or packaging proposed by the RMS, the product is subsequently granted a national MA in all the Member States (i.e., in the RMS and the Member States Concerned).

Under the above described procedures, before granting the MA, the EMA or the competent authorities of the Member States of the EEA make an assessment of the risk benefit balance of the product on the basis of scientific criteria concerning its quality, safety and efficacy.

European Union New Chemical Entity Exclusivity

In the EU, new chemical entities, sometimes referred to as new active substances, qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. The data exclusivity, if granted, prevents regulatory authorities in the EU from referencing the innovator's data to assess a generic application for eight years, after which generic marketing authorization can be submitted, and the innovator's data may be referenced, but not approved for two years. The overall 10 year period will be extended to a maximum of 11 years if, during the first eight years of those 10 years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are determined to bring a significant clinical benefit in comparison with currently approved therapies.

European Union Orphan Designation and Exclusivity

In the EU, 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 life threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the EU community (or where it is unlikely that the development of the medicine would generate sufficient return to justify the investment) and for which no satisfactory method of diagnosis, prevention or treatment has been authorized (or, if a method exists, the product would be a significant benefit to those affected).

In the EU, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and 10 years of market exclusivity is granted following medicinal product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

European Data Collection

The collection and use of personal health data in the European Union is governed by the provisions of the Data Protection Directive, and as of May 2018 the General Data Protection Regulation, or GDPR. This directive imposes several requirements relating to the consent of the individuals to whom the personal data relates, the information provided to the individuals, notification of data processing obligations to the competent national data protection authorities and the security and confidentiality of the personal data. The Data Protection Directive and GDPR also impose strict rules on the transfer of personal data out of the EU to the United States. Failure to comply with the requirements of the Data Protection Directive, the GDPR, and the related national data protection laws of the EU Member States may result in fines and other administrative penalties. The GDPR introduces new data protection requirements in the EU and substantial fines for breaches of the data protection rules. The GDPR regulations may impose additional responsibility and liability in relation to personal data that we process and we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules. This may be onerous and adversely affect our business, financial condition, results of operations and prospects.

Rest of the World Regulation

For other countries outside of the EU and the United States, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. Additionally, the clinical trials must be conducted in accordance with GCP requirements and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Additional Laws and Regulations Governing International Operations

If we further expand our operations outside of the United States, we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The U.S. Securities and Exchange Commission, or SEC, also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

Reimbursement

Sales of our products will depend, in part, on the extent to which our products, if approved, will be covered by third-party payors, such as government health programs, commercial insurers and managed healthcare organizations, as well as the level of reimbursement such third-party payors provide for our products. Patients and providers are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our products. In the United States no uniform policy of coverage and reimbursement for drugs or biological products exists, and one payor's determination to provide coverage and adequate reimbursement for a product does not assure that other payors will make a

similar determination. Accordingly, decisions regarding the extent of coverage and amount of reimbursement to be provided for any of our products candidates, if approved, will be made on a payor by payor basis. As a result, the coverage determination process may be a time consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be obtained.

The Medicaid Drug Rebate Program requires pharmaceutical manufacturers to enter into and have in effect a national rebate agreement with the Secretary of the DHHS as a condition for states to receive federal matching funds for the manufacturer's outpatient drugs furnished to Medicaid patients. The ACA made several changes to the Medicaid Drug Rebate Program, including increasing pharmaceutical manufacturers' rebate liability by raising the minimum basic Medicaid rebate on most branded prescription drugs from 15.1% of average manufacturer price, or AMP, to 23.1% of AMP and adding a new rebate calculation for "line extensions" (i.e., new formulations, such as extended release formulations) of solid oral dosage forms of branded products, creating a new method by which rebates owed by are calculated for drugs that are inhaled, infused, instilled, implanted or injected, as well as potentially impacting their rebate liability by modifying the statutory definition of AMP. The ACA also expanded the universe of Medicaid utilization subject to drug rebates by requiring pharmaceutical manufacturers to pay rebates on Medicaid managed care utilization and by enlarging the population potentially eligible for Medicaid drug benefits. Pricing and rebate programs must also comply with the Medicaid rebate requirements of the U.S. Omnibus Budget Reconciliation Act of 1990.

The Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA, established the Medicare Part D program to provide a voluntary prescription drug benefit to Medicare beneficiaries. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities that provide coverage of outpatient prescription drugs. Unlike Medicare Part A and B, Part D coverage is not standardized. While all Medicare drug plans must give at least a standard level of coverage set by Medicare, Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. However, Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a Part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee. Government payment for some of the costs of prescription drugs may increase demand for products for which we receive marketing approval. However, any negotiated prices for our products covered by a Part D prescription drug plan likely will be lower than the prices we might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non governmental payors.

For a drug product to receive federal reimbursement under the Medicaid or Medicare Part B programs or to be sold directly to U.S. government agencies, the manufacturer must extend discounts to entities eligible to participate in the 340B drug pricing program. The required 340B discount on a given product is calculated based on the AMP and Medicaid rebate amounts reported by the manufacturer. As of 2010, the ACA expanded the types of entities eligible to receive discounted 340B pricing, although, under the current state of the law, with the exception of children's hospitals, these newly eligible entities will not be eligible to receive discounted 340B pricing on orphan drugs. In addition, as 340B drug pricing is determined based on AMP and Medicaid rebate data, the revisions to the Medicaid rebate formula and AMP definition described above could cause the required 340B discount to increase.

As noted above, the marketability of any products for which we receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide coverage and adequate reimbursement. An increasing emphasis on cost containment measures in the United States has increased and we expect will continue to increase the pressure on pharmaceutical pricing. 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.

These laws, and future state and federal healthcare reform measures may be adopted in the future, any of which may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

In addition, in most foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing and reimbursement vary widely from country to country. For example, the EU provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. Reference pricing used by various EU Member States and parallel distribution, or arbitrage between low priced and high priced member states, can further reduce prices. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. In some countries, we may be required to conduct a clinical study or other studies that compare the cost effectiveness of any of our product candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products. Historically, products launched in the EU do not follow price structures of the United States and generally prices tend to be significantly lower. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries.

Employees

As of April 25, 2018, we had 49 full-time employees, including 22 employees with M.D. or Ph.D. degrees. Of these full-time employees, 39 employees are engaged in research and development activities and ten are engaged in general and administrative activities. None or our employees is represented by a labor union or covered by a collective bargaining agreement.

Facilities

Our facility comprises 21,000 square feet (including over 9,000 square feet of expansion space which we have not yet occupied) of office and laboratory space in Cambridge, Massachusetts. We executed a lease amendment on February 22, 2018 for the additional expansion space and expect to occupy the expansion space in the second quarter of 2018. The lease expires five years after our landlord delivers the expansion space to us. We have an option to extend the lease term for five additional years. We believe that our existing facilities, including our expansion space, are adequate to meet our current needs, and that suitable additional space will be available as and when needed.

Legal Proceedings

From time to time, we may be involved in various claims and legal proceedings relating to claims arising out of our operations. We are not currently a party to any material legal proceedings.

MANAGEMENT

The following table sets forth the name, age and position of each of our executive officers and directors, as of April 25, 2018:

Name	Age	Position
Executive Officers		
Nagesh K. Mahanthappa, Ph.D.	53	President, Chief Executive Officer and Director
Alan J. Buckler, Ph.D.	58	Chief Scientific Officer
Rhonda M. Chicko, C.P.A.	52	Chief Financial Officer
Yung H. Chyung, M.D.	42	Chief Medical Officer
Elan Z. Ezickson	54	Chief Operating Officer & Head of Corporate Development
Non-Employee Directors		
David Hallal	51	Chairman of the Board of Directors
Kristina Burow	44	Director
Jeffrey S. Flier, M.D.	70	Director
Michael Gilman, Ph.D.	63	Director
Amir Nashat, Sc.D.	45	Director
Timothy A. Springer, Ph.D.	70	Director

(1) Member of the audit committee

(2) Member of the compensation committee

(3) Member of the nominating and corporate governance committee

Executive Officers

Nagesh K. Mahanthappa, Ph.D. is the founding employee of Scholar Rock and has served as a director and our President and Chief Executive Officer since October 2012. Prior to joining us, from February 2007 to May 2012, Dr. Mahanthappa was a founding employee and Vice President, Corporate Development & Operations at Avila Therapeutics, Inc. (acquired by Celgene Corporation in March 2012). Previously, from August 2002 to February 2007, he served in roles of increasing responsibility at Alnylam Pharmaceuticals, Inc., most recently as Vice President, Scientific & Strategic Development. He was also a founder of TwistDx, Inc. a DNA diagnostics company acquired by Inverness Medical Innovations, Inc. (now Alere, Inc.) in 2010. Dr. Mahanthappa received his Ph.D. in Neurobiology from the California Institute of Technology, and completed his post-doctoral training at the E.K. Shriver Center for Mental Retardation (then affiliated with Massachusetts General Hospital) and Harvard Medical School. He received his M.B.A. from the F.W. Olin Graduate School of Management at Babson College and his B.A. in Biology and Chemistry from the University of Colorado, Boulder. Our board of directors believes that Dr. Mahanthappa's extensive experience in the pharmaceutical industry qualifies him to serve on our board of directors.

Alan J. Buckler, Ph.D. has served as our Chief Scientific Officer since November 2016. Prior to joining us, Dr. Buckler served as Vice President, Cell and Protein Sciences, at Biogen Inc. from 2014 to 2016. From 2005 to 2014, Dr. Buckler served as Director, Developmental and Molecular Pathways in the Novartis Institutes for Biomedical Research. Prior to Novartis, Dr. Buckler served as the Chief Scientific Officer of Ardais Corporation from 1999 to 2004 and as Vice President of Molecular Genetics at Sequana Therapeutics/Axys Pharmaceuticals from 1996 to 1999. Prior to joining the private sector, Dr. Buckler served on the Neurology faculty of Massachusetts General Hospital and Harvard Medical School from 1991 to 1996. Dr. Buckler received his A.B. in Biology from the University of Chicago, Ph.D. in Microbiology from the Boston University School of Medicine, and completed his post-doctoral training at the Center for Cancer Research, Massachusetts Institute of Technology.

Rhonda M. Chicko, C.P.A. has served as our Chief Financial Officer since April 2018. Prior to joining us, she served as Vice President, Finance at Editas Medicine, Inc. where she worked from September 2015 to March 2018. From 2005 to 2015, Ms. Chicko worked at Ironwood Pharmaceuticals, Inc. in financial roles of increasing responsibility, culminating as Senior Director, Finance and Tax. Earlier in her career, Ms. Chicko held a range of positions at investment management and accounting firms, including Wellington Management Company, LLP and PricewaterhouseCoopers, LLP. Ms. Chicko holds a B.S. in accounting from Le Moyne College and an M.S.T. from Bentley University.

Yung H. Chyung, M.D. has served as our Chief Medical Officer since February 2016. Prior to joining us, Dr. Chyung served in roles of increasing responsibility at Dyax Corp. (acquired by Shire Plc in January 2016) from 2011 to February 2016, most recently as Vice President of Medical Research, where he was responsible for clinical research and medical affairs. From 2010 to 2011, Dr. Chyung worked at Genzyme Corporation where he was responsible for medical affairs efforts globally for multiple rare disease programs. Dr. Chyung earned his M.D. from Harvard Medical School and completed his internal medicine residency and allergy and immunology fellowship at Massachusetts General Hospital. Dr. Chyung also holds an A.B. in Biochemical Sciences from Harvard College.

Elan Z. Ezickson has served as our Chief Operating Officer & Head of Corporate Development since August 2014. Prior to joining us, Mr. Ezickson served most recently as Executive Vice President and Chief Operating Officer of Aveo Pharmaceuticals, Inc., where he worked from 2003 to July 2013. From 1994 to 2003, he worked at Biogen Inc. in roles that included President of Biogen Canada, Program Executive and Associate General Counsel. Mr. Ezickson holds a B.A. in Political Science from Yale University and a J.D. from the Columbia University School of Law.

Non-Employee Directors

David Hallal has served as the Chairman of our board of directors since July 2017. Most recently, from June 2006 to December 2016, Mr. Hallal served in executive roles of increasing responsibility at Alexion Pharmaceuticals, Inc., most recently serving as Chief Executive Officer and a board member. Prior to his role as CEO, Mr. Hallal served Alexion as COO and Director as well as Chief Commercial Officer and Head of Commercial Operations. Prior to Alexion from 2004 to 2006, Mr. Hallal served as Vice President of Sales for OSI Eyetech, Inc. From 2002 to 2004, Mr. Hallal served as Head of Sales at Biogen Inc. From 1992 to 2002, Mr. Hallal held various leadership roles at Amgen Inc. From 1988 to 1992, Mr. Hallal began his pharmaceutical career at The Upjohn Company as a sales representative. Mr. Hallal holds a B.A. in psychology from the University of New Hampshire. Mr. Hallal also currently serves as an independent director at Seer Biosciences, Inc. Our board of directors believes that Mr. Hallal's experience as an executive at numerous pharmaceutical companies qualifies him to serve as our Chairman of the board of directors.

Kristina Burow has served as a member of our board of directors since August 2014. Ms. Burow has served as Managing Director of ARCH Venture Partners, or ARCH, since November 2011 and previously held roles of increasing responsibility at ARCH from August 2002 to November 2011. Ms. Burow currently serves on the board of directors of several biopharmaceutical and biotechnology companies, including Vividion Therapeutics, Inc., Lycera Corp., BlackThorn Therapeutics, Inc., Metacrine, Inc., Unity Biotechnology, Inc., AgBiome Inc., Vir Biotechnology Inc., and AgTech Accelerator, an agricultural technology startup accelerator. Ms. Burow also serves on the board of directors of Sienna. She previously was a co-founder and member of the board of directors of Receptos, Inc., a public pharmaceutical company, until its acquisition by Celgene Corporation, a public biopharmaceutical company, and of Sapphire Energy, Inc., energy company. Ms. Burow has participated in a number of other ARCH portfolio companies including KYTHERA, Siluria Technologies, Inc., an energy company, and Ikaria, Inc., a biotechnology company, acquired by Madison Dearborn Partners, a private equity firm. Prior to joining ARCH, Ms. Burow was an Associate with the Novartis BioVenture Fund in San Diego and an early employee at the Genomics Institute of the Novartis Research Foundation. Ms. Burow received a B.A. in Chemistry from the University of California, Berkeley, an M.A. in Chemistry from Columbia University, and an M.B.A. from the University of Chicago. We believe that

Ms. Burow is qualified to serve on our board of directors due to her extensive experience investing in biopharmaceutical and biotechnology companies and her experience on boards of directors in the medical industry.

Jeffrey S. Flier, M.D. has served as member of our board of directors since October 2016. Since August 2016, Dr. Flier has served as the Higginson Professor of Physiology and Medicine and Harvard University Distinguished Service Professor, and from 2007 to August 2016 served as the twenty-first Dean of the Faculty of Medicine at Harvard University. Previously, from 2002 to 2007, Dr. Flier served as Chief Academic Officer of Beth Israel Deaconess Medical Center and served as Harvard Medical School Faculty Dean for Academic Programs. An elected member of the National Academy of Medicine and a fellow of the American Academy of Arts and Sciences, his many honors include the Eli Lilly Award of the American Diabetes Association, and the Berson Lecture of the American Physiological Society. He was the recipient of the 2005 Banting Medal from the American Diabetes Association, its highest scientific honor. Dr. Flier received his B.S. from City College of New York and his M.D. from Mount Sinai School of Medicine with highest academic honors, and he completed his residency training at Mount Sinai School of Medicine. Our board of directors believes that Dr. Flier's extensive medical and scientific experience and his leadership skills qualify him to serve on our board of directors.

Michael Gilman, Ph.D. has served as a member of our board of directors since November 2013. Dr. Gilman is currently Chairman and Chief Executive Officer for Arrakis Therapeutics, Inc., a role he has served in since 2016, and Chief Executive Officer and Director for Obsidian Therapeutics, Inc., a role he has served in since 2016. Previously, from 2014 to 2016 Dr. Gilman was Founder and Chief Executive Officer of Padlock Therapeutics, Inc. Prior to Padlock, Dr. Gilman served as Senior Vice President, Early-Stage Pipeline, at Biogen Idec Inc. from 2012 to 2013. He joined Biogen Idec Inc. in 2012 following its acquisition of Stromedix, Inc., where he was Founder and Chief Executive Officer. Prior to founding Stromedix in 2006, from 1999 to 2005, Dr. Gilman served in a variety of capacities, most recently as Executive Vice President, Research at Biogen Idec. From 1994 to 1999, Dr. Gilman was at ARIAD Pharmaceuticals, Inc., where he was Executive Vice President and Chief Scientific Officer. From 1986 to 1994, Dr. Gilman was on the scientific staff of Cold Spring Harbor Laboratory in New York. He also serves on the Board of Directors of X4 Pharmaceuticals, Inc. and the Scientific Advisory Board of FutuRx, an Israeli biotech accelerator. Dr. Gilman was a postdoctoral fellow with Dr. Robert Weinberg at the Whitehead Institute. He holds a Ph.D. in Biochemistry from University of California, Berkeley, and an S.B. in Life Sciences from Massachusetts Institute of Technology. Our board of directors believes that Dr. Gilman's extensive experience in the pharmaceuticals industry qualifies him to serve on our board of directors

Amir Nashat, Sc.D. has served as a member of our board of directors since October 2012. Dr. Nashat is a managing partner at Polaris Partners, a venture capital firm, where he has worked since 2002. Dr. Nashat was also the founding Chief Executive Officer of Living Proof, Inc. and Sun Catalytix Corporation. Dr. Nashat currently represents Polaris as a Director of Agbiome, Inc., aTyr Pharmaceuticals, Inc., Fate Therapeutics, Inc., Jnana Therapeutics, where he also serves as the CEO, CAMP4, Metacine, Inc., Morphic Therapeutic, Inc., Olivo Labs, Promedior, Inc., Selecta Biosciences Inc., Syros Pharmaceuticals, Inc., and Taris Biomedical, LLC. Dr. Nashat also serves on the Partners Innovation Fund, the Investment Advisory Committee for The Engine at MIT, and helped launch the MIT Sandbox Innovation Fund as its active president. Dr. Nashat previously served on the Board of the New England Venture Capital Association. Dr. Nashat received an M.S. and B.S. in materials science and mechanical engineering from the University of California, Berkeley and a Sc.D. as a Hertz Fellow in Chemical Engineering at the Massachusetts Institute of Technology with a minor in Biology under Dr. Robert Langer. Our board of directors believes that Dr. Nashat's biotechnology investment experience qualifies him to serve on our board of directors.

Timothy A. Springer, Ph.D. is a co-founder and investor in Scholar Rock and has served as a member of our board of directors since October 2012. Since 1989, Dr. Springer has served as the Latham Family Professor of Pathology at Harvard Medical School. He has also served as Senior Investigator in the Program in Cellular and Molecular Medicine at Boston Children's Hospital since 2012 and as Professor of Biological Chemistry

and Molecular Pharmacology at Harvard Medical School and Professor of Medicine at Boston Children's Hospital since 2011. Dr. Springer was the Founder and Chairman of the Scientific Advisory Board of LeukoSite, Inc., a biotechnology company acquired by Millennium Pharmaceuticals, Inc. in 1999. He is a founder, investor and board member of Morphic Therapeutic, Inc. and an investor and board member of Selecta Biosciences Inc. Dr. Springer is the Chairman of the Institute for Protein Innovation and is a member of the National Academy of Sciences. His honors include the Crafoord Prize, the American Association of Immunologists Meritorious Career Award, the Stratton Medal from the American Society of Hematology, and the Basic Research Prize from the American Heart Association. Dr. Springer received a B.A. from the University of California, Berkeley, and a Ph.D. from Harvard University. Our board of directors believes that Dr. Springer's extensive knowledge of our business and the biotechnology field qualifies him to serve on our board of directors.

Composition of Our Board of Directors

As of April 25, 2018, our board of directors consisted of seven members, each of whom are members pursuant to the board composition provisions of our certificate of incorporation and agreements with our stockholders. These board composition provisions will terminate upon the completion of this offering. Upon the termination of these provisions, there will be no further contractual obligations regarding the election of our directors. Our nominating and corporate governance committee and our board of directors may therefore consider a broad range of factors relating to the qualifications and background of nominees. Our nominating and corporate governance committee's and our board of directors' priority in selecting board members is the identification of persons who will further the interests of our stockholders through their established record of professional accomplishment, the ability to contribute positively to the collaborative culture among board members, knowledge of our business, understanding of the competitive landscape, and professional and personal experiences and expertise relevant to our growth strategy. Our directors hold office until their successors have been elected and qualified or until the earlier of their resignation or removal. Our amended and restated certificate of incorporation and amended and restated by-laws that will become effective upon the completion of this offering also provide that our directors may be removed only for cause by the affirmative vote of the holders of at least two-thirds of the votes that all our stockholders would be entitled to cast in an annual election of directors, and that any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office.

Director Independence

Our board of directors has determined that all members of the board of directors, except Dr. Mahanthappa, are independent directors, including for purposes of the rules of The Nasdaq Global Market and the Securities and Exchange Commission, or SEC. In making such independence determination, our board of directors considered the relationships that each non-employee director has with us and all other facts and circumstances that our board of directors deemed relevant in determining their independence, including the beneficial ownership of our capital stock by each non-employee director. In considering the independence of the directors listed above, our board of directors considered the association of our directors with the holders of more than 5% of our common stock. Upon the completion of this offering, we expect that the composition and functioning of our board of directors and each of our committees will comply with all applicable requirements of The Nasdaq Global Market and the rules and regulations of the SEC. There are no family relationships among any of our directors or executive officers. Dr. Mahanthappa is not an independent director under these rules because he is an executive officer of our company.

Staggered Board

In accordance with the terms of our amended and restated certificate of incorporation and amended and restated by-laws that will become effective upon the completion of this offering, our board of directors will be divided into three staggered classes of directors and each director will be assigned to one of the three classes. At each annual meeting of the stockholders, a class of directors will be elected for a three-year term to succeed the directors of the same class whose terms are then expiring. The terms of the directors will expire upon the election and qualification of successor directors at the annual meeting of stockholders

to be held during the years 2019 for Class I directors, 2020 for Class II directors and 2021 for Class III directors.

- Our Class I directors will be ;
- Our Class II directors will be ; and
- Our Class III directors will be .

Our amended and restated certificate of incorporation and amended and restated by-laws that will become effective upon the completion of this offering will provide that the number of directors shall be fixed from time to time by a resolution of the majority of our board of directors.

The division of our board of directors into three classes with staggered three-year terms may delay or prevent stockholder efforts to effect a change of our management or a change in control.

Board Leadership Structure and Board's Role in Risk Oversight

Mr. Hallal is the current chairman of our board of directors and Dr. Mahanthappa is our current Chief Executive Officer, hence the roles of chairman of our board of directors and Chief Executive Officer are separated. We believe that separating these positions allows our Chief Executive Officer to focus on our day-to-day business, while allowing our chairman of the board to lead the board of directors in its fundamental role of providing advice to and independent oversight of management. Our board of directors recognizes the time, effort and energy that the Chief Executive Officer is required to devote to his position in the current business environment, as well as the commitment required to serve as our chairman, particularly as the board of directors' oversight responsibilities continue to grow. While our amended and restated by-laws and corporate governance guidelines do not require that our chairman and Chief Executive Officer positions be separate, our board of directors believes that having separate positions is the appropriate leadership structure for us at this time and demonstrates our commitment to good corporate governance.

Risk is inherent with every business, and how well a business manages risk can ultimately determine its success. We face a number of risks, including risks relating to our financial condition, development and commercialization activities, operations, strategic direction and intellectual property as more fully discussed in the section entitled "Risk Factors" appearing elsewhere in this prospectus. Management is responsible for the day-to-day management of risks we face, while our board of directors, as a whole and through its committees, has responsibility for the oversight of risk management. In its risk oversight role, our board of directors has the responsibility to satisfy itself that the risk management processes designed and implemented by management are adequate and functioning as designed.

The role of the board of directors in overseeing the management of our risks is conducted primarily through committees of the board of directors, as disclosed in the descriptions of each of the committees below and in the charters of each of the committees. The full board of directors (or the appropriate board committee in the case of risks that are under the purview of a particular committee) discusses with management our major risk exposures, their potential impact on us, and the steps we take to manage them. When a board committee is responsible for evaluating and overseeing the management of a particular risk or risks, the chairman of the relevant committee reports on the discussion to the full board of directors during the committee reports portion of the next board meeting. This enables the board of directors and its committees to coordinate the risk oversight role, particularly with respect to risk interrelationships.

Committees of Our Board of Directors

Our board of directors has established an audit committee, a compensation committee, a nominating and corporate governance committee and a science, innovation and technology committee, each of which will operate pursuant to a charter adopted by our board of directors and will be effective upon the effectiveness of the registration statement of which this prospectus is a part. Upon the effectiveness of the registration statement of which this prospectus is a part, the composition and functioning of all of our committees will

comply with all applicable requirements of the Sarbanes-Oxley Act of 2002, Nasdaq and SEC rules and regulations.

Audit Committee

Effective upon effectiveness of the registration statement of which this prospectus forms a part, will serve on the audit committee, which will be chaired by . Our board of directors has determined that each member of the audit committee is "independent" for audit committee purposes as that term is defined in the rules of the SEC and the applicable Nasdaq rules, and each has sufficient knowledge in financial and auditing matters to serve on the audit committee. Our board of directors has designated as an "audit committee financial expert," as defined under the applicable rules of the SEC. The audit committee's responsibilities include:

- appointing, approving the compensation of, and assessing the independence of our independent registered public accounting firm;
- pre-approving auditing and permissible non-audit services, and the terms of such services, to be provided by our independent registered public accounting firm;
- reviewing the overall audit plan with our independent registered public accounting firm and members of management responsible for preparing our financial statements;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures as well as critical accounting policies and practices used by us;
- coordinating the oversight and reviewing the adequacy of our internal control over financial reporting;
- establishing policies and procedures for the receipt and retention of accounting-related complaints and concerns;
- recommending based upon the audit committee's review and discussions with management and our independent registered public accounting firm whether our audited financial statements shall be included in our Annual Report on Form 10-K;
- monitoring the integrity of our financial statements and our compliance with legal and regulatory requirements as they relate to our financial statements and accounting matters;
- preparing the audit committee report required by SEC rules to be included in our annual proxy statement;
- reviewing all related person transactions for potential conflict of interest situations and approving all such transactions; and
- reviewing quarterly earnings releases.

Compensation Committee

Effective upon effectiveness of the registration statement of which this prospectus forms a part, will serve on the compensation committee, which will be chaired by . Our board of directors has determined that each member of the compensation committee is "independent" as defined in the applicable Nasdaq rules. The compensation committee's responsibilities include:

- annually reviewing and recommending to the board of directors the corporate goals and objectives relevant to the compensation of our Chief Executive Officer;
- evaluating the performance of our Chief Executive Officer in light of such corporate goals and objectives and based on such evaluation: (i) recommending to the board of directors the cash compensation of our Chief Executive Officer and (ii) reviewing and approving grants and awards to our Chief Executive Officer under equity-based plans;
- reviewing and approving or recommending to the board of directors the cash compensation of our other executive officers;
- reviewing and establishing our overall management compensation, philosophy and policy;
- overseeing and administering our compensation and similar plans;

- evaluating and assessing potential and current compensation advisors in accordance with the independence standards identified in the applicable Nasdaq rules;
- reviewing and approving our policies and procedures for the grant of equity-based awards;
- reviewing and recommending to the board of directors the compensation of our directors;
- preparing the compensation committee report required by SEC rules, if and when required, to be included in our annual proxy statement; and
- reviewing and approving the retention, termination or compensation of any consulting firm or outside advisor to assist in the evaluation of compensation matters.

Nominating and Corporate Governance Committee

Effective upon effectiveness of the registration statement of which this prospectus forms a part, will serve on the nominating and corporate governance committee, which will be chaired by . Our board of directors has determined that each member of the nominating and corporate governance committee is "independent" as defined in the applicable Nasdaq rules. The nominating and corporate governance committee's responsibilities include:

- developing and recommending to the board of directors criteria for board and committee membership;
- establishing procedures for identifying and evaluating board of director candidates, including nominees recommended by stockholders;
- reviewing the composition of the board of directors to ensure that it is composed of members containing the appropriate skills and expertise to advise us;
- identifying individuals qualified to become members of the board of directors;
- recommending to the board of directors the persons to be nominated for election as directors and to each of the board's committees;
- developing and recommending to the board of directors a code of business conduct and ethics and a set of corporate governance guidelines; and
- overseeing the evaluation of our board of directors and management.

Science, Innovation and Technology Committee

Effective upon effectiveness of the registration statement of which this prospectus forms a part, our science, innovation and technology committee will be composed of , with serving as chairman of the committee. The science, innovation and technology committee's responsibilities upon completion of this offering will include:

- providing a general oversight function regarding preclinical and clinical decision-making through a series of semi-annual pipeline reviews and in-depth assessments of select project strategies and plans;
- providing recommendations regarding key molecules in our discovery and development pipelines through reports and select in-depth project reviews;
- providing recommendations regarding our pipeline/portfolio balance from a scientific and clinical perspective, including new molecular entity versus new indication balance, mechanism balance, target balance and general risk balance;
- providing recommendations regarding intellectual property strategies;
- providing recommendations regarding key discovery and development strategies to align with our business needs; and
- providing feedback to the board of directors and to our research and development group.

Our board of directors may from time to time establish other committees.

Compensation Committee Interlocks and Insider Participation

None of the members of our compensation committee has at any time during the prior three years been one of our officers or employees. None of our executive officers currently serves, or in the past fiscal year has served, as a member of the board of directors or compensation committee of any entity that has one or more executive officers serving on our board of directors or compensation committee.

Corporate Governance

We have adopted a written code of business conduct and ethics, effective upon the effectiveness of the registration statement of which this prospectus is a part, that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. Following the effectiveness of the registration statement of which this prospectus is a part, a current copy of the code will be posted on the investor relations section of our website, which is located at http://www.scholarrock.com. If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website or in a current report on Form 8-K.

EXECUTIVE COMPENSATION

Executive Compensation Overview

Our executive compensation program has reflected our growth and development-oriented corporate culture. To date, the compensation of the individuals listed below, whom we refer to as our named executive officers, has primarily consisted of a combination of base salary, bonuses and long-term incentive compensation. Our named executive officers, like all of our full-time employees, are eligible to participate in our health and welfare benefit plans. As we transition from a private company to a publicly traded company, we will evaluate our compensation values and philosophy and compensation plans and arrangements as circumstances require. At a minimum, we expect to review executive compensation annually with input from a compensation consultant. As part of this review process, we expect the board of directors and the compensation committee to apply our values and philosophy, while considering the compensation levels needed to ensure our executive compensation program remains competitive. We will also review whether we are meeting our retention objectives and the potential cost of replacing a key employee.

Summary Compensation Table — 2017

The following table presents information regarding the total compensation awarded to, earned by, and paid to our named executive officer for services rendered to us in all capacities for the year ended December 31, 2017.

Name and Principal Position	Year	Salary ($)	Stock Awards ($)	Non-Equity Incentive Plan Compensation ($)[2]	Total ($)
Nagesh K. Mahanthappa Ph.D., President and Chief Executive Officer	2017	382,454	590,810[1]	147,245	1,120,509
Yung H. Chyung M.D., Chief Medical Officer	2017	359,660	165,200[3]	118,688	643,548
Elan Z. Ezickson, Chief Operating Officer & Head of Corporate Development	2017	354,447	108,643[4]	116,968	580,058
		1,096,561	864,653	$ 382,901	2,344,115

(1) $391,065 of this amount reflects the aggregate grant date fair value of a stock award granted during the year calculated in accordance with the provisions of Financial Accounting Standards Board Accounting Standard Codification Topic 718, *Compensation — Stock Compensation*. For information regarding assumptions underlying the valuation of this stock award, see Note 12 to our financial statements appearing at the end of this prospectus. The remaining $199,745 of this amount represents the incremental fair value resulting from the exchange of incentive units of Scholar Rock, LLC into shares of our common stock and restricted common stock in connection with the Reorganization, as further described in the section titled "Reorganization."

(2) Amounts reflect the cash incentive bonuses received by our named executive officers in 2018 for performance of services in 2017 and were based upon achievement of corporate performance goals.

(3) The amount reported for Dr. Chyung represents the incremental fair value resulting from the exchange of incentive units of Scholar Rock, LLC into shares of our common stock and restricted common stock in connection with the Reorganization. Dr. Chyung did not receive a stock award in 2017.

(4) The amount reported for Mr. Ezickson represents the incremental fair value resulting from the exchange of incentive units of Scholar Rock, LLC into shares of our common stock and restricted common stock in connection with the Reorganization. Mr. Ezickson did not receive a stock award in 2017.

Narrative Disclosure to 2017 Summary Compensation Table

Base Salary

Each named executive officer's base salary is a fixed component of annual compensation for performing specific duties and functions, and has been established by our board of directors taking into account each individual's role, responsibilities, skills, and experience.

Non-Equity Incentive Plan Compensation

Our annual bonus program is intended to reward our named executive officers for meeting objective or subjective individual and/or company-wide performance goals for a fiscal year. For 2017, our named executive officers received incentive compensation based upon achievement of corporate objectives.

Long-Term Equity Incentives

Our equity grant program is intended to align the interests of our named executive officers with those of our stockholders and to motivate them to make important contributions to our performance.

Employment Arrangements and Severance Agreements with Our Named Executive Officers

Nagesh K. Mahanthappa, Ph.D.

For the year ended December 31, 2017, the annual base salary for Dr. Mahanthappa was $382,454. For 2017, Dr. Mahanthappa was eligible to earn an annual cash incentive bonus targeted at 35% of his base salary, with the actual cash incentive bonus determined by the board of directors based on the achievement of specified corporate goals. Dr. Mahanthappa is also eligible to participate in the employee benefit plans available to our employees, subject to the terms of those plans.

Pursuant to Dr. Mahanthappa's offer letter, dated October 10, 2012, in the event that he is terminated by us without "cause" or he resigns for "good reason," subject to his execution of a separation agreement and general release, he will be entitled to (1) continuation of his base salary for a period of six successive months plus one additional successive month for each full year of service to us, up to a maximum of 12 total months following his termination of employment; provided, however, that such continuation of his base salary is subject to reduction in the event of his employment or self-employment after the initial three-month period, and (2) payment of health insurance premiums provided under COBRA following his termination of employment at the same rate as was in effect on the date of termination for a period of six successive months plus one additional successive month for each full year of service to us, up to a maximum of 12 total months. Additionally, in the event Dr. Mahanthappa is terminated without "cause" or he resigns for "good reason" within 18 months following a "sale event" (each as defined in his offer letter), he will be entitled to full acceleration of any unvested equity awards.

Yung H. Chyung, M.D.

For the year ended December 31, 2017, the annual base salary for Dr. Chyung was $359,660. For 2017, Dr. Chyung was eligible to earn an annual cash incentive bonus targeted at 30% of his base salary, with the actual cash incentive bonus determined by the board of directors based on the achievement of specified corporate goals. Dr. Chyung is also eligible to participate in the employee benefit plans available to our employees, subject to the terms of those plans.

Pursuant to Dr. Chyung's offer letter, dated February 2, 2016, in the event that he is terminated by us without "cause" or he resigns for "good reason," subject to his execution of a separation agreement and general release, he will be entitled to (1) continuation of his base salary for a period of six successive months plus one additional successive month for each full year of service to us, up to a maximum of nine total months following his termination of employment; provided, however, that such continuation of his base salary is subject to reduction in the event of his employment or self-employment after the initial three-month period, and (2) payment of health insurance premiums provided under COBRA following his termination of employment at the same rate as was in effect on the date of termination for a period of six successive months plus one additional successive month for each full year of service to us, up to a

maximum of nine total months. Additionally, in the event Dr. Chyung is terminated without "cause" or he resigns for "good reason" within 18 months following a "sale event" (each as defined in his offer letter), he will be entitled to full acceleration of any unvested equity awards.

Elan Z. Ezickson

For the year ended December 31, 2017, the annual base salary for Mr. Ezickson was $354,447. For 2017, Mr. Ezickson was eligible to earn an annual cash incentive bonus targeted at 30% of his base salary, with the actual cash incentive bonus determined by the board of directors based on the achievement of specified corporate goals. Mr. Ezickson is also eligible to participate in the employee benefit plans available to our employees, subject to the terms of those plans.

Pursuant to Mr. Ezickson's offer letter, dated July 17, 2014, in the event that he is terminated by us without "cause" or he resigns for "good reason," subject to his execution of a separation agreement and general release, he will be entitled to (1) continuation of his base salary for a period of six successive months plus one additional successive month for each full year of service to us, up to a maximum of nine total months following his termination of employment and (2) payment of health insurance premiums provided under COBRA following his termination of employment at the same rate as was in effect on the date of termination for a period of six successive months plus one additional successive month for each full year of service to us, up to a maximum of nine total months. Additionally, in the event Mr. Ezickson is terminated without "cause" or he resigns for "good reason" within 18 months following a "sale event" (each as defined in his offer letter), he will be entitled to full acceleration of any unvested equity awards.

Other Agreements

We have also entered into employee confidentiality, inventions assignment, non-solicitation and non-competition agreements with each of our named executive officers. Under such agreements, each named executive officer has agreed (1) not to compete with us during his or her employment and for a period of one year after the termination of such employment, (2) not to solicit our employees during his or her employment and for a period of one year after the termination of such employment, (3) to protect our confidential and proprietary information and (4) to assign to us related intellectual property developed during the course of his or her employment.

Outstanding Equity Awards as of December 31, 2017

The following table sets forth information concerning outstanding equity awards held by our named executive officers as of December 31, 2017:

Name and Principal Position	Number of Shares That Have Not Vested (#)[1]	Market Value of Shares That Have Not Vested ($)[2]
Nagesh K. Mahanthappa Ph.D., President and Chief Executive Officer[3]	515,576	1,041,464
Yung H. Chung M.D., Chief Medical Officer[4]	331,875	670,388
Elan Z. Ezickson, Chief Operating Officer & Head of Corporate Development[5]	381,252	770,129

(1) Stock award totals include shares of our restricted common stock received by the applicable named executive officer upon the exchange of incentive units of Scholar Rock, LLC in connection with the Reorganization.

(2) There was no public market for our common stock as of December 31, 2017. The fair market value of our common stock as of December 31, 2017, as determined by our board of directors, was $2.02 per share.

(3) Represents incentive units that were exchanged for restricted common stock in connection with the Reorganization from the following grants: (1) 617,000 units granted on November 12, 2014, which vest as follows: 20% vested on November 12, 2015 and the remainder vesting in equal quarterly installments for a period of 16 quarters thereafter, and (2) 403,160 units

granted on February 14, 2017, which vest in equal monthly installments over a period of four years beginning on August 12, 2016.

(4) Represents incentive units that were exchanged for restricted common stock in connection with the Reorganization from the following grants: (1) 492,000 share award granted on April 25, 2016, which shares vest as follows: 25% vested on February 25, 2017, with the remainder vesting in equal quarterly installments over a three year period thereafter, and (2) 98,000 share award granted on August 12, 2016, which shares vest as follows: 25% vested on February 25, 2017, with the remainder vesting in equal quarterly installments over a three year period thereafter.

(5) Represents incentive units that were exchanged for restricted common stock in connection with the Reorganization from the following grants: (1) 745,537 units granted on November 12, 2014, which vest as follows: 20% vested on August 1, 2015 and the remainder vesting in equal quarterly installments for a period of 16 quarters thereafter, and (2) 175,000 units granted on August 12, 2016, which vest in equal quarterly installments over a period of four years beginning on August 12, 2016.

Compensation Risk Assessment

We believe that although a portion of the compensation provided to our executive officers and other employees is performance-based, our executive compensation program does not encourage excessive or unnecessary risk taking. This is primarily due to the fact that our compensation programs are designed to encourage our executive officers and other employees to remain focused on both short-term and long-term strategic goals. As a result, we do not believe that our compensation programs are reasonably likely to have a material adverse effect on us.

Employee Benefit and Equity Compensation Plans

2018 Stock Option and Incentive Plan

Our 2018 Stock Option and Incentive Plan, or 2018 Plan, was adopted by our board of directors on , and approved by our stockholders on , and will become effective on the date immediately prior to the date on which the registration statement of which this prospectus is part is declared effective by the SEC. The 2018 Plan will replace our 2017 Stock Option and Grant Plan as our board of directors has determined not to make additional awards under that plan following the consummation of our initial public offering. The 2018 Plan allows the board of directors' compensation committee to make equity-based incentive awards to our officers, employees, directors and other key persons (including consultants).

We have initially reserved shares of our common stock for the issuance of awards under the 2018 Plan, the Initial Limit. The 2018 Plan provides that the number of shares reserved and available for issuance under the plan will automatically increase each January 1, beginning on January 1, 2019, by % of the outstanding number of shares of our common stock on the immediately preceding December 31 or such lesser number of shares as determined by our compensation committee, or the Annual Increase. These limits are subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

The shares we issue under the 2018 Plan will be authorized but unissued shares or shares we reacquire. The shares of common stock underlying any awards that are forfeited, cancelled, held back upon exercise or settlement of an award to satisfy the exercise price or tax withholding, reacquired by us prior to vesting, satisfied without the issuance of stock, expire or are otherwise terminated (other than by exercise) under the 2018 Plan and 2017 Plan will be added back to the shares of common stock available for issuance under the 2018 Plan.

The maximum aggregate number of shares that may be issued in the form of incentive stock options shall not exceed the Initial Limit cumulatively increased on January 1, 2019 and on each January 1 thereafter by the lesser of the Annual Increase for such year or shares of common stock.

The 2018 Plan will be administered by our compensation committee. Our compensation committee has full power to select, from among the individuals eligible for awards, the individuals to whom awards will be

granted, to make any combination of awards to participants, and to determine the specific terms and conditions of each award, subject to the provisions of the 2018 Plan. Persons eligible to participate in the 2018 Plan will be those full or part-time officers, employees, non-employee directors and other key persons (including consultants) as selected from time to time by our compensation committee in its discretion.

The 2018 Plan permits the granting of both options to purchase common stock intended to qualify as incentive stock options under Section 422 of the Code and options that do not so qualify. The option exercise price of each option will be determined by our compensation committee but may not be less than 100% of the fair market value of our common stock on the date of grant. The term of each option will be fixed by our compensation committee and may not exceed ten years from the date of grant. Our compensation committee will determine at what time or times each option may be exercised.

Our compensation committee may award stock appreciation rights subject to such conditions and restrictions as it may determine. Stock appreciation rights entitle the recipient to shares of common stock, or cash, equal to the value of the appreciation in our stock price over the exercise price. The exercise price may not be less than 100% of the fair market value of our common stock on the date of grant. The term of each stock appreciation right will be fixed by our compensation committee and may not exceed ten years from the date of grant. Our compensation committee will determine at what time or times each stock appreciation right may be exercised.

Our compensation committee may award restricted shares of common stock and restricted stock units to participants subject to such conditions and restrictions as it may determine. These conditions and restrictions may include the achievement of certain performance goals and/or continued employment with us through a specified vesting period. Our compensation committee may also grant shares of common stock that are free from any restrictions under the 2018 Plan. Unrestricted stock may be granted to participants in recognition of past services or for other valid consideration and may be issued in lieu of cash compensation due to such participant. Our compensation committee may grant dividend equivalent rights to participants that entitle the recipient to receive credits for dividends that would be paid if the recipient had held a specified number of shares of common stock.

Our compensation committee may grant cash bonuses under the 2018 Plan to participants, subject to the achievement of certain performance goals.

The 2018 Plan provides that upon the effectiveness of a "sale event," as defined in the 2018 Plan, an acquirer or successor entity may assume, continue or substitute outstanding awards under the 2018 Plan. To the extent that awards granted under the 2018 Plan are not assumed or continued or substituted by the successor entity, upon the effective time of the sale event, such awards under the 2018 Plan shall terminate. In the event of such termination, individuals holding options and stock appreciation rights will be permitted to exercise such options and stock appreciation rights (to the extent exercisable) within a specified period of time prior to the sale event. In addition, in connection with the termination of the 2018 Plan upon a sale event, we may make or provide for a cash payment to participants holding vested and exercisable options and stock appreciation rights equal to the difference between the per share cash consideration payable to stockholders in the sale event and the exercise price of the options or stock appreciation rights and we may make or provide for a cash payment to participants holding other vested awards.

Our board of directors may amend or discontinue the 2018 Plan and our compensation committee may amend or cancel outstanding awards for purposes of satisfying changes in law or any other lawful purpose, but no such action may adversely affect rights under an award without the holder's consent. Certain amendments to the 2018 Plan require the approval of our stockholders.

No awards may be granted under the 2018 Plan after the date that is ten years from the date of stockholder approval of the 2018 Plan. No awards under the 2018 Plan have been made prior to the date hereof.

2017 Stock Option and Incentive Plan

Our 2017 Stock Option and Incentive Plan, or 2017 Plan, was approved and adopted by our board of directors on December 22, 2017, and approved by our stockholders on that same day. Under the 2017 Plan we reserved for issuance an aggregate of 9,864,278 shares of our common stock, subject to adjustment in the event of a stock split, reverse stock split, stock dividend, recapitalization, reclassification of shares, reorganization, or other similar change in our capitalization.

The shares of common stock underlying awards that are forfeited, cancelled, terminated, reacquired prior to vesting, satisfied without the issuance of shares of common stock, or withheld to cover the exercise price or tax withholding are added back to the shares of common stock available for issuance under the 2017 Plan.

Our board of directors has acted as administrator of the 2017 Plan. The administrator has full power to select, from among the individuals eligible for awards, the individuals to whom awards will be granted, and to determine the specific terms and conditions of each award, subject to the provisions of the 2017 Plan. Persons eligible to participate in the 2017 Plan are those full or part-time employees, key persons, officers and directors of, and consultants to, our company as selected from time to time by the administrator in its discretion.

The 2017 Plan permits the granting of (1) options to purchase common stock intended to qualify as incentive stock options under Section 422 of the Internal Revenue Code of 1986, as amended, (2) options that do not so qualify, (3) restricted stock, (4) unrestricted stock, or (5) restricted stock units. For stock options, the administrator will determine the per share option exercise price and at what time or times each option may be exercised.

The 2017 Plan provides that upon the occurrence of a merger, reorganization, consolidation, liquidation, dissolution, sale of all or substantially all of the Company's assets, acquisition of a majority of our voting stock, or any other transaction that our board determines to be an acquisition of the business, or a Sale Event, all outstanding stock options shall terminate and all outstanding restricted stock and restricted stock units shall be forfeited if not assumed, continued, or substituted with comparable awards by the successor entity. In the event of such termination, holders will be permitted to exercise any vested options (including those that will become vested as a result of the Sale Event) or we may, in our sole discretion, cancel such options in exchange for a cash payment equal to the value payable per share of stock in the Sale Event multiplied by the number of shares subject to the vested portion of the option, less the aggregate exercise price. In the event that restricted stock is forfeited in connection with a Sale Event, the 2017 Plan provides that the holder shall be paid the lower of the purchase price paid for the restricted stock or the fair market value of the stock at the time of the sale event. The Company may, but is not required to, cancel the restricted stock in exchange for a price per share equal to the value payable per share of stock in the Sale Event.

Our board of directors may amend or discontinue the 2017 Plan at any time, subject to stockholder approval where such approval is required by applicable law. Our board of directors may also amend or cancel any outstanding award, provided that no amendment to an award may adversely affect a participant's rights without his or her consent.

The 2017 Plan will terminate automatically on December 22, 2027; however awards previously granted may extend beyond that date. As of April 25, 2018, 6,758,945 shares of common stock and restricted common stock and options to purchase 2,900,156 shares of common stock were outstanding under the 2017 Plan. Our board of directors has determined not to make any further awards under the 2017 Plan following the completion of this offering.

2018 Employee Stock Purchase Plan

On , our board of directors adopted the 2018 Employee Stock Purchase Plan, or 2018 ESPP, and on , our stockholders approved the 2018 ESPP. The 2018 ESPP is intended to qualify as an "employee stock purchase plan" within the meaning of Section 423 of the Code. The 2018 ESPP initially reserves and authorizes the issuance of up to a total of shares of common stock to participating employees. The ESPP provides that the number of shares reserved and available for issuance will automatically increase each January 1, beginning on January 1, 2019, by the lesser of (i) shares of common stock, (ii) % of the outstanding number of shares of our common stock on the immediately preceding December 31 or (iii) such lesser number of shares as determined by the 2018 ESPP administrator. The number of shares reserved under the 2018 ESPP is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

All employees whose customary employment is for more than 20 hours per week are eligible to participate in the ESPP. However, any participating employee who would own 5% or more of the total combined voting power or value of all classes of stock after an option were granted under the ESPP would not be eligible to purchase shares under the 2018 ESPP.

We will make one or more offerings each year to our employees to purchase shares under the ESPP. Offerings will usually begin on each January 1 and July 1 and will continue for six-month periods, referred to as offering periods. Each eligible employee may elect to participate in any offering by submitting an enrollment form at least 15 business days before the relevant offering date.

Each employee who is a participant in the 2018 ESPP may purchase shares by authorizing payroll deductions of up to % of his or her base compensation during an offering period. Unless the participating employee has previously withdrawn from the offering, his or her accumulated payroll deductions will be used to purchase shares on the last business day of the offering period at a price equal to 85% of the fair market value of the shares on the first business day or the last business day of the offering period, whichever is lower. Under applicable tax rules, an employee may purchase no more than $25,000 worth of shares of common stock, valued at the start of the purchase period, under the ESPP in any calendar year.

The accumulated payroll deductions of any employee who is not a participant on the last day of an offering period will be refunded. An employee's rights under the 2018 ESPP terminate upon voluntary withdrawal from the plan or when the employee ceases employment with us for any reason.

The 2018 ESPP may be terminated or amended by our board of directors at any time. An amendment that increases the number of shares of common stock authorized under the ESPP and certain other amendments require the approval of our stockholders.

Senior Executive Cash Incentive Bonus Plan

On , our board of directors adopted the Senior Executive Cash Incentive Bonus Plan, or the Bonus Plan. The Bonus Plan provides for cash bonus payments based upon the attainment of performance targets established by our compensation committee. The payment targets will be related to financial and operational measures or objectives with respect to our company, or Corporate Performance Goals, as well as individual performance objectives.

Our compensation committee may select Corporate Performance Goals from among the following: cash flow (including, but not limited to, operating cash flow and free cash flow); sales or revenue; corporate revenue; earnings before interest, taxes, depreciation and amortization; net income (loss) (either before or after interest, taxes, depreciation and/or amortization); changes in the market price of our common stock; economic value-added; development, clinical, regulatory or commercial milestones; acquisitions or strategic transactions, partnerships or joint ventures; operating income (loss); return on capital, assets, equity, or

investment; stockholder returns; return on sales; gross or net profit levels; productivity; expense efficiency; margins; operating efficiency; customer satisfaction; working capital; earnings (loss) per share of our common stock; sales or market shares; number of customers; operating income and/or other strategic, financial or operational objectives, any of which may be measured in absolute terms, as compared to any incremental increase, in terms of growth, as compared to results of a peer group, against the market as a whole, compared to applicable market indices and/or measured on a pre-tax or post-tax basis.

Each executive officer who is selected to participate in the Bonus Plan will have a target bonus opportunity set for each performance period. The bonus formulas will be adopted in each performance period by the compensation committee and communicated to each executive. The Corporate Performance Goals will be measured at the end of each performance period after our financial reports have been published or such other appropriate time as the compensation committee determines. If the Corporate Performance Goals and individual performance objectives are met, payments will be made as soon as practicable following the end of each performance period. The Bonus Plan also permits the compensation committee to approve additional bonuses to executive officers in its sole discretion.

401(k) Plan

We maintain the Scholar Rock Holding Corporation 401(k) Plan, a tax-qualified retirement plan for our employees. Our 401(k) plan is intended to qualify under Section 401(k) of the Code so that contributions to our 401(k) plan by employees or by us, and the investment earnings thereon, are not taxable to the employees until withdrawn from our 401(k) plan, and so that contributions by us, if any, will be deductible by us when made. Under our 401(k) plan, employees may elect to reduce their current compensation by up to the statutorily prescribed annual limit and to have the amount of such reduction contributed to our 401(k) plan. We have not historically made any discretionary matching contributions under our 401(k) plan but may do so in the future.

Limitations on Liability and Indemnification Matters

Our amended and restated certificate of incorporation 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 are not 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 amended and restated bylaws 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 amended and 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 amended and restated certificate of incorporation and amended 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 amended and restated certificate of incorporation, amended and restated 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 amended and restated certificate of incorporation and amended 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.

DIRECTOR COMPENSATION

The following table presents the total compensation for each person who served as a non-employee member of our board of directors and received compensation for such service during the year ended December 31, 2017. Other than as set forth in the table and described more fully below, we did not pay any compensation, make any additional equity awards or non-equity awards to, or pay any other compensation to any of the non-employee members of our board of directors in 2017. We reimburse non-employee members of our board of directors for reasonable travel and out-of-pocket expenses incurred in attending meetings of our board of directors and committees of our board of directors.

We also do not, and do not expect to, provide separate compensation to our directors who are also our employees, such as Dr. Mahanthappa, our President and Chief Executive Officer. Dr. Mahanthappa's compensation as an executive officer is reported above in "Executive Compensation — Summary Compensation Table."

Name	Fees Earned or Paid in Cash ($)	Stock Awards ($)(1)	Total ($)
David Hallal	75,000(3)	1,453,425(2)(3)	1,528,425
Katrine Bosley(4)	—	61,594(5)	61,594
Kristina Burow(6)	—	—	—
Jeffrey Flier(11)	—	27,440(7)	27,440
Michael Gilman(11)	—	11,780(8)	11,780
Amir Nashat, Sc.D.(9)	—	—	—
Timothy A. Springer, Ph.D.(11)	—	—	—

(1) As of December 31, 2017, our directors held the following number of shares of restricted common stock: David Hallal (730,890 shares), Jeffrey Flier (73,500 shares) and Michael Gilman (30,407 shares). Katrine Bosley, Kristina Burow, Amir Nashat, Sc.D. and Timothy A. Springer, Ph.D. did not hold any unvested shares as of December 31, 2017.

(2) $1,152,717 of this amount reflects the aggregate grant date fair value of stock awards granted during 2017 calculated in accordance with the provisions of Financial Accounting Standards Board Accounting Standard Codification Topic 718, *Compensation — Stock Compensation*. For information regarding assumptions underlying the valuation of stock awards, see Note 12 to our financial statements appearing at the end of this prospectus. The amount reported for Mr. Hallal includes the incremental fair value of $300,708 resulting from the exchange of incentive units of Scholar Rock, LLC into shares of restricted and unrestricted common stock of Scholar Rock Holding Corporation in connection with the Reorganization. Pursuant to the Hallal Letter Agreement, Mr. Hallal's equity is subject to (1) an additional six months of vesting if Mr. Hallal's service is terminated without "cause" (as defined in the Hallal Letter Agreement) and Mr. Hallal has provided at least six months of service as of that date and (2) full acceleration upon the consummation of a "sale event" (as defined in the Hallal Letter agreement).

(3) Pursuant to a letter agreement entered into between Mr. Hallal and the Company (the "Hallal Letter Agreement"), Mr. Hallal is eligible to receive an annual retainer equal to $150,000, payable on a quarterly basis. The amounts reported in this column represent Mr. Hallal's annual retainer, from July 1, 2017, the date he joined the Board.

(4) Ms. Bosley resigned from our board of directors on February 21, 2017. Any unvested portion of her stock award accelerated and became fully vested as of the date of her resignation.

(5) The amount reported for Ms. Bosley includes the incremental fair value of $11,780 resulting from the exchange of incentive units of Scholar Rock, LLC into shares of common stock of Scholar Rock Holding Corporation in connection with the Reorganization.

(6) Ms. Burow did not receive compensation in connection with serving as a director.

(7) The amount reported for Mr. Flier represents the incremental fair value resulting from of the exchange of incentive units of the Scholar Rock, LLC into shares of restricted and unrestricted common stock of Scholar Rock Holding Corporation in connection with the Reorganization.

(8) The amount reported for Mr. Gilman represents the incremental fair value resulting from the exchange of incentive units of Scholar Rock, LLC into shares our shares of restricted and unrestricted common stock of Scholar Rock Holding Corporation in connection with the Reorganization.

(9) Dr. Nashat did not receive compensation in connection with serving as a director.

(10) Dr. Springer did not receive compensation in connection with serving as a director.

(11) Drs. Flier, Gilman and Springer each received compensation in connection with consulting services provided by each individual to us. See the section titled "Certain Relationships and Related Party Transactions" for more details on the consulting services provided to us by Dr. Springer.

Non-Employee Director Compensation Policy

Our board of directors intends to adopt a non-employee director compensation policy, to be effective upon effectiveness of the registration statement of which this prospectus forms a part, that is designed to enable us to attract and retain, on a long-term basis, highly qualified non-employee directors. Under the policy, each director who is not an employee will be paid cash compensation from and after the completion of this offering, as set forth below:

	Member Annual Fee ($)	Chairman Additional Annual Fee ($)
Board of Directors		
Audit Committee		
Compensation Committee		
Nominating and Corporate Governance Committee		

In addition, each non-employee director serving on our board of directors upon completion of this offering and each non-employee director elected or appointed to our board of directors following the completing of this offering will be granted a one-time equity award of shares on the date of such director's election or appointment to the board of directors, which will vest annually over three years, subject to continued service through such vesting dates. On the date of each annual meeting of stockholders of our company, each non-employee director will be granted an annual equity award of shares, which will vest in full of the earlier to occur of the first anniversary of the date of grant or the next annual meeting, subject to continued service as a director through such vesting date.

CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

Other than the compensation agreements and other arrangements described under "Executive Compensation" and "Director Compensation" in this prospectus and the transactions described below, since December 31, 2014, there has not been and there is not currently proposed, any transaction or series of similar transactions to which we were, or will be, a party in which the amount involved exceeded, or will exceed, $120,000 and in which any director, executive officer, holder of 5% or more of any class of our capital stock or any member of the immediate family of, or entities affiliated with, any of the foregoing persons, had, or will have, a direct or indirect material interest.

Series A-3 Convertible Preferred Unit Financing

On February 12, 2015, we sold an aggregate of 5,579,709 Series A-3 convertible preferred units at a purchase price of $1.38 per unit, pursuant to a unit purchase agreement entered into with certain of our investors. Certain investors holding bridge units, originally issued in 2014, exchanged such bridge units for shares of our Series A-3 convertible preferred units. The following table summarizes purchases of our Series A-3 convertible preferred units by related persons:

5% Stockholder	Series A-3 Convertible Preferred Units[1] (#)	Total Purchase Price ($)
Entities Affiliated with Polaris Venture Partners VI, L.P.[2]	1,449,275	2,000,000
ARCH Venture Fund VIII, L.P.[3]	1,811,595	2,500,001
Timothy A. Springer, Ph.D.[4]	1,449,275	2,000,000
Entities Affiliated with EcoRl Capital Fund Qualified, L.P.[5]	513,043	707,999

(1) All outstanding Series A-3 convertible preferred units were exchanged for our shares of Series A-3 convertible preferred stock on a one-for-one basis on December 22, 2017 in connection with the Reorganization.

(2) Polaris Venture Partners VI, L.P. is an affiliate fund of Polaris Venture Partners Founders' Fund VI, L.P. and is a holder of 5% or more of our capital stock. The amount set forth in the table consists of (1) 1,369,259 Series A-3 convertible preferred units purchased by Polaris Venture Partners VI, L.P. and (2) 80,016 Series A-3 convertible preferred units purchased by Polaris Venture Partners Founders' Fund VI, L.P. Amir Nashat, Sc.D., a partner at Polaris Venture Partners VI, is a member of our board of directors.

(3) Kristina Burow is a managing director of ARCH Venture Fund VIII, L.P. ARCH Venture Fund VIII, L.P. is a holder of 5% or more of our capital stock.

(4) Timothy A. Springer, Ph.D. is holder of 5% or more of our capital stock and is a member of our board of directors.

(5) EcoRl Capital Fund Qualified, L.P. is an affiliate fund of EcoRl Capital Fund, L.P., and is a holder of 5% or more of our capital stock. The amount set forth in the table consists of (1) 318,076 Series A-3 convertible preferred units purchased by EcoRl Capital Fund Qualified, L.P. and (2) 194,967 Series A-3 convertible preferred units purchased by EcoRl Capital Fund, L.P.

Series A-4 Convertible Preferred Unit Financing

On September 21, 2015, we sold an aggregate of 3,906,738 Series A-4 convertible preferred units at a purchase price of $1.587 per unit, pursuant to a unit purchase agreement entered into with certain of our investors. The following table summarizes purchases of our Series A-4 convertible preferred units by related persons:

5% Stockholder	Series A-3 Convertible Preferred Units[1] (#)	Total Purchase Price ($)
Entities Affiliated with Polaris Venture Partners VI, L.P.[2]	787,649	1,249,999
ARCH Venture Fund VIII, L.P.[3]	1,575,299	2,500,000
Timothy A. Springer, Ph.D.[4]	787,649	1,249,999
Entities Affiliated with EcoRl Capital Fund Qualified, L.P.[5]	446,123	707,997

(1) All outstanding Series A-4 convertible preferred units were exchanged for our shares of Series A-4 convertible preferred stock on a one-for-one basis on December 22, 2017 in connection with the Reorganization.

(2) Polaris Venture Partners VI, L.P. is an affiliate fund of Polaris Venture Partners Founders' Fund VI, L.P. and is a holder of 5% or more of our capital stock. The amount set forth in the table consists of (1) 744,162 Series A-4 convertible preferred units purchased by Polaris Venture Partners VI, L.P. and (2) 43,487 Series A-4 convertible preferred units purchased by Polaris Venture Partners Founders' Fund VI, L.P. Dr. Nashat, Sc.D., a partner at Polaris Venture Partners VI, is a member of our board of directors.

(3) Kristina Burow is a managing director of ARCH Venture Fund VIII, L.P. ARCH Venture Fund VIII, L.P. is a holder of 5% or more of our capital stock.

(4) Timothy A. Springer, Ph.D. is holder of 5% or more of our capital stock and is a member of our board of directors.

(5) EcoRl Capital Fund Qualified, L.P. is an affiliate fund of EcoRl Capital Fund, L.P., and is a holder of 5% or more of capital stock. The amount set forth in the table consists of (1) 276,587 Series A-4 convertible preferred units purchased by EcoRl Capital Fund Qualified, L.P. and (2) 169,536 Series A-4 convertible preferred units purchased by EcoRl Capital Fund, L.P.

Series B Convertible Preferred Unit Financing

On December 17, 2015, and May 18, 2017, we sold an aggregate of 13,526,994 Series B convertible preferred units at a purchase price of $3.00 per unit, pursuant to a unit purchase agreement entered into with certain of our investors. The following table summarizes purchases of our Series B convertible preferred units by related persons:

5% Stockholder	Series B Convertible Preferred Units[1](#)	Total Purchase Price ($)
Entities Affiliated with Polaris Venture Partners VI, L.P.[2]	2,094,875	6,284,625
ARCH Venture Fund VIII, L.P.[3]	2,054,197	6,162,591
Timothy A. Springer, Ph.D.[4]	2,094,875	6,284,625
Entities Affiliated with EcoRl Capital Fund Qualified, L.P.[5]	976,068	2,928,204
Entities Affiliated with Fidelity Management and Research Company[6]	5,360,334	16,081,002

(1) All outstanding Series B convertible preferred units were exchanged for our shares of our Series B convertible preferred stock on a one-for-one basis on December 22, 2017 in connection with the Reorganization.

(2) Polaris Venture Partners VI, L.P. is an affiliate fund of Polaris Venture Partners Founders' Fund VI, L.P. and is a holder of 5% or more of our capital stock. The amount set forth in the table consists of (1) 1,979,216 Series B convertible preferred units purchased by Polaris Venture Partners VI, L.P. and (2) 115,659 Series B convertible preferred units purchased by Polaris Venture Partners Founders' Fund VI, L.P. Dr. Nashat, Sc.D., a partner at Polaris Venture Partners VI, is a member of our board of directors.

(3) Kristina Burow is a managing director of ARCH Venture Fund VIII, L.P. ARCH Venture Fund VIII, L.P. is a holder of 5% or more of our capital stock.

(4) Timothy A. Springer, Ph.D. controls TAS Partners, LLC. Dr. Springer is holder of 5% or more of our capital stock and a member of our board of directors. The amount set forth in the table consists of (1) 666,666 shares of Series B convertible preferred units purchased by TAS Partners, LLC and (2) 1,428,209 shares of Series B convertible preferred units purchased by Dr. Springer.

(5) EcoRl Capital Fund Qualified, L.P. is an affiliate fund of EcoRl Capital Fund, L.P. and is a holder of 5% or more of our capital stock. The amount set forth in the table consists of (1) 687,152 shares of Series B convertible preferred units purchased by EcoRl Capital Fund Qualified, L.P. and (2) 288,916 shares of Series B convertible preferred units purchased by EcoRl Capital Fund, L.P.

(6) Fidelity Management and Research Company is an affiliate of Fidelity Advisor Series VII: Biotechnology Fund and Fidelity Select Portfolios: Biotechnology Portfolio and is a beneficial owner of 5% or more of our capital stock. The amount set forth in the table consists of (1) 1,083,994 shares of Series B convertible preferred units held by Fidelity Advisor Series VII: Biotechnology Fund and (2) 4,276,340 shares of Series B convertible preferred units purchased by Fidelity Select Portfolios: Biotechnology Portfolio.

Series C Convertible Preferred Stock Financing

On December 22, 2017, immediately following the Reorganization, we sold an aggregate of 13,055,555 shares of our Series C convertible preferred stock at a purchase price of $3.60 per share, pursuant to stock purchase agreement entered into with certain of our investors. The following table summarizes purchases of our Series C convertible preferred stock by related persons:

5% Stockholder	Series C Preferred Stock (#)	Total Purchase Price ($)
Entities Affiliated with Polaris Venture Partners VI, L.P.(1)	855,391	3,079,408
ARCH Venture Fund VIII, L.P.(2)	838,780	3,019,608
Timothy A. Springer, Ph.D.(3)	855,391	3,079,408
Entities Affiliated with EcoRl Capital Fund Qualified, L.P.(4)	415,343	1,495,235
Entities Affiliated with Fidelity Management and Research Company(5)	2,777,778	10,000,001
Artal International SCA(6)	5,555,556	20,000,002

(1) Polaris Venture Partners VI, L.P. is an affiliate fund of Polaris Venture Partners Founders' Fund VI, L.P. and is a holder of 5% or more of our capital stock. The amount set forth in the table consists of (1) 808,166 shares of Series C convertible preferred stock purchased by Polaris Venture Partners VI, L.P. and (2) 47,225 shares of Series C convertible preferred stock purchased by Polaris Venture Partners Founders' Fund VI, L.P. Dr. Amir Nashat, Sc.D., a partner at Polaris Venture Partners VI, is a member of our board of directors.

(2) Kristina Burow is a managing director of ARCH Venture Fund VIII, L.P. ARCH Venture Fund VIII, L.P. is a holder of 5% or more of our capital stock.

(3) Timothy A. Springer, Ph.D. is holder of 5% or more of our capital stock and a member of our board of directors

(4) EcoRl Capital Fund Qualified, L.P. is an affiliate fund of EcoRl Capital Fund, L.P., and is a holder of 5% or more of our capital stock. The amount set forth in the table consists of (1) 328,036 shares of Series C convertible preferred stock held by EcoRl Capital Fund Qualified, L.P. and (2) 87,307 shares of Series C convertible preferred stock held by EcoRl Capital Fund, L.P.

(5) Fidelity Management and Research Company is an affiliate of Fidelity Mt. Vernon Street Trust: Fidelity Series Growth Company Fund State Street Bank & Trust, Fidelity Growth Company Commingled Pool and Fidelity Mt. Vernon Street Trust: Fidelity Series Growth Company Fund BNY Mellon, and is a beneficial owner of 5% or more of our capital stock. The amount set forth in the table consists of (1) 267,635 shares of Series C convertible preferred stock purchased by Fidelity Mt. Vernon Street Trust: Fidelity Series Growth Company Fund State Street Bank & Trust, (2) 1,166,154 shares of Series C convertible preferred stock purchased by Fidelity Growth Company Commingled Pool, and (3) 1,343,989 shares of Series C convertible preferred stock purchased by Fidelity Mt. Vernon Street Trust: Fidelity Series Growth Company Fund BNY Mellon.

(6) Artal International SCA is a holder of 5% or more of our capital stock.

Consulting Agreements

The Company entered into a consulting agreement on October 10, 2012 with Timothy A. Springer, Ph.D. to provide services related to the advancement of the research and development platform of the company.

The consulting arrangement is on a fixed-fee basis, paid quarterly. The initial contract term was four years and terminated on October 10, 2016. The contract was extended for an additional four year period. The Company incurred $80,000 of consulting expense related to this contract, in each year, for the years ended December 31, 2016 and 2017.

Indemnification Agreements

In connection with this offering, we intend to enter into agreements to indemnify our directors and executive officers. These agreements will, among other things, require us to indemnify these individuals for certain expenses (including attorneys' fees), judgments, fines and settlement amounts reasonably incurred by such person in any action or proceeding, including any action by or in our right, on account of any services undertaken by such person on behalf of our company or that person's status as a member of our board of directors to the maximum extent allowed under Delaware law.

Investors' Rights Agreement

In connection with our Series C convertible preferred stock financing, we entered into an investors' rights agreement with certain of our stockholders, including related persons. The investors' rights agreement among other things:

- grants such stockholders certain registration rights with respect to shares of our common stock, including shares of common stock issued or issuable upon conversion of our convertible preferred stock;
- obligates us to deliver periodic financial statements to any stockholder who holds at least 1,000,000 shares of our convertible preferred stock, which we refer to as a "Majority Investors;"
- grants a right of first offer with respect to sales of our shares by us, subject to specified exclusions (which exclusions include the sale of the shares in connection with this offering), to qualified holders; and
- requires us to reimburse certain legal expenses of the investors in connection with future financings or a liquidation event.

For more information regarding the registration rights provided in this agreement, please refer to the section of this prospectus titled "Description of Capital Stock — Registration Rights."

Certain provisions of this agreement, including the covenants described above, will terminate automatically upon completion of this offering. This is not a complete description of the investors' rights agreement and is qualified by the full text of the investors' rights agreement filed as an exhibit to the registration statement of which this prospectus is a part.

Voting Agreement

In connection with our Series C convertible preferred stock financing, we entered into a voting agreement with certain of our stockholders, including related persons. The voting agreement among other things: provides the terms for the voting of shares with respect to the constituency of our board of directors.

This voting agreement will terminate automatically upon completion of this offering. This is not a complete description of the voting agreement and is qualified by the full text of the voting agreement filed as an exhibit to the registration statement of which this prospectus is a part.

Right of First Refusal and Co-Sale Agreement

In connection with our Series C convertible preferred stock financing, we entered into a right of first refusal and co-sale agreement with certain of our stockholders, including related persons. The right of first refusal and co-sale agreement, among other things:

- grants our investors certain rights of first refusal and co-sale with respect to proposed transfers of our securities by certain stockholders; and
- grants us certain rights of first refusal with respect to proposed transfers of our securities by certain stockholders.

The right of first refusal and co-sale agreement will terminate automatically upon completion of this offering. This is not a complete description of the right of first refusal and co-sale agreement and is qualified by the full text of right of first refusal and co-sale agreement filed as an exhibit to the registration statement of which this prospectus is a part.

Policies for Approval of Related Party Transactions

Our board of directors reviews and approves transactions with directors, officers and holders of five percent or more of our voting securities and their affiliates, each a related party. Prior to this offering, the material facts as to the related party's relationship or interest in the transaction are disclosed to our board of directors prior to their consideration of such transaction, and the transaction is not considered approved by our board of directors unless a majority of the directors who are not interested in the transaction approve the transaction. Further, when stockholders are entitled to vote on a transaction with a related party, the material facts of the related party's relationship or interest in the transaction are disclosed to the stockholders, who must approve the transaction in good faith.

In connection with this offering, we expect to adopt a written related party transactions policy that such transactions must be approved by our audit committee. This policy will become effective on the date on which the registration statement of which this prospectus is part is declared effective by the SEC. Pursuant to this policy, the audit committee has the primary responsibility for reviewing and approving or disapproving "related party transactions," which are transactions between us and related persons in which the aggregate amount involved exceeds or may be expected to exceed $120,000 and in which a related person has or will have a direct or indirect material interest. For purposes of this policy, a related person will be defined as a director, executive officer, nominee for director, or greater than 5% beneficial owner of our common stock, in each case since the beginning of the most recently completed year, and their immediate family members.

PRINCIPAL STOCKHOLDERS

The following table sets forth certain information known to us regarding beneficial ownership of our capital stock as of December 31, 2017, as adjusted to reflect the sale of common stock offered by us in this offering, for:

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

Beneficial ownership is determined in accordance with the rules of the SEC and generally includes voting or investment power with respect to securities. Under those rules, beneficial ownership includes any shares as to which the individual or entity has sole or shared voting power or investment power, and includes securities that the individual or entity has the right to acquire, such as through the exercise of stock options, within 60 days of December 31, 2017. Except as noted by footnote, and subject to community property laws where applicable, we believe, based on the information provided to us, that the persons and entities named in the table below have sole voting and investment power with respect to all common stock shown as beneficially owned by them.

The percentage of beneficial ownership prior to this offering in the table below is based on shares of common stock deemed to be outstanding as of December 31, 2017, assuming the conversion of all outstanding shares of our convertible preferred stock upon the completion of this offering into an aggregate of 43,135,911 shares of common stock upon the completion of this offering, and the percentage of beneficial ownership after this offering in the table below is based on shares of common stock assumed to be outstanding after the completion of the offering.

Name of Beneficial Owner[1]	Number of Shares Beneficially Owned Prior to Offering	Percentage of Shares Beneficially Owned	
		Before Offering	After Offering
5% or greater stockholders:			
Timothy A. Springer, Ph.D.[2]	9,928,982	18.23%	
Affiliates of Fidelity Management and Research Company[3]	8,138,112	14.94	
Entities Affiliated with Polaris Venture Partners VI, L.P[4]	7,678,981	14.10	
ARCH Venture Fund VIII, L.P.[5]	7,529,869	13.82	
Artal International SCA[6]	5,555,556	10.20	
EcoRl Capital Fund Qualified, LP[7]	2,842,244	5.22	
Named executive officers and directors:			
Nagesh K. Mahanthappa, Ph.D.[8]	1,770,160	3.25	
Yung H. Chyung, M.D.[9]	590,000	1.08	
Elan Z. Ezickson[10]	920,537	1.69	
David Hallal[11]	835,302	1.53	
Kristina Burow[12]	7,529,869	13.82	
Jeffrey S. Flier, M.D.[13]	125,000	*	
Michael Gilman, Ph.D.[14]	125,500	*	
Amir Nashat, Sc.D.[15]	7,678,981	14.10	
Timothy A. Springer, Ph.D.[2]	9,928,982	18.23	
All executive officers and directors as a group (10 persons)	29,954,331	54.99%	

* Represents less than 1%.

(1) Address is c/o Scholar Rock Holding Corporation, 620 Memorial Dr., 2nd Floor, Cambridge, MA, unless otherwise indicated.

(2) Consists of (i) 2,250,000 shares of common stock, (ii) 1,000,000 shares of common stock issuable upon conversion of shares of Series A-1 convertible preferred stock, (iii) 1,491,792 shares of common stock issuable upon conversion of shares of Series A-2 convertible preferred stock, (iv) 1,449,275 shares of common stock issuable upon conversion of shares of Series A-3 convertible preferred stock, (v) 787,649 shares of common stock issuable upon conversion of shares of Series A-4 convertible preferred stock held by TAS Partners LLC, (vi) 2,094,875 shares of common stock issuable upon conversion of shares of Series B convertible preferred stock, and (vii) 855,391 shares of common stock issuable upon conversion of shares of Series C convertible preferred stock.

(3) Consists of (i) 1,083,994 shares of common stock issuable upon conversion of shares of Series B convertible preferred stock held by Fidelity Advisor Series VII: Biotechnology Fund, (ii) 4,276,340 shares of common stock issuable upon conversion of shares of Series B convertible preferred stock held by Fidelity Select Portfolios: Biotechnology Portfolio, (iii) 267,635 shares of common stock issuable upon conversion of shares of Series C convertible preferred stock held by Fidelity Mt. Vernon Street Trust: Fidelity Series Growth Company Fund State Street Bank & Trust, (iv) 1,166,154 shares of common stock issuable upon conversion of shares of Series C convertible preferred stock held by Fidelity Growth Company Commingled Pool, and (v) 1,343,989 shares of common stock issuable upon conversion of shares of Series C convertible preferred stock held by Fidelity Mt. Vernon Street Trust: Fidelity Growth Company Fund. The Funds that hold shares of our capital stock are managed by direct or indirect subsidiaries of Fidelity Management & Research, LLC, or FMR LLC. Edward C. Johnson 3rd is a Director and the Chairman of FMR LLC and Abigail P. Johnson is a Director, the Vice Chairman and the President of FMR LLC. Members of the family of Edward C. Johnson 3rd, including Abigail P. Johnson, are the predominant owners, directly or through trusts, of Series B voting common shares of FMR LLC, representing 49% of the voting power of FMR LLC. The Johnson family group and all other Series B shareholders have entered into a shareholders' voting agreement under which all Series B voting common shares will be voted in accordance with the majority vote of Series B voting common shares. Accordingly, through their ownership of voting common shares and the execution of the shareholders' voting agreement, members of the Johnson family may be deemed, under the Investment Company Act of 1940, to form a controlling group with respect to FMR LLC. Neither FMR LLC nor Edward C. Johnson 3rd nor Abigail P. Johnson has the sole power to vote or direct the voting of the shares owned directly by the various investment companies registered under the Investment Company Act, or Fidelity Funds, advised by Fidelity Management & Research Company, or FMR Co, a wholly owned subsidiary of FMR LLC, which power resides with the Fidelity Funds' Boards of Trustees. Fidelity Management & Research Company carries out the voting of the shares under written guidelines established by the Fidelity Funds' Boards of Trustees.

(4) Consists of (i) 944,789 shares of common stock issuable upon conversion of shares of Series A-1 convertible preferred stock held by Polaris Venture Partners VI, L.P., (ii) 55,211 shares of common stock issuable upon conversion of shares of Series A-1 convertible preferred stock held by Polaris Venture Partners Founders Fund VI, L.P., (iii) 1,409,429 shares of common stock issuable upon conversion of shares of Series A-2 convertible preferred stock held by Polaris Venture Partners VI, L.P., (iv) 82,363 shares of common stock issuable upon conversion of shares of Series A-2 convertible preferred stock held by Polaris Venture Partners Founders Fund VI, L.P., (v) 1,369,259 shares of common stock issuable upon conversion of shares of Series A-3 convertible preferred stock held by Polaris Venture Partners VI, L.P., (vi) 80,015 shares of common stock issuable upon conversion of shares of Series A-3 convertible preferred stock held by Polaris Venture Partners Founders Fund VI, L.P., (vii) 744,162 shares of common stock issuable upon conversion of shares of Series A-4 convertible preferred stock held by Polaris Venture Partners VI, L.P., (viii) 43,487 shares of common stock issuable upon conversion of shares of Series A-4 convertible preferred stock held by Polaris Venture Partners Founders Fund VI, L.P., (ix) 1,979,216 shares of common stock issuable upon conversion of shares of Series B convertible preferred stock held by Polaris Venture Partners VI, L.P., (x) 115,659 shares of common stock issuable upon conversion of shares of Series B convertible preferred stock held by Polaris Venture Partners Founders Fund VI, L.P., (xi) 808,166 shares of common stock issuable upon conversion of shares of Series C convertible preferred stock held by Polaris Venture Partners VI, L.P., and (xii) 47,225 shares of common stock issuable upon conversion of shares of Series C convertible preferred stock held by Polaris Venture Partners Founders Fund VI, L.P. Polaris Venture Management Co. VI, L.L.C. is a general partner of each of the funds that hold shares of our capital stock and may be deemed to have the sole voting and dispositive power over the shares held by these funds. Dr. Amir Nashat, a member of our board of directors, is a Managing Partner of Polaris Partners and may be deemed to share voting and dispositive power over the shares held by these funds. The address of the Polaris funds is Polaris Funds is One Marina Park Drive, 10th Floor, Boston, Massachusetts 02210.

(5) Consists of (i) 1,249,999 shares of common stock issuable upon conversion of shares of Series A-2 convertible preferred stock, (ii) 1,811,594 shares of common stock issuable upon conversion of shares of Series A-3 convertible preferred stock, (iii) 1,575,299 shares of common stock issuable upon conversion of shares of Series A-4 convertible preferred stock, (iv) 2,054,197 shares of common stock issuable upon conversion of shares of Series B convertible preferred stock, and (v) 838,780 shares of common stock issuable upon conversion of shares of Series C convertible preferred stock. The sole general partner of this fund is ARCH Venture Partners VIII, L.P. or ARCH Partners VIII, which may be deemed to beneficially own the shares held by this fund. The sole general partner of ARCH Partners VIII is ARCH Venture Partners VIII, LLC or ARCH VIII LLC, which may be deemed to beneficially own the shares held by this fund. ARCH Partners VIII and ARCH VIII LLC disclaim beneficial ownership of such shares, except to the extent of any pecuniary interest therein. The managing

directors of ARCH VIII LLC are Keith L. Crandell, Clinton Bybee and Robert Nelsen, and they may be deemed to beneficially own the shares held by this fund. Messrs. Crandell, Bybee and Nelsen disclaim beneficial ownership of such shares, except to the extent of any pecuniary interest therein.

(6) Consists of 5,555,556 shares of Series C convertible preferred stock.

(7) Consists of (i) 186,843 shares of common stock issuable upon conversion of shares of Series A-2 convertible preferred stock held by EcoRl Capital Fund, LP, (ii) 304,823 shares of common stock issuable upon conversion of shares of Series A-2 convertible preferred stock held by EcoRl Capital Fund Qualified, LP, (iii) 194,967 shares of common stock issuable upon conversion of shares of Series A-3 convertible preferred stock held by EcoRl Capital Fund, LP, (iv) 318,076 shares of common stock issuable upon conversion of shares of Series A-3 convertible preferred stock held by EcoRl Capital Fund Qualified, LP, (v) 169,536 shares of common stock issuable upon conversion of shares of Series A-4 convertible preferred stock held by EcoRl Capital Fund, LP, (vi) 276,588 shares of common stock issuable upon conversion of shares of Series A-4 convertible preferred stock held by EcoRl Capital Fund Qualified, LP, (vii) 288,916 shares of common stock issuable upon conversion of shares of Series B convertible preferred stock held by EcoRl Capital Fund, LP, (viii) 687,152 shares of common stock issuable upon conversion of shares of Series B convertible preferred stock held by EcoRl Capital Fund Qualified, LP, (ix) 87,307 shares of common stock issuable upon conversion of shares of Series C convertible preferred stock held by EcoRl Capital Fund, LP, and (x) 328,036 shares of common stock issuable upon conversion of shares of Series C convertible preferred stock held by EcoRl Capital Fund Qualified, LP. EcoR1 Capital, LLC, as the sole general partner of EcoR1 Capital Fund, L.P. and EcoR1 Capital Fund Qualified, L.P., may be deemed to beneficially own the shares held of record by EcoR1 Capital Fund, L.P. and EcoR1 Capital Fund Qualified, L.P. The address of the EcoR1 funds is 409 Illinois Street, San Francisco, CA 94158.

(8) Consists of 1,770,160 shares of common stock and restricted common stock.

(9) Consists of 590,000 shares of common stock and restricted common stock.

(10) Consists of 920,537 shares of common stock and restricted common stock.

(11) Consists of 835,302 shares of common stock and restricted common stock.

(12) Consists of the shares described in footnote (5) above. Kristina Burow one of our directors, is a managing director at ARCH Venture Partners. Ms. Burow owns an interest in ARCH Partners VIII but does not have voting or investment control over the shares held by the fund, and disclaims beneficial ownership of such shares, except to the extent of any pecuniary interest therein. The address of the fund is 8755 West Higgins Road, Suite 1025, Chicago, Illinois 60631.

(13) Consists of 125,000 shares of common stock and restricted common stock.

(14) Consists of 125,500 shares of common stock and restricted common stock.

(15) Consists of the shares described in footnote (4) above. Dr. Nashat is a partner at Polaris Venture Partners VI, L.P. and shares voting and investment control with respect to these shares. Dr. Nashat disclaims beneficial ownership of all shares held by Polaris Venture Partners VI, L.P. except to the extent of any pecuniary interest therein.

DESCRIPTION OF CAPITAL STOCK

The following descriptions are summaries of the material terms of our amended and restated certificate of incorporation, which will be effective upon the completion of this offering and amended and restated by-laws, which will be effective upon the effectiveness of the registration statement of which this prospectus is a part. The descriptions of the common stock and convertible preferred stock give effect to changes to our capital structure that will occur immediately prior to the completion of this offering.

General

Upon completion of this offering, our authorized capital stock will consist of shares of common stock, par value $0.001 per share, and shares of convertible preferred stock, par value $0.001 per share, all of which shares of convertible preferred stock will be undesignated.

As of , 2018, shares of our common stock and shares of convertible preferred stock were outstanding and held by stockholders of record. This amount does not take into account the conversion of all outstanding shares of our convertible preferred stock into common stock upon the completion of this offering.

Common Stock

The holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of the stockholders. The holders of our common stock do not have any cumulative voting rights. Holders of our common stock are entitled to receive ratably any dividends declared by our board of directors out of funds legally available for that purpose, subject to any preferential dividend rights of any outstanding convertible preferred stock. Our common stock has no preemptive rights, conversion rights or other subscription rights or redemption or sinking fund provisions.

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in all assets remaining after payment of all debts and other liabilities and any liquidation preference of any outstanding convertible preferred stock. The shares to be issued by us in this offering will be, when issued and paid for, validly issued, fully paid and non-assessable.

Preferred Stock

Upon the completion of this offering, all outstanding shares of our convertible preferred stock will be converted into shares of our common stock. Upon the consummation of this offering, our board of directors will have the authority, without further action by our stockholders, to issue up to shares of convertible preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, terms of redemption, liquidation preferences, sinking fund terms and the number of shares constituting, or the designation of, such series, any or all of which may be greater than the rights of common stock. The issuance of our convertible preferred stock could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend payments and payments upon our liquidation. In addition, the issuance of convertible preferred stock could have the effect of delaying, deferring or preventing a change in control of our company or other corporate action. Immediately after consummation of this offering, no shares of convertible preferred stock will be outstanding, and we have no present plan to issue any shares of convertible preferred stock.

Registration Rights

Upon the completion of this offering, the holders of 43,135,911 shares of our common stock, including those issuable upon the conversion of convertible preferred stock will be entitled to rights with respect to the registration of these securities under the Securities Act. These rights are provided under the terms of an

investors' rights agreement between us and holders of our convertible preferred stock. The investors' rights agreement includes demand registration rights, short-form registration rights and piggyback registration rights. All fees, costs and expenses of underwritten registrations under this agreement will be borne by us and all selling expenses, including underwriting discounts and selling commissions, will be borne by the holders of the shares being registered.

Demand Registration Rights

Beginning 180 days after the effective date of this registration statement, the holders of 43,135,911 shares of our common stock, including those issuable upon the conversion of convertible preferred stock upon completion of this offering, are entitled to demand registration rights. Under the terms of the investors' rights agreement, we will be required, upon the written request of holders of at least 25% of these securities that would result in an aggregate offering price of at least $3.0 million, to file a registration statement and use best efforts to effect the registration of all or a portion of these shares for public resale. We are required to effect only two registrations pursuant to this provision of the investors' rights agreement.

Short-Form Registration Rights

Pursuant to the investors' rights agreement, if we are eligible to file a registration statement on Form S-3, upon the written request of at least 10% of these holders to sell registrable securities at an aggregate price of at least $1.0 million, we will be required to use commercially reasonable efforts to effect a registration of such shares. We are required to effect only two registrations in any twelve month period pursuant to this provision of the investors' rights agreement. The right to have such shares registered on Form S-3 is further subject to other specified conditions and limitations.

Piggyback Registration Rights

Pursuant to the investors' rights agreement, if we register any of our securities either for our own account or for the account of other security holders, the holders of these shares are entitled to include their shares in the registration. Subject to certain exceptions contained in the investors' rights agreement, we and the underwriters may limit the number of shares included in the underwritten offering to the number of shares which we and the underwriters determine in our sole discretion will not jeopardize the success of the offering.

Indemnification

Our investors' rights agreement contains customary cross-indemnification provisions, under which we are obligated to indemnify holders of registrable securities in the event of material misstatements or omissions in the registration statement attributable to us, and they are obligated to indemnify us for material misstatements or omissions attributable to them.

Expiration of Registration Rights

The demand registration rights and short form registration rights granted under the investors' rights agreement will terminate on the fifth anniversary of the completion of this offering or at such time after this offering when the holders' shares may be sold without restriction pursuant to Rule 144 within a three month period.

Anti-Takeover Effects of Our Amended and Restated Certificate of Incorporation and Amended and Restated By-laws and Delaware Law

Our amended and certificate of incorporation and amended and restated by-laws include a number of provisions that may have the effect of delaying, deferring or preventing another party from acquiring control of us and encouraging persons considering unsolicited tender offers or other unilateral takeover proposals to negotiate with our board of directors rather than pursue non-negotiated takeover attempts. These provisions include the items described below.

Board Composition and Filling Vacancies

Our amended and restated certificate of incorporation provides for the division of our board of directors into three classes serving staggered three-year terms, with one class being elected each year. Our amended and restated certificate of incorporation also provides that directors may be removed only for cause and then only by the affirmative vote of the holders of two-thirds or more of the shares then entitled to vote at an election of directors. Furthermore, any vacancy on our board of directors, however occurring, including a vacancy resulting from an increase in the size of our board, may only be filled by the affirmative vote of a majority of our directors then in office even if less than a quorum. The classification of directors, together with the limitations on removal of directors and treatment of vacancies, has the effect of making it more difficult for stockholders to change the composition of our board of directors.

No Written Consent of Stockholders

Our amended and restated certificate of incorporation provides that all stockholder actions are required to be taken by a vote of the stockholders at an annual or special meeting, and that stockholders may not take any action by written consent in lieu of a meeting. This limit may lengthen the amount of time required to take stockholder actions and would prevent the amendment of our amended and restated bylaws or removal of directors by our stockholders without holding a meeting of stockholders.

Meetings of Stockholders

Our amended and restated certificate of incorporation and amended and restated by-laws provide that only a majority of the members of our board of directors then in office may call special meetings of stockholders and only those matters set forth in the notice of the special meeting may be considered or acted upon at a special meeting of stockholders. Our amended and restated by-laws limit the business that may be conducted at an annual meeting of stockholders to those matters properly brought before the meeting.

Advance Notice Requirements

Our amended and restated by-laws establish advance notice procedures with regard to stockholder proposals relating to the nomination of candidates for election as directors or new business to be brought before meetings of our stockholders. These procedures provide that notice of stockholder proposals must be timely given in writing to our corporate secretary prior to the meeting at which the action is to be taken. Generally, to be timely, notice must be received at our principal executive offices not less than 90 days nor more than 120 days prior to the first anniversary date of the annual meeting for the preceding year. Our by-laws specify the requirements as to form and content of all stockholders' notices. These requirements may preclude stockholders from bringing matters before the stockholders at an annual or special meeting.

Amendment to Amended and Restated Certificate of Incorporation and Amended and Restated By-laws

Any amendment of our amended and restated certificate of incorporation must first be approved by a majority of our board of directors, and if required by law or our amended and restated certificate of incorporation, must thereafter be approved by a majority of the outstanding shares entitled to vote on the amendment and a majority of the outstanding shares of each class entitled to vote thereon as a class, except that the amendment of the provisions relating to stockholder action, board composition, limitation of liability and the amendment of our by-laws and certificate of incorporation must be approved by not less than two-thirds of the outstanding shares entitled to vote on the amendment, and not less than two-thirds of the outstanding shares of each class entitled to vote thereon as a class. Our amended and restated by-laws may be amended by the affirmative vote of a majority of the directors then in office, subject to any limitations set forth in the amended and restated by-laws, and may also be amended by the affirmative vote of at least two-thirds of the outstanding shares entitled to vote on the amendment, or, if our board of directors recommends that the stockholders approve the amendment, by the affirmative vote of the majority of the outstanding shares entitled to vote on the amendment, in each case voting together as a single class.

Undesignated Preferred Stock

Our amended and restated certificate of incorporation provides for authorized shares of convertible preferred stock. The existence of authorized but unissued shares of convertible preferred stock may enable

our board of directors to discourage an attempt to obtain control of us by means of a merger, tender offer, proxy contest or otherwise. For example, if in the due exercise of its fiduciary obligations, our board of directors were to determine that a takeover proposal is not in the best interests of our stockholders, our board of directors could cause shares of convertible preferred stock to be issued without stockholder approval in one or more private offerings or other transactions that might dilute the voting or other rights of the proposed acquirer or insurgent stockholder or stockholder group. In this regard, our amended and restated certificate of incorporation grants our board of directors broad power to establish the rights and preferences of authorized and unissued shares of convertible preferred stock. The issuance of shares of convertible preferred stock could decrease the amount of earnings and assets available for distribution to holders of shares of common stock. The issuance may also adversely affect the rights and powers, including voting rights, of these holders and may have the effect of delaying, deterring or preventing a change in control of us.

Choice of Forum

Our amended and restated certificate of incorporation provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for: (1) any derivative action or proceeding brought on our behalf; (2) any action asserting a claim of breach of a fiduciary duty or other wrongdoing by any of our directors, officers, employees or agents to us or our stockholders; (3) any action asserting a claim against us arising pursuant to any provision of the General Corporation Law of the State of Delaware or our certificate of incorporation or by-laws; (4) any action to interpret, apply, enforce or determine the validity of our certificate of incorporation or by-laws; or (5) any action asserting a claim governed by the internal affairs doctrine. Our amended and restated certificate of incorporation also provides that any person or entity purchasing or otherwise acquiring any interest in shares of our capital stock will be deemed to have notice of and to have consented to this choice of forum provision. It is possible that a court of law could rule that the choice of forum provision contained in our amended and restated certificate of incorporation is inapplicable or unenforceable if it is challenged in a proceeding or otherwise.

In addition, our amended and restated certificate of incorporation that will become effective upon the closing of this offering will contain a provision by virtue of which, unless we consent in writing to the selection of an alternative forum, the United States District Court for the District of Massachusetts will be the exclusive forum for any private action asserting violations by us or any of our directors or officers of the Securities Act, or the rules and regulations promulgated thereunder, and of all suits in equity and actions at law brought to enforce any liability or duty created by those statutes or the rules and regulations under such statutes. If any action the subject matter of which is within the scope of the preceding sentence is filed in a court other than the United States District Court for the District of Massachusetts, the plaintiff or plaintiffs shall be deemed by this provision of our amended and restated certificate of incorporation (i) to have consented to removal of the action by us to the United States District Court for the District of Massachusetts, in the case of an action filed in a state court, and (ii) to have consented to transfer of the action to the United States District Court for the District of Massachusetts.

Section 203 of the Delaware General Corporation Law

Upon completion of this offering, we will be subject to the provisions of Section 203 of the Delaware General Corporation Law. In general, Section 203 prohibits a publicly held Delaware corporation from engaging in a "business combination" with an "interested stockholder" for a three-year period following the time that this stockholder becomes an interested stockholder, unless the business combination is approved in a prescribed manner. Under Section 203, a business combination between a corporation and an interested stockholder is prohibited unless it satisfies one of the following conditions:

- before the stockholder became interested, our board of directors approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;

- upon consummation of 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, shares owned by persons who are directors and also officers, and employee stock plans, in some instances, but not the outstanding voting stock owned by the interested stockholder; or

- at or after the time the stockholder became interested, the business combination was approved by our board of directors and authorized at an annual or special meeting of the stockholders by the affirmative vote of at least two-thirds of the outstanding voting stock which is not owned by the interested stockholder.

Section 203 defines a business combination to include:

- any merger or consolidation involving the corporation and the interested stockholder;

- any sale, transfer, lease, pledge or other disposition involving the interested stockholder of 10% or more of the assets of the corporation;

- subject to exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;

- subject to exceptions, any transaction involving the corporation that has the effect of increasing the proportionate share of the stock of any class or series of the corporation beneficially owned by the interested stockholder; and

- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits provided by or through the corporation.

In general, Section 203 defines an interested stockholder as any entity or person beneficially owning 15% or more of the outstanding voting stock of the corporation and any entity or person affiliated with or controlling or controlled by the entity or person.

Nasdaq Global Market Listing

We have applied to list our common stock on The Nasdaq Global Market under the trading symbol "SRRK."

Transfer Agent and Registrar

The transfer agent and registrar for our common stock will be .

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our shares. Future sales of our common stock in the public market, or the availability of such shares for sale in the public market, could adversely affect market prices prevailing from time to time. As described below, only a limited number of shares will be available for sale shortly after this offering due to contractual and legal restrictions on resale. Nevertheless, sales of our common stock in the public market after such restrictions lapse, or the perception that those sales may occur, could adversely affect the prevailing market price at such time and our ability to raise equity capital in the future.

Based on the number of shares outstanding as of , 2018 upon the completion of this offering, shares of our common stock will be outstanding. Of the outstanding shares, all of the shares sold in this offering will be freely tradable, except that any shares held by our affiliates, as that term is defined in Rule 144 under the Securities Act, may only be sold in compliance with the limitations described below. All remaining shares of common stock held by existing stockholders immediately prior to the completion of this offering will be "restricted securities" as such term is defined in Rule 144. These restricted securities were issued and sold by us, or will be issued and sold by us, in private transactions and are eligible for public sale only if registered under the Securities Act or if they qualify for an exemption from registration under the Securities Act, including the exemptions provided by Rule 144 or Rule 701, summarized below.

Rule 144

In general, a person who has beneficially owned restricted stock for at least six months would be entitled to sell their securities provided that (i) such person is not deemed to have been one of our affiliates at the time of, or at any time during the 90 days preceding, a sale and (ii) we are subject to the Securities Exchange Act of 1934, as amended, or the Exchange Act, periodic reporting requirements for at least 90 days before the sale. Persons who have beneficially owned restricted shares for at least six months but who are our affiliates at the time of, or any time during the 90 days preceding, a sale, would be subject to additional restrictions, by which such person would be entitled to sell within any three-month period only a number of securities that does not exceed the greater of either of the following:

- 1% of the number of shares then outstanding, which will equal approximately shares immediately after this offering, assuming no exercise of the underwriters' option to purchase additional shares, based on the number of shares outstanding as of December 31, 2017; or
- the average weekly trading volume of our common stock on The Nasdaq Global Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to the sale;

provided, in each case, that we are subject to the Exchange Act periodic reporting requirements for at least 90 days before the sale. Such sales both by affiliates and by non-affiliates must also comply with the manner of sale, current public information and notice provisions of Rule 144.

Rule 701

Rule 701 under the Securities Act, as in effect on the date of this prospectus, permits resales of shares in reliance upon Rule 144 but without compliance with certain restrictions of Rule 144, including the holding period requirement. Most of our employees, executive officers or directors who purchased shares under a written compensatory plan or contract may be entitled to rely on the resale provisions of Rule 701, but all holders of Rule 701 shares are required to wait until 90 days after the date of this prospectus before selling their shares.

However, substantially all Rule 701 shares are subject to lock-up agreements as described below and under "Underwriting" included elsewhere in this prospectus and will become eligible for sale upon the expiration of the restrictions set forth in those agreements.

Lock-Up Agreements

We, our directors and executive officers and holders of substantially all of our common stock have signed a lock-up agreement that prevent us and them from selling any of our common stock or any securities convertible into or exercisable or exchangeable for common stock for a period of not less than 180 days from the date of this prospectus without the prior written consent of the Underwriters, subject to certain exceptions. See the section entitled "Underwriters" appearing elsewhere in this prospectus for more information.

Registration Rights

Upon completion of this offering, certain holders of our securities will be entitled to various rights with respect to registration of their shares under the Securities Act. Registration of these shares under the Securities Act would result in these shares becoming fully tradable without restriction under the Securities Act immediately upon the effectiveness of the registration. See the section entitled "Description of Capital Stock — Registration Rights" appearing elsewhere in this prospectus for more information.

Equity Incentive Plans

We intend to file one or more registration statements on Form S-8 under the Securities Act to register our shares issued or reserved for issuance under our equity incentive plans. The first such registration statement is expected to be filed soon after the date of this prospectus and will automatically become effective upon filing with the SEC. Accordingly, shares registered under such registration statement will be available for sale in the open market, unless such shares are subject to vesting restrictions with us or the lock-up restrictions described above. As of , 2018 we estimate that such registration statement on Form S-8 will cover approximately shares.

CERTAIN MATERIAL U.S. FEDERAL INCOME TAX CONSIDERATIONS FOR NON-U.S. HOLDERS

The following discussion is a summary of the material U.S. federal income tax considerations applicable to non-U.S. holders (as defined below) with respect to their ownership and disposition of shares of our common stock issued pursuant to this offering. For purposes of this discussion, a non-U.S. holder means a beneficial owner of our common stock that is for U.S. federal income tax purposes:

- a non-resident alien individual;
- a foreign corporation or any other foreign organization taxable as a corporation for U.S. federal income tax purposes; or
- a foreign estate or trust, the income of which is not subject to U.S. federal income tax on a net income basis.

This discussion does not address the tax treatment of partnerships or other entities that are pass-through entities for U.S. federal income tax purposes or persons that hold their common stock through partnerships or other pass-through entities. A partner in a partnership or other pass-through entity that will hold our common stock should consult his, her or its tax advisor regarding the tax consequences of acquiring, holding and disposing of our common stock through a partnership or other pass-through entity, as applicable.

This discussion is based on current provisions of the U.S. Internal Revenue Code of 1986, as amended, which we refer to as the Code, existing and proposed U.S. Treasury Regulations promulgated thereunder, current administrative rulings and judicial decisions, all as in effect as of the date of this prospectus and, all of which are subject to change or to differing interpretation, possibly with retroactive effect. Any such change or differing interpretation could alter the tax consequences to non-U.S. holders described in this prospectus. There can be no assurance that the Internal Revenue Service, which we refer to as the IRS, will not challenge one or more of the tax consequences described herein. We assume in this discussion that a non-U.S. holder holds shares of our common stock as a capital asset within the meaning of Section 1221 of the Code, generally property held for investment.

This discussion does not address all aspects of U.S. federal income taxation that may be relevant to a particular non-U.S. holder in light of that non-U.S. holder's individual circumstances nor does it address any aspects of any U.S. federal tax other than the income tax, U.S. state, local or non-U.S. taxes, the alternative minimum tax, the rules regarding qualified small business stock within the meaning of Section 1202 of the Code or the Medicare tax on net investment income. This discussion also does not consider any specific facts or circumstances that may apply to a non-U.S. holder and does not address the special tax rules applicable to particular non-U.S. holders, such as:

- insurance companies;
- tax-exempt or governmental organizations;
- financial institutions;
- brokers or dealers in securities;
- regulated investment companies;
- pension plans;
- "controlled foreign corporations," "passive foreign investment companies," and corporations that accumulate earnings to avoid U.S. federal income tax;
- "qualified foreign pension funds," or entities wholly owned by a "qualified foreign pension fund";
- partnerships or other entities or arrangements treated as partnerships for U.S. federal income tax purposes (and partners and investors therein);
- persons deemed to sell our common stock under the constructive sale provisions of the Code;

- persons that hold our common stock as part of a straddle, hedge, conversion transaction, synthetic security or other integrated investment;
- persons who hold or receive our common stock pursuant to the exercise of any employee stock option or otherwise as compensation; and
- certain U.S. expatriates.

This discussion is for general information only and is not tax advice. Accordingly, all prospective non-U.S. holders of our common stock should consult their tax advisors with respect to the U.S. federal, state, local and non-U.S. tax consequences of the purchase, ownership and disposition of our common stock.

Distributions on Our Common Stock

Distributions, if any, on our common stock will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. If a distribution exceeds our current and accumulated earnings and profits, the excess will be treated as a tax-free return of the non-U.S. holder's investment, up to such holder's tax basis in the common stock. Any remaining excess will be treated as capital gain, subject to the tax treatment described below in "Gain on Sale or Other Taxable Disposition of Our Common Stock." Any such distributions will also be subject to the discussions below under the sections titled "Backup Withholding and Information Reporting" and "Withholding and Information Reporting Requirements — FATCA."

Subject to the discussion in the following two paragraphs in this section, dividends paid to a non-U.S. holder generally will be subject to withholding of U.S. federal income tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

Dividends that are treated as effectively connected with a trade or business conducted by a non-U.S. holder within the United States and, if an applicable income tax treaty so provides, that are attributable to a permanent establishment or a fixed base maintained by the non-U.S. holder within the United States, are generally exempt from the 30% withholding tax if the non-U.S. holder satisfies applicable certification and disclosure requirements. However, such U.S. effectively connected income, net of specified deductions and credits, is taxed at the same graduated U.S. federal income tax rates applicable to United States persons (as defined in the Code). Any U.S. effectively connected income received by a non-U.S. holder that is a corporation may also, under certain circumstances, be subject to an additional "branch profits tax" at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

A non-U.S. holder of our common stock who claims the benefit of an applicable income tax treaty between the United States and such holder's country of residence generally will be required to provide a properly executed IRS Form W-8BEN or W-8BEN-E (or successor form) to the applicable withholding agent and satisfy applicable certification and other requirements. Non-U.S. holders are urged to consult their tax advisors regarding their entitlement to benefits under a relevant income tax treaty. A non-U.S. holder that is eligible for a reduced rate of U.S. withholding tax under an income tax treaty may obtain a refund or credit of any excess amounts withheld by timely filing a U.S. tax return with the IRS.

Gain on Sale or Other Taxable Disposition of Our Common Stock

Subject to the discussions below under "Backup Withholding and Information Reporting" and "Withholding and Information Reporting Requirements — FATCA," a non-U.S. holder generally will not be subject to any U.S. federal income tax on any gain realized upon such holder's sale or other taxable disposition of shares of our common stock unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a U.S. trade or business and, if an applicable income tax treaty so provides, is attributable to a permanent establishment or a fixed-base maintained by such non-U.S. holder in the United States, in which case the non-U.S. holder generally will be taxed on a net income basis at the graduated U.S. federal income tax rates applicable to United States persons (as defined in the Code) and, if the non-U.S. holder is a foreign corporation, the branch profits tax described above in "Distributions on Our Common Stock" also may apply;

- the non-U.S. holder is a nonresident alien individual who is present in the United States for 183 days or more in the taxable year of the disposition and certain other conditions are met, in which case the non-U.S. holder will be subject to a 30% tax (or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence) on the net gain derived from the disposition, which may be offset by certain U.S. source capital losses of the non-U.S. holder, if any (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; or

- we are, or have been, at any time during the five-year period preceding such sale of other taxable disposition (or the non-U.S. holder's holding period, if shorter) a "U.S. real property holding corporation," unless our common stock is regularly traded on an established securities market and the non-U.S. holder holds no more than 5% of our outstanding common stock, directly or indirectly, actually or constructively, during the shorter of the 5-year period ending on the date of the disposition or the period that the non-U.S. holder held our common stock. Generally, a corporation is a U.S. real property holding corporation only if the fair market value of its U.S. real property interests equals or exceeds 50% of the sum of the fair market value of its worldwide real property interests plus its other assets used or held for use in a trade or business. Although there can be no assurance, we do not believe that we are, or have been, a U.S. real property holding corporation, or that we are likely to become one in the future. No assurance can be provided that our common stock will be regularly traded on an established securities market for purposes of the rules described above. If we are a U.S. real property holding corporation and either our common stock is not regularly traded on an established securities market or a non-U.S. holder holds, or is treated as holding, more than 5% of our outstanding common stock, directly or indirectly, during the applicable testing period, any gain recognized by such non-U.S. holder will generally be subject to U.S. federal income tax rates in the same manner as if the non-U.S. holder were a resident of the United States. If we are a U.S. real property holding corporation and our common stock is not regularly traded on an established securities market, such non-U.S. holder's proceeds received on the disposition of shares will also generally be subject to withholding at a rate of 15%. Prospective investors are encouraged to consult their own tax advisors regarding the possible consequences to them if we are, or were to become, a U.S. real property holding corporation.

Backup Withholding and Information Reporting

We must report annually to the IRS and to each non-U.S. holder the gross amount of the distributions on our common stock paid to such holder and the tax withheld, if any, with respect to such distributions. Non-U.S. holders may have to comply with specific certification procedures to establish that the holder is not a United States person (as defined in the Code) in order to avoid backup withholding at the applicable rate with respect to dividends on our common stock. Dividends paid to non-U.S. holders subject to

withholding of U.S. federal income tax, as described above in "Distributions on Our Common Stock," generally will be exempt from U.S. backup withholding.

Information reporting and backup withholding will generally apply to the proceeds of a disposition of our common stock by a non-U.S. holder effected by or through the U.S. office of any broker, U.S. or foreign, unless the holder certifies its status as a non-U.S. holder and satisfies certain other requirements, or otherwise establishes an exemption. Generally, information reporting and backup withholding will not apply to a payment of disposition proceeds to a non-U.S. holder where the transaction is effected outside the United States through a non-U.S. office of a broker. However, for information reporting purposes, dispositions effected through a non-U.S. office of a broker with substantial U.S. ownership or operations generally will be treated in a manner similar to dispositions effected through a U.S. office of a broker. Non-U.S. holders should consult their tax advisors regarding the application of the information reporting and backup withholding rules to them. Copies of information returns may be made available to the tax authorities of the country in which the non-U.S. holder resides or is incorporated under the provisions of a specific treaty or agreement. Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules from a payment to a non-U.S. holder can be refunded or credited against the non-U.S. holder's U.S. federal income tax liability, if any, provided that an appropriate claim is filed with the IRS in a timely manner.

Withholding and Information Reporting Requirements — FATCA

The Foreign Account Tax Compliance Act, or FATCA, generally imposes a U.S. federal withholding tax at a rate of 30% on payments of dividends on, or gross proceeds from the sale or other disposition of, our common stock paid to a foreign entity unless (i) if the foreign entity is a "foreign financial institution," such foreign entity undertakes certain due diligence, reporting, withholding, and certification obligations, (ii) if the foreign entity is not a "foreign financial institution," such foreign entity identifies certain of its U.S. investors, if any, or (iii) the foreign entity is otherwise exempt under FATCA. Under applicable U.S. Treasury regulations, withholding under FATCA currently applies to payments of dividends on our common stock, but will only apply to payments of gross proceeds from a sale or other disposition of our common stock made after December 31, 2018. Under certain circumstances, a non-U.S. holder may be eligible for refunds or credits of this withholding tax. An intergovernmental agreement between the United States and an applicable foreign country may modify the requirements described in this paragraph. Non-U.S. holders should consult their tax advisors regarding the possible implications of this legislation on their investment in our common stock and the entities through which they hold our common stock, including, without limitation, the process and deadlines for meeting the applicable requirements to prevent the imposition of the 30% withholding tax under FATCA.

UNDERWRITING

Subject to the terms and conditions set forth in the underwriting agreement, dated , 2018, by and among us and Jefferies LLC, Cowen and Company, LLC and BMO Capital Markets Corp., as the representatives of the underwriters named below, we have agreed to sell to the underwriters, and each of the underwriters has agreed, severally and not jointly, to purchase from us, the respective number of shares of common stock shown opposite its name below:

Underwriter	Number of Shares
Jefferies LLC	
Cowen and Company, LLC	
BMO Capital Markets Corp.	
Wedbush Securities Inc.	
Total	

The underwriting agreement provides that the obligations of the several underwriters are subject to certain conditions precedent such as the receipt by the underwriters of officers' certificates and legal opinions and approval of certain legal matters by their counsel. The underwriting agreement provides that the underwriters will purchase all of the shares of common stock if any of them are purchased. If an underwriter defaults, the underwriting agreement provides that the purchase commitments of the nondefaulting underwriters may be increased or the underwriting agreement may be terminated. We have agreed to indemnify the underwriters and certain of their controlling persons against certain liabilities, including liabilities under the Securities Act, and to contribute to payments that the underwriters may be required to make in respect of those liabilities.

The underwriters have advised us that, following the completion of this offering, they currently intend to make a market in the common stock as permitted by applicable laws and regulations. However, the underwriters are not obligated to do so, and the underwriters may discontinue any market-making activities at any time without notice in their sole discretion. Accordingly, no assurance can be given as to the liquidity of the trading market for the common stock, that you will be able to sell any of the common stock held by you at a particular time or that the prices that you receive when you sell will be favorable.

The underwriters are offering the shares of common stock subject to their acceptance of the shares of common stock from us and subject to prior sale. The underwriters reserve the right to withdraw, cancel or modify offers to the public and to reject orders in whole or in part.

Commission and Expenses

The underwriters have advised us that they propose to offer the shares of common stock to the public at the initial public offering price set forth on the cover page of this prospectus and to certain dealers, which may include the underwriters, at that price less a concession not in excess of $ per share of common stock. After the offering, the initial public offering price and concession to dealers may be reduced by the representatives. No such reduction will change the amount of proceeds to be received by us as set forth on the cover page of this prospectus.

The following table shows the public offering price, the underwriting discounts and commissions that we are to pay the underwriters and the proceeds, before expenses, to us in connection with this offering. Such amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

	Per Share		Total	
	Without Option to Purchase Additional Shares	With Option to Purchase Additional Shares	Without Option to Purchase Additional Shares	With Option to Purchase Additional Shares
Public offering price	$	$	$	$
Underwriting discounts and commissions paid by us	$	$	$	$
Proceeds to us, before expenses	$	$	$	$

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 also agreed to reimburse the underwriters for certain of their expenses in an amount up to $.

Determination of Offering Price

Prior to this offering, there has not been a public market for our common stock. Consequently, the initial public offering price for our common stock will be determined by negotiations between us and the representatives. Among the factors to be considered in these negotiations will be prevailing market conditions, our financial information, market valuations of other companies that we and the underwriters believe to be comparable to us, estimates of our business potential, the present state of our development and other factors deemed relevant.

We offer no assurances that the initial public offering price will correspond to the price at which the common stock will trade in the public market subsequent to the offering or that an active trading market for the common stock will develop and continue after the offering.

Listing

We have applied to have our common stock approved for listing on The Nasdaq Global Market under the trading symbol "SRRK."

Stamp Taxes

If you purchase shares of common stock offered in this prospectus, you may be required to pay stamp taxes and other charges under the laws and practices of the country of purchase, in addition to the offering price listed on the cover page of this prospectus.

Option to Purchase Additional Shares

We have granted to the underwriters an option, exercisable for 30 days from the date of this prospectus, to purchase, from time to time, in whole or in part, up to an aggregate of shares from us at the public offering price set forth on the cover page of this prospectus, less underwriting discounts and commissions. If the underwriters exercise this option, each underwriter will be obligated, subject to specified conditions, to purchase a number of additional shares proportionate to that underwriter's initial purchase commitment as indicated in the table above. This option may be exercised only if the underwriters sell more shares than the total number set forth on the cover page of this prospectus.

No Sales of Similar Securities

We, our officers, directors and holders of all or substantially all our outstanding capital stock and other securities have agreed, subject to specified exceptions, not to directly or indirectly:

- sell, offer, contract or grant any option to sell (including any short sale), pledge, transfer, establish an open "put equivalent position" within the meaning of Rule 16a-l(h) under the Securities Exchange Act of 1934, as amended, or
- otherwise dispose of any shares of common stock, options or warrants to acquire shares of common stock, or securities exchangeable or exercisable for or convertible into shares of common stock currently or hereafter owned either of record or beneficially, or
- publicly announce an intention to do any of the foregoing for a period of 180 days after the date of this prospectus without the prior written consent of Jefferies LLC and Cowen and Company, LLC

This restriction terminates after the close of trading of the common stock on and including the 180th day after the date of this prospectus.

Jefferies LLC and Cowen and Company, LLC may, in their sole discretion and at any time or from time to time before the termination of the 180-day period release all or any portion of the securities subject to lock-up agreements. There are no existing agreements between the underwriters and any of our shareholders who will execute a lock-up agreement, providing consent to the sale of shares prior to the expiration of the lock-up period.

Stabilization

The underwriters have advised us that they, pursuant to Regulation M under the Securities Exchange Act of 1934, as amended, certain persons participating in the offering may engage in short sale transactions, stabilizing transactions, syndicate covering transactions or the imposition of penalty bids in connection with this offering. These activities may have the effect of stabilizing or maintaining the market price of the common stock at a level above that which might otherwise prevail in the open market. Establishing short sales positions may involve either "covered" short sales or "naked" short sales.

"Covered" short sales are sales made in an amount not greater than the underwriters' option to purchase additional shares of our common stock in this offering. The underwriters may close out any covered short position by either exercising their option to purchase additional shares of our common stock or purchasing shares of our common stock in the open market. In determining the source of shares to close out the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase shares through the option to purchase additional shares.

"Naked" short sales are sales in excess of the option to purchase additional shares of our common stock. The underwriters must close out any naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the shares of our common stock in the open market after pricing that could adversely affect investors who purchase in this offering.

A stabilizing bid is a bid for the purchase of shares of common stock on behalf of the underwriters for the purpose of fixing or maintaining the price of the common stock. A syndicate covering transaction is the bid for or the purchase of shares of common stock on behalf of the underwriters to reduce a short position incurred by the underwriters in connection with the offering. Similar to other purchase transactions, the underwriter's purchases to cover the syndicate short sales 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 may be higher than the price that might otherwise exist in the open market. A penalty bid is an arrangement permitting the underwriters to reclaim the selling concession otherwise accruing to a syndicate member in connection with the offering if the common stock

originally sold by such syndicate member are purchased in a syndicate covering transaction and therefore have not been effectively placed by such syndicate member.

Neither we nor any of the underwriters make any representation or prediction as to the direction or magnitude of any effect that the transactions described above may have on the price of our common stock. The underwriters are not obligated to engage in these activities and, if commenced, any of the activities may be discontinued at any time.

The underwriters may also engage in passive market making transactions in our common stock on The Nasdaq Global Market in accordance with Rule 103 of Regulation M during a period before the commencement of offers or sales of shares of our common stock in this offering and extending through the completion of distribution. A passive market maker must display its bid at a price not in excess of the highest independent bid of that security. However, if all independent bids are lowered below the passive market maker's bid, that bid must then be lowered when specified purchase limits are exceeded.

Electronic Distribution

A prospectus in electronic format may be made available by e-mail or on the web sites or through online services maintained by one or more of the underwriters or their affiliates. In those cases, prospective investors may view offering terms online and may be allowed to place orders online. The underwriters may agree with us to allocate a specific number of shares of common stock for sale to online brokerage account holders. Any such allocation for online distributions will be made by the underwriters on the same basis as other allocations. Other than the prospectus in electronic format, the information on the underwriters' web sites and any information contained in any other web site maintained by any of the underwriters is not part of this prospectus, has not been approved and/or endorsed by us or the underwriters and should not be relied upon by investors.

Other Activities and Relationships

The underwriters and certain of their 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 underwriters and certain of their affiliates have, from time to time, performed, and may in the future perform, various commercial and investment banking and financial advisory services for us and our affiliates, for which they received or will receive customary fees and expenses.

In the ordinary course of their various business activities, the underwriters and certain of their affiliates may make or hold a broad array of investments and actively trade debt and equity securities (or related derivative securities) and financial instruments (including bank loans) for their own account and for the accounts of their customers, and such investment and securities activities may involve securities and/or instruments issued by us and our affiliates. If the underwriters or their respective affiliates have a lending relationship with us, they routinely hedge their credit exposure to us consistent with their customary risk management policies. The underwriters and their respective affiliates may hedge such exposure by entering into transactions which consist of either the purchase of credit default swaps or the creation of short positions in our securities or the securities of our affiliates, including potentially the common stock offered hereby. Any such short positions could adversely affect future trading prices of the common stock offered hereby. The underwriters and certain of their respective affiliates may also communicate independent investment recommendations, market color or trading ideas and/or publish or express independent research views in respect of such securities or instruments and may at any time hold, or recommend to clients that they acquire, long and/or short positions in such securities and instruments.

Disclaimers About Non-U.S. Jurisdictions

Australia

This prospectus is not a disclosure document for the purposes of Australia's Corporations Act 2001 (Cth) of Australia, or Corporations Act, has not been lodged with the Australian Securities & Investments Commission and is only directed to the categories of exempt persons set out below. Accordingly, if you receive this prospectus in Australia:

You confirm and warrant that you are either:

- a "sophisticated investor" under section 708(8)(a) or (b) of the Corporations Act;
- a "sophisticated investor" under section 708(8)(c) or (d) of the Corporations Act and that you have provided an accountant's certificate to the Company which complies with the requirements of section 708(8)(c)(i) or (ii) of the Corporations Act and related regulations before the offer has been made;
- a person associated with the Company under Section 708(12) of the Corporations Act; or
- a "professional investor" within the meaning of section 708(11)(a) or (b) of the Corporations Act.

To the extent that you are unable to confirm or warrant that you are an exempt sophisticated investor, associated person or professional investor under the Corporations Act any offer made to you under this prospectus is void and incapable of acceptance.

You warrant and agree that you will not offer any of the securities issued to you pursuant to this prospectus for resale in Australia within 12 months of those securities being issued unless any such resale offer is exempt from the requirement to issue a disclosure document under section 708 of the Corporations Act.

Canada

(A) Resale Restrictions. The distribution of common stock in Canada is being made only in the provinces of Ontario, Quebec, Alberta and British Columbia on a private placement basis exempt from the requirement that we prepare and file a prospectus with the securities regulatory authorities in each province where trades of these securities are made. Any resale of the common stock in Canada must be made under applicable securities laws which may vary depending on the relevant jurisdiction, and which may require resales to be made under available statutory exemptions or under a discretionary exemption granted by the applicable Canadian securities regulatory authority. Purchasers are advised to seek legal advice prior to any resale of the securities.

(B) Representations of Canadian Purchasers. By purchasing common stock in Canada and accepting delivery of a purchase confirmation, a purchaser is representing to us and the dealer from whom the purchase confirmation is received that:

- the purchaser is entitled under applicable provincial securities laws to purchase the common stock without the benefit of a prospectus qualified under those securities laws as it is an "accredited investor" as defined under National Instrument 45-106 — *Prospectus Exemptions*,
- the purchaser is a "permitted client" as defined in National Instrument 31-103 — *Registration Requirements, Exemptions and Ongoing Registrant Obligations*,
- where required by law, the purchaser is purchasing as principal and not as agent, and
- the purchaser has reviewed the text above under Resale Restrictions.

(C) Conflicts of Interest. Canadian purchasers are hereby notified that Jefferies and Cowen are relying on the exemption set out in section 3A.3 or 3A.4, if applicable, of National Instrument 33-105 — *Underwriting Conflicts* from having to provide certain conflict of interest disclosure in this document.

(D) Statutory Rights of Action. Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if the offering memorandum (including any amendment thereto) such as this document contains a misrepresentation, provided that the remedies

for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser of these securities in Canada should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

(E) Enforcement of Legal Rights. All of our directors and officers as well as the experts named herein may be located outside of Canada and, as a result, it may not be possible for Canadian purchasers to effect service of process within Canada upon us or those persons. All or a substantial portion of our assets and the assets of those persons may be located outside of Canada and, as a result, it may not be possible to satisfy a judgment against us or those persons in Canada or to enforce a judgment obtained in Canadian courts against us or those persons outside of Canada.

(F) Taxation and Eligibility for Investment. Canadian purchasers of common stock should consult their own legal and tax advisors with respect to the tax consequences of an investment in the common stock in their particular circumstances and about the eligibility of the common stock for investment by the purchaser under relevant Canadian legislation.

European Economic Area

Any distributor subject to MiFID II that is offering, selling or recommending the common stock is responsible for undertaking its own target market assessment in respect of the common stock and determining its own distribution channels for the purposes of the MiFID product governance rules under Commission Delegated Directive (EU) 2017/593, or the Delegated Directive. Neither we nor the underwriters make any representations or warranties as to a distributor's compliance with the Delegated Directive.

In relation to each member state of the European Economic Area which has implemented the Prospectus Directive, or a Relevant Member State, an offer to the public of any shares of common stock which are the subject of the offering contemplated by this prospectus 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 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 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 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 underwriters or the underwriters nominated by us 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 common stock shall require us or any of the underwriters to publish 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, the expression an "offer shares of common stock to the public" in relation to the shares of 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 the shares of common stock to be offered so as to enable an investor to decide to purchase or subscribe to the shares of common stock, as the same may be varied in that Relevant Member State by any measure implementing the Prospectus Directive in that Relevant Member State and 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 the expression "2010 PD Amending Directive" means Directive 2010/73/EU.

Hong Kong

No securities have been offered or sold, and no securities may be offered or sold, in Hong Kong, by means of any document, other than to persons whose ordinary business is to buy or sell shares or debentures, whether as principal or agent; or to "professional investors" as defined in the Securities and Futures Ordinance (Cap. 571) of Hong Kong, or SFO, and any rules made under that Ordinance; or in other circumstances which do not result in the document being a "prospectus" as defined in the Companies Ordinance (Cap. 32) of Hong Kong, or CO, or which do not constitute an offer or invitation to the public for the purpose of the CO or the SFO. No document, invitation or advertisement relating to the securities has been issued or may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public of Hong Kong (except if permitted under the securities laws of Hong Kong) other than with respect to securities which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" as defined in the SFO and any rules made under that Ordinance.

This prospectus has not been registered with the Registrar of Companies in Hong Kong. Accordingly, this prospectus may not be issued, circulated or distributed in Hong Kong, and the securities may not be offered for subscription to members of the public in Hong Kong. Each person acquiring the securities will be required, and is deemed by the acquisition of the securities, to confirm that he is aware of the restriction on offers of the securities described in this prospectus and the relevant offering documents and that he is not acquiring, and has not been offered any securities in circumstances that contravene any such restrictions.

Israel

This document does not constitute a prospectus under the Israeli Securities Law, 5728-1968, or the Securities Law, and has not been filed with or approved by the Israel Securities Authority. In Israel, this prospectus is being distributed only to, and is directed only at, and any offer of the common stock is directed only at, (i) a limited number of persons in accordance with the Israeli Securities Law and (ii) investors listed in the first addendum, or the Addendum, to the Israeli Securities Law, consisting primarily of joint investment in trust funds, provident funds, insurance companies, banks, portfolio managers, investment advisors, members of the Tel Aviv Stock Exchange, underwriters, venture capital funds, entities with equity in excess of NIS 50 million and "qualified individuals," each as defined in the Addendum (as it may be amended from time to time), collectively referred to as qualified investors (in each case, purchasing for their own account or, where permitted under the Addendum, for the accounts of their clients who are investors listed in the Addendum). Qualified investors are required to submit written confirmation that they fall within the scope of the Addendum, are aware of the meaning of same and agree to it.

Japan

The offering has not been and will not be registered under the Financial Instruments and Exchange Law of Japan (Law No. 25 of 1948 of Japan, as amended), or FIEL, and the underwriters will not offer or sell any securities, directly or indirectly, in Japan or to, or for the benefit of, any resident of Japan (which term as used herein means any person resident in Japan, including any corporation or other entity organized under the laws of Japan), or to others for re-offering or resale, directly or indirectly, in Japan or to, or for the benefit of, any resident of Japan, except pursuant to an exemption from the registration requirements of, and otherwise in compliance with, the FIEL and any other applicable laws, regulations and ministerial guidelines of Japan.

Singapore

This prospectus has not been and will not be lodged or registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the common stock may not be circulated or distributed, nor may the common stock be offered or sold, or be made the subject of an invitation for

subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor under Section 274 of the Securities and Futures Act, Chapter 289 of Singapore, or SFA, (ii) to a relevant person pursuant to Section 275(1), or any person pursuant to Section 275(1A), and in accordance with the conditions specified in Section 275, of the SFA, or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA.

Where the securities are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

- a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or

- a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary of the trust is an individual who is an accredited investor,

securities (as defined in Section 239(1) of the SFA) of that corporation or the beneficiaries' rights and interest (howsoever described) in that trust shall not be transferred within six months after that corporation or that trust has acquired the securities pursuant to an offer made under Section 275 of the SFA except:

- to an institutional investor or to a relevant person defined in Section 275(2) of the SFA, or to any person arising from an offer referred to in Section 275(1A) or Section 276(4)(i)(B) of the SFA;

- where no consideration is or will be given for the transfer;

- where the transfer is by operation of law;

- as specified in Section 276(7) of the SFA; or

- as specified in Regulation 32 of the Securities and Futures (Offers of Investments) (Shares and Debentures) Regulations 2005 of Singapore.

Switzerland

The securities may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or SIX, or on any other stock exchange or regulated trading facility in Switzerland. This prospectus has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this prospectus nor any other offering or marketing material relating to the securities or the offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this prospectus nor any other offering or marketing material relating to the offering, the Company or the securities have been or will be filed with or approved by any Swiss regulatory authority. In particular, this prospectus will not be filed with, and the offer of securities will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA, and the offer of securities has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of securities.

United Kingdom

This prospectus is only being distributed to, and is only directed at, persons in the United Kingdom that are qualified investors within the meaning of Article 2(1)(e) of the Prospectus Directive that are also (i) investment professionals falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, as amended, or Order, and/or (ii) high net worth entities falling within Article 49(2)(a) to (d) of the Order and other persons to whom it may lawfully be communicated, or a Relevant Person.

This prospectus and its contents are confidential and should not be distributed, published or reproduced (in whole or in part) or disclosed by recipients to any other persons in the United Kingdom. Any person in the United Kingdom that is not a Relevant Person should not act or rely on this document or any of its content.

LEGAL MATTERS

The validity of the shares of common stock offered by this prospectus will be passed upon for us by Goodwin Procter LLP, Boston, Massachusetts. Certain legal matters related to this offering will be passed upon for the underwriters by Cooley LLP, Boston, Massachusetts.

EXPERTS

The consolidated financial statements of Scholar Rock Holding Corporation at December 31, 2016 and 2017, and for the years then ended, appearing in this prospectus and registration statement have been audited by Ernst & Young LLP, independent registered accounting firm, as set forth in their report thereon (which contains an explanatory paragraph describing conditions that raise substantial doubt about our ability to continue as a going concern as described in Note 1 to the consolidated financial statements) appearing elsewhere herein and are included in reliance upon such report given on the authority of such firm 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 (File Number 333-) under the Securities Act with respect to the common stock we are offering by this prospectus. This prospectus does not contain all of the information included in the registration statement. For further information pertaining to us and our common stock, you should refer to the registration statement and to its exhibits. Whenever we make reference in this prospectus to any of our contracts, agreements or other documents, the references are not necessarily complete, and you should refer to the exhibits attached to the registration statement for copies of the actual contract, agreement or other document.

Upon the completion of the offering, we will be subject to the informational requirements of the Exchange Act and will file annual, quarterly and current reports, proxy statements and other information with the SEC. You can read our SEC filings, including the registration statement, at the SEC's website at www.sec.gov. You may also read and copy any document we file with the SEC at its public reference facility at 100 F Street, N.E., Room 1580, Washington, D.C. 20549. We also maintain a website at http://www.scholarrock.com. Upon completion of the offering, you may access, free of charge, our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendment to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC.

You may also obtain copies of the documents at prescribed rates by writing to the Public Reference Section of the SEC at 100 F Street, N.E., Washington, D.C. 20549. Please call the SEC at 1-800-SEC-0330 for further information on the operation of the public reference facilities.

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Stockholders and the Board of Directors of Scholar Rock Holding Corporation

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Scholar Rock Holding Corporation (the Company) as of December 31, 2016 and 2017, the related consolidated statements of operations and comprehensive loss, convertible preferred stock and stockholders' equity (deficit) and cash flows for the years then ended, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2016 and 2017, and the results of its operations and its cash flows for the years then ended, in conformity with U.S. generally accepted accounting principles.

The Company's Ability to Continue as a Going Concern

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the consolidated financial statements, the Company has suffered recurring losses from operations, will require substantial additional capital to fund operations, and has stated that substantial doubt exists about the Company's ability to continue as a going concern. Management's evaluation of the events and conditions and management's plans regarding these matters are also described in Note 1. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB and in accordance with auditing standards generally accepted in the United States of America. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting 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.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2015.
Boston, Massachusetts
March 23, 2018

SCHOLAR ROCK HOLDING CORPORATION
CONSOLIDATED BALANCE SHEETS
(In thousands, except unit, share, and per share data)

	December 31,		Pro Forma December 31,
	2016	2017	2017 (unaudited)
Assets			
Current assets:			
Cash and cash equivalents	$ 10,033	$ 56,461	$ 56,461
Marketable securities	18,002	1,498	1,498
Prepaid expenses and other current assets	461	1,242	1,242
Total current assets	28,496	59,201	59,201
Marketable securities	1,496	—	—
Property and equipment, net	2,430	2,181	2,181
Restricted cash	205	205	205
Other long-term assets	155	50	50
Total assets	$ 32,782	$ 61,637	$ 61,637
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)			
Current liabilities:			
Accounts payable	$ 663	$ 1,359	$ 1,359
Accrued expenses	1,487	2,796	2,796
Deferred rent	209	228	228
Loan payable	647	641	641
Total current liabilities	3,006	5,024	5,024
Long-term portion of deferred rent	696	468	468
Long-term portion of loan payable	1,023	398	398
Warrant to purchase redeemable security	27	37	—
Total liabilities	4,752	5,927	5,890
Commitments and contingencies (Note 14)			
Convertible preferred units (Series A-1, A-2, A-3, A-4 and B), 30,102,095 units authorized and 28,652,147 units issued and outstanding at December 31, 2016; (aggregate liquidation preference of $58,277 at December 31, 2016); no units authorized, issued or outstanding as of December 31, 2017 or pro forma as of December 31, 2017 (unaudited)	58,057	—	—
Convertible preferred stock (Series A-1, A-2, A-3, A-4, B and C), $0.001 par value; no shares authorized, issued or outstanding as of December 31, 2016; 43,157,651 shares authorized and 43,135,911 shares issued and outstanding as of December 31, 2017; (aggregate liquidation preference of $109,561 as of December 31, 2017); no shares issued or outstanding, pro forma as of December 31, 2017 (unaudited)	—	109,232	—
Stockholders' equity (deficit):			
Common units, 21,130,140 units authorized and 4,576,500 units issued and outstanding at December 31, 2016; no units authorized, issued or outstanding as of December 31, 2017 or pro forma as of December 31, 2017 (unaudited)	1,471	—	—
Common stock, $0.001 par value; no shares authorized, issued or outstanding as of December 31, 2016; 60,000,000 shares authorized and 11,335,445 shares issued and outstanding as of December 31, 2017; 54,471,356 shares issued and outstanding, pro forma as of December 31, 2017 (unaudited)	—	11	54
Additional paid-in capital	1,052	3,994	113,220
Accumulated other comprehensive loss	(20)	(2)	(2)
Accumulated deficit	(32,530)	(57,525)	(57,525)
Total stockholders' equity (deficit)	(30,027)	(53,522)	55,747
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 32,782	$ 61,637	$ 61,637

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

SCHOLAR ROCK HOLDING CORPORATION
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(In thousands, except unit, share, per unit and per share data)

	Year Ended December 31,	
	2016	2017
Collaboration revenue	$ 379	$ —
Operating expenses:		
Research and development	12,477	19,944
General and administrative	4,112	5,085
Total operating expenses	16,589	25,029
Loss from operations	(16,210)	(25,029)
Other income (expense):		
Interest income (expense), net	(19)	44
Other income (expense), net	22	(10)
Total other income	3	34
Net loss	$ (16,207)	$ (24,995)
Net loss per unit, basic and diluted	$ (3.54)	
Net loss per share, basic and diluted		$ (5.36)
Weighted average common units outstanding, basic and diluted	4,576,500	
Weighted average common shares outstanding, basic and diluted		4,665,036
Pro forma net loss per share, basic and diluted (unaudited)		$ (0.72)
Pro forma weighted average common shares outstanding, basic and diluted (unaudited)		34,567,011
Comprehensive loss:		
Net loss	$ (16,207)	$ (24,995)
Other comprehensive income (loss):		
Unrealized gain (loss) on marketable securities	(20)	18
Total other comprehensive income (loss)	(20)	18
Comprehensive loss	$ (16,227)	$ (24,977)

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

SCHOLAR ROCK HOLDING CORPORATION

CONSOLIDATED STATEMENTS OF CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)

(In thousands, except unit and share data)

	Convertible Preferred Units		Convertible Preferred Stock		Common Units		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders Equity (Deficit)
	Units	Amount	Shares	Amount	Units	Amount	Shares	Amount				
Balance at December 31, 2015	28,652,147	$ 58,057	—	$ —	4,576,500	$ 1,471	—	$ —	$ 496	$ —	$ (16,323)	$ (14,356
Unrealized loss on marketable securities	—	—	—	—	—	—	—	—	—	(20)	—	(20
Equity-based compensation expense	—	—	—	—	—	—	—	—	556	—	—	556
Net loss	—	—	—	—	—	—	—	—	—	—	(16,207)	(16,207
Balance at December 31, 2016	28,652,147	58,057	—	—	4,576,500	1,471	—	—	1,052	(20)	(32,530)	(30,027
Unrealized gain on marketable securities	—	—	—	—	—	—	—	—	—	18	—	18
Issuance of Series B convertible preferred units	1,428,209	4,285	—	—		—		—	—	—	—	—
Effect of Reorganization	(30,080,356)	(62,342)	30,080,356	62,342	(4,576,500)	(1,471)	4,576,500	4	1,467	—	—	—
Issuance of common stock and restricted common stock as part of Reorganization	—	—	—	—	—	—	6,758,945	7	(7)			
Issuance of Series C convertible preferred stock, net of issuance costs of $109	—	—	13,055,555	46,890	—	—	—	—	—	—	—	—
Equity-based compensation expense	—	—	—	—	—	—		—	1,482	—	—	1,482
Net loss	—	—	—	—	—	—		—	—	—	(24,995)	(24,995
Balance at December 31, 2017	—	—	43,135,911	109,232	—	—	11,335,445	11	3,994	(2)	(57,525)	(53,522
Conversion of convertible preferred stock into common stock (unaudited)	—	—	(43,135,911)	(109,232)	—	—	43,135,911	43	109,189	—	—	109,232
Reclassification of warrant to purchase convertible preferred stock to stockholders' equity (deficit) (unaudited)	—	—	—	—	—	—	—	—	37	—	—	37
Pro forma balance at December 31, 2017 (unaudited)	—	$ —	—	$ —	—	$ —	54,471,356	$ 54	$ 113,220	$ (2)	$ (57,525)	$ 55,747

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

SCHOLAR ROCK HOLDING CORPORATION
CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands)

	Year Ended December 31,	
	2016	2017
Cash flows from operating activities:		
Net loss	$ (16,207)	$ (24,995)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	575	669
Equity-based compensation	556	1,482
Amortization of deferred rent	(184)	(209)
Deferred payroll tax credit	(250)	(199)
Other	22	47
Change in operating assets and liabilities:		
Accounts receivable	380	—
Prepaid expenses and other current assets	(141)	(477)
Accounts payable	91	636
Accrued expenses	17	1,309
Net cash used in operating activities	(15,141)	(21,737)
Cash flows from investing activities:		
Purchase of property and equipment	(794)	(361)
Purchases of marketable securities	(19,525)	—
Maturities of marketable securities	—	18,026
Net cash (used in) provided by investing activities	(20,319)	17,665
Cash flows from financing activities:		
Proceeds from loan payable and warrants, net of issuance costs	1,318	—
Principal payments on loan payable	(302)	(667)
Proceeds from issuance of Series B convertible preferred units	—	4,285
Proceeds from issuance of Series C convertible preferred shares, net of issuance costs	—	46,890
Principal payments on capital lease obligation and other	(14)	(8)
Net cash provided by financing activities	1,002	50,500
Net (decrease) increase in cash and cash equivalents	(34,458)	46,428
Cash and cash equivalents, beginning of period	44,491	10,033
Cash and cash equivalents, end of period	$ 10,033	$ 56,461
Supplemental disclosure of non-cash items:		
Property and equipment purchases in accounts payable	$ 98	$ 159
Supplemental cash flow information:		
Cash paid for interest	$ 42	$ 53

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

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements

1. Nature of the Business and Basis of Presentation

Organization

Scholar Rock, LLC was formed on May 22, 2012, as a Delaware limited liability company to discover and develop a new class of biologics.

On December 22, 2017, a series of transactions were completed pursuant to which Scholar Rock Merger Sub, LLC, a wholly owned subsidiary of Scholar Rock Holding Corporation, a Delaware corporation, was merged with and into Scholar Rock, LLC (the "Reorganization"). As part of the Reorganization, each issued and outstanding convertible preferred and common unit of Scholar Rock, LLC outstanding immediately prior to the Reorganization was exchanged for shares of Scholar Rock Holding Corporation capital stock of the same class and/or series on a one-for-one basis. Previously outstanding vested and unvested incentive units, irrespective of any strike price or voting rights, were exchanged for an equal number of shares of common stock or restricted common stock, respectively. The restricted common stock was issued with the same vesting terms as the unvested incentive units held immediately prior to the Reorganization.

Upon consummation of the Reorganization, the historical consolidated financial statements of Scholar Rock, LLC became the historical consolidated financial statements of Scholar Rock Holding Corporation, the entity whose shares are being offered in this offering. For purposes of these consolidated financial statements, "the Company" refers to Scholar Rock, LLC prior to the Reorganization and Scholar Rock Holding Corporation after the Reorganization. The Company is based in Cambridge, Massachusetts.

The Company is a biopharmaceutical company focused on the discovery and development of innovative medicines for the treatment of serious diseases in which signaling by protein growth factors plays a fundamental role. Since its inception, the Company's operations have focused on organizing and staffing, business planning, raising capital, establishing the Company's intellectual property portfolio and performing research and development of monoclonal antibodies that selectively inhibit activation of growth factors for therapeutic effect. Revenue generation activities have been limited to research services and the issuance of a license, in each case, pursuant to an option and license agreement with Janssen Biotech, Inc. ("Janssen"), a subsidiary of Johnson & Johnson. No revenues have been recorded from the sale of any commercial product.

Basis of Presentation

The consolidated financial statements prior to the Reorganization include the accounts of Scholar Rock, LLC and its wholly owned subsidiary, Scholar Rock, Inc. The consolidated financial statements subsequent to the Reorganization include the accounts of Scholar Rock Holding Corporation and its wholly owned subsidiaries. All intercompany balances have been eliminated in consolidation.

These consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States ("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").

Going Concern

In accordance with Accounting Standards Update 2014-15, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern (Subtopic 205-40),* the Company has evaluated whether there are conditions and events considered in the aggregate that raise substantial doubt about the Company's ability to continue as a going concern.

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

1. Nature of the Business and Basis of Presentation (Continued)

Since inception, the Company has primarily funded its operations with proceeds from sale of convertible preferred units and convertible preferred stock and a credit facility with Silicon Valley Bank ("SVB"). From inception through December 31, 2017, the Company has raised an aggregate of $111.2 million, of which $109.2 million was from the issuance of convertible preferred units and convertible preferred stock and $2.0 million was from the Company's credit facility. The Company has incurred recurring losses and negative cash flows from operations since inception, including net losses of $16.2 million and $25.0 million for the years ended December 31, 2016 and 2017, respectively, and negative cash flows from operating activities of $15.1 million and $21.7 million for the years ended December 31, 2016 and 2017, respectively. As of December 31, 2017, the Company had an accumulated deficit of $57.5 million.

The Company is also subject to a number of risks similar to other life science companies, including, but not limited to, successful discovery and development of its drug candidates, raising additional capital, development by its competitors of new technological innovations, protection of proprietary technology and regulatory approval and market acceptance of the Company's products. The Company anticipates that it will continue to incur significant operating losses for the next several years as it continues to develop its product candidates.

The Company expects it operating losses and negative cash flows to continue into the foreseeable future as it continues to develop, manufacture, and commercialize its products. As of December 31, 2017, the Company had cash, cash equivalents and marketable securities of $58.0 million. Based on its current operating plan, the Company expects its cash, cash equivalents and marketable securities will be sufficient to fund operating expenses and capital expenditure requirements into the first quarter of 2019. Based on the Company's available cash resources, the Company does not have sufficient cash on hand to support current operations for at least the next twelve months from the date that these financial statements are issued. This condition results in substantial doubt about the Company's ability to continue as a going concern.

The Company is seeking to complete an initial public offering ("IPO") of its common stock. Upon the closing of a qualified public offering on specified terms, the Company's outstanding convertible preferred stock will automatically convert to common stock. In the event the Company does not complete an IPO, the Company expects to seek additional funding through private or public equity financings, debt financings, government funding arrangements, collaborations, strategic alliances and marketing, distribution or licensing agreements. The Company may not be able to obtain funding on acceptable terms, or at all. The terms of any financing may adversely affect the holdings or rights of the Company's shareholders.

If the Company is unable to obtain financing, the Company could be forced to delay, reduce or eliminate some or all of its research and development programs, product portfolio expansion or commercialization efforts, which could adversely affect its business prospects, or the Company may be unable to continue operations. 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 accompanying consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the ordinary course of business. The consolidated financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of the uncertainties described above.

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in accordance with GAAP requires management to make estimates and judgments that may affect the reported amounts of assets and liabilities and related disclosures of contingent assets and liabilities at the date of the financial statements and the related reporting of revenues and expenses during the reporting period. Significant estimates of accounting reflected in these consolidated financial statements include, but are not limited to, estimates related to accrued expenses, the valuation of equity-based compensation, including incentive units, common stock and restricted common stock, and income taxes. Actual results could differ from those estimates.

The Company utilizes significant estimates and assumptions in determining the fair value of its equity-based compensation, including incentive units, common stock and restricted common stock. The Company utilized various valuation methodologies in accordance with the framework of the 2013 American Institute of Certified Public Accountants Technical Practice Aid, *Valuation of Privately-Held Company Equity Securities Issued as Compensation,* to estimate the fair value of its equity awards. Each valuation methodology includes estimates and assumptions that require the Company's judgment. These estimates and assumptions include a number of objective and subjective factors, including external market conditions, guideline public company information, the prices at which the Company sold convertible preferred units and convertible preferred stock, the superior rights and preferences of securities senior to the Company's common units and common stock at the time and the likelihood of achieving a liquidity event such as an initial public offering or sale. Significant changes to the assumptions used in the valuations could result in different fair values of common units, incentive units, common stock and restricted common stock at each valuation date, as applicable.

Unaudited Pro Forma Information

On January 26, 2018, the Company's board of directors authorized the management of the Company to file a registration statement with the Securities and Exchange Commission to sell shares of its common stock to the public. Upon the closing of a qualified initial public offering (as defined in the Company's Certificate of Incorporation), all of the Company's outstanding shares of convertible preferred stock will automatically convert into shares of common stock and the outstanding warrant for the purchase of shares of convertible preferred stock will automatically convert into a warrant for the purchase of shares of common stock. The accompanying unaudited pro forma consolidated balance sheet and consolidated statement of convertible preferred stock and stockholders' equity (deficit) as of December 31, 2017 have been prepared to give effect to (1) the automatic conversion of all outstanding shares of convertible preferred stock into 43,135,911 shares of common stock and (2) the automatic conversion of the outstanding warrant to purchase 21,739 shares of convertible preferred stock into a warrant to purchase 21,739 shares of common stock, resulting in the reclassification of the warrant liability to additional paid-in capital, as if the Company's proposed IPO had occurred on December 31, 2017. The shares of common stock issuable and the proceeds expected to be received in the proposed IPO are excluded from such pro forma financial information.

The unaudited pro forma basic and diluted net loss per share in the accompanying consolidated statement of operations and comprehensive income (loss) for the year ended December 31, 2017 has been computed to give effect to the automatic conversion of all outstanding shares of convertible preferred stock into shares of common stock and the automatic conversion of the warrant to purchase shares of convertible preferred stock into a warrant to purchase shares of common stock. The unaudited pro forma basic and diluted net loss per share for the year ended December 31, 2017 was computed using the weighted average number of

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

shares of common stock outstanding, including the pro forma effect of the conversion of all outstanding shares of convertible preferred stock into shares of common stock, as if the Company's proposed IPO had occurred on the later of January 1, 2017 or the original issuance dates of the convertible preferred units or convertible preferred stock. The unaudited pro forma net loss used in the calculation of unaudited basic and diluted pro forma net loss per share for the year ended December 31, 2017 excludes the impact of the change in fair value of the warrant liability that was recorded by the Company during such period. The unaudited pro forma net loss per share does not include the shares expected to be sold or related proceeds to be received in the proposed IPO.

Concentration of Credit Risk and Off-Balance Sheet Risk

The Company has no off-balance sheet risk, such as foreign exchange contracts, option contracts or other foreign-hedging arrangements.

The primary objectives for the Company's investment portfolio are the preservation of capital and maintenance of liquidity. In 2016, the Company expanded its investment policy to allow funds to be held outside bank accounts, but to be invested only in fixed income instruments denominated and payable in U.S. dollars including obligations of the U.S. government and its agencies and money market funds registered according to SEC Rule 2a-7 of the Investment Company Act of 1940. Investments in the money market fund shall be consistent with approved instruments and assets under management must be at least $10 billion.

All securities must have a readily ascertainable market value, must be readily marketable and be U.S. dollar denominated.

Cash and Cash Equivalents

The Company considers highly liquid investments with a maturity of three months or less when purchased to be cash equivalents. At December 31, 2016 and 2017, cash and cash equivalents include bank demand deposits and money market funds that invest primarily in U.S. government-backed securities and treasuries. Cash equivalents are stated at cost, which approximates market value.

Property and Equipment

Property and equipment are recorded at cost. Expenditures for major renewals or betterments that extend the useful lives of property and equipment are capitalized; expenditures for maintenance and repairs are charged to expense as incurred. Depreciation is calculated on a straight-line basis over the estimated useful lives of the related asset. Property and equipment are depreciated as follows:

	Estimated Useful Life (in Years)
Laboratory equipment	3 - 5
Machinery and equipment	3 - 5
Furniture and fixtures	5

Leasehold improvements are amortized over the shorter of the useful life or remaining term of the lease.

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

Impairment of Long-Lived Assets

Long-lived assets consist of property and equipment. Long-lived assets to be held and used are tested for recoverability whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends and significant changes or planned changes in the use of the assets. If an impairment review is performed to evaluate a long-lived asset group for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long-lived asset group to its carrying value. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of an asset group are less than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset group over its fair value, determined based on discounted cash flows. The Company did not record any impairment losses on long-lived assets during the years ended December 31, 2016 or 2017.

Fair Value Measurements

ASC Topic 820, *Fair Value Measurement* ("ASC 820"), establishes a fair value hierarchy for instruments measured at fair value that distinguishes between assumptions based on market data (observable inputs) and the Company's own assumptions (unobservable inputs). Observable inputs are inputs that market participants would use in pricing the asset or liability 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 asset or liability and are developed based on the best information available in the circumstances. ASC 820 identifies fair value as the exchange price, or exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As a basis for considering market participant assumptions in fair value measurements, ASC 820 establishes a three-tier fair value hierarchy that distinguishes between the following:

Level 1 — Quoted market prices in active markets for identical assets or liabilities.

Level 2 — Inputs other than Level 1 inputs that are either directly or indirectly observable, such as quoted market prices, interest rates and yield curves.

Level 3 — Unobservable inputs developed using estimates of assumptions developed by the Company, which reflect those that a market participant would use.

To the extent the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair values requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized as 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.

Segment Information

Operating segments are defined as components of an entity 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 views its operations and manages its business in one operating segment operating exclusively in the United States.

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

Revenue Recognition

To date, all revenue has been generated from an agreement with Janssen.

The Company recognizes revenue in accordance with ASC 605, *Revenue Recognition* ("ASC 605"). Accordingly, revenue is recognized for each unit of accounting when all of the following criteria are met:

- Persuasive evidence of an arrangement exists;
- Delivery has occurred or services have been rendered;
- The seller's price to the buyer is fixed or determinable; and
- Collectability is reasonably assured.

Amounts received prior to satisfying the revenue recognition criteria are recorded as deferred revenue. Amounts expected to be recognized as revenue within the twelve months following the balance sheet date are classified in current liabilities. Amounts not expected to be recognized as revenue within the twelve months following the balance sheet date are classified as deferred revenue, net of current portion.

The Company evaluates multiple-element arrangements based on the guidance in ASC Topic 605-25, *Revenue Recognition-Multiple-Element Arrangements* ("ASC 605-25"). Pursuant to the guidance in ASC 605-25, the Company evaluates multiple-element arrangements to determine (1) the deliverables included in the arrangement and (2) whether the individual deliverables represent separate units of accounting or whether they must be accounted for as a combined unit of accounting. This evaluation involves subjective determinations and requires the Company to make judgments about the individual deliverables and whether such deliverables are separable from the other aspects of the contractual relationship. Deliverables are considered separate units of accounting provided that the delivered item has value to the customer on a standalone basis and, if the arrangement includes a general right of return relative to the delivered item, delivery or performance of the undelivered item is considered probable and substantially in the Company's control. In assessing whether an item has standalone value, the Company considers factors such as the research, development, manufacturing and commercialization capabilities of the collaboration partner and the availability of the associated expertise in the general marketplace. In addition, the Company considers whether the collaboration partner can use a deliverable for its intended purpose without the receipt of the remaining deliverable, whether the value of the deliverable is dependent on the undelivered item and whether there are other vendors that can provide the undelivered items. The consideration received under the arrangement that is fixed or determinable is then allocated among the separate units of accounting using the relative selling price method. The Company determines the estimated selling price for units of accounting within each arrangement using vendor-specific objective evidence ("VSOE") of selling price, if available, third-party evidence ("TPE") of selling price if VSOE is not available, or best estimate of selling price ("BESP") if neither VSOE nor TPE is available. Determining the BESP for a unit of accounting requires significant judgment. In developing the BESP for a unit of accounting, the Company considers applicable market conditions and relevant entity-specific factors, including factors that were contemplated in negotiating the agreement with the customer and estimated costs. The Company validates the BESP for units of accounting by evaluating whether changes in the key assumptions used to determine the BESP will have a significant effect on the allocation of arrangement consideration between multiple units of accounting.

The Company recognizes arrangement consideration allocated to each unit of accounting when all of the revenue recognition criteria in ASC 605 are satisfied for that particular unit of accounting. In the event that a deliverable does not represent a separate unit of accounting, the Company recognizes revenue from the

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

combined unit of accounting over the Company's contractual or estimated performance period for the undelivered elements, which is typically the term of the Company's research and development obligations. If there is no discernible pattern of performance or objectively measurable performance measures do not exist, then the Company recognizes revenue under the arrangement on a straight-line basis over the period the Company is expected to complete its performance obligations. Conversely, if the pattern of performance in which the service is provided to the customer can be determined and objectively measurable performance measures exist, then the Company recognizes revenue under the arrangement using the proportional performance method. At the inception of an arrangement that includes milestone payments, the Company evaluates whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. This evaluation includes an assessment of whether: (1) the consideration is commensurate with either the Company's performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from its performance to achieve the milestone, (2) the consideration relates solely to past performance and (3) the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. The Company evaluates factors such as the scientific, clinical, regulatory, commercial and other risks that must be overcome to achieve the particular milestone and the level of effort and investment required to achieve the particular milestone in making this assessment. There is considerable judgment involved in determining whether a milestone satisfies all of the criteria required to conclude that a milestone is substantive. Milestones that are not considered substantive are recognized as earned if there are no remaining performance obligations or over the remaining period of performance, assuming all other revenue recognition criteria are met.

Research and Development Expenses

Research and development expenses are expensed as incurred and consist of costs incurred in performing research and development activities, including compensation related expenses for research and development personnel, preclinical and clinical activities including cost of supply, overhead expenses including facilities expenses, materials and supplies, amounts paid to consultants and outside service providers, and depreciation of equipment. Upfront license payments related to acquired technologies which have not yet reached technological feasibility and have no alternative future use are also included in research and development expense.

Research Contract Costs and Accruals

The Company has entered into various research service arrangements under which vendors perform various services. The Company records accrued expenses for estimated costs incurred under the arrangements. When evaluating the adequacy of the accrued expenses, the Company analyzed the progress of the studies, trials or other services performed, including invoices received and contracted costs. Significant judgments and estimates are made in determining the accrued expense balances at the end of any reporting period.

Equity-Based Compensation

The Company accounts for equity awards, including incentive units, common stock and restricted common stock, granted to employees as equity award compensation in accordance with ASC Topic 718, *Compensation — Stock Compensation* ("ASC 718"). ASC 718 requires all stock-based payments to employees, which includes grants of employee equity awards, to be recognized as expense in the statements of operations based on their grant date fair values. For equity awards granted to employees and to members of the Board of Directors for their services on the Board of Directors, the Company estimates the grant date fair value of each equity award using an appropriate valuation methodology, which, in 2016 and 2017, was the guideline public company (GPC) method or the precedent transaction method which "backsolves" to a preferred price. The use of these valuation approaches requires management to make assumptions with

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

respect to the expected volatility of its common units and common stock, time until a liquidity event and risk-free interest rates. Compensation expense related to equity awards to employees are subject to graded vesting is recognized on a straight-line basis, based on the grant date fair value, over the requisite service period of the award, which is generally the vesting term. For awards subject to performance conditions, the Company recognizes equity award compensation expense using an accelerated recognition method over the remaining service period when management determines that achievement of the milestone is probable. Management evaluates when the achievement of a performance-based milestone is probable based on the relative satisfaction of the performance conditions as of the reporting date.

For equity awards granted to non-employees, the Company accounts for the related equity award compensation in accordance with the provisions of ASC 718 and ASC Topic 505, *Equity,* and recognizes equity award compensation expense over the related service period of the non-employee award. Equity awards issued to non-employees are recorded at their fair values, using the GPC method or the precedent transaction method, and are periodically revalued as the equity instruments vest.

Convertible Preferred Units and Convertible Preferred Stock

The Company records all convertible preferred units or convertible preferred shares at their respective fair values on the dates of issuance less issuance costs. The Company classifies its convertible preferred units and convertible preferred shares outside of stockholders' equity (deficit) when the redemption of such units or shares is outside the Company's control. The Company does not adjust the carrying values of the convertible preferred units or convertible preferred stock to the liquidation preferences of such units or shares until such time as a deemed liquidation event is probable of occurring.

Comprehensive Loss

Comprehensive loss is the change in equity of a business enterprise during a period from transactions and other events and circumstances from non-owner sources. Comprehensive loss includes net loss and the change in accumulated other comprehensive loss for the period. Accumulated other comprehensive loss consisted entirely of unrealized gains and losses on available-for-sale marketable securities.

Net Loss per Unit and Share

The Company applies the two-class method to compute basic and diluted net loss per unit and net loss per share because it has issued units and shares that meet the definition of participating securities. The two-class method determines net income (loss) per unit and share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income (losses) available to common unit holders and common stockholders for the period to be allocated between common and participating securities based upon their respective rights to share in the earnings as if all income (losses) for the period had been distributed. During periods of loss, there is no allocation required under the two-class method since the participating securities do not have a contractual obligation to fund the losses of the Company.

Prior to the Reorganization, the Company calculates basic net loss per unit by dividing net loss by the weighted average number of common units outstanding. Subsequent to the Reorganization, the Company calculates basic net loss per share by dividing net loss by the weighted average number of common shares outstanding, excluding restricted common stock. The Company calculates diluted net loss per unit and diluted net loss per share by dividing net loss by the weighted average number of common units outstanding or weighted average number of common shares outstanding, as applicable, after giving consideration to the dilutive effect of convertible preferred units, convertible preferred stock, incentive

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

units, restricted common stock and warrants that are outstanding during the period. The Company has generated a net loss in all periods presented, so the basic and diluted net loss per unit and net loss per share are the same, as the inclusion of the potentially dilutive securities would be anti-dilutive.

Income Taxes

Prior to the Reorganization, Scholar Rock, LLC elected to be treated under the partnership provisions of the Internal Revenue Service code. Accordingly, all income and deductions of Scholar Rock, LLC were recorded on the members' individual tax returns and no taxes were recorded by Scholar Rock, LLC. Scholar Rock, Inc., the wholly-owned subsidiary of Scholar Rock, LLC, was taxed as a C-corporation for federal income tax purposes and filed a separate corporate income tax return from the LLC entity. All operations were recorded at Scholar Rock, Inc. Subsequent to the Reorganization, Scholar Rock Holding Corporation became the 100% owner of Scholar Rock, LLC, creating a new ultimate parent company, and a consolidated group for income tax reporting. The Reorganization and change in tax status of the reporting entity did not have an impact on the consolidated tax provision.

Income taxes for Scholar Rock Holding Corporation and Scholar Rock, Inc. are recorded in accordance with ASC Topic 740, *Income Taxes ("ASC 740")*, which provides for deferred taxes using an asset and liability approach. Under this method, deferred income tax assets and liabilities are recognized based on future income tax consequences attributable to differences between the financial statement carrying amount of existing assets and liabilities, and their respective income tax basis. Deferred income tax assets and liabilities are measured using enacted income tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect of changes in income tax rates on deferred income tax assets and liabilities is recognized as income or expense in the period that a valuation allowance for any income tax benefits of which future realization is not more likely than not.

The Company provides reserves for potential payments of tax to various tax authorities related to uncertain tax positions. The tax benefits recorded are based on a determination of whether and how much of a tax benefit taken by the Company in its tax filings or positions is "more likely than not" to be realized following resolution of any uncertainty related to the tax benefit, assuming that the matter in question will be raised by the tax authorities.

The Company is open to examination by the Internal Revenue Service for the tax years ended December 31, 2012 to December 31, 2017. The Company is currently not under examination by the Internal Revenue Service or any other jurisdictions for any tax years. The Company has not recorded any interest or penalties on any unrecognized tax benefits since its inception.

Recently Adopted Accounting Pronouncements

In March 2016, the FASB issued Accounting Standards Update No. 2016-09, *Improvements to Employee Share-Based Payment Accounting* (ASU 2016-09). ASU 2016-09 simplified several aspects of the accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities and classification on the statement of cash flows. ASU 2016-09 is effective for annual periods beginning after December 15, 2017, and interim periods within annual periods beginning after December 15, 2018. Early adoption is permitted for any entity in any interim or annual period. The Company elected to early adopt ASU 2016-09 effective for the year ended December 31, 2016 and has elected to account for forfeitures when they occur instead of estimating the number of awards that are expected to vest. The adoption of ASU No. 2016-09 did not have a material impact on the Company's

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

financial statements. The Company did not have any excess tax benefits associated with equity exercises and therefore there was no deferred tax asset recorded upon adoption.

In January 2017, the FASB issued ASU No. 2017-01, *Business Combinations* ("ASU 2017-01"), which clarified the definition of a business and provides a screen to determine when an integrated set of assets and activities is not a business. The screen requires that when substantially all of the fair value of the gross assets acquired, or disposed of, is concentrated in a single identifiable asset or a group of similar identifiable assets, the set is not a business. This new standard is effective for fiscal years beginning after December 15, 2017, and interim periods within those years. Early adoption was permitted. The Company adopted this new standard as of January 1, 2017, with prospective application to any business development transactions.

Recently Issued Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies and adopted by the Company as of the specified effective date.

In May 2014, the FASB issued Accounting Standards Update (ASU) No. 2014-09, *Revenue from Contracts with Customers (Topic 606)*, which supersedes all existing revenue recognition requirements, including most industry-specific guidance. The new standard requires a company to recognize revenue when it transfers goods or services to customers in an amount that reflects the consideration that the company expects to receive for those goods or services. In April 2016, the FASB issued ASU No. 2016-10, Revenue from Contracts with Customers (Topic 606): Identifying Performance Obligations and Licensing, which amends the guidance on accounting for licenses of intellectual property and identifying performance obligations under the new standard. ASU No. 2014-09 and ASU No. 2016-10 is effective for public business entities for annual reporting periods beginning after December 15, 2017. The Company currently plans to adopt the standard on January 1, 2018. The Company is currently evaluating the potential impact these updates may have on the Company's financial position and results of operations related to its agreement with Janssen. The Company currently anticipates that it will adopt the new standard using the full retrospective method.

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)*, which supersedes all existing lease guidance. The new standard requires a company to recognize lease assets and liabilities for leases previously classified as operating leases. The new standard will be effective for the Company on January 1, 2019. The Company is currently evaluating the potential impact that this update may have on the Company's financial position and results of operations.

In August 2016, the FASB issued ASU No. 2016-15, *Statement of Cash Flows: Classification of Certain Cash Receipts and Cash Payments* ("Topic 230"). The new standard clarifies certain aspects of the statement of cash flows, including the clarification of restricted cash and several clarifications not currently applicable to the Company. The new standard also clarifies that an entity should determine each separately identifiable source or use within the cash receipts and cash payments on the basis of the nature of the underlying cash flows. In situations in which cash receipts and payments have aspects of more than one class of cash flows and cannot be separated by source or use, the appropriate classification should depend on the activity that is likely to be the predominant source or use of cash flows for the item. The new standard will be effective for the Company on January 1, 2019. The adoption of this standard is not expected to have a material impact on the Company's consolidated statements of cash flows.

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

In May 2017, the FASB issued ASU No. 2017-09, *Compensation — Stock Compensation* ("ASU 2017-09"), which provided guidance about which changes to the terms or conditions of a share-based payment award require an entity to apply modification accounting. The new guidance requires modification accounting if the vesting condition, fair value or award classification is not the same both before and after a change to the terms and conditions of the award. This new standard is effective for fiscal years beginning after December 15, 2017, and interim periods within those years. Early adoption was permitted. The Company does not anticipate a material impact to its consolidated financial statements as a result of the adoption of this standard.

Marketable Securities

The Company classifies its marketable securities as available-for-sale. Marketable securities with a remaining maturity date greater than one year are classified as non-current. Marketable securities are maintained by an investment manager and consist of U.S. treasury securities. Marketable securities are carried at fair value with the unrealized gains and losses included in accumulated other comprehensive loss as a component of stockholders' equity (deficit) until realized. Any premium or discount arising at purchase is amortized and/or accreted to interest income and/or expense over the life of the underlying marketable security.

Although available to be sold to meet operating needs or otherwise, securities are generally held through maturity. The cost of securities sold is determined on a specific identification basis, and realized gains and losses are included in interest income (expense) within the statement of operations and comprehensive loss. During 2016 and 2017, there were no realized gains of losses on sales of marketable securities and no marketable securities were adjusted for other than temporary declines in fair value.

The Company evaluates its marketable securities with unrealized losses for other-than-temporary impairment. When assessing marketable securities for other-than-temporary declines in value, the Company considers such factors as, among other things, how significant the decline in value is as a percentage of the original cost, how long the market value of the investment has been less than its original cost, the Company's ability and intent to retain the investment for a period of time sufficient to allow for any anticipated recovery in fair value and market conditions in general. If any adjustment to fair value reflects a decline in the value of the investment that the Company considers to be "other than temporary," the Company would reduce the investment to fair value through a charge to the statement of operations and comprehensive loss. No such adjustments were necessary during the periods presented.

Warrant Liability

The Company accounts for warrant instruments that either conditionally or unconditionally obligate the issuer to transfer assets or issue equity shares as a liability, if it permits the holder to purchase redeemable shares, including shares that are contingently redeemable outside the control of the Company and the warrant itself is indexed to an underlying share that embodies the issuer's obligation to repurchase the share and the issuer has a conditional obligation to transfer the assets if the shares are put back. These warrants are subject to revaluation at each balance sheet date, and any change in fair value are recorded as a component of other expense, until the earlier of their exercise or expiration or upon the completion of a liquidation event.

Subsequent Events

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the consolidated financial statements for potential recognition or disclosure in the consolidated

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

financial statements. Subsequent events have been evaluated through the date these consolidated financial statements were issued for potential recognition or disclosure in the consolidated financial statements.

3. Fair Value of Financial Assets and Liabilities

The following table summarizes the assets and liabilities measured at fair value on a recurring basis at December 31, 2016 and 2017 (in thousands):

	Fair Value Measurements at December 31, 2016			
	Total	Level 1	Level 2	Level 3
Assets:				
Money market funds, included in cash and cash equivalents	$ 8,166	$ 8,166	$ —	$ —
Marketable securities:				
U.S. Treasury obligations	19,498	19,498	—	—
Total assets	$ 27,664	$ 27,664	$ —	$ —
Liabilities:				
Warrant to purchase redeemable security	$ 27	$ —	$ —	$ 27
Total liabilities	$ 27	$ —	$ —	$ 27

	Fair Value Measurements at December 31, 2017			
	Total	Level 1	Level 2	Level 3
Assets:				
Money market funds, included in cash and cash equivalents	$ 55,291	$ 55,291	$ —	$ —
Marketable securities:				
U.S. Treasury obligations	1,498	1,498	—	—
Total assets	$ 56,789	$ 56,789	$ —	$ —
Liabilities:				
Warrant to purchase redeemable security	$ 37	$ —	$ —	$ 37
Total liabilities	$ 37	$ —	$ —	$ 37

Cash and cash equivalents and marketable securities include investments in money market funds that invest in U.S. government securities that are valued using quoted market prices. Accordingly, money market funds and government funds are categorized as Level 1 as of December 31, 2016 and 2017.

The carrying amounts reflected in the balance sheets for accounts receivable, prepaid expenses and other current assets, accounts payable, and accrued expenses approximate their fair values at December 31, 2016 and 2017, due to their short-term nature. The Company believes the terms of the loan payable reflect

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

3. Fair Value of Financial Assets and Liabilities (Continued)

current market conditions for an instrument with similar terms and maturity, therefore the carrying value of the Company's debt approximates its fair value based on Level 3 of the fair value hierarchy.

Warrants to Purchase Convertible Preferred Units or Stock Subject to Redemption

In conjunction with the loan and security agreement, the Company issued a warrant to SVB to purchase 21,739 Series A-3 Convertible Preferred Units at a purchase price of $1.38 per unit. In connection with the Reorganization, the warrant was converted to or exchanged for a warrant to purchase 21,739 shares of Series A-3 Convertible Preferred Stock at the same purchase price. The warrant is exercisable immediately and expires on August 10, 2025. The Company determined that the warrant represents an instrument to purchase a redeemable security, and, therefore, is required to be classified as a liability on the balance sheet. Because the warrant is classified as a liability, the liability is re-measured at its fair value at each balance sheet date.

The fair value of the warrant is estimated using the Black-Scholes option pricing model. The estimates in the Black-Scholes option pricing model are based, in part, on subjective assumptions, including stock price volatility, term of the warrants, risk free interest rate, dividend yield and fair value of the preferred stock underlying the warrants. Such assumptions could differ materially in the future. The warrant was initially valued at $20,300 and was included in issuance costs incurred in connection with the loan payable, recorded as a discount to the debt and amortized over the term of the loan as interest expense. The change in fair value of the warrant is recorded as other expense in the consolidated statement of operations.

The following assumptions were used in valuing the warrant at December 31, 2016 and 2017:

	December 31,	
	2016	2017
Interest rate	2.35%	2.33%
Expected dividend yield	0.00%	0.00%
Expected term (years)	8.61	7.61
Expected volatility	60.40%	77.40%

The following table sets forth a summary of changes in the fair value of the warrant, which represented a recurring measurement classified within Level 3 of the fair value hierarchy, wherein fair value was estimated using significant unobservable inputs (in thousands):

Balance at December 31, 2015	$ 28
Change in fair value of warrant included in other income (expense), net	(1)
Balance at December 31, 2016	27
Change in fair value of warrant included in other income (expense), net	10
Balance at December 31, 2017	$ 37

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

4. Marketable Securities

The following table summarizes the Company's investments as of December 31, 2016 (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Marketable securities available-for-sale:				
U.S. Treasury obligations	$ 19,518	$ —	$ (20)	$ 19,498
Total available-for-sale securities	$ 19,518	$ —	$ (20)	$ 19,498

The aggregate fair value of marketable securities with unrealized losses was $19.5 million at December 31, 2016. At December 31, 2016, 13 investments were in an unrealized loss position. All such investments have been in an unrealized loss position for less than a year and these losses are considered temporary. The Company has the ability and intent to hold these investments until a recovery of their amortized cost. As of December 31, 2016, the Company held one investment with a fair value of $1.5 million with a maturity greater than one year. This investment matures in March 2018.

The following table summarizes the Company's investments as of December 31, 2017 (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Marketable securities available-for-sale:				
U.S. Treasury obligations	$ 1,480	$ 18	$ —	$ 1,498
Total available-for-sale securities	$ 1,480	$ 18	$ —	$ 1,498

The Company did not have any marketable securities in an unrealized loss position at December 31, 2017.

5. Property and Equipment, Net

At December 31, 2016 and 2017, property and equipment consists of the following (in thousands):

	As of December 31, 2016	2017
Laboratory equipment	$ 1,673	$ 2,074
Furniture & fixtures	132	151
Machinery & equipment	7	7
Leasehold improvements	1,498	1,498
	3,310	3,730
Less: Accumulated depreciation and amortization	(880)	(1,549)
	$ 2,430	$ 2,181

Depreciation and amortization expense was $575,000 and $669,000 for the years ended December 31, 2016 and 2017, respectively.

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

6. Accrued Expenses

At December 31, 2016 and 2017, accrued expenses consist of the following (in thousands):

	As of December 31,	
	2016	2017
Accrued external research and development expense	$ 451	$ 1,225
Accrued payroll and related expenses	817	1,174
Accrued professional and consulting expense	201	382
Accrued other	18	15
	$ 1,487	$ 2,796

7. Member Units

Prior to the Reorganization, all interests of members in distributions and other amounts were represented by their units of membership in the Company as specified in its operating agreement. There were two classes of units, capital units and incentive units. Capital units were comprised of common units and convertible preferred units, which represent a capital interest in the Company, while incentive units represent profits interests within the meaning of IRS Revenue Procedures 93-27 and 2001-43. The various classes of capital units are described below.

8. Common Units

As of December 31, 2015, the Company had outstanding 4,576,500 common units. There were no additional common units issued during the years ended December 31, 2016 and 2017.

Holders of common units were entitled to one vote per unit and receive dividends, when and if declared by the Board of Directors. No common unit dividends were declared. The voting, dividend, and liquidation rights of the holders of the common units were subject to, and qualified by, the rights of the holders of the Series A Convertible Preferred Units and the Series B Convertible Preferred Units.

9. Convertible Preferred Units

Prior to 2016, the Company had sold Series A-1, A-2, A-3 and A-4 Convertible Preferred Units. The Series A-1, A-2, A-3 and A-4 Convertible Preferred Units are collectively referred to as the Series A Convertible Preferred Units. Additionally, the Company sold 12,098,785 Series B Convertible Preferred Units. The Series A Convertible Preferred Units and Series B Convertible Preferred Units are collectively considered the Convertible Preferred Units.

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

9. Convertible Preferred Units (Continued)

As of December 31, 2016, the Convertible Preferred Units consisted of the following (in thousands, except unit amounts):

	As of December 31, 2016				
	Preferred Units Authorized	Preferred Units Issued and Outstanding	Carrying Value	Liquidation Preference	Common Units Issuable Upon Conversion
Series A-1 convertible preferred units	2,000,000	2,000,000	$ 1,942	$ 2,000	2,000,000
Series A-2 convertible preferred units	5,066,915	5,066,915	6,004	6,080	5,066,915
Series A-3 convertible preferred units	5,601,448	5,579,709	7,698	7,700	5,579,709
Series A-4 convertible preferred units	3,906,738	3,906,738	6,198	6,200	3,906,738
Series B convertible preferred units	13,526,994	12,098,785	36,215	36,297	12,098,785
	30,102,095	28,652,147	$ 58,057	$ 58,277	28,652,147

In May 2017, the Company issued 1,428,209 shares of Series B Convertible Preferred Units for net proceeds of $4.3 million.

The rights, preferences and privileges of the Convertible Preferred Units were as follows:

Liquidation

In the event of any liquidation, dissolution or winding up of affairs of the Company (including a change of control), distributions would have first been made to holders of the Series B Convertible Preferred Units equal to their original issue price plus any declared but unpaid dividends. The Series B Convertible Preferred Units were issued at $3.00 per unit. After distribution to the Series B Convertible Preferred Unit holders, the holders of the Series A Convertible Preferred Units as a class would have received a distribution equal to their original issue price plus any declared but unpaid dividends. The Series A-1, A-2, A-3 and A-4 Convertible Preferred Units were issued at $1.00, $1.20, $1.38 and $1.587, respectively. Next, the common unit holders and incentive unit holders would have received a distribution in proportion to the respective aggregate number of common units and incentive units held by each member until each common and incentive unit holder had received, on a per unit basis, the sum of the amounts distributed to the holders of the Series A Convertible Preferred Units less any amounts distributed for dividends. Next, the Series A Convertible Preferred Unit holders, the common unit holders, and incentive unit holders would have received a distribution in proportion to the respective aggregate number of Series A Convertible Preferred Units, common units, and incentive units held by each member until each Series A Convertible Preferred Unit holder, common unit holder, and incentive unit holder had received, on a per unit basis, the sum of the amounts distributed to the holders of the Series B Convertible Preferred Units less any amounts distributed for dividends. Any remaining amount available for distribution would have been made to holders of the Convertible Preferred Units, on an as converted basis, common units and incentive units, in proportion to the respective number of units held by each member.

Incentive unit holders would have participated in distributions as described above only after the distribution met the strike price with respect to such unit. The strike price is an amount per incentive unit determined by the Board of Directors based on the amount of distributions that the holders of a common unit would have been entitled to receive in a hypothetical liquidation of the Company on the date of issuance of the

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

9. Convertible Preferred Units (Continued)

incentive unit in which the Company sold its assets at fair market value, satisfied its liabilities and distributed the net proceeds to the holders of units in liquidation of the Company. Incentive unit holders would have participated in distributions only after the distribution met the strike price with respect to such unit. The Board of Directors had the discretion to determine the extent to which an incentive unit would have been excluded from participating in distributions.

Conversion

The Convertible Preferred Units could have been converted by the holder at any time into a number of common units equal to the number of Convertible Preferred Units being converted. Upon either the closing of a firm-commitment underwritten public offering pursuant to an effective registration statement under the Securities Act of 1933, as amended, in which the price per common unit was at least $3.00 per share and the aggregate gross proceeds were at least $40 million or the occurrence of an event, specified by vote or written consent of the supermajority interest (members holding at least 70% of the outstanding Convertible Preferred Units, voting or consenting together as a single class on an as-converted basis), all outstanding Convertible Preferred Units would have been automatically converted into a number of common units equal to the number of Convertible Preferred Units at the then applicable conversion rate which was equal to their issuance price noted above. Further, the automatic conversion of the Series B Convertible Preferred Units would not have been automatically converted into common units without the affirmative vote or written consent of the members holding at least 61% of the outstanding Series B Convertible Preferred Units.

Dividends

Dividends, if declared, were payable to each Convertible Preferred Unit holder as follows:

Convertible Preferred Unit	Dividend Per Unit
B	$ 0.240
A-4	0.127
A-3	0.110
A-2	0.096
A-1	0.080

Dividends were subject to appropriate adjustment in the event of any unit dividend, unit split, combination or other similar recapitalization with respect to the units. Dividends were payable when and as declared by the Board of Directors, were not cumulative and did not accrue to unit holders by reason of the fact that they are not declared or paid in any calendar year. No dividends have been declared by the Board of Directors since inception.

Voting

On any matter to be approved by the unit holders, holders of Convertible Preferred Units had the right to cast a number of votes equal to the number of common units into which the Convertible Preferred Units held by such holder would have converted.

Redemption

The Company's Convertible Preferred Units have been classified as temporary equity on the accompanying consolidated balance sheets in accordance with authoritative guidance for the classification and measurement of redeemable securities as the Convertible Preferred Units are redeemable upon the

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

9. Convertible Preferred Units (Continued)

occurrence of a liquidation event. The carrying value of the Company's Convertible Preferred Units was not adjusted because a liquidation event was not probable and did not occur.

The Company has evaluated the Convertible Preferred Units and determined that they should be considered an "equity host" and not a "debt host." The evaluation was necessary to determine if any embedded features required bifurcation and separate accounting as a derivative financial instrument. The Company's analysis was based on a consideration of the economic characteristics and risks and more specifically, evaluated all the stated and implied substantive terms and features including (i) whether the Convertible Preferred Unit included redemption features, (ii) how and when any redemption features could have been exercised, (iii) whether the Convertible Preferred Units were entitled to dividends, (iv) the voting rights of the Convertible Preferred Unit and (v) the existence and nature of any conversion rights. As a result of its evaluation that the Convertible Preferred Unit is an "equity host," the various embedded conversion options are not considered a separate, embedded derivative.

10. Reorganization and Convertible Preferred Stock

In connection with the Reorganization:

- Holders of Scholar Rock, LLC Series B Convertible Preferred Units received one share of Scholar Rock Holding Corporation Series B Convertible Preferred Stock for each outstanding Series B Convertible Preferred Units held immediately prior to the Reorganization, with an aggregate of 13,526,994 shares of Scholar Rock Holding Corporation Series B Convertible Preferred Stock issued in the Reorganization;
- Holders of Scholar Rock, LLC Series A-4 Convertible Preferred Units received one share of Scholar Rock Holding Corporation Series A-4 Convertible Preferred Stock for each outstanding Series A-4 Convertible Preferred Units held immediately prior to the Reorganization, with an aggregate of 3,906,738 shares of Scholar Rock Holding Corporation Series A-4 Convertible Preferred Stock issued in the Reorganization;
- Holders of Scholar Rock, LLC Series A-3 Convertible Preferred Units received one share of Scholar Rock Holding Corporation Series A-3 Convertible Preferred Stock for each outstanding Series A-3 Convertible Preferred Units held immediately prior to the Reorganization, with an aggregate of 5,579,709 shares of Scholar Rock Holding Corporation Series A-3 Convertible Preferred Stock issued in the Reorganization;
- Holders of Scholar Rock, LLC Series A-2 Convertible Preferred Units received one share of Scholar Rock Holding Corporation Series A-2 Convertible Preferred Stock for each outstanding Series A-2 Convertible Preferred Units held immediately prior to the Reorganization, with an aggregate of 5,066,915 shares of Scholar Rock Holding Corporation Series A-2 Convertible Preferred Stock issued in the Reorganization;
- Holders of Scholar Rock, LLC Series A-1 Convertible Preferred Units received one share of Scholar Rock Holding Corporation Series A-1 Convertible Preferred Stock for each outstanding Series A-1 Convertible Preferred Units held immediately prior to the Reorganization, with an aggregate of 2,000,000 shares of Scholar Rock Holding Corporation Series A-1 Convertible Preferred Stock issued in the Reorganization;
- Holders of Scholar Rock, LLC Common Units received one share of Scholar Rock Holding Corporation common stock for each outstanding Common Unit held immediately prior to the Reorganization, with an aggregate of 4,576,500 shares of common stock issued in the Reorganization;

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

10. Reorganization and Convertible Preferred Stock (Continued)

- Holders of Scholar Rock, LLC vested and unvested incentive units, irrespective of any strike price or voting rights on any such outstanding incentive units, exchanged such incentive units for an equal number of shares of common stock or restricted common stock, respectively. The restricted common stock was issued with the same vesting terms as the unvested incentive units held immediately prior to the Reorganization. An aggregate of 6,758,945 shares of common stock and restricted common stock were issued in the Reorganization; and

- The outstanding warrant to purchase 21,739 shares of Series A-3 Convertible Preferred Units at a purchase price of $1.38 per share was converted to a warrant to purchase 21,739 shares of Series A-3 Convertible Preferred Stock at the same purchase price.

In evaluating the Reorganization, the Company considered that (i) with the exception of holders of Incentive Units, there were no changes in ownership interest held by each stockholder as a result of the Reorganization, (ii) the changes in the overall ownership interest of the Company resulting from the changes in ownership interest related to the holders of Incentive Units as a result of the Reorganization is not significant and (iii) the Reorganization occurred between a parent and wholly owned subsidiary, where the parent, Scholar Rock, LLC, had no substantive operations. Based on this evaluation, the Company determined that the Reorganization lacked economic substance and should be accounted for in a manner consistent with a common control transaction. Similarly, there was no change in fair value between the stockholders, individually or as a class, the Company determined that the exchange of shares occurring in the Reorganization should be accounted for as a modification of equity securities.

11. Convertible Preferred Stock and Common Stock

Convertible Preferred Stock

The Series A-1, A-2, A-3 and A-4 Convertible Preferred Stock issued in connection with the Reorganization are collectively referred to as Series A Convertible Preferred Stock.

On December 22, 2017, the Company entered into the Series C Preferred Stock Purchase Agreement pursuant to which the Company issued 13,055,555 shares of Series C Convertible Preferred Stock for net proceeds of $46.9 million.

The Series A Convertible Preferred Stock, Series B Convertible Preferred Stock and the Series C Convertible Preferred Stock are collectively referred to as Convertible Preferred Stock.

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

11. Convertible Preferred Stock and Common Stock (Continued)

As of December 31, 2017, the Convertible Preferred Stock consisted of the following (in thousands, except share amounts):

	As of December 31, 2017				
	Preferred Shares Authorized	Preferred Shares Issued and Outstanding	Carrying Value	Liquidation Preference	Common Shares Issuable Upon Conversion
Series A-1 convertible preferred stock	2,000,000	2,000,000	$ 1,942	$ 2,000	2,000,000
Series A-2 convertible preferred stock	5,066,915	5,066,915	6,004	6,080	5,066,915
Series A-3 convertible preferred stock	5,601,448	5,579,709	7,698	7,700	5,579,709
Series A-4 convertible preferred stock	3,906,739	3,906,738	6,198	6,200	3,906,738
Series B convertible preferred stock	13,526,994	13,526,994	40,500	40,581	13,526,994
Series C convertible preferred stock	13,055,555	13,055,555	46,890	47,000	13,055,555
	43,157,651	43,135,911	$ 109,232	$ 109,561	43,135,911

The terms of the Convertible Preferred Stock are as follows:

Liquidation

In the event of any liquidation, dissolution or winding up of affairs of the Company (or upon a deemed liquidation event), distributions are first made to holders of the Series C Convertible Preferred Stock equal to the greater of (i) their original issuance price, plus any declared but unpaid dividends or (ii) the amount that the holder would be entitled upon conversion into common stock. The original issue price of the Series C Convertible Preferred Stock was $3.60 per share. After distribution to the Series C Convertible Preferred Stockholders, distributions are made to holders of the Series B Convertible Preferred Stock equal to the greater of (i) their original issue price plus any declared but unpaid dividends or (ii) the amount that the holder would be entitled upon conversion into common stock. The original issue price of Series B Convertible Preferred Stock is $3.00 per share. After distribution to the Series B Convertible Preferred Stockholders, the holders of the Series A Convertible Preferred Stock, as a class, will receive a distribution equal to the greater of (i) their original issue price plus any declared but unpaid dividends or (ii) the amount that the holder would be entitled upon conversion into common stock. The original issue price of the Series A-1, A-2, A-3 and A-4 Convertible Preferred Stock is $1.00, $1.20, $1.38 and $1.587, respectively. Upon completion of the preferential payments to holders of Convertible Preferred Stock, all of the remaining assets shall be distributed among the holders of Convertible Preferred Stock and common stock pro rata based on the number of shares of common stock held by each, assuming conversion of all outstanding shares of Convertible Preferred Stock.

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

11. Convertible Preferred Stock and Common Stock (Continued)

A deemed liquidation event is defined as a merger (unless the shares of capital stock prior to the transaction represent a majority of the post merger voting rights) or the sale or transfer of substantially all of the assets of the Company unless the holders of at least (i) 80% of the Convertible Preferred Stock, (ii) 61% of the Series B Convertible Preferred Stock and (iii) a majority of the Series C Convertible Preferred Stock elect otherwise.

Conversion

Shares of Convertible Preferred Stock may be converted by the holder at any time into a number of common shares equal to such number of shares as determined by dividing the original issue price by the conversion price in effect at the time. The conversion price is equal to the original issue price for all Convertible Preferred Stock as of December 31, 2017. The conversion price is subject to adjustments in the event of any stock dividend, stock split, combination or other similar recapitalization and other adjustments as set forth in the Company's certificate of incorporation. Upon either the closing of a firm-commitment underwritten public offering pursuant to an effective registration statement under the Securities Act of 1933, as amended, in which the aggregate gross proceeds are at least $55 million or the occurrence of an event, specified by vote or written consent of the supermajority interest (members holding at least 80% of the outstanding Convertible Preferred Stock, voting or consenting together as a single class on an as-converted basis), all outstanding Convertible Preferred Stock will be automatically converted into a number of common shares equal to the number of Convertible Preferred Stock at the then applicable conversion rate which is equal to their original issue price noted above.

Dividends

Dividends, if declared, are payable to each holder of Convertible Preferred Stock as follows:

Convertible Preferred Stock	Dividend Per Share
C	$ 0.288
B	0.240
A-4	0.127
A-3	0.110
A-2	0.096
A-1	0.080

Dividends are subject to appropriate adjustment in the event of any stock split, stock dividend, combination or other similar recapitalization with respect to the shares. Dividends are payable when and as declared by the Board of Directors, are not cumulative and do not accrue to shareholders by reason of the fact that they are not declared or paid in any calendar year. No dividends have been declared or paid by the Company to the holders of Convertible Preferred Stock since issuance of the Convertible Preferred Stock.

Voting

On any matter to be approved by the stockholders, holders of Convertible Preferred Stock have the right to cast a number of votes equal to the number of shares of common stock into which the shares of Convertible Preferred Stock held by such holder convert.

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

11. Convertible Preferred Stock and Common Stock (Continued)

Redemption

The Company's Convertible Preferred Stock has been classified as temporary equity on the accompanying consolidated balance sheets in accordance with authoritative guidance for the classification and measurement of redeemable securities as the Convertible Preferred Stock is redeemable upon the occurrence of a deemed liquidation event. The carrying value of the Company's Convertible Preferred Stock is not being adjusted because a deemed liquidation event is not probable.

The Company has evaluated the Convertible Preferred Stock and determined that they should be considered an "equity host" and not a "debt host." The evaluation was necessary to determine if any embedded features require bifurcation and separate accounting as a derivative financial instrument. The Company's analysis was based on a consideration of the economic characteristics and risks and more specifically, evaluated all the stated and implied substantive terms and features including (i) whether the Convertible Preferred Stock includes redemption features, (ii) how and when any redemption features could be exercised, (iii) whether the Convertible Preferred Stock is entitled to dividends, (iv) the voting rights of the Convertible Preferred Stock and (v) the existence and nature of any conversion rights. As a result of its evaluation that the Convertible Preferred Stock is an "equity host," the various embedded conversion options are not considered a separate, embedded derivative.

Common Stock

The voting, dividend and liquidation rights of the holders of common stock are subject to and qualified by the rights, powers and preferences of the holders of Convertible Preferred Stock. The common stock has the following characteristics:

Voting

The holders of shares of common stock are entitled to one vote for each share of common stock held at any meeting of stockholders and at the time of any written action in lieu of a meeting.

Dividends

The holders of shares of common stock are entitled to receive dividends, if and when declared by the Company's board of directors. Cash dividends may not be declared or paid to holders of shares of common stock until all unpaid dividends on Convertible Preferred Stock have been paid in accordance with their terms. No dividends have been declared or paid by the company to the holders of common stock since the issuance of the common stock.

Liquidation

After payment of the respective liquidation preferences to the holders of Convertible Preferred Stock, the holders of common stock are entitled to share ratably in the Company's remaining assets available for distribution to its stockholders in the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company or upon the occurrence of a deemed liquidation event.

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

11. Convertible Preferred Stock and Common Stock (Continued)

Shares reserved for future issuance

As of December 31, 2017, the Company had reserved common shares for the conversion of outstanding Convertible Preferred Stock and for future issuance under the 2017 Stock Option and Incentive Plan (the "2017 Plan") as follows:

	Shares
Common shares reserved for conversion of convertible preferred stock outstanding	43,135,911
Common shares reserved for conversion of convertible preferred shares issuable upon exercise of a warrant	21,739
Common shares reserved for future issuance under the 2017 Plan	3,105,333
	46,262,983

12. Equity-Based Compensation

Prior to the Reorganization, the Company's operating agreement, as amended and restated, provided for the granting of incentive units to employees, officers, directors and consultants under the 2013 Equity Incentive Plan (the "2013 Plan"), as determined by the Board of Directors. At December 31, 2016, 6,086,500 incentive units were authorized to be granted under the 2013 Plan.

2013 Equity Incentive Plan

The terms of the incentive units granted prior to the Reorganization were determined by the Board of Directors and included vesting, forfeiture, repurchase and other provisions. The Board of Directors also determined whether each incentive unit granted was a voting incentive unit, having the right to vote on any matter the common units had the right to vote on, or a non-voting incentive unit and not carry the right to vote. At December 31, 2016, there were 3,765,771 incentive units that were voting, and 1,253,687 incentive units that were non-voting, respectively. Incentive units had no rights to dividends and were entitled to distributions. Incentive unit holders were not required to purchase or "exercise" their incentive units in order to receive such distributions. However, distributions to incentive unit holders began only after the cumulative amount distributed to common unit holders exceeded the strike price with respect to such incentive unit. Distributions were entitled to be made to incentive unit holders whether vested or unvested. Unvested distributions were to be held by the Company until the incentive units vest, at which time they would be released to the incentive unit holder. The Board of Directors had the discretion to determine the extent to which an incentive unit would be excluded from participating in distributions. Unless otherwise approved by the Board of Directors, the incentive units generally vested over a four year period with the first 25% vesting following 12 months of employment or service and the remaining incentive units vesting in equal quarterly installments over the following 36 months. The incentive units had no contractual term.

In connection with the issuance of each incentive unit, the Board of Directors set a strike price based on the amount of distributions that the holders of a common unit would be entitled to receive in a hypothetical liquidation of the Company on the date of issuance of the incentive unit in which the Company sold its assets at fair market value, satisfied its liabilities and distributed the net proceeds to the holders of units in liquidation of the Company.

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

12. Equity-Based Compensation (Continued)

A summary of the Company's incentive unit activity under the 2013 Plan and related information is as follows:

	Number of Units	Weighted Average Fair Value per Unit	Weighted Average Strike Price per Unit
Outstanding at December 31, 2016	5,019,458	$ 0.71	$ 0.38
Granted	1,774,462	1.27	0.92
Forfeited	(34,975)	1.00	0.78
Exchanged for common stock and restricted common stock pursuant to the Reorganization	(6,758,945)	0.85	0.52
Outstanding at December 31, 2017	—		

The weighted average grant date fair value for incentive units granted in 2016 was $0.98 per unit.

A summary of vested incentive units is as follows:

	Number of Units
Vested at December 31, 2016	1,815,362
Vesting through the date of the Reorganization	1,403,140
Vested as of the Reorganization	3,218,502

The total fair value of employee incentive units vested during 2016 and during 2017 through the date of the Reorganization was $391,000 and $1.1 million, respectively.

Incentive Unit Compensation Expense Assumptions

In 2017, the fair value of incentive units granted to both employees and non-employees was determined using the market approach, including the guideline public company method and a precedent transaction method which "backsolves" to a preferred price. Equity value was allocated to the common units, incentive units and convertible preferred units using either an option-pricing method ("OPM"), or a hybrid method, which is a hybrid between the OPM and the probability-weighted expected return method. The OPM treats common securities and preferred securities as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under this method, the common and incentive units have value only if the funds available for distribution to members exceed the value of the preferred security liquidation preference at the time of the liquidity event, such as a strategic sale or a merger. The hybrid method estimates the probability-weighted value across multiple scenarios but uses the OPM to estimate the allocation of value within at least one of the scenarios. In addition to the OPM, the hybrid method considers an IPO scenario in which preferred shares are assumed to convert to common stock. The future value of the incentive units

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

12. Equity-Based Compensation (Continued)

in the IPO scenario is discounted back to the valuation date at an appropriate risk adjusted discount rate. In the hybrid method, the present value indicated for each scenario is probability weighted to arrive at an indication of value for the common and incentive units.

The following assumptions were used in determining the fair value of incentive units granted to both employees and non-employees during 2016 and 2017:

	2016	2017
Risk-free interest rate	1.61%	1.48% - 1.97%
Expected dividend yield	—	—
Expected term (years to liquidity)	3.61	2.63 - 3.23
Expected volatility	69%	71% - 77%

The Company determined the risk-free rate based on constant maturing U.S. Treasury rates commensurate with the expected term, not seasonally adjusted. The Company has never paid, and does not anticipate paying, any cash dividends in the foreseeable future, and therefore uses an expected dividend yield of zero. Incentive units do not have an explicit contractual term. The Company, therefore, based its assumption on a weighted average of various liquidation scenarios, which would require a distribution to the incentive units. Since the Company was privately held as of the date of these financial statements, it does not have relevant historical data to support its expected volatility. As such, the Company has used a weighted-average of expected volatility based on the volatilities of a representative group of publicly traded biopharmaceutical companies. For purposes of identifying representative companies, the Company considered characteristics such as stage of development and area of therapeutic focus. Forfeitures of incentive units are accounted for when they occur, pursuant to the Company's early adoption of ASU 2016-09, *Improvements to Employee Share-Based Payment Accounting* ("ASU 2016-09"). In 2016 and 2017, the Company recorded a reduction in compensation expense for forfeitures of incentive units of $18,000 and $15,000, respectively, in accordance with ASU 2016-09. No prior periods were retrospectively adjusted as the impact of this change in accounting was immaterial to the financial statements.

Incentive Units Granted to Non-Employees

During the years ended December 31, 2016 and 2017, the Company granted incentive units to non-employees. The Company valued these incentive units based on their fair value on the date of grant.

The unvested incentive units granted to non-employees have been re-measured using the Company's estimate of fair value at each vesting date through the remaining vesting period. Equity-based compensation expense of $22,000 and $60,000 was recorded for the years ended December 31, 2016 and 2017, respectively relating to non-employee incentive unit awards.

Compensation Expense related to Incentive Units

The Company recorded equity-based compensation expense for incentive units granted to employees, directors and non-employees of $556,000 and $1,059,000 for the years ended December 31, 2016 and 2017, respectively.

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

12. Equity-Based Compensation (Continued)

2017 Stock Option and Incentive Plan

The 2017 Plan provides for the grant of incentive stock options, non-qualified stock options, restricted stock awards, unrestricted stock awards and restricted stock units. The 2017 Plan is administered by the Board of Directors, or at the discretion of the Board of Directors, by a committee of the board comprised of not less than two directors. The exercise prices, vesting and other restrictions are determined at the discretion of the Board of Directors, or their committee if so delegated, except that the exercise price per share of stock options may not be less than 100% of the fair market value of the share of common stock on the date of grant and the term of stock option may not be greater than ten years. The number of shares initially reserved for issuance under the 2017 Plan was 9,864,278 shares of common stock. The shares of common stock underlying any awards that are forfeited, cancelled, repurchased or are otherwise terminated by the Company under the 2017 Plan will be added back to the shares of common stock available for issuance under the 2017 Plan.

Reorganization

As part of the Reorganization, the Company terminated the 2013 Plan and instituted the 2017 Plan. At December 31, 2017, 9,864,278 shares were authorized to be granted under the 2017 Plan.

Pursuant to the Reorganization, all vested and unvested incentive units granted under the 2013 Plan which were outstanding immediately prior to the Reorganization, irrespective of any strike price or voting rights on any such outstanding incentive units, were exchanged for an equal number of shares of common stock or restricted common stock, respectively, under the 2017 Plan. The restricted common stock was issued with the same vesting terms as the unvested incentive units held immediately prior to the Reorganization.

The following table summarizes the common stock and restricted common stock activity under the 2017 Plan:

	Number of Shares	Weighted Average Fair Value per Share at Issuance
Common stock and restricted common stock issued as part of the Reorganization	6,758,945	$ 2.02
Vested as of and after the Reorganization	3,275,708	$ 2.02
Restricted common stock as of December 31, 2017	3,483,237	$ 2.02

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

12. Equity-Based Compensation (Continued)

The Company accounted for the exchange of incentive units in Scholar Rock, LLC for common stock and restricted common stock of Scholar Rock Holding Corporation, as a modification in accordance with the requirements of ASC 718. The Company determined the fair value of the common stock and restricted common stock using the market approach, including the guideline public company method and the precedent transaction method which "backsolves" to a preferred price. Accordingly, the Company determined there was an excess fair value of the replacement awards over the fair value of the incentive units exchanged in connection with the Reorganization, which resulted in incremental compensation expense of $1.4 million of which $423,000 was recognized in 2017. The incremental fair value related to vested awards was recognized immediately as compensation expense. The incremental fair value of unvested awards and any remaining unrecognized compensation of the original awards will be recognized as compensation expense over the remaining vesting period.

As of December 31, 2017, the Company had unrecognized equity-based compensation expense of $4.4 million related to restricted common stock issued to employees, which is expected to be recognized over 2.9 years.

Total Equity-based Compensation Expense

During the years ended December 31, 2016 and 2017, the Company recorded compensation expense related to incentive units, common stock, and restricted common stock for employees and non-employees, which was allocated as follows in the consolidated statements of operations (in thousands):

	Year Ended December 31,	
	2016	2017
Research and development expense	$ 264	$ 704
General and administrative expense	292	778
	$ 556	$ 1,482

Performance-based Awards

The Company had granted incentive units, which contain both performance-based and service-based vesting criteria. Milestone events are specific to the Company's corporate goals, including but not limited to certain research and funding milestones. Equity-based compensation expense associated with these performance-based awards is recognized if the performance condition is considered probable of achievement using management's best estimates. Consistent with all incentive units, as part of the Reorganization, the incentive units granted with performance-based and service-based vesting criteria were exchanged for restricted common stock with the same vesting terms. In 2016 and 2017, compensation expense of $101,000 and $91,000 was recorded related to performance-based awards.

13. Income Taxes

The Company has not recorded a current or deferred tax provision for the years ended December 31, 2016 and 2017.

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

13. Income Taxes (Continued)

The effective income tax rate differed from the amount computed by applying the federal statutory rate to our loss before income taxes as follows:

	For Year Ended December 31,	
	2016	2017
Tax effected at statutory rate	34.0%	34.0%
State taxes	6.0	8.1
Stock compensation	(1.2)	(2.0)
Non-taxable income	0.5	0.3
Non deductible expenses	0.0	0.1
Impact of federal rate change on net deferred taxes	0.0	(27.0)
Federal research and development credits	1.2	2.3
Change in valuation allowance	40.5	(15.8)
	0.0%	0.0%

Deferred tax assets (liabilities) consist of the following at December 31, 2016 and 2017 (in thousands):

	As of December 31,	
	2016	2017
Long-term net deferred tax assets:		
Reserve and accruals	$ 341	$ 340
Net operating loss carryforwards	10,532	13,734
Deferred rent	355	190
Tax credits	847	1,665
Fixed and intangible assets	(235)	(128)
Total long-term net deferred tax assets:	11,840	15,801
Valuation allowance	(11,840)	(15,801)
Total net deferred tax assets	$ —	$ —

Total Net Deferred Tax Assets

Deferred tax assets are reduced by a valuation allowance if, based on the weight of available positive and negative evidence, it is more likely than not that some portion or all of the deferred tax assets will not be realized. For the years ended December 31, 2016 and 2017, the valuation allowance for deferred tax assets increased by $6.6 million and $4.0 million, respectively. This increase mainly relates to the establishment of valuation allowance against the Company's net domestic deferred tax assets in connection with net operating losses generated in each year, and the recording of additional net operating losses and credit carryforwards, partially offset by a revaluation of the federal deferred tax assets in 2017 based on the tax law change discussed below. As of December 31, 2017, the Company had $50.4 million and

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

13. Income Taxes (Continued)

$49.8 million of Federal and state operating loss carryforwards respectively, which begin to expire in 2034. These loss carryforwards are available to reduce future taxable income, if any. In addition, as of December 31, 2017, the Company had $1.1 million and $0.7 million of Federal and state credit carryovers which begin to expire in 2034 and 2020, respectively. These loss and credit carryforwards are subject to review and possible adjustment by the appropriate taxing authorities. The amount of loss and credit carryforwards that may be utilized in any future period may be limited based upon changes in the ownership of the company's ultimate parent.

On December 22, 2017, the Tax Cuts and Jobs Act ("TCJA") was signed into law. The TCJA significantly revises the U.S. corporate income tax by, among other things, lowering corporate income tax rates, implementing a hybrid territorial tax system, and imposing a one-time repatriation tax on foreign cash and earnings. The Company has assessed the impact of this law change on the realization of its deferred tax assets and the remeasurement of the deferred tax assets and liabilities to the lower statutory federal US tax rate. The remeasurement of the deferred tax assets and liabilities did not result in a change to the current year income tax provision or balance sheet, as an offsetting adjustment was also recorded to the valuation allowance maintained on these accounts. The Company also had no investments in specified foreign corporations as of December 31, 2017. The Company's assessment of the TCJA is ongoing and assumptions and estimates may need to be revised based on new information available and as additional transition guidance is related by the IRS. The assessment is expected to be completed no later than the fourth quarter of calendar year 2018.

The Company follows the provisions of ASC 740-10, "Accounting for Uncertainty in Income Taxes," which specifies how tax benefits for uncertain tax positions are to be recognized, measured, and recorded in financial statements; requires certain disclosures of uncertain tax matters; specifies how reserves for uncertain tax positions should be classified on the balance sheet; and provides transition and interim period guidance, among other provisions. As of December 31, 2016 and 2017, the Company has not recorded any amounts for uncertain tax positions. The Company's policy is to recognize interest and penalties accrued on any uncertain tax positions as a component of income tax expense, if any, in its statements of income. For the years ended December 31, 2016 and 2017, no estimated interest or penalties were recognized on uncertain tax positions. The Company has not yet conducted a study of its research and development credit carry forwards. Such a study may result in an adjustment to the Company's research and development credit carryforwards; however, until a study is completed and any adjustment is known, no amount is being presented as an uncertain tax position. A full valuation allowance has been provided against the Company's research and development credits, and, if an adjustment is required, this adjustment would be offset by an adjustment to the valuation allowance. Thus, there would be no impact to the balance sheet or statement of operations and comprehensive loss if an adjustment were required.

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

14. Commitments and Contingencies

Operating Leases

Office Equipment

The Company leases certain office equipment under non-cancelable operating leases. Total costs for such leases was $11,000 and $11,000 for the year ended December 31, 2016 and 2017, respectively. The future minimum lease payments for these leases as of December 31, 2017 are as follows (in thousands):

Year ending December 31,	
2018	$ 7
Total minimum lease payments	$ 7

Facility Lease

In March 2015, the Company entered into a 5-year facility lease for approximately 11,600 square feet of space at 620 Memorial Drive, Cambridge, Massachusetts. The lease provided for a landlord contribution towards normal tenant improvements which was capitalized and recorded as deferred rent and amortizes as a reduction to rent expense over the lease term.

Minimum annual rent payments under this lease for the remaining three years, excluding operating expenses and taxes which are not fixed for future periods as of December 31, 2017, are as follows (in thousands):

Year ending December 31,	
2018	$ 689
2019	709
2020	604
Total minimum lease payments	$ 2,002

In accordance with the lease, the Company entered into a cash-collateralized irrevocable standby letter of credit in the amount of $205,000 naming the landlord as beneficiary and the amount is included in restricted cash in the consolidated balance sheets. The Company has a single option to extend the lease by an additional three years at market rental rates.

Legal Proceedings

The Company is not currently a party to any material legal proceedings.

15. Loan Payable

In August 2015, the Company entered into a Loan and Security Agreement with Silicon Valley Bank ("SVB"), which provided the Company an equipment line of credit of up to $2 million to finance the purchase of eligible equipment. Pursuant to the agreement, SVB was obligated to make up to five equipment advances, each in an amount of at least $100,000 during the draw period which began on the

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

15. Loan Payable (Continued)

effective date, August 11, 2015, and ended on June 30, 2016. The Company borrowed $677,000 against the line of credit as of December 31, 2015.

In August 2016, the Company entered into the First Amendment to the Loan and Security Agreement ("First Amendment"), which provided the Company an extension of the draw period to December 31, 2016. The Company borrowed $1.3 million in 2016 against the line of credit, which fulfilled the maximum credit line of $2 million at December 31, 2016. The loan balance at December 31, 2017 was $1.0 million.

In addition, the First Amendment expanded the operating accounts provision to require the Company to, at all times, have on deposit in operating, depository and securities accounts maintained with SVB or SVB's affiliates, unrestricted and unencumbered cash in an amount equal to the lesser of (a) 105% of the then outstanding obligations or (b) 100% of the dollar value of the Company's accounts at all financial institutions. The Company was in compliance with this covenant at December 31, 2017, which required the Company to hold a minimum of $1.1 million in accounts with SVB.

Amounts borrowed bear interest at an annual prime rate as published in the Wall Street Journal less 0.25%, which, at December 31, 2016 and 2015 was 3.50% and 3.25%, respectively. For each advance, interest only payments were due and paid through June 2016. Principal and interest payments commenced on July 1, 2016 for a period of 36 months. A final payment fee equal to 4% of the aggregate advances is also due on June 1, 2019. The final payment is being accrued over the term of the loan and is being recorded as interest expense.

Future principal payments on this loan as of December 31, 2017 are as follows (in thousands):

Year ending December 31,	
2018	$ 667
2019	365
	$ 1,032

The Company incurred costs on behalf of the lender recorded as a debt discount of $53,000 and incurred issuance costs recorded as deferred financing costs of $19,000, both of which are recorded as a deduction from the carrying amount of the loan and are being amortized as interest expense over the term of the loan.

The Company has granted SVB a security interest in the equipment financed under the agreement. The Loan and Security Agreement contains negative covenants restricting the Company's activities, including limitations on dispositions, change in business ownership or location, mergers or acquisitions, incurring indebtedness or liens, paying dividends or making investments and certain other business transactions.

The Company has the option to prepay the loan and upon prepayment will pay the outstanding principal and interest, the final payment fee and the prepayment premium. The prepayment premium is equal to 1% of the then outstanding principal if made on or prior to the second anniversary and 0.5% of the principal balance if made after August 11, 2017. The Company evaluated the prepayment option ("Call Option") to determine if the features should be separated from the loan and recognized as a derivative under ASC Topic

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

15. Loan Payable (Continued)

815, *Derivatives and Hedging*, (ASC 815), concluding that the Call Option is clearly and closely related to the Loan and does not meet the criteria for bifurcation from the loan.

In the event of a default, and during such an event, the interest rate will increase by 5% per year. The Company evaluated this increase to determine if it should be separated from the loan and recognized as a derivative. The Company determined that it met the requirements of ASC 815 in that a default could occur due to non-credit related matters. Therefore, the economic characteristics and risks are not closely related to that of the debt, and the interest rate feature requires bifurcation from the loan, however, the value associated with this embedded feature is *de minimis*, and thus not recorded at the issuance date through December 31, 2017.

The Company recorded total interest expense for this loan of $42,000 and $101,000 for the years ended December 31, 2016 and 2017, respectively.

16. Option and License Agreement

Overview

On December 17, 2013, the Company entered into an option and license agreement with Janssen. Under this agreement, the Company conducted drug discovery research to identify molecules with either one or two pharmacological profiles, and Janssen funded such research during a two-year period beginning on December 17, 2013. During the two-year period, the Company granted Janssen a research license to research, develop and use the collaboration molecule(s) and/or lead molecule(s) for use in the field and in the territory. Janssen was not granted a license to commercialize any collaboration molecule, lead molecule or licensed product unless and until Janssen exercised its license option in accordance with the agreement. The Company also granted an option to exclusively license molecules identified during the term that meet either one or both pharmacological profiles. If Janssen were not to exercise its license option by the end of this period, the term could be extended for up to one additional year by mutual written agreement of the parties. The activities under the agreement were governed by a program committee, which met quarterly and consisted of three members each from the Company and Janssen.

The Company received funding from Janssen based on a set rate per annual full-time equivalent personnel working on the research plus actual external costs incurred by the Company up to a maximum dollar amount as defined in the agreement. Costs approximated the funding provided. All amounts billed by the Company to Janssen were made quarterly, in arears, based on time and actual costs incurred. There are no refund provisions in the agreement. Pursuant to the contract, if a molecule was not identified under either pharmacological profile, or Janssen did not exercise its option to the molecule(s) identified, the agreement would expire at the end of the term, December 17, 2015, unless extended by the parties.

At any time during the two-year collaboration period, Janssen held the right to exercise its license option for molecules with either or both pharmacological profiles by providing written notice to the Company and paying an option exercise fee of $1 million per option exercised (up to two). Once Janssen exercises its option, the Company's obligations under the program plan for the molecule and related pharmacological profile cease and Janssen assumes full responsibility for further development of the molecules at its sole cost. The Company is obligated to transfer any and all manufacturing related activities to Janssen at Janssen's cost. In addition, after Janssen exercises its option, it is obligated to pay the Company certain development milestones totaling up to $25 million and regulatory milestones totaling up to $97 million for each pharmacological profile as detailed in the agreement during the development period and through

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

16. Option and License Agreement (Continued)

successful regulatory approval. Development milestones are triggered upon the achievement of specified development criteria or dosing of a specified number of patients in phases of clinical trials. Regulatory milestones are triggered upon approval to market a product candidate by the United States Food and Drug Administration ("FDA") or other global regulatory authorities. Additional commercial milestone payments totaling up to $130 million for each pharmacological profile are eligible to be earned as certain sales thresholds are achieved by Janssen and royalties are also required to be paid by Janssen to the Company based on annual net sales thresholds, based on Janssen's sales of a product derived from the collaboration molecule(s). The next potential milestone the Company may be entitled to receive under the agreement is a milestone payment of $2 million upon achievement of a development milestone.

Accounting Analysis

The Company accounts for this agreement pursuant to ASC Topic 605-25*, Revenue Recognition — Multiple Element Arrangements,* or ASC 605-25. The Company identified the following deliverables in this agreement:

- a non-exclusive research and development license to Janssen over the initial two-year term of the agreement ("research license deliverable");
- the Company's obligation to provide research services in accordance with the Research Plan ("research services deliverable") and
- the Company's participation on the Program Committee ("program committee deliverable").

The Company evaluated whether the exclusive option to obtain a commercialization and development license at the end of the collaboration period constituted a deliverable at the inception of the arrangement. The Company determined that the option is "substantive" and therefore not considered a deliverable at the inception of the arrangement.

The Company determined that the research license deliverable did not have standalone value from the research services to be provided by the Company and, therefore it will be combined with the research services as a single unit of account. The Company determined that the program committee deliverable has standalone value from the research license and research services unit of account. However, the Company determined that the best estimate of selling price of the program committee deliverable is *de minimis*. Based on the foregoing, the Company has accounted for the research license, research services and the program committee deliverables as a single combined unit of account. The non-contingent arrangement consideration has been allocated to the combined unit of account. The Company also considered whether the future development, regulatory and commercial milestones were substantive at the inception of the arrangement. Although there is substantive uncertainty that the milestone events will be achieved and the achievement of the milestones would result in additional payments being due to the Company, the milestones are not triggered by events that will be achieved based solely in whole or in part on the Company's performance or on the occurrence of a specific outcome resulting from the Company's performance. As a result, none of the milestones are deemed to be substantive at the inception of the arrangement, requiring their consideration in a unit of account. As the Company's performance obligations are complete at the time that each milestone is achieved, the Company will recognize the development, regulatory and commercial milestone payments upon achievement of the milestones, provided all other criteria for revenue recognition are met. Additionally, royalties will be recognized upon Janssen's reporting of such sales to the Company, provided all other criteria for revenue recognition are met.

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

16. Option and License Agreement (Continued)

The Company recognized the initial arrangement consideration as revenue as the research services were provided. The costs incurred by the Company pursuant to the research agreement are recorded as research and development expense in the consolidated statement of operations. In December 2015, Janssen delivered notice to Scholar Rock of its exercise of the license option for collaboration molecules for one of the pharmacological profiles, upon which the Company received $1.0 million. Following Janssen's exercise of its license option for molecules with one of the pharmacological profiles, the Company evaluated and determined that there were no remaining deliverables related to that pharmacological profile license and as a result, recognized the entire license fee in 2015 upon exercise of the option. The Company and Janssen also agreed to extend the collaboration period for the second pharmacological profile through March 31, 2016. The option exercise period for this profile expired unexercised on March 31, 2016.

During 2016, the Company recognized revenue related to the initial consideration under the collaboration of $379,000. There was no revenue recognized in 2017.

17. Net Loss per Unit and Share and Unaudited Pro Forma Net Loss per Share

Basic and diluted net loss per unit is calculated as follows (in thousands, except unit and per unit data):

	Year Ended December 31, 2016
Net loss	$ (16,207)
Weighted average common units outstanding, basic and diluted	4,576,500
Net loss per unit, basic and diluted	$ (3.54)

Following the Reorganization, the Company calculates net loss per share based on its outstanding shares of common stock. For the year ended December 31, 2017, the weighted average number of common shares outstanding includes the weighted average number of common units outstanding prior to the Reorganization.

Basic and diluted net loss per share is calculated as follows (in thousands, except share and per share data):

	Year Ended December 31, 2017
Net loss	$ (24,995)
Weighted average common shares outstanding, basic and diluted	4,665,036
Net loss per share, basic and diluted	$ (5.36)

The following table sets forth the outstanding common unit or common stock equivalents, presented based on amounts outstanding at each period end, that have been excluded from the calculation of diluted net

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

17. Net Loss per Unit and Share and Unaudited Pro Forma Net Loss per Share (Continued)

loss per unit or share for the periods indicated because their inclusion would have been anti-dilutive (in common unit or common stock equivalent shares, as applicable):

	Year Ended December 31,	
	2016	2017
Convertible preferred units	28,652,147	—
Convertible preferred stock	—	43,135,911
Incentive units	5,019,458	—
Restricted common stock	—	3,483,237
Warrant	21,739	21,739
	33,693,344	46,640,887

Unaudited pro forma basic and diluted net loss per share is calculated as follows (in thousands, except share and per share data):

	Year Ended December 31, 2017
Numerator:	
Net loss	$ (24,995)
Change in fair value of warrant liability	10
Pro forma net loss	$ (24,985)
Denominator:	
Weighted average common shares outstanding, basic and diluted	4,665,036
Pro forma adjustment for the automatic conversion of all outstanding shares of convertible preferred stock in shares of common stock	29,901,975
Pro forma weighted average common shares outstanding, basic and diluted	34,567,011
Pro forma net loss per share, basic and diluted	$ (0.72)

18. Retirement Plan

The Company sponsors a 401(K) retirement plan, in which substantially all of its full-time employees are eligible to participate. Participants may contribute a percentage of their annual compensation to this plan, subject to statutory limitations. The Company did not provide any contributions to this plan during the years ended December 31, 2016 and 2017.

19. Deferred Payroll Tax Credit

In December 2015, the Protecting Americans from Tax Hikes (PATH) Act of 2015 was signed into law, which created several new research and development ("R&D") tax credit provisions, including allowing qualified small businesses to utilize the R&D tax credit against the employer's portion of payroll tax up to a maximum of $250,000 per year. This provision is available for R&D tax credits generated in tax years

SCHOLAR ROCK HOLDING CORPORATION

Notes to Consolidated Financial Statements (Continued)

19. Deferred Payroll Tax Credit (Continued)

beginning after 2015. The Company qualified as a small business under PATH for both 2016 and 2017, and has elected to apply the maximum $250,000 for each of the 2016 R&D tax credit and the 2017 R&D tax credit generated against future employer payroll tax liabilities. The $250,000 benefit was recorded as a reduction of research and development costs for both of the years ended December 31, 2016 and 2017. The R&D tax credit of $95,000 and $399,000 is recorded in prepaid expenses and other current assets as of December 31, 2016 and 2017, respectively. The R&D tax credit of $155,000 and $50,000 is recorded in other long term assets as of December 31, 2016 and 2017, respectively.

20. Related Party Transactions

Licensing Agreement

Pursuant to a license agreement with Children's Medical Center Corporation ("CMCC"), a common share holder, the Company paid CMCC an annual license maintenance fee of $5,000 in 2016. Beginning in 2017, this obligation increased to $10,000 per year, and continues until the agreement is terminated. The Company will also be responsible for up to $1.3 million of development milestone payments through the first regulatory approval of a licensed product, tiered royalty payments of low single-digit percentages on net sales of licensed product in the event that the Company realizes sales from products covered by the license agreement, and between 10% to 20% of non-royalty income attributable to a sublicense of the CMCC rights. The Company recorded research and development expense in the statements of operations of $5,000 and $10,000 for the years ended December 31, 2016 and 2017, respectively. There are no amounts due at December 31, 2016 and 2017.

Consulting Agreements

The Company entered into consulting agreements on October 10, 2012 with its two scientific co-founders to provide services related to the advancement of the research and development platform of the company.

The consulting arrangements are on a fixed-fee basis, paid quarterly. The initial contract terms were four years and terminated on October 10, 2016. The contracts were extended for an additional four year period. The Company incurred $160,000 of consulting expense related to these contracts, in each year, for the years ended December 31, 2016 and 2017. There are no amounts due at December 31, 2016 and 2017.

21. Subsequent Events

For its consolidated financial statements as of December 31, 2017, and for the year then ended, the Company evaluated subsequent events through the date the consolidated financial statements were issued.

On February 22, 2018, the Company amended its lease for office space (the "original lease") with its landlord to lease an additional 9,132 square feet (the "expansion space") at its current location and to extend the lease term. The amended lease is set to expire 5 years from the Company's occupancy of the expansion space which is expected to occur in the second quarter of 2018. Rent for the expansion space increases from $667,000 a year to $750,000 a year over the term of the lease. The rent for the original space will increase to an amount based on the rental amount per square foot of the expansion space for the period beginning after the original term of the lease expires (November 1, 2018) through the extended term of the lease. The landlord provided the Company with a tenant improvement allowance of $91,000 for costs to perform alterations of the expansion space. The Company can elect to receive an additional $137,000 of tenant improvement allowances to be repaid as rent expense over the lease term at an interest rate of 8% per annum. The Company has the option to extend the term of the amended lease for one additional term of 5 years commencing after the amended lease expires.

On February 20, 2018, the Company granted stock options to purchase 1,885,156 shares of common stock at an exercise price of $2.02 per share out of the 2017 Plan.

Table of Contents

Shares

Scholar Rock Holding Corporation

Common Stock

PRELIMINARY PROSPECTUS

Joint Book-Running Managers

Jefferies
Cowen
BMO Capital Markets

Co-Manager

Wedbush PacGrow

Until , 2018 (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 is in addition to the dealers' obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

PART II

Information Not Required in Prospectus

Item 13. Other Expenses of Issuance and Distribution.

The following table sets forth the fees and expenses, other than underwriting discounts and commissions, payable in connection with the registration of the common stock hereunder. All amounts are estimates except the SEC registration fee.

	Amount to be Paid
SEC registration fee	$ *
FINRA filing fee	*
Nasdaq Global Market listing fee	*
Printing and mailing	*
Legal fees and expenses	*
Accounting fees and expenses	*
Transfer agent and registrar fees and expenses	*
Miscellaneous	*
Total	$ *

* To be completed by amendment.

Item 14. Indemnification of Directors and Officers.

Section 145 of the Delaware General Corporation Law or the DGCL, authorizes a corporation to indemnify its directors and officers against liabilities arising out of actions, suits and proceedings to which they are made or threatened to be made a party by reason of the fact that they have served or are currently serving as a director or officer to a corporation. The indemnity may cover expenses (including attorneys' fees) judgments, fines and amounts paid in settlement actually and reasonably incurred by the director or officer in connection with any such action, suit or proceeding. Section 145 permits corporations to pay expenses (including attorneys' fees) incurred by directors and officers in advance of the final disposition of such action, suit or proceeding. In addition, Section 145 provides that a corporation has the power to purchase and maintain insurance on behalf of its directors and officers against any liability asserted against them and incurred by them in their capacity as a director or officer, or arising out of their status as such, whether or not the corporation would have the power to indemnify the director or officer against such liability under Section 145.

We have adopted provisions in our certificate of incorporation to be in effect upon the completion of this offering and by-laws to be in effect upon the effectiveness of this registration statement that limit or eliminate the personal liability of our directors to the fullest extent permitted by the DGCL, as it now exists or may in the future be amended. Consequently, a director will not be personally liable to us or our stockholders for monetary damages or breach of fiduciary duty as a director, except for 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;
- § any unlawful payments related to dividends or unlawful stock purchases, redemptions or other distributions; or
- § any transaction from which the director derived an improper personal benefit.

These limitations of liability do not alter director liability under the federal securities laws and do not affect the availability of equitable remedies such as an injunction or rescission.

In addition, our by-laws provide that:

- we will indemnify our directors, officers and, in the discretion of our board of directors, certain employees to the fullest extent permitted by the DGCL, as it now exists or may in the future be amended; and
- we will advance reasonable expenses, including attorneys' fees, to our directors and, in the discretion of our board of directors, to our officers and certain employees, in connection with legal proceedings relating to their service for or on behalf of us, subject to limited exceptions.

We have entered into indemnification agreements with each of our directors and intend to enter into such agreements with certain of our executive officers. These agreements provide that we will indemnify each of our directors, certain of our executive officers and, at times, their affiliates to the fullest extent permitted by Delaware law. We will advance expenses, including attorneys' fees (but excluding judgments, fines and settlement amounts), to each indemnified director, executive officer or affiliate in connection with any proceeding in which indemnification is available and we will indemnify our directors and officers for any action or proceeding arising out of that person's services as a director or officer brought on behalf of us or in furtherance of our rights. Additionally, certain of our directors or officers may have certain rights to indemnification, advancement of expenses or insurance provided by their affiliates or other third parties, which indemnification relates to and might apply to the same proceedings arising out of such director's or officer's services as a director referenced herein. Nonetheless, we have agreed in the indemnification agreements that our obligations to those same directors or officers are primary and any obligation of such affiliates or other third parties to advance expenses or to provide indemnification for the expenses or liabilities incurred by those directors are secondary.

We also maintain general liability insurance which covers certain liabilities of our directors and officers arising out of claims based on acts or omissions in their capacities as directors or officers, including liabilities under the Securities Act of 1933, as amended, or the Securities Act.

The underwriting agreement filed as Exhibit 1.1 to this registration statement provides for indemnification of us and our directors and officers by the underwriters against certain liabilities under the Securities Act and the Securities Exchange Act of 1934.

Item 15. Recent Sales of Unregistered Securities.

In the three years preceding the filing of this registration statement, we have issued the following securities that were not registered under the Securities Act:

(a) Reorganization and Issuance of Convertible Preferred Stock

Reorganization

In connection with the Reorganization:

- Holders of Scholar Rock, LLC outstanding Series B convertible preferred units received one share of Scholar Rock Holding Corporation Series B convertible preferred stock for each Series B convertible preferred unit held immediately prior to the Reorganization, with an aggregate of 13,526,994 shares of Scholar Rock Holding Corporation Series B convertible Preferred stock issued in the Reorganization;
- Holders of Scholar Rock, LLC outstanding Series A-4 convertible preferred units received one share of Scholar Rock Holding Corporation Series A-4 convertible preferred stock for each Series A-4 convertible preferred unit held immediately prior to the Reorganization, with an aggregate of 3,906,738 shares of Scholar Rock Holding Corporation Series A-4 convertible preferred stock issued in the Reorganization;

- Holders of Scholar Rock, LLC outstanding Series A-3 convertible preferred units received one share of Scholar Rock Holding Corporation Series A-3 convertible preferred stock for each Series A-3 convertible preferred unit held immediately prior to the Reorganization, with an aggregate of 5,579,709 shares of Scholar Rock Holding Corporation Series A-3 convertible preferred stock issued in the Reorganization;

- Holders of Scholar Rock, LLC outstanding Series A-2 convertible preferred units received one share of Scholar Rock Holding Corporation Series A-2 convertible preferred stock for each Series A-2 convertible preferred unit held immediately prior to the Reorganization, with an aggregate of 5,066,915 shares of Scholar Rock Holding Corporation Series A-2 convertible preferred stock issued in the Reorganization;

- Holders of Scholar Rock, LLC outstanding Series A-1 convertible preferred units received one share of Scholar Rock Holding Corporation Series A-1 convertible preferred stock for each Series A-1 convertible preferred unit held immediately prior to the Reorganization, with an aggregate of 2,000,000 shares of Scholar Rock Holding Corporation Series A-1 convertible preferred stock issued in the Reorganization;

- Holders of Scholar Rock, LLC outstanding common units received one share of Scholar Rock Holding Corporation common stock for each common unit held immediately prior to the Reorganization, with an aggregate of 4,576,500 shares of common stock issued in the Reorganization; and

- Holders of Scholar Rock, LLC outstanding vested and unvested incentive units, irrespective of any strike price or voting rights on any such outstanding incentive units, exchanged such units for an equal number of shares of common stock or restricted common stock, respectively. The restricted common stock was issued with the same vesting terms as the unvested incentive units held immediately prior to the Reorganization. An aggregate of 6,758,945 shares of restricted common stock and common stock were issued to the prior holders of incentive units in the Reorganization. The restricted common stock was issued with the same vesting terms as the incentive units held immediately prior the Reorganization.

Issuances of Capital Stock

In December 2017, we issued and sold an aggregate of 13,055,555 shares of Series C convertible preferred stock at a purchase price of $3.60 per share, for an aggregate purchase price of $47.0 million to Artal International SCA, Redmile Capital Fund, LP, Redmile Biopharma Investments I, L.P. (and affiliates), Fidelity Mt. Vernon Street Trust: Fidelity Series Growth Company Fund (and affiliates), Cormorant Private Healthcare Fund I, LP (and affiliates), ARCH Venture Fund VIII, L.P., Polaris Venture Partners VI, L.P. (and affiliates), Timothy A. Springer, Ph.D, EcoR1 Capital Fund, LP (and affiliates), KPC Venture Capital, LLC, and JAKII, LLC.

In May 2017, we issued and sold an aggregate of 1,428,209 shares of Series B convertible preferred units at a purchase price of $3.00 per unit, for an aggregate purchase price of $4.3 million to Timothy A. Springer, Ph.D. Series B convertible preferred units converted on a one-to-one basis for Series B convertible preferred stock in connection with the Reorganization.

In December 2015, we issued and sold an aggregate of 12,098,785 Series B convertible preferred units at a purchase price of $3.00 per unit, for an aggregate purchase price of $36.3 million to Fidelity Advisor Biotechnology Fund and affiliates, Cormorant Global Healthcare Master Fund, LP, Arch Venture Fund VII, L.P., Polaris Venture Partners VI, L.P. (and affiliates), TAS Partners, LLC, EcoR1 Capital Fund, LP (and affiliates), KPC Venture Capital, LLC, and JAKII, LLC. Series B convertible preferred units converted on a one-to-one basis for Series B convertible preferred stock in connection with the Reorganization.

No underwriters were involved in the foregoing sales of securities. Unless otherwise stated, the sales of securities described above were deemed to be exempt from registration pursuant to Section 4(a)(2) of the Securities Act, including Regulation D and Rule 506 promulgated thereunder, as transactions by an issuer not involving a public offering. All of the purchasers in these transactions represented to us in connection

with their purchase that they were acquiring the securities for investment and not distribution, that they could bear the risks of the investment and could hold the securities for an indefinite period of time. Such purchasers received written disclosures that the securities had not been registered under the Securities Act and that any resale must be made pursuant to a registration or an available exemption from such registration. All of the foregoing securities are deemed restricted securities for the purposes of the Securities Act.

(b) Grants and Exercises of Stock Options

We have granted stock options to purchase an aggregate of 2,810,156 shares of our common stock, of which 1,885,156 have an exercise price of $2.02 per share, and 1,015,000 have an exercise price of $2.51 per share, to employees, directors and consultants pursuant to the 2017 Plan. Since April 25, 2018, no shares of common stock have been issued upon the exercise of stock options pursuant to the 2017 Plan.

The issuances of the securities described above were deemed to be exempt from registration pursuant to Section 4(a)(2) of the Securities Act or Rule 701 promulgated under the Securities Act as transactions pursuant to compensatory benefit plans. The shares of common stock issued upon the exercise of options are deemed to be restricted securities for purposes of the Securities Act.

Item 16. Exhibits and Financial Statement Schedules.

(a) Exhibits

Exhibit No.	Exhibit Index
1.1*	Form of Underwriting Agreement
3.1**	Amended and Restated Certificate of Incorporation of the Registrant, as currently in effect
3.2*	Form of Amended and Restated Certificate of Incorporation of the Registrant (to be effective upon the completion of this offering)
3.3**	By-laws of the Registrant, as currently in effect
3.4*	Form of Amended and Restated By-laws (to be effective upon the completion of this offering)
4.1**	Investors' Rights Agreement among the Registrant and certain of its stockholders, dated December 22, 2017
4.2*	Specimen Stock Certificate evidencing shares of common stock
4.3**	Amended and Restated Warrant to Purchase Stock, by and between Silicon Valley Bank and the Registrant, dated December 22, 2017
5.1*	Opinion of Goodwin Procter LLP
10.1#**	2017 Stock Option and Incentive Plan and forms of award agreements thereunder
10.2#*	2018 Stock Option and Incentive Plan and forms of award agreements thereunder
10.3#*	Senior Executive Cash Incentive Bonus Plan
10.4#*	Employee Stock Purchase Plan
10.5#*	Form of Indemnification Agreement
10.6†**	Exclusive License Agreement by and between the Registrant, and Children's Medical Center, dated as December 16, 2013
10.7#**	Offer Letter by and between Nagesh K. Mahanthappa, Ph.D. and the Registrant, dated October 10, 2012
10.8#**	Offer Letter by and between Yung H. Chyung, M.D. and the Registrant, dated February 2, 2016
10.9#**	Offer Letter by and between Elan Z. Ezickson and the Registrant, dated July 17, 2014
10.10#**	Non-Competition, Non-Solicitation, Confidentiality and Assignment Agreement, by Nagesh K. Mahanthappa, dated October 10, 2012
10.11#**	Non-Competition, Non-Solicitation, Confidentiality and Assignment Agreement, by Yung H. Chyung, M.D., dated February 2, 2016
10.12#**	Non-Competition, Non-Solicitation, Confidentiality and Assignment Agreement, by Elan Z. Ezickson, dated July 17, 2014
10.13†**	Option and License Agreement by and between the Registrant and Janssen Biotech, Inc., dated as of December 17, 2013
10.14**	Lease Agreement by and between 620 Memorial Leasehold LLC and the Registrant, dated March 5, 2015, as amended by the First Amendment dated February 22, 2016 and the Second Amendment dated February 22, 2018

Exhibit No.	Exhibit Index
21.1**	Subsidiaries of the Registrant
23.1*	Consent of Ernst and Young, Independent Registered Public Accounting Firm
23.2*	Consent of Goodwin Procter LLP (included in Exhibit 5.1)
24.1*	Power of Attorney (included on the signature page hereto)

* To be included by amendment.

** Previously filed

† Application has been made to the Securities and Exchange Commission for confidential treatment of certain provisions. Omitted material for which confidential treatment has been requested has been filed separately with the Securities and Exchange Commission.

# Indicates a management contract or any compensatory plan, contract or arrangement.

(b) Financial Statements Schedules:

Schedules have been omitted because the information required to be set forth therein is not applicable or is shown in the financial statements or notes thereto.

Item 17. Undertakings.

Insofar as indemnification for liabilities arising under the Securities Act of 1933, as amended, or the 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 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 Act and will be governed by the final adjudication of such issue.

The Registrant hereby undertakes that:

(a) The Registrant will provide to the underwriter at the closing as specified in the underwriting agreement, certificates in such denominations and registered in such names as required by the underwriter to permit prompt delivery to each purchaser.

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

(c) For the purpose of determining any liability under the Act, as amended, 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.

SIGNATURES

Pursuant to the requirements of the Securities Act of 1933, the registrant has duly caused this Registration Statement on Form S-1 to be signed on its behalf by the undersigned, thereunto duly authorized, in the City of Cambridge, Massachusetts, on the day of , 2018.

SCHOLAR ROCK HOLDING CORPORATION

By: _____

Nagesh K. Mahanthappa, Ph.D.
President and Chief Executive Officer, and Director

POWER OF ATTORNEY AND SIGNATURES

Each individual whose signature appears below hereby constitutes and appoints each of Nagesh K. Mahanthappa, Ph.D., Elan Z. Ezickson and Rhonda M. Chicko, C.P.A. as such person's true and lawful attorney-in-fact and agent with full power of substitution and resubstitution, for such person in such person's name, place and stead, in any and all capacities, to sign any and all amendments (including post-effective amendments) to this Registration Statement (or any Registration Statement for the same offering that is to be effective upon filing pursuant to Rule 462(b) under the Securities Act of 1933), and to file the same, with all exhibits thereto, and all documents in connection therewith, with the Securities and Exchange Commission granting unto each said attorney-in-fact and agent 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 such person might or could do in person, hereby ratifying and confirming all that any said attorney-in-fact and agent, or any substitute or substitutes of any of them, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Act of 1933, as amended, this Registration Statement and Power of Attorney has been signed by the following person in the capacities and on the date indicated.

Name	Title	Date
Nagesh K. Mahanthappa, Ph.D.	President, Chief Executive Officer, and Director (Principal Executive Officer)	, 2018
Rhonda M. Chicko, C.P.A.	Chief Financial Officer (Principal Financial and Accounting Officer)	, 2018
David Hallal	Chairman	, 2018
Kristina Burow	Director	, 2018
Jeffrey S. Flier, M.D.	Director	, 2018
Michael Gilman, Ph.D.	Director	, 2018
Amir Nashat, Sc.D.	Director	, 2018
Timothy A. Springer, Ph.D.	Director	, 2018