

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, D.C. 20549**

**FORM 10-Q**

**QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

FOR THE QUARTERLY PERIOD ENDED **June 30, 2018**  
OR

**TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

FOR THE TRANSITION PERIOD FROM \_ TO \_

COMMISSION FILE NUMBER **001-38501**

**SCHOLAR ROCK HOLDING CORPORATION**

(Exact name of registrant as specified in its charter)

Delaware (State or other jurisdiction of incorporation or organization)	82-3750435 (I.R.S. Employer Identification No.)
620 Memorial Drive, 2nd Floor Cambridge, Massachusetts (Address of principal executive offices)	02139 (Zip Code)
(857) 259-3860 (Registrant's telephone number, including area code)	

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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	<input type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input checked="" type="checkbox"/> (Do not check if a smaller reporting company)	Smaller reporting company	<input type="checkbox"/>
		Emerging growth company	<input checked="" type="checkbox"/>

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

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

The number of outstanding shares of the Registrant's Common Stock as of August 3, 2018 was 25,243,684.

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## SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q (“Quarterly Report”), contains forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Quarterly Report are forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as “may”, “will”, “should”, “expects”, “intends”, “plans”, “anticipates”, “believes”, “estimates”, “predicts”, “potential”, “continue” or the negative of these terms or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- the timing of the completion of, and progress 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 identify a potential product 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, including our expected use of proceeds from the initial public offering; and
- other risks and uncertainties, including those listed under Part II, Item 1A, Risk Factors.

Any forward-looking statements in this Quarterly Report reflect our current views with respect to future events and with respect to our future financial performance, and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those described under Part II, Item 1A, Risk Factors and elsewhere in this Quarterly Report. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

We may from time to time provide estimates, projections and other information concerning our industry, the general business environment, and the markets for certain diseases, including estimates regarding the potential size of those markets and the estimated incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties, and actual events, circumstances or numbers, including actual disease prevalence rates and market size, may differ materially from the information reflected in this Quarterly Report. Unless otherwise expressly stated, we obtained this industry, business information, market data, prevalence information and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data, and similar sources, in some cases applying our own assumptions and analysis that may, in the future, prove not to have been accurate.

**SCHOLAR ROCK HOLDING CORPORATION**  
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**PART I - FINANCIAL INFORMATION****Item 1. Financial Statements**

**SCHOLAR ROCK HOLDING CORPORATION**  
**CONSOLIDATED BALANCE SHEETS**  
**(Unaudited)**  
**(In thousands, except unit, share, and per share data)**

	June 30, 2018	December 31, 2017
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 115,061	\$ 56,461
Marketable securities	—	1,498
Prepaid expenses and other current assets	1,856	1,242
Total current assets	116,917	59,201
Property and equipment, net	1,856	2,181
Restricted cash	205	205
Other long-term assets	—	50
Total assets	<u>\$ 118,978</u>	<u>\$ 61,637</u>
<b>Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)</b>		
Current liabilities:		
Accounts payable	\$ 2,598	\$ 1,359
Accrued expenses	3,356	2,796
Deferred rent	—	228
Loan payable	731	641
Total current liabilities	6,685	5,024
Long-term portion of deferred rent	681	468
Long-term portion of loan payable	—	398
Warrant to purchase redeemable security	—	37
Total liabilities	<u>7,366</u>	<u>5,927</u>
Commitments and contingencies (Note 9)		
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 June 30, 2018; 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)	—	109,232
Stockholders' equity (deficit):		
Preferred stock, \$0.001 par value; 10,000,000 and no shares authorized at June 30, 2018 and December 31, 2017, respectively; no shares issued and outstanding at June 30, 2018 and December 31, 2017, respectively	—	—
Common stock, \$0.001 par value; 150,000,000 shares authorized and 25,243,684 shares issued and outstanding as of June 30, 2018, respectively; 60,000,000 shares authorized and 3,970,586 shares issued and outstanding as of December 31, 2017, respectively	25	4
Additional paid-in capital	192,711	4,001
Accumulated other comprehensive loss	—	(2)
Accumulated deficit	(81,124)	(57,525)
Total stockholders' equity (deficit)	<u>111,612</u>	<u>(53,522)</u>
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	<u>\$ 118,978</u>	<u>\$ 61,637</u>

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

**SCHOLAR ROCK HOLDING CORPORATION**  
**CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**  
**(Unaudited)**  
**(In thousands, except unit, share, per unit and per share data)**

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2018</u>	<u>2017</u>	<u>2018</u>	<u>2017</u>
Operating expenses:				
Research and development	11,424	4,711	18,125	8,279
General and administrative	3,460	1,177	5,775	2,350
Total operating expenses	<u>14,884</u>	<u>5,888</u>	<u>23,900</u>	<u>10,629</u>
Loss from operations	(14,884)	(5,888)	(23,900)	(10,629)
Other income (expense):				
Interest income, net	213	11	357	23
Other expense, net	(36)	(2)	(56)	(3)
Total other income	<u>177</u>	<u>9</u>	<u>301</u>	<u>20</u>
Net loss	<u>\$ (14,707)</u>	<u>\$ (5,879)</u>	<u>\$ (23,599)</u>	<u>\$ (10,609)</u>
Net loss per unit, basic and diluted		<u>\$ (3.67)</u>		<u>\$ (6.62)</u>
Net loss per share, basic and diluted	<u>\$ (1.39)</u>		<u>\$ (3.51)</u>	
Weighted average common units outstanding, basic and diluted		<u>1,603,088</u>		<u>1,603,088</u>
Weighted average common shares outstanding, basic and diluted	<u>10,593,987</u>		<u>6,716,283</u>	
Comprehensive loss:				
Net loss	<u>\$ (14,707)</u>	<u>\$ (5,879)</u>	<u>\$ (23,599)</u>	<u>\$ (10,609)</u>
Other comprehensive income:				
Unrealized gain on marketable securities	<u>2</u>	<u>4</u>	<u>2</u>	<u>1</u>
Total other comprehensive income	<u>2</u>	<u>4</u>	<u>2</u>	<u>1</u>
Comprehensive loss	<u>\$ (14,705)</u>	<u>\$ (5,875)</u>	<u>\$ (23,597)</u>	<u>\$ (10,608)</u>

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

**SCHOLAR ROCK HOLDING CORPORATION**  
**CONSOLIDATED STATEMENTS OF CASH FLOWS**  
**(Unaudited)**  
**(In thousands)**

	Six Months Ended	
	June 30,	
	2018	2017
<b>Cash flows from operating activities:</b>		
Net loss	\$ (23,599)	\$ (10,609)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	359	330
Equity-based compensation	1,571	488
Amortization of deferred rent	(15)	(103)
Deferred payroll tax credit	190	—
Other	82	35
Change in operating assets and liabilities:		
Prepaid expenses and other current assets	(753)	(456)
Accounts payable	464	638
Accrued expenses	191	(257)
Net cash used in operating activities	<u>(21,510)</u>	<u>(9,934)</u>
<b>Cash flows from investing activities:</b>		
Purchase of property and equipment	(192)	(209)
Sales and maturities of marketable securities	1,498	3,001
Net cash provided by investing activities	<u>1,306</u>	<u>2,792</u>
<b>Cash flows from financing activities:</b>		
Principal payments on loan payable	(333)	(333)
Proceeds from initial public offering of common stock, net of issuance costs	79,137	—
Proceeds from issuance of Series B convertible preferred units, net of issuance costs	—	4,285
Net cash provided by financing activities	<u>78,804</u>	<u>3,952</u>
Net increase (decrease) in cash and cash equivalents and restricted cash	58,600	(3,190)
Cash and cash equivalents and restricted cash, beginning of period	56,666	10,238
Cash and cash equivalents and restricted cash, end of period	<u>\$ 115,266</u>	<u>\$ 7,048</u>
<b>Supplemental disclosure of non-cash items:</b>		
Offering costs in accounts payable and accrued liabilities	<u>\$ 1,302</u>	<u>\$ —</u>
Supplemental cash flow information:		
Cash paid for interest	<u>\$ 19</u>	<u>\$ 28</u>

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

## **SCHOLAR ROCK HOLDING CORPORATION**

### **Notes to Consolidated Financial Statements**

#### **(Unaudited)**

#### **1. Nature of the Business**

Scholar Rock Holding Corporation (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. The Company’s lead product candidate, SRK-015, is a selective inhibitor of the activation of the growth factor myostatin in skeletal muscle that the Company has advanced into clinical development for the treatment of spinal muscular atrophy. The Company was originally formed as a Delaware limited liability company in May 2012. In December 2017, the Company converted into a Delaware corporation through a series of transactions (the “Reorganization”). Its principal offices are in Cambridge, Massachusetts.

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 activities, including research and development of monoclonal antibodies that selectively inhibit activation of growth factors for therapeutic effect. The Company has primarily financed its operations through various equity and debt financings, including the initial public offering of its common stock (the “IPO”) in May 2018.

The Company is 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.

During the second quarter of 2018, the Company completed its IPO, in which the Company sold 6,164,000 shares of common stock, including all additional shares available to cover over-allotments, at a price of \$14.00 per share. The Company received aggregate net proceeds of approximately \$77.8 million, after deducting underwriting discounts and commissions and other offering expenses payable by the Company. Upon the closing of the IPO, all outstanding shares of convertible preferred stock converted into 15,109,950 shares of common stock and the Company’s outstanding warrant to purchase preferred stock converted into a warrant to purchase 7,614 shares of common stock.

The Company has determined that the receipt of the aggregate net proceeds from the IPO has alleviated the substantial doubt about the Company’s ability to continue as a going concern. The Company has incurred annual net operating losses in every year since its inception. The Company had an accumulated deficit of \$81.1 million at June 30, 2018, and will require substantial additional capital to fund its operations. The Company has never generated any product revenue. There can be no assurance that the Company will be able to obtain additional debt or equity financing or generate product revenue or revenues from collaborative partners, on terms acceptable to the Company, on a timely basis or at all. The failure of the Company to obtain sufficient funds on acceptable terms when needed could have a material adverse effect on the Company’s business, results of operations, and financial condition.

#### **2. Summary of Significant Accounting Policies**

##### ***Summary of Significant Accounting Policies***

The Company’s significant accounting policies are described in Note 2, “Summary of Significant Accounting Policies,” to the Consolidated Financial Statements included in the Company’s final prospectus filed with the SEC pursuant to Rule 424(b)(4) on May 24, 2018 (the “Prospectus”). There have been no material changes to the significant accounting policies previously disclosed in the Prospectus.

##### ***Unaudited Interim Financial Information***

The consolidated financial statements of the Company included herein have been prepared, pursuant to the rules and regulations of the Securities and Exchange Commission (the “SEC”). Certain information and footnote disclosures normally included in financial statements prepared in accordance with accounting principles generally accepted in the United States of America (“GAAP”) have been omitted from this report, as is permitted by such rules and regulations.



Accordingly, these consolidated financial statements should be read in conjunction with the financial statements and notes thereto included in the Prospectus.

The unaudited consolidated financial statements include the accounts of Scholar Rock Holding Corporation and its wholly owned subsidiaries. All intercompany transactions and balances of the subsidiaries have been eliminated in consolidation. In the opinion of management, the information furnished reflects all adjustments, all of which are of a normal and recurring nature, necessary for a fair presentation of the results for the reported interim periods. The Company considers events or transactions that occur after the balance sheet date but before the financial statements are issued to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure. The results of operations for interim periods are not necessarily indicative of results to be expected for the full year or any other interim period.

#### ***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, restricted common stock and stock options, and income taxes. The Company utilizes significant estimates and assumptions in determining the fair value of its equity-based compensation, including incentive units, common stock, restricted common stock and stock options that it believes to be reasonable under the circumstances. Actual results could differ from those estimates.

#### ***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.

#### ***Recently Issued Accounting Pronouncements***

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.

### **3. Fair Value of Financial Assets and Liabilities**

The following tables summarize the assets and liabilities measured at fair value on a recurring basis at June 30, 2018 and December 31, 2017 (in thousands):

	Fair Value Measurements at June 30, 2018			
	Total	Level 1	Level 2	Level 3
<b>Assets:</b>				
Money market funds, included in cash and cash equivalents	\$ 112,699	\$ 112,699	\$ —	\$ —
Total assets	\$ 112,699	\$ 112,699	\$ —	\$ —

	Fair Value Measurements at December 31, 2017			
	Total	Level 1	Level 2	Level 3
<b>Assets:</b>				
Money market funds, included in cash and cash equivalents	\$ 55,291	\$ 55,291	\$ —	\$ —
Marketable securities:				
U.S. Treasury obligations	1,498	1,498	—	—
<b>Total assets</b>	<b>\$ 56,789</b>	<b>\$ 56,789</b>	<b>\$ —</b>	<b>\$ —</b>
<b>Liabilities:</b>				
Warrant to purchase redeemable security	\$ 37	\$ —	\$ —	\$ 37
<b>Total liabilities</b>	<b>\$ 37</b>	<b>\$ —</b>	<b>\$ —</b>	<b>\$ 37</b>

There were no transfers between fair value measurements levels during the three or six months ended June 30, 2018 or 2017.

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 June 30, 2018 and December 31, 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 June 30, 2018 and December 31, 2017, due to their short-term nature. The Company believes the terms of the loan payable reflect 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.

Upon the completion of the IPO, the Company's outstanding warrant to purchase preferred stock converted into a warrant to purchase common stock and the Company reclassified the fair value of the warrant to additional paid-in capital. The following table presents activity in the preferred stock warrant during the six months ended June 30, 2018 (in thousands):

Balance at December 31, 2017	\$ (37)
Change in fair value of warrant included in other income (expense), net	(56)
Reclassification to additional paid-in capital upon consummation of the IPO	93
Balance at June 30, 2018	<u>\$ —</u>

The change in fair value of the warrant included in other income (expense), net for the three and six months ended June 30, 2018 was \$(36) and \$(56), respectively.

#### 4. Marketable Securities

The Company did not have any investments as of June 30, 2018.

The following table summarizes the Company's investments as of December 31, 2017 (in thousands):

	Amortized Cost	Gross Unrealized		Estimated Fair Value
		Gains	Losses	
Marketable securities available-for-sale:				
U.S. Treasury obligations	\$ 1,500	\$ —	\$ (2)	\$ 1,498
<b>Total available-for-sale securities</b>	<b>\$ 1,500</b>	<b>\$ —</b>	<b>\$ (2)</b>	<b>\$ 1,498</b>

## 5. Property and Equipment, Net

As of June 30, 2018 and December 31, 2017, property and equipment, net consists of the following (in thousands):

	As of	
	June 30, 2018	December 31, 2017
Laboratory equipment	\$ 2,100	\$ 2,074
Furniture & fixtures	159	151
Machinery & equipment	7	7
Leasehold improvements	1,498	1,498
	<u>3,764</u>	<u>3,730</u>
Less: Accumulated depreciation	(1,908)	(1,549)
	<u>\$ 1,856</u>	<u>\$ 2,181</u>

## 6. Accrued Expenses

As of June 30, 2018 and December 31, 2017, accrued expenses consist of the following (in thousands):

	As of	
	June 30, 2018	December 31, 2017
Accrued external research and development expense	\$ 1,411	\$ 1,225
Accrued payroll and related expenses	1,260	1,174
Accrued professional and consulting expense	631	382
Accrued other	54	15
	<u>\$ 3,356</u>	<u>\$ 2,796</u>

## 7. Common Stock and Preferred Stock

In connection with the consummation of the IPO, on May 29, 2018 the Company filed an amended and restated certificate of incorporation, which increased the number of common shares authorized for issuance thereunder by 90,000,000 shares to 150,000,000 shares and also authorized for issuance 10,000,000 shares of Preferred Stock, par value \$0.001. As of June 30, 2018, no shares of the Preferred Stock were issued or outstanding.

## 8. Equity-Based Compensation

The Company recorded equity-based compensation expense related to all equity-based awards for employees and non-employees, which was allocated as follows in the consolidated statements of operations (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Research and development expense	\$ 542	\$ 118	\$ 786	\$ 225
General and administrative expense	548	114	785	263
	<u>\$ 1,090</u>	<u>\$ 232</u>	<u>\$ 1,571</u>	<u>\$ 488</u>

**Restricted Stock**

The following table summarizes restricted common stock activity as of June 30, 2018:

	<u>Number of Shares</u>	<u>Weighted Average Fair Value per Share at Issuance</u>
Restricted common stock as of December 31, 2017	1,220,085	\$ 5.77
Granted	—	\$ —
Vested	(216,011)	\$ 5.77
Forfeited	(852)	\$ 5.77
Restricted common stock as of June 30, 2018	<u>1,003,222</u>	<u>\$ 5.77</u>

As of June 30, 2018, the Company had unrecognized equity-based compensation expense of \$3.6 million related to restricted stock issued to employees and directors, which is expected to be recognized over a period of 2.4 years.

**Stock Options**

The following table summarizes the Company's stock option activity for the six months ended June 30, 2018:

	<u>Number of Shares</u>	<u>Weighted Average Exercise Price</u>	<u>Weighted Average Remaining Contractual Term</u> (in years)	<u>Aggregate Intrinsic Value</u> (in thousands)
Outstanding as of December 31, 2017	—	\$ —	—	\$ —
Granted	1,484,105	9.51	—	—
Exercised	—	—	—	—
Forfeited	(784)	10.21	—	—
Outstanding as of June 30, 2018	<u>1,483,321</u>	<u>\$ 9.51</u>	<u>9.77</u>	<u>\$ 9,984</u>
Options exercisable as of June 30, 2018	53,217	\$ 6.77	9.67	\$ 490

Using the Black-Scholes option pricing model, the weighted average fair value of options granted to employees and directors during the six months ended June 30, 2018 was \$6.72.

The following assumptions were used in determining the fair value of options granted to employees and non-employees:

	<u>Six Months Ended June 30, 2018</u>	
	<u>Employees</u>	<u>Non-Employees</u>
Risk-free interest rate	2.76 %	2.85 %
Expected dividend yield	— %	— %
Expected term (years to liquidity)	6.12	9.64
Expected volatility	81.66 %	78.00 %

As of June 30, 2018, the Company has unrecognized equity-based compensation expense related to its employee stock options of \$9.4 million which the Company expects to recognize over the remaining weighted average vesting period of 3.6 years.

## **9. Commitments and Contingencies**

The Company enters 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.

### ***Operating Leases***

#### ***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, as amended in February 2016 (the “lease”). The lease was amended in February 2018, to add an additional 9,132 square feet (the “expansion space”) at the current location and to extend the lease term (the “amended lease”). The amended lease is set to expire 5 years from the date the landlord delivers the expansion space which occurred in July 2018. Rent for the facility lease, including the expansion space, increases from \$1.4 million a year to \$1.7 million a year over the term of the lease.

### ***Legal Proceedings***

The Company is not currently a party to any material legal proceedings.

## **10. 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 \$2.0 million to finance the purchase of eligible equipment. The loan balance at June 30, 2018 and December 31, 2017 was \$731,000 and \$1.0 million, respectively.

## **11. Option and License Agreement**

### ***Overview***

On December 17, 2013, the Company entered into an option and license agreement with Janssen. Janssen is obligated to pay the Company certain development milestones totaling up to \$25.0 million and regulatory milestones totaling up to \$97.0 million for the pharmacological profile licensed by Janssen during the development period and through successful regulatory approval, as further described in the option and license agreement. 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 U.S. Food and Drug Administration (“FDA”) or other global regulatory authorities. Additionally, commercial milestone payments totaling up to \$130.0 million for the 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 \$2.0 million upon achievement of a development milestone.

The Company did not recognize any revenue from its option and license agreement with Janssen in the three or six-month periods ended June 30, 2018 or June 30, 2017, respectively.

## **12. Net Loss per Share**

Prior to the Reorganization, the Company calculated basic net loss per unit by dividing net loss by the weighted average number of common units outstanding. Subsequent to 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 units, restricted common stock, warrants and stock options 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. The significant increase in shares outstanding in the second quarter of 2018 is expected to impact the year-over-year comparability of the Company's net loss per share calculations for the next twelve months.

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 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):

	<b>Three and Six Months Ended June 30,</b>	
	<b>2018</b>	<b>2017</b>
Convertible preferred units	—	10,536,765
Incentive units	—	1,930,649
Restricted common stock	1,003,222	—
Warrant	7,614	7,614
Stock options	1,483,321	—
	<u>2,494,157</u>	<u>12,475,028</u>

### **13. Related Party Transactions**

#### ***Licensing Agreement***

Pursuant to a license agreement with Children's Medical Center Corporation ("CMCC"), a holder of shares of the Company's common stock, the Company paid CMCC an annual license maintenance fee of \$10,000 in 2017. The Company did not make any payments for the CMCC license maintenance fees during three and six months ended June 30, 2017, respectively. The Company paid \$0 and \$10,000 during three and six months ended June 30, 2018, respectively. The obligation 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 products in the event that the Company realizes sales from products covered by the license agreement, which are products developed using the Company's proprietary platform that are covered by a valid claim contained in any patent under the license agreement, and between 10% to 20% of non-royalty income attributable to a sublicense of the CMCC rights. The Company recorded such payments as research and development expense in the statements of operations. There are no amounts due to CMCC under this license agreement at December 31, 2017 or June 30, 2018.

#### ***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 \$40,000 and \$80,000 for the three and six months ended June 30, 2018 and June 30, 2017, respectively. There are no amounts due under these consulting agreements at December 31, 2017 or June 30, 2018.

## ITEM 2 MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

*The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and the audited financial information and the notes thereto included in our final prospectus for our initial public offering filed pursuant to Rule 424(b) under the Securities Act of 1933, as amended, or the Securities Act, which was filed with the Securities and Exchange Commission, or the SEC on May 24, 2018, or the Prospectus.*

*Our actual results and timing of certain events may differ materially from the results discussed, projected, anticipated, or indicated in any forward-looking statements. We caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this Quarterly Report. In addition, even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this Quarterly Report, they may not be predictive of results or developments in future periods.*

*The following information and any forward-looking statements should be considered in light of factors discussed elsewhere in this Quarterly Report on Form 10-Q, including those risks identified under Part II, Item 1A. Risk Factors.*

*We caution readers not to place undue reliance on any forward-looking statements made by us, which speak only as of the date they are made. We disclaim any obligation, except as specifically required by law and the rules of the SEC, to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.*

### 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 inhibitor of the activation of the growth factor myostatin in skeletal muscle, into clinical development for the treatment of spinal muscular atrophy, or SMA. 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 initiated our Phase 1 clinical trial in May 2018. The FDA has granted orphan drug designation for SRK-015 for the treatment of SMA. In addition, 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.

In May 2018, we completed an initial public offering, or IPO, of our common stock, and issued and sold 6,164,000 shares of common stock, including all additional shares available to cover over-allotments, at a public offering price of \$14.00 per share, resulting in net proceeds of \$77.8 million after deducting underwriting discounts and commissions and other offering expenses payable by the Company. Upon the closing of the IPO, all outstanding shares of convertible preferred stock converted into 15,109,950 shares of common stock and the Company's outstanding warrant to purchase preferred stock converted into a warrant to purchase 7,614 shares of common stock.

Since inception, we have incurred significant operating losses. Our net losses were \$14.7 million and \$5.9 million for the three months ended June 30, 2018 and 2017, respectively, and \$23.6 million and \$10.6 million for the six months ended June 30, 2018 and 2017, respectively. As of June 30, 2018, we had an accumulated deficit of \$81.1 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 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 TGFβ1;
- maintain, expand and protect our intellectual property portfolio;
- hire additional research, development and business personnel; and
- operate as a public company subsequent to the completion the IPO.

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.

## **Financial Operations Overview**

### ***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;
- costs of purchasing laboratory supplies and non-capital equipment used in our preclinical studies;
- costs related to compliance with clinical regulatory requirements; and
- facility costs and other allocated expenses, which include expenses for rent and maintenance of facilities, insurance, depreciation and other supplies.

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 goods and 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 continued development of our 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, Net**

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

#### **Other Expense, Net**

Other expense, net consists primarily of non-cash changes in the fair value of warrants issued in connection with our credit facility.

**Results of Operations****Comparison of the Three Months Ended June 30, 2018 and 2017**

The following table summarizes our results of operations for the three months ended June 30, 2018 and 2017 (in thousands, except percentages):

	<b>Three Months Ended June 30,</b>		<b>Change</b>	
	<b>2018</b>	<b>2017</b>	<b>\$</b>	<b>%</b>
<b>Operating expenses:</b>				
Research and development	\$ 11,424	\$ 4,711	\$ 6,713	142.5 %
General and administrative	3,460	1,177	2,283	194.0 %
Total operating expenses	14,884	5,888	8,996	152.8 %
Loss from operations	(14,884)	(5,888)	(8,996)	152.8 %
<b>Other income (expense):</b>				
Interest income, net	213	11	202	NM *
Other expense, net	(36)	(2)	(34)	NM *
Total other income	177	9	168	NM *
Net loss	\$ (14,707)	\$ (5,879)	\$ (8,828)	150.2 %

\* NM means not meaningful.

**Research and Development**

Research and development expense increased by \$6.7 million from \$4.7 million for the three months ended June 30, 2017 to \$11.4 million for the three months ended June 30, 2018, an increase of 142.5%. The following table summarizes our research and development expense for the three months ended June 30, 2018 and 2017 (in thousands, except percentages):

	<b>Three Months Ended June 30,</b>		<b>Change</b>	
	<b>2018</b>	<b>2017</b>	<b>\$</b>	<b>%</b>
<b>External costs by program</b>				
SRK-015	\$ 5,536	\$ 1,288	\$ 4,248	329.8 %
Other early development candidates and unallocated costs	1,936	835	1,101	131.9 %
Total external costs	7,472	2,123	5,349	252.0 %
<b>Internal costs:</b>				
Employee compensation and benefits	2,556	1,570	986	62.8 %
Facility and other	1,396	1,018	378	37.1 %
Total internal costs	3,952	2,588	1,364	52.7 %
Total research and development expense	\$ 11,424	\$ 4,711	\$ 6,713	142.5 %

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

- The \$5.3 million increase in external costs primarily related to increased research, preclinical and clinical development and manufacturing costs associated with our lead product candidate, SRK-015, and other external research costs associated with our other early development candidates.
- The \$1.4 million increase in internal costs was primarily driven by an 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 \$2.3 million from \$1.2 million for the three months ended June 30, 2017 to \$3.5 million for the three months ended June 30, 2018. The increase in general and administrative expense was primarily attributable to an increase of \$1.6 million in employee compensation and benefits related to increased headcount and an increase of \$0.5 million in professional services and consulting fees primarily related to increases in legal fees, accounting and audit fees, and public and investor relations fees due to ongoing business activities and public company costs.

### Interest Income, Net

The increase in interest income, net was attributable to increased income earned on our investment portfolio, which was significantly larger during the three months ended June 30, 2018 as compared to the three months ended June 30, 2017.

### Comparison of the Six Months Ended June 30, 2018 and 2017

The following table summarizes our results of operations for the six months ended June 30, 2018 and 2017 (in thousands, except percentages):

	Six Months Ended June 30,		Change	
	2018	2017	\$	%
Operating expenses:				
Research and development	\$ 18,125	\$ 8,279	\$ 9,846	118.9 %
General and administrative	5,775	2,350	3,425	145.7 %
Total operating expenses	23,900	10,629	13,271	124.9 %
Loss from operations	(23,900)	(10,629)	(13,271)	124.9 %
Other income (expense):				
Interest income, net	357	23	334	NM *
Other expense, net	(56)	(3)	(53)	NM *
Total other income	301	20	281	NM *
Net loss	\$ (23,599)	\$ (10,609)	\$ (12,990)	122.4 %

\* NM means not meaningful.

### Research and Development

Research and development expense increased by \$9.8 million from \$8.3 million for the six months ended June 30, 2017 to \$18.1 million for the six months ended June 30, 2018, an increase of 118.9%. The following table summarizes our research and development expense for the six months ended June 30, 2018 and 2017 (in thousands, except percentages):

	Six Months Ended June 30,		Change	
	2018	2017	\$	%
External costs by program:				
SRK-015	\$ 7,531	\$ 1,627	\$ 5,904	362.9 %
Other early development candidates and unallocated costs	3,232	1,718	1,514	88.1 %
Total external costs	10,763	3,345	7,418	221.8 %
Internal costs:				
Employee compensation and benefits	4,695	2,985	1,710	57.3 %
Facility and other	2,667	1,949	718	36.8 %
Total internal costs	7,362	4,934	2,428	49.2 %
Total research and development expense	\$ 18,125	\$ 8,279	\$ 9,846	118.9 %

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

- The \$7.4 million increase in external costs primarily related to increased research, and preclinical and clinical development and manufacturing costs associated with our lead product candidate, SRK-015 and other external research costs associated with our other early development candidates.
- The \$2.4 million increase in internal costs was primarily driven by an 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 \$3.4 million from \$2.4 million for the six months ended June 30, 2017 to \$5.8 million for the six months ended June, 2018. The increase in general and administrative expense was primarily attributable to an increase of \$1.9 million in employee compensation and benefits due to increased headcount and an increase of \$1.3 million in professional services and consulting fees primarily related to increases in legal fees, accounting and audit fees, and public and investor relations fees due to ongoing business activities and public company costs.

### **Interest Income, Net**

The increase in interest income, net was attributable to increased income earned on our investment portfolio, which was significantly larger during the six months ended June 30, 2018 as compared to the six months ended June 30, 2017.

### **Liquidity and Capital Resources**

#### ***Sources of Liquidity***

In May 2018, we completed an initial public offering, or IPO, of our common stock, and issued and sold 6,164,000 shares of common stock, including all additional shares available to cover over-allotments, resulting in net proceeds of \$77.8 million after deducting underwriting discounts and commissions and other offering costs payable by the Company.

Prior to the IPO, we have funded our operations from inception through the IPO with the net 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 June 30, 2018 and December 31, 2017 (in thousands):

	June 30, 2018	December 31, 2017
Cash and cash equivalents	\$ 115,061	\$ 56,461
Marketable securities	—	1,498
Total cash, cash equivalents and marketable securities	<u>\$ 115,061</u>	<u>\$ 57,959</u>

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 June 30, 2018 was \$0.7 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 that is currently exercisable for 7,614 shares of our common stock at an exercise price of \$3.94 per share.

### **Cash Flows**

The following table provides information regarding our cash flows for the six months ended June 30, 2018 and 2017 (in thousands):

	<b>Six Months Ended June 30,</b>	
	<b>2018</b>	<b>2017</b>
Net cash used in operations	\$ (21,510)	\$ (9,934)
Net cash provided by investing activities	1,306	2,792
Net cash provided by financing activities	78,804	3,952
Net increase (decrease) in cash and cash equivalents and restricted cash	\$ 58,600	\$ (3,190)

### **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 \$21.5 million for the six months ended June 30, 2018 compared to \$9.9 million for the six months ended June 30, 2017. The increase in cash used in operating activities was primarily due to an increase in net loss of \$13.0 million, partially offset by an increase of \$1.1 million in non-cash equity-based compensation for the six months ended June 30, 2018 as compared to the six months ended June 30, 2017.

### **Net Cash Provided by Investing Activities**

Net cash provided by investing activities was \$1.3 million for the six months ended June 30, 2018 compared to \$2.8 million for the six months ended June 30, 2017. Net cash provided by investing activities for the six months ended June 30, 2018 consisted primarily of the maturity of marketable securities. Net cash provided by investing activities for the six months ended June 30, 2017 consisted primarily of sales of marketable securities.

### **Net Cash Provided by Financing Activities**

Net cash provided in financing activities was \$78.8 million for the six months ended June 30, 2018 compared to \$4.0 million for the six months ended June 30, 2017. Net cash provided by financing activities for the six months ended June 30, 2018 consisted primarily of proceeds from our initial public offering of common stock. Net cash provided by financing activities for the six months ended June 30, 2017 consisted primarily of proceeds from the issuance of Series B convertible preferred units. These amounts were partially offset by principal payments made on outstanding debt.

### **Funding Requirements**

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development for, continue and 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, we expect to incur additional costs associated with operating as a public company.

We expect that our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2020.

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.

There have been no significant changes to our critical accounting policies from those described in our final prospectus for our initial public offering filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on May 24, 2018.

#### **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**

There were no material changes to our contractual obligations and commitments described under Management's Discussion and Analysis of Financial Condition and Results of Operations as previously disclosed in the Prospectus.

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.

#### **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 Quarterly Report on Form 10-Q, such standards will not have a material impact on our financial statements or do not otherwise apply to our operations.

### **ITEM 3 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.

As such, 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 three and six months ended June 30, 2018 or 2017.

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

#### **Management's Evaluation of our Disclosure Controls and Procedures**

We maintain "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is (1) recorded, processed, summarized and reported within the time periods specified in the Securities and Exchange Commission's rules and forms and (2) accumulated and communicated to our management, including our principal executive and principal financial officer, as appropriate to allow timely decisions regarding required disclosure. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible

controls and procedures. Our disclosure controls and procedures are designed to provide reasonable assurance of achieving their control objectives.

Our management, with the participation of our chief executive officer and chief financial officer has evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2018, the end of the period covered by this Quarterly Report on Form 10-Q. Based upon such evaluation, our chief executive officer and chief financial officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of such date. We continue to review and document our disclosure controls and procedures, including our internal controls and procedures for financial reporting, and may from time to time make changes aimed at enhancing their effectiveness and to ensure that our systems evolve with our business.

**Changes in Internal Controls Over Financial Reporting**

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the three months ended June 30, 2018 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.



**Part II. Other Information**

**Item 1. Legal Proceedings**

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. The outcome of litigation cannot be predicted with certainty and some lawsuits, claims or proceedings may be disposed of unfavorably to us, which could materially affect our financial condition or results of operations.

## Item 1A. Risk Factors

*Careful consideration should be given to the following risk factors, in addition to the other information set forth in this Quarterly Report on Form 10-Q (“Quarterly Report”) and in other documents that we file with the Securities and Exchange Commission (the “SEC”), in evaluating Scholar Rock Holding Corporation and our subsidiaries (collectively, the “Company”) and our business. Investing in our common stock involves a high degree of risk. If any of the following risks and uncertainties actually occurs, our business, prospects, financial condition and results of operations could be materially and adversely affected. The risks described below are not intended to be exhaustive and are not the only risks facing the Company. New risk factors can emerge from time to time, and it is not possible to predict the impact that any factor or combination of factors may have on our business, prospects, financial condition and results of 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. For the three months ended June 30, 2017 and 2018, we reported a net loss of \$5.9 million and \$14.7 million, respectively. As of December 31, 2017 and June 30, 2018, we had an accumulated deficit of \$57.5 million and \$81.1 million, respectively. 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, through 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 June 30, 2018, we had approximately \$115.1 million in cash, cash equivalents and marketable securities. Based on our current operating plan, we believe that our existing cash, cash equivalents and marketable securities as of June 30, 2018, will be sufficient to fund our operating expenses and capital expenditure requirements into the second half of 2020. 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 and potential 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, strategic alliance, or licensing 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 (the "FDA"), the European Medicines Agency (the "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 developing research cell lines and development and 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 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 in Phase 1 clinical development for the treatment of spinal muscular atrophy ("SMA"), which we initiated in May 2018. All of our other programs are in preclinical development, and we have yet to nominate a product candidate from these programs.

SRK-015 and any future 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 (“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 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 June 30, 2018, we had 54 employees. As our clinical development and commercialization plans and strategies develop 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 (“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. We have adopted 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.

**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 potential product candidate for any 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 any planned product candidates, including 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 safe or 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 certain potential 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 existing product candidate 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 (“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 (“cGMPs”) and good clinical practices (“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 (“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 (“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;
- our failure to establish an appropriate safety profile for a product candidate based on clinical or preclinical data for such product candidate as well as data emerging from other molecules in the same class as our product candidate; 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 (“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 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.

***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," (the "TCJA"), that significantly reforms the Internal Revenue Code of 1986, as amended (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 \$49.8 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 our initial public offering, may trigger such an ownership change pursuant to Section 382. Any such limitation, whether as the result of our initial public 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, the facilities at any clinical trial site, 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 one or more 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 from the FDA for SRK-015 for the treatment of SMA, and we may seek Orphan Drug Designation from regulatory authorities in other jurisdictions for SRK-015 and Orphan Drug Designation from the FDA or regulatory authorities in other jurisdictions for our future product candidates. In any of these instances, we may not receive the requested designation or 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 from the FDA for SRK-015 for the treatment of SMA, or if we obtain Orphan Drug Designation from regulatory authorities in other jurisdictions for SRK-015 for the treatment of SMA or 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 SRK-015 from regulatory authorities in other jurisdictions or 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, and we may not be successful in obtaining such designation, or if received, 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. 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 (“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 (“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 (“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 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 (“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 ("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 (“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 (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 (“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 (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 2027, 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 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 ("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 introduced 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 could negatively impact or restrict our 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 (the “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 (the “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 (“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.

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 patents 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 (the "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 and/or methods of medical treatment, 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 product candidate that includes such an antibody and advance that product 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 Quarterly Report 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**

#### ***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 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 Quarterly Report, these factors include:

- the results of our Phase 1 clinical trial for SRK-015;
- any delay in identifying potential product candidates 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. 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.***

As of June 30, 2018, our executive officers, directors and their affiliates beneficially hold, in the aggregate, approximately 6.7% of our outstanding voting stock. These stockholders, acting together, are able to significantly influence all matters requiring stockholder approval. For example, these stockholders are able to significantly influence 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.

***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 (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, (the “Sarbanes-Oxley Act”), reduced disclosure obligations regarding executive compensation in 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 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 our initial public 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 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 may 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.

***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 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 publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.***

The trading market for our common stock depends in part on the research and reports that securities or industry analysts publish about us or our business. 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 bylaws 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 bylaws 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. In addition, our amended and restated bylaws 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. In addition, our amended and restated bylaws provide that any person or entity purchasing or otherwise acquiring any interest in shares of our common stock is deemed to have notice of and consented to the foregoing provisions. We have chosen the United States District Court for the District of Massachusetts as the exclusive forum for such causes of action because our principal executive offices are located in Cambridge, Massachusetts. Some companies that have adopted similar federal district court forum selection provisions are currently subject to a suit in the Court of Chancery of the State of Delaware brought by stockholders who assert that the federal district court forum selection provision is not enforceable. We recognize that the federal district court forum selection clause may impose additional litigation costs on stockholders who assert the provision is not enforceable and may impose more general additional litigation costs in pursuing any such claims, particularly if the stockholders do not reside in or near the Commonwealth of Massachusetts. Additionally, 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 bylaws 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.

## **Item 2. Unregistered Sales of Equity Securities and Use of Proceeds**

### ***Recent Sales of Unregistered Securities***

During the period between April 1, 2018 and May 24, 2018, we issued to certain of our employees options to purchase an aggregate of 665,464 common shares at a weighted-average exercise price of \$10.35 per share. We deemed these issuances to be exempt from registration under the Securities Act either in reliance on Rule 701 of the Securities Act as sales and offers under compensatory benefit plans and contracts relating to compensation in compliance with Rule 701, or in reliance on Section 4(a)(2), as transactions by an issuer not involving a public offering. All recipients either received adequate information about our company or had access, through employment or other relationships, to such information. No underwriters were involved in the foregoing issuances of securities.

In May 2018, upon the closing of our initial public offering, all shares of our then-outstanding convertible preferred stock were automatically converted into 15,109,950 shares of common stock. The issuance of such shares of common stock was exempt from the registration requirements of the Securities Act, pursuant to Section 3(a)(9) of the Securities Act, involving an exchange of securities exchanged by the issuer with its existing security holders exclusively where no commission or other remuneration is paid or given directly or indirectly for soliciting such exchange. No underwriters were involved in this issuance of shares. In addition, upon the closing of our initial public offering, there was an automatic conversion of the outstanding warrant to purchase 21,739 shares of convertible preferred stock into a warrant to purchase 7,614 shares of common stock. The issuance of such shares of common stock was exempt from the registration requirements of the Securities Act, pursuant to Section 3(a)(9) of the Securities Act, involving an exchange of securities exchanged by the issuer with its existing security holders exclusively where no commission or other remuneration is paid or given directly or indirectly for soliciting such exchange.

### ***Issuer Purchases of Equity Securities***

In the quarter ended June 30, 2018, we did not repurchase any shares of our common stock.

### ***Use of Proceeds from Initial Public Offering of Common Stock***

On May 29, 2018, we completed the sale of 5,360,000 shares of our common stock in our initial public offering at a price to the public of \$14.00 per share. The underwriters fully exercised their over-allotment option on June 1, 2018, and purchased an additional 804,000 shares of our common stock. The offer and sale of the shares in our IPO was registered under the Securities Act pursuant to registration statements on Form S-1 (File No. 333-224493), which was filed with the SEC on April 27, 2018 and amended subsequently and declared effective by the SEC on May 23, 2018. Following the sale of the shares in connection with the closing of our IPO, the offering terminated. The offering did not terminate before all the securities registered in the registration statements were sold. Jefferies LLC, Cowen and Company, LLC and BMO Capital Markets Corp. acted as lead book-running managers for the offering. Wedbush PacGrow acted as the co-manager for the offering. We raised approximately \$77.8 million in net proceeds after deducting underwriting discounts and commissions and offering expenses payable by us. None of these expenses consisted of direct or indirect payments made by us to directors, officers or persons owning 10% or more of our common stock or to their associates, or to our affiliates. There has been no material change in the planned use of proceeds from our IPO as described in our final prospectus filed with the SEC on May 24, 2018. We invested the funds received in cash, cash equivalents and other short-term investments in accordance with our investment policy. As of June 30, 2018, the entire amount of the net proceeds is included as cash, cash equivalents and other short-term investments.

## **Item 3. Defaults Upon Senior Securities**

None.

## **Item 4. Mine Safety Disclosures**

None.

## **Item 5. Other Information**

None.

**Item 6. Exhibits**

## EXHIBIT INDEX

Exhibit Number	Description	Incorporated by Reference to:			
		Form	File No.	Exhibit No.	Filing Date
3.1	<a href="#">Amended and Restated Certificate of Incorporation of the Registrant</a>	S-1/A	333-224493	3.2	May 8, 2018
3.2	<a href="#">Amendment to Amended and Restated Certificate of Incorporation of the Registrant</a>	S-1/A	333-224493	3.1.1	May 14, 2018
3.3	<a href="#">Amended and Restated By-laws of the Registrant</a>	S-1/A	333-224493	3.4	May 8, 2018
4.1	<a href="#">Investors' Rights Agreement among the Registrant and certain of its stockholders, dated December 22, 2017</a>	S-1	333-224493	4.1	April 27, 2018
4.2	<a href="#">Specimen Stock Certificate evidencing shares of common stock</a>	S-1/A	333-224493	4.2	May 14, 2018
4.3	<a href="#">Amended and Restated Warrant to Purchase Stock, by and between Silicon Valley Bank and the Registrant, dated December 22, 2017</a>	S-1	333-224493	4.3	April 27, 2018
31.1*	<a href="#">Certification of Principal Executive Officer Pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934</a>				
31.2*	<a href="#">Certification of Principal Financial Officer Pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934</a>				
32.1**	<a href="#">Certification of Principal Executive Officer and Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002</a>				
101*	The following materials from the Registrant's Quarterly Report on Form 10-Q for the quarter ended June 30, 2018, formatted in XBRL (eXtensible Business Reporting Language): (i) the Consolidated Balance Sheets, (ii) the Consolidated Statements of Operations and Comprehensive Loss, (iii) the Consolidated Statements of Cash Flows, and (iv) related notes to these financial statements				

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\* Filed herewith

\*\* Furnished herewith

**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

SCHOLAR ROCK HOLDING CORPORATION

Date: August 8, 2018

By: /s/ Nagesh K. Mahanthappa

Nagesh K. Mahanthappa, Ph.D.  
President and Chief Executive Officer  
(Principal Executive Officer)

Date: August 8, 2018

By: /s/ Rhonda M. Chicko

Rhonda M. Chicko  
Chief Financial Officer  
(Principal Financial Officer)



**Certifications**

I, Nagesh K. Mahanthappa, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Scholar Rock Holding Corporation;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 8, 2018

/s/ Nagesh K. Mahanthappa  
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Nagesh K. Mahanthappa  
President and Chief Executive Officer  
(Principal Executive Officer)

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**Certifications**

I, Rhonda M. Chicko, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Scholar Rock Holding Corporation;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 8, 2018

/s/ Rhonda M. Chicko  
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Rhonda M. Chicko  
Chief Financial Officer  
(Principal Financial Officer)

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**CERTIFICATION PURSUANT TO**  
**18 U.S.C. SECTION 1350**  
**AS ADOPTED PURSUANT TO**  
**SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Quarterly Report on Form 10-Q of Scholar Rock Holding Corporation (the "Company") for the period ended June 30, 2018 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers of the Company certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, to his or her knowledge, that:

- (1) the Report fully complies with the requirements of Section 13(a) or 15(d), as applicable, of the Securities Exchange Act of 1934, as amended; and
- (2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

This certification is being provided pursuant to 18 U.S.C. 1350 and is not to be deemed a part of the Report, nor is it to be deemed to be "filed" for any purpose whatsoever.

Date: August 8, 2018

/s/ Nagesh K. Mahanthappa  
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Nagesh K. Mahanthappa  
President and Chief Executive Officer

Date: August 8, 2018

/s/ Rhonda M. Chicko  
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Rhonda M. Chicko  
Chief Financial Officer

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