

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

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**FORM 10-Q**

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

FOR THE QUARTERLY PERIOD ENDED March 31, 2019  
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

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**SCHOLAR ROCK HOLDING CORPORATION**

(Exact name of registrant as specified in its charter)

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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)

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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"/>	Smaller reporting company	<input checked="" 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

**Securities registered pursuant to Section 12(b) of the Exchange Act:**

Title of each class	Trading symbol(s)	Name of each exchange on which registered
Common Stock, par value \$0.001 per share	SRRK	The Nasdaq Global Select Market

The number of outstanding shares of the Registrant's Common Stock as of May 1, 2019 was 26,226,204.

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## SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q (“Quarterly Report”), including the documents incorporated by reference, contains forward-looking statements within the meaning of the federal securities laws, Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. We intend these forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995 and are including this statement for purposes of complying with those safe harbor provisions. 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. Some of the risks and uncertainties that may cause our actual results, performance or achievements to differ materially from those expressed or implied by forward-looking statements include, among others, the following:

- the success, cost and timing of clinical trials for SRK-015, including the progress and completion of our Phase 2 clinical trial and any future clinical trials for 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 for SRK-181 for the treatment of cancers resistant to checkpoint blockade therapies (“CBTs”) 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, SRK-181 and our other programs;
- 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, SRK-181 or any of our future product candidates;
- the potential for our identified research priorities to advance our proprietary platform, development programs or product candidates;
- the timing, scope, or likelihood of our ability to obtain and maintain regulatory approval from the U.S. Food and Drug Administration, European Medicines Agency and other applicable regulatory authorities for SRK-015, SRK-181 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;
- our ability to establish or maintain collaborations or strategic relationships, including our collaboration with Gilead Sciences, Inc.;
- our ability to obtain additional funding when necessary;
- 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 expectations related to the use of our cash reserves;
- the impact of the implementation of new laws and regulations or changes to existing laws and regulations;
- developments and projections relating to our competitors and our industry;

- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing, including our expected use of proceeds from our initial public offering; and
- other risks and uncertainties, including those listed under Part II, Item 1A, Risk Factors.

The risks set forth above are not exhaustive. Other sections of this report may include additional factors that could adversely affect our business and financial performance. Moreover, we operate in a very competitive and rapidly changing environment. New risk factors emerge from time to time and it is not possible for management to predict all risk factors, nor can we assess the impact of all risk factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements. Given these risks and uncertainties, investors should not place undue reliance on forward-looking statements as a prediction of actual results. Investors should also refer to our most recent Annual Reports on Form 10-K and our Quarterly Reports on Form 10-Q for future periods and Current Reports on Form 8-K as we file them with the SEC, and to other materials we may furnish to the public from time to time through Current Reports on Form 8-K or otherwise, for a discussion of risks and uncertainties that may cause actual results, performance or achievements to differ materially from those expressed or implied by forward-looking statements. We expressly disclaim any responsibility to update any forward-looking statements to reflect changes in underlying assumptions or factors, new information, future events, or otherwise, and you should not rely upon these forward-looking statements after the date of this report.

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 share and per share data)**

	March 31, 2019	December 31, 2018
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 67,004	\$ 115,069
Marketable securities	92,662	60,576
Prepaid expenses and other current assets	2,362	2,296
Total current assets	162,028	177,941
Property and equipment, net	3,644	3,190
Operating lease right-of-use asset	5,105	—
Restricted cash	205	205
Total assets	<u>\$ 170,982</u>	<u>\$ 181,336</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 3,249	\$ 3,303
Accrued expenses	4,141	7,157
Deferred rent	—	16
Operating lease liability	1,036	—
Loan payable	270	424
Deferred revenue	25,364	20,209
Other current liabilities	15	14
Total current liabilities	34,075	31,123
Long-term portion of deferred rent	—	871
Long-term portion of operating lease liability	4,930	—
Other long-term liabilities	20	24
Long-term portion of deferred revenue	34,434	42,695
Total liabilities	73,459	74,713
Commitments and contingencies (Note 8)		
Stockholders' equity:		
Preferred stock, \$0.001 par value; 10,000,000 shares authorized at March 31, 2019 and December 31, 2018; no shares issued and outstanding at March 31, 2019 and December 31, 2018	—	—
Common stock, \$0.001 par value; 150,000,000 shares authorized and 26,217,447 shares issued and outstanding as of March 31, 2019; 150,000,000 shares authorized and 26,217,701 shares issued and outstanding as of December 31, 2018	26	26
Additional paid-in capital	215,084	213,453
Accumulated other comprehensive loss	16	(8)
Accumulated deficit	(117,603)	(106,848)
Total stockholders' equity	97,523	106,623
Total liabilities and stockholders' equity	<u>\$ 170,982</u>	<u>\$ 181,336</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 share and per share data)**

	<b>Three Months Ended March 31,</b>	
	<b>2019</b>	<b>2018</b>
Revenue	\$ 3,106	\$ —
Operating expenses:		
Research and development	10,739	6,701
General and administrative	4,070	2,315
Total operating expenses	<u>14,809</u>	<u>9,016</u>
Loss from operations	(11,703)	(9,016)
Other income (expense):		
Interest income, net	948	144
Other expense, net	<u>—</u>	<u>(20)</u>
Total other income (expense)	948	124
Net loss	<u>\$ (10,755)</u>	<u>\$ (8,892)</u>
Net loss per share, basic and diluted	<u>\$ (0.42)</u>	<u>\$ (3.18)</u>
Weighted average common shares outstanding, basic and diluted	<u>25,592,659</u>	<u>2,795,497</u>
Comprehensive loss:		
Net loss	\$ (10,755)	\$ (8,892)
Other comprehensive income (loss):		
Unrealized gain on marketable securities	24	1
Total other comprehensive income (loss)	<u>24</u>	<u>1</u>
Comprehensive loss	<u>\$ (10,731)</u>	<u>\$ (8,891)</u>

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

**SCHOLAR ROCK HOLDING CORPORATION**  
**CONSOLIDATED STATEMENTS OF CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY**  
**(DEFICIT)**  
**(Unaudited)**  
(In thousands, except share and per share data)

	Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount				
<b>Balance at December 31, 2017</b>	<b>43,135,911</b>	<b>\$ 109,232</b>	<b>3,970,586</b>	<b>\$ 4</b>	<b>\$ 4,001</b>	<b>\$ (2)</b>	<b>\$ (57,525)</b>	<b>\$ (53,522)</b>
Unrealized gain on marketable securities			—	—	—	1	—	1
Equity-based compensation expense	—	—	—	—	482	—	—	482
Net loss	—	—	—	—	—	—	(8,892)	(8,892)
<b>Balance at March 31, 2018</b>	<b>43,135,911</b>	<b>\$ 109,232</b>	<b>3,970,586</b>	<b>\$ 4</b>	<b>\$ 4,483</b>	<b>\$ (1)</b>	<b>\$ (66,417)</b>	<b>\$ (61,931)</b>

	Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount				
<b>Balance at December 31, 2018</b>	<b>—</b>	<b>\$ —</b>	<b>26,217,701</b>	<b>\$ 26</b>	<b>\$ 213,453</b>	<b>\$ (8)</b>	<b>\$ (106,848)</b>	<b>\$ 106,623</b>
Unrealized loss on marketable securities	—	—	—	—	—	24	—	24
Restricted shares forfeited during the period	—	—	(2,237)	—	—	—	—	—
Exercise of stock options	—	—	1,983	—	13	—	—	13
Equity-based compensation expense	—	—	—	—	1,618	—	—	1,618
Net Loss	—	—	—	—	—	—	(10,755)	(10,755)
<b>Balance at March 31, 2019</b>	<b>—</b>	<b>\$ —</b>	<b>26,217,447</b>	<b>\$ 26</b>	<b>\$ 215,084</b>	<b>\$ 16</b>	<b>\$ (117,603)</b>	<b>\$ 97,523</b>

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

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

	Three Months Ended	
	March 31,	
	2019	2018
<b>Cash flows from operating activities:</b>		
Net loss	\$ (10,755)	\$ (8,892)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	271	179
Equity-based compensation	1,618	482
Amortization of investment securities	(365)	(31)
Amortization of operating lease right-of-use asset	244	—
Deferred payroll tax credit	—	42
Change in operating assets and liabilities:		
Prepaid expenses and other current assets	(206)	(1,923)
Accounts payable	(98)	1,529
Accrued expenses	(3,016)	(1,271)
Deferred rent	—	(32)
Operating lease liabilities	(130)	—
Deferred revenue	(3,106)	—
Other liabilities	13	33
Net cash used in operating activities	<u>(15,530)</u>	<u>(9,884)</u>
<b>Cash flows from investing activities:</b>		
Purchase of property and equipment	(681)	(176)
Purchase of marketable securities	(77,297)	(23,412)
Sales and maturities of marketable securities	45,600	—
Net cash used in investing activities	<u>(32,378)</u>	<u>(23,588)</u>
<b>Cash flows from financing activities:</b>		
Principal payments on loan payable	(167)	(167)
Proceeds from stock option exercises	13	—
Other	(3)	—
Net cash used in financing activities	<u>(157)</u>	<u>(167)</u>
Net decrease in cash and cash equivalents and restricted cash	(48,065)	(33,639)
Cash and cash equivalents and restricted cash, beginning of period	115,274	56,666
Cash and cash equivalents and restricted cash, end of period	<u>\$ 67,209</u>	<u>\$ 23,027</u>
<b>Supplemental disclosure of non-cash items:</b>		
Property and equipment purchases in accounts payable	\$ 483	\$ 13
Deferred offering costs in accounts payable and accrued expenses	\$ —	\$ 985
Operating lease right-of-use asset obtained in exchange for operating lease obligation	\$ 5,349	\$ —
<b>Supplemental cash flow information:</b>		
Cash paid for interest	\$ 4	\$ 10

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 highly 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’s second product candidate, SRK-181, is being developed for the treatment of cancers resistant to checkpoint blockade therapies. The Company was originally formed in May 2012. Its principal offices are in Cambridge, Massachusetts.

Since its inception, the Company’s operations have focused on research and development of monoclonal antibodies that selectively inhibit activation of growth factors for therapeutic effect, as well as establishing the Company’s intellectual property portfolio and performing research and development activities. 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, as well as research and development collaboration agreements.

Revenue generation activities have been limited to two collaborations, both containing research services and the issuance of a license. The first agreement, executed in 2013 was with Janssen Biotech, Inc. (“Janssen”), a subsidiary of Johnson & Johnson. The second agreement, executed in December 2018 was with Gilead Sciences, Inc. (“Gilead”), and the Company began recognizing revenue on the Gilead Collaboration Agreement in 2019. No revenues have been recorded from the sale of any commercial product.

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 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. The Company anticipates that it will continue to incur significant operating losses for the next several years as it continues to develop its product candidates. The Company believes that its existing cash and cash equivalents, and marketable securities at March 31, 2019 will be sufficient to allow the Company to fund its current operations through at least a period of one year after the date the financial statements are issued.

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

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

The significant accounting policies used in preparation of the unaudited consolidated financial statements are described in the Company’s audited consolidated financial statements as of and for the year ended December 31, 2018, and the notes thereto, which are included in the Company’s Annual Report on Form 10-K. Material changes to the significant accounting policies previously disclosed in the Company’s Annual Report on Form 10-K for the year ended December 31, 2018 are reflected below.

### **Leases**

Effective January 1, 2019, the Company adopted Accounting Standards Codification (“ASC”), Topic 842, Leases (“ASC 842”), using the modified retrospective approach and utilizing the effective date as its date of initial application, for which prior periods are presented in accordance with the previous guidance in ASC 840, Leases.

At the inception of an arrangement, the Company determines whether the arrangement is or contains a lease based on the unique facts and circumstances present. Leases with a term greater than one year are recognized on the balance sheet as right-of-use assets, lease liabilities and, if applicable, long-term lease liabilities. Operating lease liabilities and their corresponding right-of-use assets are recorded based on the present value of lease payments over the expected remaining lease term. However, certain adjustments to the right-of-use asset may be required for items such as incentives received. The interest rate implicit in lease contracts is typically not readily determinable. As a result, the Company utilizes its incremental borrowing rates, which are the rates incurred to borrow on a collateralized basis over a similar term an amount equal to the lease payments in a similar economic environment.

In accordance with the guidance in ASC 842, components of a lease should be split into three categories: lease components (e.g. land, building, etc.), non-lease components (e.g. common area maintenance, consumables, etc.), and non-components (e.g. property taxes, insurance, etc.) Then the fixed and in-substance fixed contract consideration (including any related to non-components) must be allocated based on the respective relative fair values to the lease components and non-lease components.

### **Reclassifications**

Certain reclassifications have been made to prior year financial statements to conform to classifications used in the current year.

### **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”). 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. Management bases its estimates on historical experience and on various assumptions that are believed to be reasonable under the circumstances. Actual results could differ from those estimates.

### **Recently Adopted Accounting Pronouncements**

ASU 2016-02, Leases and ASU 2018-11, Leases, Targeted Improvements

In February 2016, the FASB issued ASU 2016-02, Leases, (“ASU 2016-02”), which superseded the lease accounting requirements in ASC 840, Leases and created a new Topic 842, Leases.

In adopting the new standard, the Company elected to utilize the available package of practical expedients permitted under the transition guidance within the new standard, which removed the requirement to reassess previous accounting

conclusions around whether arrangements are or contain leases, the classification of leases, and the treatment of initial direct costs. The adoption of this standard resulted in the recognition of operating lease liabilities and right-of-use assets of \$6.1 million and \$5.3 million, respectively, as of January 1, 2019. There was no cumulative transition adjustment to retained earnings upon adoption of the standard and there was no material effect on the Company's statements of operations or statement of cash flows.

### Recently Issued Accounting Pronouncements

In August 2018, the FASB issued ASU 2018-15, *Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract*. The new standard will align the requirements for capitalizing implementation costs for hosting arrangements (services) with costs for internal-use software (assets). As a result, certain implementation costs incurred in hosting arrangements will be deferred and amortized. The new standard will be effective for the Company on January 1, 2020. Early adoption is permitted. The Company is currently evaluating the potential impact that adoption of this standard 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 March 31, 2019 and December 31, 2018 (in thousands):

	Fair Value Measurements at March 31, 2019			
	Total	Level 1	Level 2	Level 3
<b>Assets:</b>				
Money market funds, included in cash and cash equivalents	\$ 62,744	\$ 62,744	\$ —	\$ —
<b>Marketable securities:</b>				
U.S. Treasury obligations	92,662	92,662	—	—
<b>Total assets</b>	<b>\$ 155,406</b>	<b>\$ 155,406</b>	<b>\$ —</b>	<b>\$ —</b>

	Fair Value Measurements at December 31, 2018			
	Total	Level 1	Level 2	Level 3
<b>Assets:</b>				
Money market funds, included in cash and cash equivalents	\$ 114,593	\$ 114,593	\$ —	\$ —
<b>Marketable securities:</b>				
U.S. Treasury obligations	60,576	60,576	—	—
<b>Total assets</b>	<b>\$ 175,169</b>	<b>\$ 175,169</b>	<b>\$ —</b>	<b>\$ —</b>

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 March 31, 2019 and December 31, 2018. There were no transfers between fair value measurements levels during the three months ended March 31, 2019 or 2018.

The carrying amounts reflected in the balance sheets for prepaid expenses and other current assets, accounts payable, and accrued expenses approximate their fair values at March 31, 2019 and December 31, 2018, 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 2 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. As of March 31, 2019, the warrant is currently exercisable for 7,614 shares of the Company's common stock at an exercise price of \$3.94 per share.

#### 4. Marketable Securities

The following table summarizes the Company's investments as of March 31, 2019 (in thousands):

	Amortized Cost	Gross Unrealized		Estimated Fair Value
		Gains	Losses	
Marketable securities available-for-sale:				
U.S. Treasury obligations	\$ 92,646	\$ 18	\$ (2)	\$ 92,662
Total available-for-sale securities	\$ 92,646	\$ 18	\$ (2)	\$ 92,662

The following table summarizes the Company's investments as of December 31, 2018 (in thousands):

	Amortized Cost	Gross Unrealized		Estimated Fair Value
		Gains	Losses	
Marketable securities available-for-sale:				
U.S. Treasury obligations	\$ 60,584	\$ —	\$ (8)	\$ 60,576
Total available-for-sale securities	\$ 60,584	\$ —	\$ (8)	\$ 60,576

#### 5. Accrued Expenses

As of March 31, 2019 and December 31, 2018, accrued expenses consist of the following (in thousands):

	As of	
	March 31, 2019	December 31, 2018
Accrued external research and development expense	\$ 2,203	\$ 3,284
Accrued payroll and related expenses	1,110	2,826
Accrued professional and consulting expense	629	890
Accrued other	199	157
	\$ 4,141	\$ 7,157

#### 6. 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 shares of common stock 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 March 31, 2019, no shares of the Preferred Stock were issued or outstanding.

#### 7. Equity-Based Compensation

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

	Three Months Ended March 31,	
	2019	2018
Research and development expense	\$ 579	\$ 245
General and administrative expense	1,039	237
	\$ 1,618	\$ 482

**Restricted Stock**

The following table summarizes restricted common stock activity as of March 31, 2019:

	Number of Shares	Weighted Average Fair Value per Share at Issuance
Restricted common stock as of December 31, 2018	664,174	\$ 5.77
Granted	—	\$ —
Vested	(90,500)	\$ 5.77
Forfeited	(2,237)	\$ 5.77
Restricted common stock as of March 31, 2019	571,437	\$ 5.77

As of March 31, 2019, the Company had unrecognized equity-based compensation expense of \$2.5 million related to restricted stock issued to employees and directors, which is expected to be recognized over a period of 1.8 years.

**Stock Options**

The following table summarizes the Company’s stock option activity for the three months ended March 31, 2019:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding as of December 31, 2018	1,627,947	\$ 10.86	9.26	\$ 19,831
Granted	660,100	14.81		
Exercised	(1,983)	6.63		
Cancelled	(14,194)	7.87		
Outstanding as of March 31, 2019	2,271,870	12.03	9.24	\$ 16,475
Options exercisable as of March 31, 2019	318,576	\$ 10.00	8.73	\$ 2,901

Using the Black-Scholes option pricing model, the weighted average fair value of options granted to employees and directors during the three months ended March 31, 2019 was \$10.60.

The following assumptions were used in determining the fair value of options granted in the three months ended March 31, 2019:

Risk-free interest rate	2.52 %
Expected dividend yield	0.0 %
Expected term (years to liquidity)	6.25
Expected volatility	81.62 %

As of March 31, 2019, the Company has unrecognized equity-based compensation expense related to its employee stock options of \$16.2 million which the Company expects to recognize over the remaining weighted average vesting period of 3.2 years.

**8. Commitments and Contingencies**

**Operating Leases**

Facility Lease

In March 2015, the Company entered into a 5-year lease for its corporate headquarters (the “lease”). The lease was further amended in February 2018, to add an additional space (the “expansion space”) at the current location and to extend the lease term (the “amended lease”). The amended lease expires in July 2023. Rent for the facility lease, including the expansion space, increases from \$1.4 million per year to \$1.7 million per year over the term of the lease.

Variable lease payments include the Company’s allocated share of costs incurred and expenditures made by the landlord in the operation and management of the building. The Company has the option to extend the term of the amended lease for one additional term of 5 years commencing after the amended lease expires.

Other information related to the Company’s lease was as follows (in thousands, except lease term and discount rate):

	<b>For Three Months Ended March 31, 2019</b>
<b>Lease Cost:</b>	
Operating lease cost	\$ 343
Variable lease cost	179
Total lease cost	<u>\$ 522</u>
<b>Other information:</b>	
Operating cash flows used for operating leases	\$ 229
Weighted average remaining lease term	4.42 years
Weighted average discount rate	6.47 %

The following is a maturity analysis of the annual undiscounted cash flows reconciled to the carrying value of the operating lease liabilities as of March 31, 2019 (in thousands):

<b>Year Ending December 31,</b>	
2019 (excluding the three months ended March 31, 2019)	\$ 1,039
2020	1,447
2021	1,623
2022	1,672
2023	1,137
Thereafter	—
Total lease payments	<u>6,918</u>
Less imputed interest	<u>(952)</u>
Total operating lease liabilities	<u>\$ 5,966</u>

#### **Legal Proceedings**

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

#### **9. 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 March 31, 2019 and December 31, 2018 was \$0.3 million and \$0.4 million, respectively.

#### **10. Agreements**

##### ***Collaboration with Gilead***

##### ***Agreement Summary***

On December 19, 2018 (the “Effective Date”), the Company entered into a Master Collaboration Agreement (the “Gilead Collaboration Agreement”) with Gilead to discover and develop specific inhibitors of TGFβ activation focused on the treatment of fibrotic diseases. Under the collaboration, Gilead has exclusive options to license worldwide rights to product candidates that emerge from three of the Company’s TGFβ programs (each a “Gilead Program”). Pursuant to the Gilead Collaboration Agreement, the Company is responsible for antibody discovery and preclinical research through product candidate nomination, after which, upon exercising the option for a

Gilead Program, Gilead will be responsible for the program's preclinical and clinical development and commercialization. Such option may be exercised by Gilead at any time from the Effective Date through a date that is 90 days following the expiration of the Research Collaboration Term (as defined below) for a given Gilead Program, or until termination of the Gilead Program, whichever is earlier (the "Option Exercise Period").

The Company received a non-refundable upfront payment of \$50 million under the Gilead Collaboration Agreement. If Gilead exercises its option to exclusively license a Gilead Program, the Company may earn a total potential aggregate option exercise fee, development, regulatory and commercial milestone payments with respect to each Gilead Program of \$475 million, or a total of \$1,425 million across all three Gilead Programs. Additionally, in partial consideration of the rights granted to Gilead pursuant to the License Agreement, Gilead shall pay to the Company certain tiered royalties at a rate ranging from the high single-digits to the low double-digits (depending on the amount of net sales) on each Licensed Product in a given calendar year, on a country-by-country basis. In addition, Gilead will pay the Company a one-time milestone payment in the amount of \$25 million following achievement of successful demonstration of *in vivo* proof of concept consistent with certain criteria detailed in the Gilead Collaboration Agreement. None of the payments under the Gilead Collaboration Agreement are refundable.

Simultaneously with the entry into the Gilead Collaboration Agreement, the Company entered into a Share Purchase Agreement with Gilead (the "Gilead Equity Agreement"). Pursuant to the terms of the Gilead Equity Agreement, Gilead purchased 980,392 shares of common stock of the Company (the "Shares") at a purchase price of \$30.60 per share, for an aggregate purchase price of \$30 million. The Company did not incur any material costs in connection with the issuance of the Shares.

The Company and Gilead have established a joint steering committee (the "JSC"). The JSC will, among other powers and responsibilities, review, oversee and have decision-making responsibilities for certain strategic activities performed under the Gilead Programs, including reviewing and amending the research plans, reviewing any development candidate nominations, selecting a development candidate, and overseeing the strategic direction of the Gilead Programs. The Company will conduct its activities for each Gilead Program under the Gilead Collaboration Agreement, on a program-by-program basis, during the period beginning on the Effective Date and ending on the earliest to occur of (a) the date that the JSC first approves a selected development candidate for such program, (b) the third anniversary of the Effective Date, or (c) the effective date of termination of the Gilead Collaboration Agreement (the "Research Collaboration Term"). During the Research Collaboration Term, for each Gilead Program, the Company will notify Gilead, through the JSC, of up to two Gilead Program antibodies (in the case that Gilead rejects one, in accordance with the terms of the Gilead Collaboration Agreement) that satisfy the development criteria for such program (the "Development Candidate Nomination").

This Gilead Collaboration Agreement will remain in effect, unless otherwise earlier terminated in accordance with the terms of the Gilead Collaboration Agreement, on a program-by-program basis, until Gilead exercises its option with respect to a given Gilead Program or until expiration of the applicable Option Exercise Period, whichever is earlier (the "Term"). Unless earlier terminated, the Term shall expire in its entirety upon the expiration of the last to expire Option Exercise Period under the Gilead Collaboration Agreement. Gilead may terminate the Gilead Collaboration Agreement in its entirety or on a program-by-program basis in its sole discretion upon prior written notice to the Company pursuant to the terms of the agreement. The Gilead Collaboration Agreement may also be terminated on a program-by-program basis by either party in the event of an uncured material breach of the Gilead Collaboration Agreement by the other party.

Prior to Gilead's exercise of an option, the Company will have the lead responsibility for drug discovery and pre-clinical development of all Gilead Programs through to Development Candidate Nomination. Within a certain period of time after receiving a data package for a Development Candidate Nomination, Gilead may exercise its option to enter into a Form of License Agreement for exclusive rights to develop, manufacture and commercialize the licensed antibodies and licensed products of such Gilead Program.

#### *Accounting Treatment*

The Company assessed this arrangement in accordance with ASC 606 and concluded that the contract counterparty, Gilead, is a customer. The Company identified the following material promises under the arrangement: (1) the non-

exclusive, royalty-free research and development license; (2) the research and development services for the Gilead Programs; and (3) the options to license each of the three Gilead Programs to develop, manufacture and commercialize licensed candidates and resulting products, which were determined to be material rights for each program. The research and development services for each of the three Gilead Programs were determined to not be distinct from the research and development license and have been combined into a single performance obligation for each Gilead Program. Additionally, the option and associated material right for each Gilead Program represent separate performance obligations. The promises under the Gilead Collaboration Agreement relate primarily to the research and development required by the Company for each of the Gilead Programs nominated by Gilead. The Company does not have significant responsibilities subsequent to Gilead's exercise of each option.

At the commencement of the arrangement, two units of accounting were identified: the issuance of 980,392 of the Company's common shares and the joint research activities during the three-year research collaboration term. The Company determined the total transaction price to be \$80 million, consisting of \$17.1 million attributed to the equity sold to Gilead and \$62.9 million attributed to the joint research activities. In determining the fair value of the common stock at closing, the Company considered the closing price of the common stock at the time of the transaction and included a lack of marketability discount because the shares were subject to certain restrictions. Of the \$30 million equity investment, \$12.9 million was determined to be a premium and therefore was included as part of the transaction price to be allocated over the performance obligations. The potential incremental payment of \$25 million is due following achievement of successful demonstration of *in vivo* proof of concept consistent with certain criteria detailed in the Gilead Collaboration Agreement and will be included in the transaction price when it becomes probable that a future material reversal will not occur. The Company will reevaluate the transaction price at the end of each reporting period and as uncertain events are resolved or other changes in circumstances occur, and, if necessary, will adjust its estimate of the transaction price. The \$62.9 million attributed to the joint research activities was allocated to the performance obligations based on their standalone selling prices (the "SSP") when the Gilead Collaboration Agreement was executed. The Company made certain estimates when determining the SSP. For the research licenses and related research and development services, the estimated SSP is primarily based on the nature of the services to be performed and estimates of the associated effort and costs of the services. The Company developed the estimated SSP for the material rights based on the intrinsic value of the license upon exercise of the underlying option, industry standards for product development and estimates for the likelihood of option exercise.

The consideration related to the underlying options will not be included in the transaction price until the options are exercised. Additionally, the subsequent potential development, regulatory and commercial milestones are excluded from the transaction price, until after Gilead exercises its respective options.

Revenue associated with the research and development and license performance obligations relating to the Gilead Programs is recognized as revenue as the research and development services are provided using an input method, according to the costs incurred on each Gilead Program and the costs expected to be incurred in the future to satisfy the performance obligation. The amounts allocated to the three material rights will be recognized when Gilead exercises each respective option and delivers the underlying license and transfer of know-how, or immediately as each option expires unexercised. The transfer of control occurs over time. In management's judgment, this input method is the best measure of progress towards satisfying the performance obligation. The amounts received that have not yet been recognized as revenue are recorded in deferred revenue on the Company's consolidated balance sheet.

None of the performance obligations have been fully satisfied as of March 31, 2019. As a result of the joint research activities conducted during the three months ended March 31, 2019, the Company recognized \$3.1 million in revenue in the Company's consolidated statements of operations and comprehensive loss under the Gilead Collaboration Agreement. The aggregate amount of the transaction price allocated to the Company's unsatisfied performance obligations and recorded in deferred revenue at March 31, 2019 is \$59.8 million. The Company will recognize the deferred revenue based on a cost input method, over the remaining research term for each respective Gilead Program, which is a maximum of 2.75 years as of March 31, 2019; each research term is dependent on the timing of Gilead either exercising its options for the Gilead Programs or terminating further development on the Gilead Programs prior to the expiration date of the research term.



## 11. Net Loss per Share

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 has generated a net loss in all periods presented, so the basic and diluted net loss per share are the same, as the inclusion of the potentially dilutive securities would be anti-dilutive.

The following table sets forth the outstanding common stock equivalents, presented based on amounts outstanding at each period end, that have been excluded from the calculation of diluted net loss per share for the periods indicated because their inclusion would have been anti-dilutive):

	<b>Three Months Ended March 31,</b>	
	<b>2019</b>	<b>2018</b>
Convertible preferred stock	—	15,109,950
Restricted common stock	571,437	1,114,089
Warrant	7,614	7,614
Stock options	2,271,870	660,319
	<u>2,850,921</u>	<u>16,891,972</u>

## 12. Related Party Transactions

### *Licensing Agreement*

Pursuant to a license agreement with Children’s Medical Center Corporation (“CMCC”), a common share holder, the Company has an obligation to pay CMCC an annual license maintenance fee of \$10,000 for each year 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 that the Company develops using its proprietary platform, and between 10% to 20% of non-royalty income attributable to a sublicense of the CMCC rights. The Company recorded research and development expense in the statements of operations of \$2,500 for the three months ended March 31, 2019 and 2018. The Company had \$0.7 million due to CMCC under this license agreement at March 31, 2019 and December 31, 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 a total of \$40,000 of consulting expense related to these contracts, for the three months ended March 31, 2019 and 2018, respectively. There are no amounts due under these consulting agreements at March 31, 2019 or December 31, 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 (the “Quarterly Report”), and the audited financial information and the notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2018.*

*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, 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 novel 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. Our lead product candidate, SRK-015, is a highly selective, fully human, monoclonal antibody, with a unique mechanism of action that results in inhibition of the activation of the growth factor, myostatin, in skeletal muscle. We have advanced SRK-015 as a potential first muscle-directed therapy for the treatment of spinal muscular atrophy (“SMA”) into clinical development. We initiated our Phase 1 clinical trial of SRK-015 in healthy volunteers in May 2018 and reported positive interim results in early 2019. We have initiated a Phase 2 clinical trial in patients with SMA and commenced patient dosing in April 2019. Our second product candidate, SRK-181, is being developed for the treatment of cancers resistant to checkpoint blockade therapies (“CBTs”) such as anti-PD1 or anti-PDL antibodies. SRK-181 is a highly selective inhibitor of the activation of transforming growth factor beta-1 (“TGFβ1”). We intend to initiate a Phase 1 clinical trial of SRK-181 in cancer immunotherapy in mid-2020. In addition, utilizing our proprietary platform, we are continuing to create 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 our initial public offering (“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 us. Upon the closing of the IPO, all outstanding shares of convertible preferred stock converted into 15,109,950 shares of common stock.

Since inception, we have incurred significant operating losses. Our net losses were \$10.8 million and \$8.9 million for the three months ended March 31, 2019 and 2018, respectively. As of March 31, 2019, we had an accumulated deficit of

\$117.6 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 development activities for SRK-015, our lead product candidate, as we conduct our Phase 2 clinical trial;
- continue research and development activities for SRK-181, our program focused on inhibitors of the activation of TGF $\beta$ 1;
- continue to discover, validate and develop additional product candidates;
- maintain, expand and protect our intellectual property portfolio;
- hire additional research, development and business personnel; and
- continue to create the infrastructure to support our operations as a public company.

To date, we have not generated any revenue from product sales and do not expect to generate any revenue from the sale of products in the near future. If we successfully complete clinical development and obtain regulatory approval for SRK-015, SRK-181 or any of our future product candidates, we may generate revenue in the future from product sales. In addition, if we obtain regulatory approval for SRK-015, SRK-181 or any of our future product candidates, we expect to incur significant expenses related to developing our commercialization capability to support product sales, marketing and distribution activities.

## **Financial Operations Overview**

### ***Revenue***

No revenues have been recorded from the sale of any commercial product. Revenue generation activities have been limited to collaborations, containing research services and the issuance of a license. Currently, revenue is being recognized related to the Master Collaboration Agreement (the “Gilead Collaboration Agreement”) with Gilead Sciences, Inc. (“Gilead”) which was executed in December 2018, and we began recognizing associated revenue in 2019.

Revenue associated with the research and development and license performance obligations relating to the Gilead Programs is recognized as revenue as the research and development services are provided using an input method, according to the costs incurred on each Gilead Program and the costs expected to be incurred in the future to satisfy the performance obligation. The transfer of control occurs over time. In management’s judgment, this input method is the best measure of progress towards satisfying the performance obligations. The amounts received that have not yet been recognized as revenue are recorded in deferred revenue on our consolidated balance sheet. We expect to recognize the deferred revenue according to costs incurred, over the remaining research term for each respective Gilead Program, which is up to three years from the execution of the agreement; each research term is dependent on the timing of Gilead either exercising its options for the Gilead Programs or terminating further development on the Gilead Programs prior to the expiration date of the research term.

### ***Operating 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, preclinical studies, manufacturing, 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;
- expenses incurred under agreements with third parties that conduct research and development and preclinical activities on our behalf;
- expenses incurred under agreements related to our clinical trials, including the costs for investigative sites and clinical research organizations (“CROs”), that conduct our clinical trials;
- manufacturing process-development, clinical supplies and technology-transfer expenses;
- consulting and professional fees related to research and development activities;

- costs of purchasing laboratory supplies and non-capital equipment used in our internal research and development activities;
- 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.

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. However, we do not allocate our internal research and development expenses, consisting primarily of employee related costs, depreciation and other indirect costs, on a program-by-program basis as they are deployed across multiple projects.

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. In addition, we expect to incur additional costs in connection with our research and development activities under our collaboration with Gilead.

The successful development of SRK-015, SRK-181 and any future product candidates is 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, SRK-181 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:

- the scope, progress, outcome and costs of our preclinical development activities, clinical trials and other research and development activities;
- 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;
- significant and changing government regulation;
- 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, SRK-181 or any of our future product candidates would significantly change the costs and timing associated with the development of that product candidate.

### 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, business development, investor relations, legal, information technology 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 product candidates. These increases will likely include increased costs related to the hiring of additional personnel, as well as 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.

### Interest Income, Net

Interest income, net consists primarily of interest income earned on our cash and 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 the warrant issued in connection with our credit facility.

### Results of Operations

#### Comparison of the Three Months Ended March 31, 2019 and 2018

The following table summarizes our results of operations for the three months ended March 31, 2019 and 2018 (in thousands, except percentages):

	Three Months Ended March 31,		Change	
	2019	2018	\$	%
Revenue	\$ 3,106	\$ —	\$ 3,106	100.0 %
Operating expenses:				
Research and development	10,739	6,701	4,038	60.3 %
General and administrative	4,070	2,315	1,755	75.8 %
Total operating expenses	14,809	9,016	5,793	64.3 %
Loss from operations	(11,703)	(9,016)	(2,687)	29.8 %
Other income (expense):				
Interest income, net	948	144	804	NM *
Other expense, net	—	(20)	20	NM *
Total other income (expense)	948	124	824	NM *
Net loss	<u>\$ (10,755)</u>	<u>\$ (8,892)</u>	<u>\$ (1,863)</u>	21.0 %

\* NM means not meaningful.

**Revenue**

Revenue was \$3.1 million for the three months ended March 31, 2019. No revenue was recognized for the three months ended March 31, 2018. The revenue for the three months ended March 31, 2019 was related to the Gilead Collaboration Agreement, which was executed in December 2018. Revenue associated with the research and development and license performance obligations relating to the Gilead Programs is recognized as the research and development services are provided using a cost input method.

**Operating Expenses****Research and Development**

Research and development expense was \$10.7 million for the three months ended March 31, 2019 compared to \$6.7 million for the three months ended March 31, 2018, an increase of \$4.0 million or 60.3%. The following table summarizes our research and development expense for the three months ended March 31, 2019 and 2018 (in thousands, except percentages):

	Three Months Ended March 31,		Change	
	2019	2018	\$	%
<b>External costs by program:</b>				
SRK-015	\$ 1,743	\$ 1,995	\$ (252)	(12.6)%
SRK-181	1,736	—	1,736	100.0 %
Other early programs and unallocated costs	2,001	1,295	706	54.5 %
Total external costs	5,480	3,290	2,190	66.6 %
<b>Internal costs:</b>				
Employee compensation and benefits	3,436	2,150	1,286	59.8 %
Facility and other	1,823	1,261	562	44.6 %
Total internal costs	5,259	3,411	1,848	54.2 %
Total research and development expense	\$ 10,739	\$ 6,701	\$ 4,038	60.3 %

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

- The \$2.2 million increase in external costs primarily related to costs associated with SRK-181, which we declared as our second product candidate in March 2019. The increase includes a one time option fee owed upon its product candidate declaration. Other increases relate to external research costs associated with our other early programs. These increases are partially offset by lower manufacturing expenses related to SRK-015.
- The \$1.8 million increase in internal research and development costs was primarily driven by an increase in employee compensation and benefits costs, associated with increased headcount and related overhead, as we continued to build out our research and development functions.

In 2019, we expect our research and development expenses to increase, as we continue to advance the development of our product candidates, including SRK-015 through our Phase 2 clinical trial, and continue development of SRK-181. Additionally, we expect to continue to conduct research under our Gilead collaboration.

**General and Administrative**

General and administrative expense was \$4.1 million for the three months ended March 31, 2019 compared to \$2.3 million for the three months ended March 31, 2018, an increase of \$1.8 million or 75.8%. The increase in general and administrative expense was primarily attributable to an increase of \$1.6 million in employee compensation and benefits, including stock compensation, related to increased headcount and \$0.2 million in other costs related to items such as premiums for directors and officers' insurance.

We expect our general and administrative expenses to increase in 2019, as we continue to invest in our internal infrastructure to support our overall company growth, and as we comply with the responsibilities of being a public company.

#### **Interest Income, Net**

The increase in interest income, net was attributable to increased income earned on our investment portfolio, associated with larger cash balances during the three months ended March 31, 2019 as compared to the three months ended March 31, 2018.

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

There was no other expense for the three months ended March 31, 2019. The other expense for the three months ended March 31, 2018 was associated with the change in fair value of the warrant during the three months ended March 31, 2018. Previously, the warrant was classified as a liability, the liability was re-measured at its fair value at each balance sheet date and recorded in other expense. Subsequent to our IPO, the warrant is classified as equity and no longer fair valued.

#### **Liquidity and Capital Resources**

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

Since our inception, we have not generated any product revenue and have incurred significant operating losses and negative cash flows from our operations. We have funded our operations to date primarily with proceeds from the sale of our convertible preferred stock and units in private placements before our IPO, and sale of our common stock through our IPO and to Gilead in an exempt private placement as well as payments from our research collaborations.

The following table provides information regarding our total cash and cash equivalents and marketable securities at March 31, 2019 and December 31, 2018 (in thousands):

	<b>March 31, 2019</b>	<b>December 31, 2018</b>
Cash and cash equivalents	\$ 67,004	\$ 115,069
Marketable securities	92,662	60,576
<b>Total cash, cash equivalents and marketable securities</b>	<b>\$ 159,666</b>	<b>\$ 175,645</b>

During the three months ended March 31, 2019, our cash, cash equivalents and marketable securities balance decreased approximately \$16.0 million. The decrease was primarily due to cash used to operate our business, including payments related to, among other things, research and development and general and administrative expenses as we continue to invest in our primary product candidates and support our internal research and development efforts. We also made capital purchases and payments on our debt.

In December 2018, we entered into the Gilead Collaboration Agreement with Gilead pursuant to which we will conduct research and pre-clinical development activities relating to the diagnosis, treatment, cure, mitigation or prevention of diseases, disorders or conditions, other than in the field of oncology in accordance with a pre-determined research plan. Pursuant to the Gilead Collaboration Agreement, Gilead made non-refundable payments of \$80.0 million, including an upfront payment and an equity investment.

In May 2018, we completed our IPO, in which we 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 us.

Prior to the IPO, we primarily funded our operations from inception through the IPO with the net proceeds of \$109.2 million from sales of our convertible preferred stock and units, as well as borrowings of \$2.0 million under our credit facility with SVB.

## Cash Flows

The following table provides information regarding our cash flows for the three months ended March 31, 2019 and 2018 (in thousands):

	Three Months Ended March 31,	
	2019	2018
Net cash used in operating activities	\$ (15,530)	\$ (9,884)
Net cash used in investing activities	(32,378)	(23,588)
Net cash used in financing activities	(157)	(167)
Net decrease in cash and cash equivalents and restricted cash	\$ (48,065)	\$ (33,639)

### Net Cash Used in Operating Activities

Net cash used in operating activities was \$15.5 million for the three months ended March 31, 2019 and consisted of our net loss of \$10.8 million and changes in our assets and liabilities of \$6.5 million, primarily related to a change of \$3.1 million in deferred revenue related to the Gilead collaboration and \$3.0 million in accrued expenses, partially offset by non-cash adjustments of \$1.8 million, primarily from equity-based compensation. Net cash used in operating activities was \$9.9 million for the three months ended March 31, 2018 and consisted of our net loss of \$8.9 million and \$1.7 million in changes in our assets and liabilities, partially offset by \$0.7 million in non-cash adjustments.

### Net Cash Used in Investing Activities

Net cash used in investing activities was \$32.4 million for the three months ended March 31, 2019 compared to net cash used in investing activities of \$23.6 million for the three months ended March 31, 2018. Net cash used in investing activities for both periods was primarily associated with transactions involving our marketable securities, including purchases, sales and maturities.

### Net Cash Used in Financing Activities

Net cash used in financing activities was \$0.2 million for both the three months ended March 31, 2019 and the three months ended March 31, 2018. These amounts are primarily attributable to 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, our product candidates, including SRK-015 and SRK-181, and any of our future product candidates. In addition, if we obtain marketing approval for SRK-015, SRK-181 or any of our future product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. Furthermore, we expect to incur additional costs associated with operating as a public company.

We expect that our existing cash and cash equivalents will enable us to fund our operating expenses and capital expenditure requirements into 2021.

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 continuing our development of our lead product candidate, SRK-015, including enrollment in the Phase 2 clinical trial, and the costs of conducting future clinical trials, including for SRK-181;
- the costs of future manufacturing of SRK-015, SRK-181 and any other product candidates;
- 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 of identifying and developing, or in-licensing or acquiring, additional product candidates and technologies;
- 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 of seeking marketing approvals for our product candidates that successfully complete clinical trials, if any;
- 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;
- the costs of expanding our infrastructure and facilities to accommodate our growing employee base, including adding equipment and physical infrastructure to support our research and development; 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, common stockholder ownership interests may be diluted, and the terms of these securities may include liquidation or other preferences that could adversely affect the rights of 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, the results of which form the basis for making judgements about the carrying values of assets and liabilities that are not readily apparent from other sources. 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. Our actual results may differ from these estimates under different assumptions or conditions.

There have been no material changes to our critical accounting policies from those described in Part II, Item 7. “Management’s Discussion and Analysis of Financial Condition and Results of Operations” included in our Annual Report on Form 10-K for the year ended December 31, 2018, except for the following.

### **Revenue Recognition**

No revenues have been recorded from the sale of any commercial product. Revenue generation activities have been limited to collaborations, containing research services and the issuance of a license. Currently, revenue is being recognized related to the Gilead Collaboration Agreement which was executed in December 2018. We began recognizing associated revenue in 2019 over the period that research is performed under the collaboration. We account for revenue under ASC Topic 606, Revenue from Contracts with Customers (“ASC 606”).

Under ASC 606, we recognize revenue when our customer obtains control of promised goods or services, in an amount that reflects the consideration which we expect to receive in exchange for those goods or services. To determine the appropriate amount of revenue to be recognized for arrangements determined to be within the scope of ASC 606, we perform the following five steps: (i) identification of the contract(s) with the customer, (ii) identification of the promised goods or services in the contract and determination of whether the promised goods or services are performance obligations, (iii) measurement of the transaction price, (iv) allocation of the transaction price to the performance obligations, and (v) recognition of revenue when (or as) we satisfy each performance obligation. We only apply the five-step model to contracts when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services we transfer to our customer.

#### **Identification of the Contract(s) with the Customer**

We account for a contract with a customer that is within the scope of ASC 606 when all of the following criteria are met: (i) the arrangement has been approved by the parties and the parties are committed to perform their respective obligations, (ii) each party’s rights regarding the goods or services to be transferred can be identified, (iii) the payment terms for the goods or services to be transferred can be identified, (iv) the arrangement has commercial substance and (v) collection of substantially all of the consideration to which we will be entitled in exchange for the goods or services that will be transferred to the customer is probable.

#### **Identification of the Performance Obligations**

Performance obligations are promised goods or services in a contract to transfer a distinct good or service to the customer. Promised goods or services are considered distinct when: (i) the customer can benefit from the good or service on its own or together with other readily available resources and (ii) the promised good or service is separately identifiable from other promises in the contract. In assessing whether promised goods or services are distinct, we consider factors such as the stage of development of the underlying intellectual property, the capabilities of our customer to develop the intellectual property on their own and whether the required expertise is readily available. Arrangements that include rights to additional goods or services that are exercisable at a customer’s discretion are generally considered options. We assess if these options provide a material right to the customer and if so, they are considered performance obligations. The identification of material rights requires judgments related to the determination of the value of the underlying license relative to the option exercise price, including assumptions about technical feasibility and the probability of developing a candidate that would be subject to the option rights.

#### **Determination of the Transaction Price**

We estimate the transaction price based on the amount of consideration we expect to receive for transferring the promised goods or services in the contract. The consideration may include both fixed consideration and variable consideration. At the inception of each arrangement that includes variable consideration, we evaluate the amount of the potential payments and the likelihood that the payments will be received. We utilize either the most likely amount method or expected value method to estimate the transaction price based on which method better predicts the amount of

consideration expected to be received. If it is probable that a significant revenue reversal would not occur, the variable consideration is included in the transaction price.

We evaluate whether development, regulatory, and commercial milestone payments are considered probable of being reached and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within our control or the licensee's control, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. At the end of each reporting period, we re-evaluate the probability of achievement of such milestones and any related constraint, and if necessary, adjust our estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect collaboration revenue and earnings in the period of adjustment. To date, no milestones have been achieved under our arrangements with customers.

For sales-based royalties, including milestone payments based on the level of sales, we determine whether the sole or predominant item to which the royalties relate is a license. When the license is the sole or predominant item to which the sales-based royalty relates, we recognize revenue at the later of: (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, we have not recognized any sales-based royalty revenue resulting from our arrangement.

#### **Allocation of Transaction Price**

We allocate the transaction price based on the estimated standalone selling price. We must develop assumptions that require judgment to determine the standalone selling price for each performance obligation identified in the contract. We utilize key assumptions to determine the standalone selling price, which may include other comparable transactions, pricing considered in negotiating the transaction and the estimated costs. Estimating costs for research and development programs is subjective as we estimate the costs anticipated to successfully complete the research performance obligations. As the research is novel, efforts to be successful may be significantly different than the estimated costs at the beginning of the contract. Certain variable consideration is allocated specifically to one or more performance obligations in a contract when the terms of the variable consideration relate to the satisfaction of the performance obligation and the resulting amounts allocated to each performance obligation are consistent with the amounts we would expect to receive for satisfying each performance obligation.

#### **Recognition of Revenue**

We utilize judgment to determine whether the performance obligation is satisfied over time or at a point in time. We determine the appropriate method of measuring progress performance obligations satisfied over time for purposes of recognizing revenue, such as by using an input method based on costs incurred compared to the costs expected to be incurred in the future to satisfy the performance obligation. We evaluate the measure of progress each reporting period and, if necessary, adjust the measure of performance and related revenue recognition. The estimated remaining costs is highly subjective, as the research is novel, therefore efforts to be successful may be significantly different than the estimated costs made at the balance sheet date. If the license to our intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, we will recognize revenue from non-refundable, up-front fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license.

We receive payments from customers based on billing schedules established in each contract. Up-front payments and fees are recorded as deferred revenue upon receipt or when due until we perform our obligations under these arrangements. Amounts are recorded as accounts receivable when our right to consideration is unconditional.

#### **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.

### **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, 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 Risk**

We are a smaller reporting company as defined by Rule 12b-2 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), and are not required to provide the information required under this item.

### **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 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 March 31, 2019, the end of the period covered by this Quarterly Report. 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 March 31, 2019 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 are subject to various legal proceedings and claims that arise in the ordinary course of our business activities. Although the results of litigation and claims cannot be predicted with certainty, as of the date of this Quarterly Report, we do not believe we are party to any claim or litigation the outcome of which, if determined adversely to us, would individually or in the aggregate be reasonably expected to have a material adverse effect on our business. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

## 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, before investing in our common stock. 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 market price of our common stock could decline if one or more of these risks or uncertainties were to occur, which may cause you to lose all or part of the money you paid to buy our common stock. 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. Certain statements below are forward-looking statements. See “Special Note Regarding Forward-Looking Statements” in this report.

### 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 three months ended March 31, 2019 and 2018, we reported a net loss of \$10.8 million and \$8.9 million, respectively. As of March 31, 2019 and December 31, 2018, we had an accumulated deficit of \$117.6 million and \$106.8 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 product candidates, SRK-015, and SRK-181 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 2 clinical development, and, if successful, later-stage clinical trials;
- advance our other preclinical development programs into clinical development, including our product candidate SRK-181, in cancer immunotherapy, and identifying our next product candidate and lead indication in our TGFβ1 program;
- 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 development, manufacturing and scientific personnel as we continue to develop our product candidates;

- 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 current or potential future collaborators must develop and eventually commercialize products with significant market potential and favorable pricing. 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 could 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 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, SRK-181 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, SRK-181 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 March 31, 2019, we had approximately \$159.7 million in cash and cash equivalents and marketable securities. Based on our current operating plan, we believe that our existing cash and cash equivalents and marketable securities as of March 31, 2019 will be sufficient to fund our operating expenses and capital expenditure requirements into 2021. 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 SRK-015, SRK-181 and any potential future product candidates;
- the clinical development plans we establish for these product candidates;
- the number and characteristics of product candidates that we identify and develop;

- the terms of any collaboration, strategic alliance, or licensing agreements we are currently party to or may choose to enter into in the future;
- 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 other than pursuant to our collaboration agreement with Gilead and our license agreement with Janssen, which payments we may not receive in full or at all, and we cannot be certain that additional funding will be available on acceptable terms, or at all. Even if we receive the maximum payments under the collaboration agreement with Gilead or license agreement with Janssen, the payments may not meet our current or future funding requirements. 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, SRK-181 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 SRK-015, SRK-181 or one or more of our future product candidates or 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 SRK-181 and 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, our lead product candidate, is our only clinical stage 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 negatively affected. Our second product candidate, SRK-181, is in preclinical development and we intend to initiate a Phase 1 clinical trial in cancer immunotherapy in mid-2020. All of



our other programs are in preclinical development, and we may nominate additional product candidates from these programs.

SRK-015, SRK-181 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. 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 May 1, 2019, we had 69 full-time employees. As our clinical development plans and strategies develop, we expect we will need to hire additional managerial, clinical development, scientific, regulatory, and administrative personnel. If our product candidates approach commercialization, we will also need to hire sales, marketing and other commercial 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 regulatory review process for SRK-015, SRK-181, 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, SRK-181 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. 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 hinder the timing and 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 existing and 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.

***Inadequate funding for the FDA, the SEC and other government agencies, including from government shut downs, could prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.***

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA and other government employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Future shutdowns could also affect other government agencies such as the SEC, which may also impact our business by delaying review of our public filings, to the extent such review is necessary, and our ability to access the public markets.

### **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 and SRK-181. 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.

***Preclinical and clinical development involve 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, SRK-181, 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, SRK-181 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 if a clinical trial is 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. For example, we anticipate some of our future trials to, in part, utilize an “open-label” trial design. An open-label trial is one where both the patient and investigator know whether the patient is receiving the test article or either an existing approved drug or placebo. Open-label trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label studies are aware that they are receiving treatment. Open-label trials may be subject to a “patient bias” where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. Patients selected for early clinical studies often include the most severe sufferers and their symptoms may have been bound to improve notwithstanding the new treatment. In addition, open-label trials may be subject to an “investigator bias” where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. The opportunity for bias in clinical trials as a result of open-label design may not be adequately handled and may cause any of our trials that utilize such design to fail and additional trials may be necessary to support future marketing applications. Further, the FDA, EMA or other regulatory authorities 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, SRK-181 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, SRK-181 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 or patients 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 an estimated 30,000 to 35,000 patients in the United States and Europe. As a result, we may encounter difficulties enrolling patients 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, including the anticipated testing of SRK-181, 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, additional 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 and cancer, 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, SRK-181 or any future product candidate we develop receives marketing approval, whether as a single agent or in combination with other therapies, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors, and others in the medical community. For example, doctors may deem it sufficient to treat patients with SMA with an SMN upregulator such as nusinersen, and therefore will not be willing to utilize SRK-015 in conjunction with such SMN upregulator. If the product candidates we develop do not achieve an adequate level of

acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of any product candidate, if approved for commercial sale, will depend on a number of factors, including:

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

***If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.***

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our research and development activities involve the use of biological and hazardous materials and produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials, which could cause an interruption of our commercialization efforts, research and development efforts and business operations, environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling and disposal of these materials and specified waste products. Although we believe that the safety procedures utilized by our third-party manufacturers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources and state or federal or other applicable authorities may curtail our use of certain materials and/or interrupt our business operations. Furthermore, environmental laws and regulations are complex, change frequently and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

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

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

On December 22, 2017, President Trump signed into law the "Tax Cuts and Jobs Act," or the TCJA, which 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 ability to use our net operating loss carryforwards and certain tax credit carryforwards may be subject to limitation.***

As of December 31, 2018, we had net operating loss carryforwards for federal and state income tax purposes of \$93.0 million and \$92.4 million, respectively, which begin to expire in 2034, except for our 2018 federal net operating loss carryforwards of \$42.6 million which do not expire. As of December 31, 2018, we also had available tax credit carryforwards for federal and state income tax purposes of \$4.0 million and \$1.1 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, European Union (“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 the EMA granted Orphan Medicinal Product Designation to SRK-015 for the treatment of SMA. We may seek Orphan Drug Designation from regulatory authorities in other jurisdictions for SRK-015 and Orphan Drug Designation from the FDA, EMA 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.***

Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is intended to treat a rare disease or condition, defined as a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the European Union, after a recommendation from the EMA's Committee for Orphan Medicinal Products ("COMP") the European Commission grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union. Additionally, designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biological product or where there is no satisfactory method of diagnosis, prevention or treatment, or, if such a method exists, the medicine must be of significant

benefit to those affected by the condition. Any approval that we are granted for our product candidates in the United States or Europe would not assure approval of product candidates in the other or in any other jurisdiction. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. In addition, if a product receives the first FDA approval for the indication for which it has orphan designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity or where the manufacturer is unable to assure sufficient product quantity. In the European Union, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity following drug or biological product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity.

We have received Orphan Drug Designation from the FDA for SRK-015 for the treatment of SMA, and the EMA's COMP has adopted a positive opinion designating SRK-015 as an orphan medicinal product for the treatment of SMA. Even if we are able to obtain orphan designation, we may not be the first to obtain marketing approval for such 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 with different active moieties can be approved for the same condition. In the United States, even after an orphan drug is approved, the FDA may subsequently approve another drug for the same condition if the FDA concludes that the latter drug is not the same drug or is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In the European Union, marketing authorization may be granted to a similar medicinal product for the same orphan indication if:

- the second applicant can establish in its application that its medicinal product, although similar to the orphan medicinal product already authorized, is safer, more effective or otherwise clinically superior;
- the holder of the marketing authorization for the original orphan medicinal product consents to a second orphan medicinal product application; or
- the holder of the marketing authorization for the original orphan medicinal product cannot supply sufficient quantities of orphan medicinal product.

***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.

On January 31, 2019, the Department of Health and Human Services (HHS) and HHS Office of Inspector General (OIG) proposed an amendment to one of the existing Anti-Kickback safe harbors (42 C.F.R. 1001.952(h)) which would prohibit certain pharmaceutical manufacturers from offering rebates to pharmacy benefit managers (“PBMs”) in the Medicare Part D and Medicaid managed care programs. The proposed amendment would remove protection for “discounts” from Anti-Kickback enforcement action, and would include criminal and civil penalties for knowingly and willfully offering, paying, soliciting, or receiving remuneration to induce or reward the referral of business reimbursable under federal health care programs. At the same time, HHS also proposed to create a new safe harbor to protect point-of-

sale discounts that drug manufacturers provide directly to patients, and adds another safe harbor to protect certain administrative fees paid by manufacturers to PBMs. If this proposal is adopted, in whole or in part, it could affect the pricing and reimbursement for any products for which we receive approval in the future.

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.

In addition, there has been a trend of increased state regulation of payments made to physicians for marketing. Some states, such as California, Massachusetts and Vermont, mandate implementation of corporate compliance programs, along with the tracking and reporting of gifts, compensation, and other remuneration to physicians.

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 or maintaining 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 may not approve the manufacturing, marketing and promotion of the product candidate in other 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 ("CMS"), the agency responsible for administering the Medicare program, 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% (70% as of January 1, 2019, pursuant to the Balanced Budget Act of 2018) 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.

There have been a number of significant changes to the ACA and its implementation. The Tax Cuts and Jobs Act of 2017 ("Tax Act"), includes a provision that repealed 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." On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, or the Texas District Court Judge, ruled that the individual mandate is a critical and inseparable feature of the ACA, and therefore, because it was repealed as part of the Tax Act, the remaining provisions of the ACA are invalid as well. While the Trump Administration and CMS have both stated that the ruling will have no immediate effect, and on December 30, 2018 the Texas District Court Judge issued an order staying the judgment pending appeal, it is unclear how this decision, subsequent appeals and other efforts to repeal and replace the ACA will impact the ACA and our business.

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 (“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 finalized 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. Additionally, CMS published a final rule permitting further collections and payments to and from certain Affordable Care Act qualified health plans and health insurance issuers under the Affordable Care Act risk adjustment program in response to the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. 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 and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

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 disclosed publicly. 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.

***Failure to comply with health and data protection laws and regulations could lead to government enforcement actions (which could include civil or criminal penalties), private litigation, and/or adverse publicity and could negatively affect our operating results and business.***

We and any potential collaborators may be subject to federal, state, and foreign data protection laws and regulations (i.e., laws and regulations that address privacy and data security). In the United States, numerous federal and state laws and regulations, including federal health information privacy laws, state data breach notification laws, state health information privacy laws, and federal and state consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), that govern the collection, use, disclosure and protection of health-related and other personal information could apply to our operations or the operations of our collaborators. In addition, we may obtain health information from third parties (including research institutions from which we obtain clinical trial data) that are subject to privacy and security requirements under HIPAA, as amended by HITECH. Depending on the facts and circumstances, we could be subject to civil, criminal, and administrative penalties if we knowingly obtain, use, or disclose individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA.

Compliance with U.S. and international data protection laws and regulations could require us to take on more onerous obligations in our contracts, restrict our ability to collect, use and disclose data, or in some cases, impact our ability to operate in certain jurisdictions. Failure to comply with these laws and regulations could result in government enforcement actions (which could include civil, criminal and administrative penalties), private litigation, and/or adverse publicity and could negatively affect our operating results and business. Moreover, clinical trial subjects, employees and other individuals about whom we or our potential collaborators obtain personal information, as well as the providers who share this information with us, may limit our ability to collect, use and disclose the information. Claims that we have violated individuals' privacy rights, failed to comply with data protection laws, or breached our contractual obligations,

even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business.

In the event we conduct clinical trial in the European Economic Area (“EEA”), we may be subject to additional privacy laws. The General Data Protection Regulation, (EU) 2016/679 (“GDPR”) became effective on May 25, 2018, and deals with the processing of personal data and on the free movement of such data. The GDPR imposes a broad range of strict requirements on companies subject to the GDPR, including requirements relating to having legal bases for processing personal information relating to identifiable individuals and transferring such information outside the EEA, including to the United States, providing details to those individuals regarding the processing of their personal information, keeping personal information secure, having data processing agreements with third parties who process personal information, responding to individuals’ requests to exercise their rights in respect of their personal information, reporting security breaches involving personal data to the competent national data protection authority and affected individuals, appointing data protection officers, conducting data protection impact assessments, and record-keeping. The GDPR increases substantially the penalties to which we could be subject in the event of any non-compliance, including fines of up to 10,000,000 Euros or up to 2% of our total worldwide annual turnover for certain comparatively minor offenses, or up to 20,000,000 Euros or up to 4% of our total worldwide annual turnover for more serious offenses. Given the new law, we face uncertainty as to the exact interpretation of the new requirements and we may be unsuccessful in implementing all measures required by data protection authorities or courts in interpretation of the new law.

In particular, national laws of member states of the EU are in the process of being adapted to the requirements under the GDPR, thereby implementing national laws which may partially deviate from the GDPR and impose different obligations from country to country, so that we do not expect to operate in a uniform legal landscape in the EU. Also, as it relates to processing and transfer of genetic data, the GDPR specifically allows national laws to impose additional and more specific requirements or restrictions, and European laws have historically differed quite substantially in this field, leading to additional uncertainty.

In the event we conduct clinical trials in the EEA, we must also ensure that we maintain adequate safeguards to enable the transfer of personal data outside of the EEA, in particular to the United States, in compliance with European data protection laws. We expect that we will continue to face uncertainty as to whether our efforts to comply with our obligations under European privacy laws will be sufficient. If we are investigated by a European data protection authority, we may face fines and other penalties. Any such investigation or charges by European data protection authorities could have a negative effect on our existing business and on our ability to attract and retain new clients or pharmaceutical partners. We may also experience hesitancy, reluctance, or refusal by European or multi-national clients or pharmaceutical partners to continue to use our products and solutions due to the potential risk exposure as a result of the current (and, in particular, future) data protection obligations imposed on them by certain data protection authorities in interpretation of current law, including the GDPR. Such clients or pharmaceutical partners may also view any alternative approaches to compliance as being too costly, too burdensome, too legally uncertain, or otherwise objectionable and therefore decide not to do business with us. Any of the foregoing could materially harm our business, prospects, financial condition and results of operations.

***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 (“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 (the “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 may 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 may be 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, SRK-181 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, and/or 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, disclosed, or misappropriated their confidential information or 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 or 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 the grant of a third-party's patent in opposition proceedings in the European Patent Office ("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, because patent applications in PCT member jurisdictions are typically not published until 18 months after the earliest filing, and because 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.

For applications filed under pre-AIA, interference proceedings declared 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 2034 through 2040, 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. While the validity of a subset of patents at issue was subsequently upheld by a district court jury, uncertainty remains as to the legal question pertaining to the written description requirement under 35 USC §112 as it relates to functional antibodies. 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. This may prevent us from asserting this patent against our competitors marketing otherwise infringing products in relevant European or foreign 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 to conduct our clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines or comply with legal and 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 to conduct our clinical trials, under agreements with universities, medical institutions, CROs, strategic partners and others. We often have to negotiate budgets and contracts with such third parties, and if we are unsuccessful or if the negotiations take longer than anticipated, this could result in delays to our development timelines and increased costs.

We rely especially heavily on third parties over the course of our clinical trials, and, as a result, 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 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 trials. 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 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 and SRK-181.

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, SRK-181 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 which we believe have 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.***

In order to continue to conduct later-stage clinical trials with SRK-015, or conduct clinical trials of SRK-181 or any of our future product candidates, we will need to manufacture such product candidate 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 of our 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 a license 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.

***The failure to maintain the Gilead Collaboration Agreement, or the failure of Gilead to perform its obligations under or our failure to achieve certain milestones under the Gilead Collaboration Agreement could negatively impact our business, financial condition, results of operations and prospects.***

On December 19, 2018, we entered into a Master Collaboration Agreement, the Gilead Collaboration Agreement, with Gilead Sciences, Inc. (“Gilead”), to discover and develop specific inhibitors of TGF $\beta$  activation focused on the treatment of fibrotic diseases. Under the collaboration, Gilead has exclusive options to license worldwide rights to product candidates that emerge from three of our TGF $\beta$  programs. Pursuant to the Gilead Collaboration Agreement, we are responsible for antibody discovery and preclinical research through product candidate nomination, after which, upon exercising the option for a Gilead Program, Gilead will be responsible for the Gilead Program’s preclinical and clinical development and commercialization. In consideration of the foregoing, we received \$80 million in upfront payments, comprised of \$50 million in cash and a \$30 million equity investment in us. In addition, we will receive a one-time milestone payment of \$25 million if we successfully complete specific preclinical studies and will be eligible to receive up to an additional \$1,425 million in potential payments aggregated across all three Gilead Programs, based on the successful achievement of certain research, development, regulatory and commercialization milestones. We would also receive high single-digit to low double-digit tiered royalties on sales of potential future products originating from the collaboration. We cannot guarantee the outcome of our efforts to achieve such milestones, and, even if we achieve such

milestones, we cannot directly control Gilead's performance of its obligations under the agreement or the amount and timing of resources that Gilead will dedicate to these efforts, and accordingly, we may not receive any of the milestone or royalty payments that are contingent upon our or Gilead's achievements.

We are subject to a number of other risks associated with our collaboration with Gilead, including:

- If we are able to identify program antibodies and present Gilead with development candidate nominations, Gilead may not exercise its option to such program or we and Gilead could disagree as to future development plans, and Gilead may delay, fail to commence, or stop future preclinical and clinical development and commercialization; and
- If Gilead exercised one or more options, following such exercise, Gilead will have sole responsibility for the development and commercialization of the product candidates from such program in the applicable field. Gilead will have the sole discretion to determine and direct its efforts and resources, including the ability to discontinue all efforts and resources it applies to the development and, if approval is obtained, commercialization and marketing of the product candidates covered by the applicable program. Gilead may not be effective in obtaining approvals for the product candidates developed from the programs or in marketing, or arranging for necessary supply, manufacturing or distribution relationships for, any approved products. Furthermore, Gilead may change its strategic focus or pursue alternative technologies in a manner that results in reduced, delayed or no revenue to us. Gilead has a variety of marketed products and product candidates under collaboration with other companies, including some of our competitors, and its own corporate objectives may not be consistent with our best interests. If Gilead fails to develop, obtain regulatory approval for or ultimately commercialize any product candidate from the programs covered by the Gilead Collaboration Agreement, or if Gilead terminates our collaboration, our business, financial condition, results of operations and prospects would be harmed.
- There may be disputes between Gilead and us, including disagreements regarding the Gilead Collaboration Agreement, that may result in the delay of development programs, creation of uncertainty as to ownership of, control of, or access to intellectual property rights, litigation or arbitration proceedings, distraction of our management from other business activities, and our incurrence of substantial expenses. Any disagreements could result in failure to achieve developmental, regulatory and sales objectives that would have otherwise resulted in milestone or royalty payments to us or the delay or termination of any future development or commercialization of a Gilead Program.

The Gilead Collaboration Agreement is also subject to early termination, including through Gilead's right under certain circumstances to terminate upon advance notice to us. If the Gilead Collaboration Agreement is terminated early, we may not be able to find another collaborator for the further development and commercialization of the three Gilead Programs covered by the Gilead Collaboration Agreement on acceptable terms, or at all, and we may be unable to pursue continued development and commercialization of such programs on our own.

***We may not be successful in our efforts to discover antibodies or identify potential product candidates under the Gilead Collaboration Agreement.***

A key element of our strategy under the Gilead Collaboration Agreement is to use our proprietary technology to identify program antibodies that meet the development criteria for such Gilead Program. Our antibody discovery process may not be successful in identifying antibodies that meet the development criteria for a Gilead Program under the Gilead Collaboration Agreement or that we believe qualify as product candidates. Even if we identify and nominate a product candidate for any Gilead Program, Gilead may not choose to exercise its option for the Gilead Program or may not be successful in developing or commercializing such product candidate. If Gilead elected not to exercise an option, we would have incurred significant discovery and research expenses but may not be eligible to receive future milestone or royalty payments related to such program. Further development of a product candidate may also be discontinued by Gilead if the product candidate is shown to have harmful side effects or if other characteristics are observed that indicate the product candidate may be unlikely to receive marketing approval or achieve market acceptance. If Gilead decides not to move forward with a product candidate, that could negatively affect our business, including our reputation, and could hinder our ability to enter into future collaborations.

***We may seek to enter into collaborations in the future with other third parties, including for SRK-015, SRK-181 or potential product candidates. If we are unable to enter into such collaborations, or if these collaborations are not successful, our business could be adversely affected.***

A part of our strategy is to evaluate and, as deemed appropriate, enter into additional collaborations or 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, capabilities and funding for our programs and underlying technology.

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

- collaborators may have significant discretion or decision making authority in determining the efforts and resources that they will apply to the collaboration or that we are required to apply to the collaboration;
- collaborators may not perform their obligations as expected or in a manner satisfactory to us;
- we may commit to certain preclinical or clinical development or commercialization efforts as part of the collaboration that we are unable to meet or our collaborators may not be satisfied with our preclinical or clinical development or commercialization efforts;
- 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 biotechnology or pharmaceutical industry, including within 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 collaborations, the terms that we agree upon may not be favorable to us, and we may not be able to maintain such strategic collaborations 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 collaboration 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 is volatile, and you could lose all or part of your investment.***

Similar to the trading prices of the common stock of other biopharmaceutical companies, the trading price of our common stock is 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:

- 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 any preclinical studies or clinical trials for SRK-015 or SRK-181, including the results of our Phase 1 clinical trial for SRK-015;
- 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, SRK-181 or any future product candidate;
- introduction of new products or services offered by us or our competitors;
- announcements of significant acquisitions, strategic collaborations or 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 or inadequacy of our internal controls and procedures;
- 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 Board members, management, and their affiliates, own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.***

As of March 31, 2019, our executive officers, directors and their affiliates beneficially hold, in the aggregate, approximately 40.8% 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. These exemptions include:

- not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended (“Sarbanes-Oxley Act”);
- reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements;
- exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not previously approved;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements; and
- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced “Management’s Discussion and Analysis of Financial Condition and Results of Operations” disclosure.

We will remain an emerging growth company until the earlier of (1) December 31, 2023 (2) the last day of the fiscal year in which we have total annual gross revenue of at least \$1.07 billion or (3) the last day of the fiscal year 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 last business date of our most recently completed second fiscal quarter, and (4) 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.

Even after we no longer qualify as an emerging growth company, we may still qualify as a “smaller reporting company” if the market value of our common stock held by non-affiliates is below \$250 million (or \$700 million if our annual revenue is less than \$100 million) as of the last business day of our second fiscal quarter in any given year, which would allow us to take advantage of many of the same exemptions from disclosure requirements, including exemption from the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements.

***We expect to continue to incur increased costs as a result of operating as a public company, and our management is required to devote substantial time to new compliance initiatives.***

As a public company, we incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act of 2002 and rules subsequently implemented by the SEC and Nasdaq have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote a substantial amount of time to these compliance initiatives. These rules and regulations have significantly increased our legal and financial compliance costs and we anticipate that these activities will become more time-consuming and costly over time.

Pursuant to Section 404, we are required to furnish a report by our management on our internal control over financial reporting, and, once we are no longer an EGC or a “smaller reporting company”, we will be required to furnish an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we are engaged in a process to document

and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that neither we nor our independent registered public accounting firm will be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction to the trading price of our common stock in the financial markets due to a loss of confidence in the reliability of our financial statements.

***If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.***

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404, or any subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our stock.

We are required to disclose changes made in our internal controls and procedures on a quarterly basis and our management will be required to assess the effectiveness of these controls annually. However, for as long as we are an EGC or a “smaller reporting company”, our independent registered public accounting firm will not be required to attest to the effectiveness of our internal controls over financial reporting pursuant to Section 404. We could be an EGC for up to five years following the completion of our IPO and will qualify as a “smaller reporting company” if the market value of our common stock held by non-affiliates is below \$250 million (or \$700 million if our annual revenue is less than \$100 million) as of June 30 in any given year. An independent assessment of the effectiveness of our internal controls over financial reporting could detect problems that our management’s assessment might not. Undetected material weaknesses in our internal controls over financial reporting could lead to financial statement restatements and require us to incur the expense of remediation.

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

If any of our existing major stockholders sell substantial amounts of our common stock in the public market, the trading price of our common stock could decline. As of March 31, 2019, over 35% of our common stock was held by members of our Board and shareholders affiliated with our Board of Directors and if any of them were to sell a portion of their holdings of our common stock, our stock price could be negatively affected.

We issued 980,392 shares to Gilead in December 2018. The shares are subject to a lock-up period, but following the expiration of such lock-up period, such shares of our common stock may be freely sold in the open market, subject to compliance with applicable securities laws. The sale of these shares in the open market could cause the market price of our common stock to decline or become highly volatile.

In addition, shares of common stock that are either subject to outstanding options or reserved for future issuance under our existing equity compensation plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, the lock-up agreements and Rule 144 and Rule 701 under the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline. Additionally, the number of shares of our common stock reserved for issuance under our 2018 Stock Option and Incentive Plan automatically increased by 4% of the total number of shares of our capital stock outstanding on December 31, 2018 and will automatically increase on January 1 of year hereafter and

continuing through and including January 1, 2029, by 4% of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by our board of directors. Unless our board of directors elects not to increase the number of shares available for future grant each year, our stockholders will experience additional dilution.

***We have broad discretion in the use of our existing cash, cash equivalents and marketable securities and may not use them effectively.***

Our management has broad discretion in the application of our existing cash, cash equivalents and marketable securities. Because of the number and variability of factors that will determine our use of our existing cash and cash equivalents, their ultimate use may vary substantially from their currently intended use. Our management might not apply our existing cash and cash equivalents in ways that ultimately increase the value of your investment. The failure by our management to apply these funds effectively could harm our business.

***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. On December 19, 2018, the Court of Chancery of the State of Delaware issued a decision declaring that federal forum selection provisions purporting to require claims under the Securities Act be brought in federal court are ineffective and invalid under Delaware law. On January 17, 2019, the decision was appealed to the Delaware Supreme Court. While the Delaware Supreme Court recently dismissed the appeal on jurisdictional grounds, we expect that the appeal will be re-filed after the Court of Chancery issues a final judgment. Unless and until the Court of Chancery's decision is reversed by the Delaware Supreme Court or otherwise abrogated, we do not intend to enforce our federal forum selection provision designating the District of Massachusetts as the exclusive forum for Securities Act claims. In the event that the Delaware Supreme Court affirms the Court of Chancery's decision or otherwise determines that federal forum selection provisions are invalid, our board of directors intends to amend promptly our amended and restated by-laws to remove our federal forum selection bylaw provision. As a result of the Court of Chancery's decision or a decision by the Delaware Supreme Court affirming the Court of Chancery's decision, or if the federal forum selection provision is otherwise found inapplicable to, or unenforceable in respect of, one or more of the specified actions or proceedings, we may incur additional costs, which could have an adverse effect on our business, financial condition or results of operations. 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***

Not applicable.

***Issuer Purchases of Equity Securities***

None.

***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. As of March 31, 2019, we had used approximately \$36.0 million of the net offering proceeds, primarily to fund research and development activities for SRK-015, to fund TGFβ1 and other preclinical research and development activities and for working capital and general corporate purposes. 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 accordance with our investment policy. As of March 31, 2019, the remaining amount of the net proceeds is included as cash, cash equivalents and marketable securities.

**Item 3. Defaults Upon Senior Securities**

None.

**Item 4. Mine Safety Disclosures**

None.

**Item 5. Other Information**

None.

**Item 6. Exhibits****EXHIBIT INDEX**

<b>Exhibit Number</b>	<b>Description</b>	<b>Incorporated by Reference to:</b>			
		<b>Form</b>	<b>File No.</b>	<b>Exhibit No.</b>	<b>Filing Date</b>
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
10.1	<a href="#">Amended and Restated Collaboration Agreement, dated March 12, 2019, by and between Scholar Rock, Inc. and Adimab, LLC</a>	8-K	001-38501	10.1	March 13, 2019
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 March 31, 2019, 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: May 15, 2019

By: /s/ Nagesh K. Mahanthappa

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

Date: May 15, 2019

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: May 15, 2019

/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: May 15, 2019

/s/ Rhonda M. Chicko  
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 March 31, 2019 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: May 15, 2019

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

Date: May 15, 2019

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

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