

Savara Inc  
Form 10-Q  
May 09, 2018

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2018

OR

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

For the transition period from \_\_\_\_\_ to \_\_\_\_\_

Commission File Number 001-32157

Savara Inc.

(Exact name of registrant as specified in its charter)

Delaware  
(State or other jurisdiction of  
incorporation or organization)

84-1318182  
(I.R.S. Employer  
Identification No.)

6836 Bee Cave Road, Building III, Suite 200

Austin, TX  
(Address of principal executive offices)

78746  
(Zip Code)

(512) 961-1891

Edgar Filing: Savara Inc - Form 10-Q

(Registrant's telephone number, including area code)

N/A

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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

Accelerated filer

Non-accelerated filer

Smaller reporting company

Emerging growth company

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

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

As of May 9, 2018, the registrant had 30,606,866 shares of common stock, \$0.001 par value per share, outstanding.

---

Table of Contents

	Page
PART I. FINANCIAL INFORMATION	
Item 1. Financial Statements (Unaudited)	
<u>Condensed Consolidated Balance Sheets</u>	1
<u>Condensed Consolidated Statements of Operations and Comprehensive Loss</u>	2
<u>Consolidated Statements of Changes in Redeemable Convertible Preferred Stock and Stockholders' Deficit</u>	3
<u>Condensed Consolidated Statements of Cash Flows</u>	4
<u>Notes to Condensed Consolidated Financial Statements</u>	5
Item 2. <u>Management's Discussion and Analysis of Financial Condition and Results of Operations</u>	19
Item 3. <u>Quantitative and Qualitative Disclosures About Market Risk</u>	25
Item 4. <u>Controls and Procedures</u>	25
PART II. <u>OTHER INFORMATION</u>	26
Item 1. <u>Legal Proceedings</u>	26
Item 1A. <u>Risk Factors</u>	26
Item 2. <u>Unregistered Sales of Equity Securities and Use of Proceeds</u>	47
Item 3. <u>Defaults Upon Senior Securities</u>	47
Item 4. <u>Mine Safety Disclosures</u>	47
Item 5. <u>Other Information</u>	47
Item 6. <u>Exhibits</u>	47
<u>Exhibit Index</u>	48
<u>Signatures</u>	49

Edgar Filing: Savara Inc - Form 10-Q

Savara Inc. and Subsidiaries

Condensed Consolidated Balance Sheets

(In thousands, except share and per share amounts)

(Unaudited)

	March 31, 2018	December 31, 2017
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$22,122	\$22,121
Short-term investments	62,862	72,192
Prepaid expenses and other current assets	2,586	3,551
Total current assets	87,570	97,864
Property and equipment, net	837	925
In-process R&D	12,238	33,626
Goodwill	27,171	27,082
Other non-current assets	1,056	131
Total assets	\$128,872	\$159,628
Liabilities and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	\$3,418	\$2,784
Accrued expenses	1,947	2,966
Current portion of capital lease obligation	21	265
Total current liabilities	5,386	6,015
Long-term liabilities:		
Debt facility	14,884	14,775
Contingent consideration	11,814	11,948
Deferred tax liability	2,692	7,181
Capital lease obligation, net of current portion	297	297
Other long-term liabilities	96	103
Total liabilities	35,169	40,319
Stockholders' equity:		
Common stock, \$0.001 par value, 500,000,000 shares authorized as of		
March 31, 2018 and December 31, 2017; 30,604,366 and 30,509,522		
shares issued and outstanding as of March 31, 2018 and December 31, 2017, respectively	32	32
Additional paid-in capital	187,448	186,522
Accumulated other comprehensive income (loss)	1,275	958
Accumulated deficit	(95,052 )	(68,203 )
Total stockholders' equity	93,703	119,309
Total liabilities and stockholders' equity	\$128,872	\$159,628

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

1

---

## Savara Inc. and Subsidiaries

## Condensed Consolidated Statements of Operations and Comprehensive Loss

(In thousands, except share and per share amounts)

(Unaudited)

	Three Months Ended	
	March 31,	2017
	2018	
Operating expenses:		
Research and development	\$8,539	\$2,948
General and administrative	1,769	1,736
Impairment of acquired IPR&D	21,692	—
Depreciation	107	90
Total operating expenses	32,107	4,774
Loss from operations	(32,107 )	(4,774 )
Other income (expense):		
Interest expense, net	(104 )	(245 )
Foreign currency exchange gain (loss)	(61 )	(32 )
Change in fair value of financial instruments	(56 )	(160 )
Total other income (expense)	(221 )	(437 )
Loss before income taxes	(32,328 )	(5,211 )
Income tax benefit	5,479	237
Net loss	\$(26,849 )	\$(4,974 )
Accretion of redeemable convertible preferred stock	—	(24 )
Net loss attributable to common stockholders	\$(26,849 )	\$(4,998 )
Other comprehensive income:		
Gain (loss) on foreign currency translation	341	143
Unrealized gain (loss) on short-term investments	(24 )	—
Total Comprehensive Loss	\$(26,532 )	\$(4,831 )
Net loss per share:		
Basic and diluted	\$(0.88 )	\$(1.65 )
Weighted average common shares outstanding		
Basic and diluted	30,543,746	3,029,223

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

## Savara Inc. and Subsidiaries

## Consolidated Statements of Changes in Stockholders' Equity (Deficit)

Period Ended March 31, 2018

(In thousands, except share amounts)

(Unaudited)

	Stockholders' Equity (Deficit)		Accumulated			Total
	Common Stock		Additional	Other		
	Number of	Paid-In	Accumulated	Comprehensive		
	Shares	Amount	Capital	Deficit	Income	
Balance on December 31, 2017	30,509,522	\$ 32	\$ 186,522	\$ (68,203 )	\$ 958	\$ 119,309
Issuance of common stock upon At The Market sales, net	46,900	—	493	—	—	493
Issuance of common stock for settlement of RSUs	11,250	—	—	—	—	—
Net issuance of common stock upon cashless exercise of stock options	28,655	—	—	—	—	—
Issuance of common stock upon exercise of stock options	6,000	—	3	—	—	3
Issuance of common stock upon exercise of warrants	2,039	—	18	—	—	18
Stock-based compensation	—	—	412	—	—	412
Foreign exchange translation adjustment	—	—	—	—	341	341
Unrealized gain (loss) on short-term investments	—	—	—	—	(24 )	(24 )
Net loss incurred	—	—	—	(26,849 )	—	(26,849 )
Balance on March 31, 2018	30,604,366	\$ 32	\$ 187,448	\$ (95,052 )	\$ 1,275	\$ 93,703

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





## Savara Inc. and Subsidiaries

## Condensed Consolidated Statements of Cash Flows

(In thousands)

(Unaudited)

	Three Months Ended	
	March 31, 2018	2017
<b>Cash flows from operating activities:</b>		
Net loss	\$(26,849)	\$(4,974)
<b>Adjustments to reconcile net loss to net cash used in operating activities:</b>		
Depreciation	107	90
Impairment of acquired IPR&D	21,692	—
Changes in fair value of financial instruments	96	60
Change in fair value of contingent consideration	(134 )	100
Noncash interest	35	98
Foreign currency gain/(loss)	61	32
Amortization of debt issuance costs	109	—
Accretion on discount to short-term investments and convertible promissory notes	(138 )	149
Stock-based compensation	412	81
Provision (benefit) for deferred taxes	(4,555 )	—
<b>Changes in operating assets and liabilities:</b>		
Grant and award receivable	—	400
Tax refund receivable	(873 )	(165 )
Prepaid expenses and other current assets	927	(32 )
Deferred rent	(7 )	(2 )
Accounts payable and accrued expenses	(554 )	1,319
Net cash used in operating activities	\$(9,671 )	\$(2,844 )
<b>Cash flows from investing activities:</b>		
Purchase of property and equipment	(19 )	(59 )
Maturities of available-for-sale securities	25,300	—
Purchase of available-for-sale securities	(15,891)	—
Net cash provided by / (used in) investing activities	\$9,390	\$(59 )
<b>Cash flows from financing activity:</b>		
Issuance of common stock upon exercise of warrants	18	—
Issuance of common stock upon at the market offerings, net	493	—
Proceeds from exercise of stock options	3	—
Capital lease obligation principal payments	(243 )	—
Net cash provided by financing activities	\$271	\$—
Effect of exchange rate changes on cash and cash equivalents	11	(6 )
Increase / (Decrease) in cash and cash equivalents	\$1	\$(2,909)
Cash and cash equivalents beginning of period	22,121	13,373
Cash and cash equivalents end of period	\$22,122	\$10,464

Non-cash transactions:

Accretion of redeemable convertible preferred stock	\$—	\$24
---	-----	------

Supplemental disclosure of cash flow information:

Cash paid for interest	\$334	\$—
------------------------	-------	-----

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

4

---

Savara Inc. and Subsidiaries

Notes to Condensed Consolidated Financial Statements (Unaudited)

1. Description of Business and Basis of Presentation

Description of Business

Savara Inc. (“Savara,” the “Company,” or as used in the context of “we” or “us”) is an orphan lung disease company. The Company’s pipeline comprises Molgradex, an inhaled granulocyte-macrophage colony-stimulating factor, or GM-CSF, in Phase 3 development for pulmonary alveolar proteinosis (“PAP”), and in Phase 2a development for nontuberculous mycobacterial (“NTM”) lung infection and AeroVanc, a Phase 3 stage inhaled vancomycin for treatment of persistent methicillin-resistant *Staphylococcus aureus* (“MRSA”) lung infection in individuals living with cystic fibrosis (“CF”). The Company and its wholly owned subsidiaries operate in one segment with its principal offices in Austin, Texas.

Since inception, Savara has devoted substantially all of its efforts and resources to identifying and developing its product candidates, recruiting personnel, and raising capital. Savara has incurred operating losses and negative cash flow from operations and has no product revenue from inception to date. The Company has not yet commenced commercial operations.

Basis of Presentation

The interim condensed consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States (“U.S. GAAP”) as defined by the Financial Accounting Standards Board (“FASB”). These condensed consolidated financial statements should be read in conjunction with the audited financial statements and notes thereto for the year ended December 31, 2017.

Unaudited Interim Financial Information

The interim condensed consolidated financial statements included in this document are unaudited. The unaudited interim financial statements have been prepared on the same basis as the annual financial statements and reflect, in the opinion of management, all adjustments of a normal and recurring nature that are necessary for a fair statement of the Company’s financial position as of March 31, 2018, and its results of operations for the three months ended March 31, 2018 and 2017, and cash flows for the three months ended March 31, 2018 and 2017. The results of operations for the three months ended March 31, 2018 are not necessarily indicative of the results to be expected for the year ending December 31, 2018 or for any other future annual or interim period. The December 31, 2017 consolidated balance sheet was derived from audited financial statements but does not include all disclosures required by U.S. GAAP. These condensed consolidated financial statements should be read in conjunction with the audited financial statements and notes thereto for the year ended December 31, 2017.

2. Summary of Significant Accounting Policies

Liquidity

As of March 31, 2018, the Company had an accumulated deficit of approximately \$95.1 million. The Company also had negative cash flow from operations of approximately \$9.7 million during the three months ended March 31, 2018. The cost to further develop and obtain regulatory approval for any drug is substantial and, as noted below, the Company may have to take certain steps to maintain a positive cash position. Accordingly, the Company will need additional capital to further fund the development of, and seek regulatory approvals for, its product candidates and

begin to commercialize any approved products.

Currently, the Company is primarily focused on the development of respiratory drugs and believes such activities will result in the Company's continued incurrence of significant research and development and other expenses related to those programs. If the clinical trials for any of the Company's product candidates fail or produce unsuccessful results and those product candidates do not gain regulatory approval, or if any of the Company's product candidates, if approved, fail to achieve market acceptance, the Company may never become profitable. Even if the Company achieves profitability in the future, it may not be able to sustain profitability in subsequent periods. The Company intends to cover its future operating expenses through cash and cash equivalents on hand and through a combination of equity offerings, debt financings, government or other third-party funding, and other collaborations and strategic alliances. The Company cannot be sure that additional financing will be available when needed or that, if available, financing will be obtained on terms favorable to the Company or its stockholders.

While the Company had cash and cash equivalents of \$22.1 million and short-term investments of \$62.9 million as of March 31, 2018, we intend to continue to raise additional capital as needed through the issuance of additional equity and potentially through borrowings, and strategic alliances with partner companies. However, if such financings are not available timely and at adequate levels, the Company will need to reevaluate its operating plans. The condensed consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

## Principles of Consolidation

The interim condensed consolidated financial statements of the Company are stated in U.S. dollars and are prepared using U.S. GAAP. These financial statements include the accounts of the Company and its wholly owned subsidiaries. The financial statements of the Company's wholly owned subsidiaries are recorded in their functional currency and translated into the reporting currency. The cumulative effect of changes in exchange rates between the foreign entity's functional currency and the reporting currency is reported in Accumulated Other Comprehensive Income. All intercompany transactions and accounts have been eliminated in consolidation.

## Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires the Company to make certain estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Management's estimates include those related to the accrual of research and development costs, the valuation of preferred and common shares, certain financial instruments recorded at fair value, stock-based compensation, and the valuation allowance for deferred tax assets. The Company bases its estimates on historical experience and on various other market-specific and relevant assumptions that it believes to be reasonable under the circumstances. Accordingly, actual results could be materially different from those estimates.

## Risks and Uncertainties

The product candidates being developed by the Company require approvals from the U.S. Food and Drug Administration (FDA) or foreign regulatory agencies prior to commercial sales. There can be no assurance that the Company's product candidates will receive the necessary approvals. If the Company is denied regulatory approval of its product candidates, or if approval is delayed, it may have a material adverse impact on the Company's business, results of operations and its financial position.

The Company is subject to a number of risks similar to other life science companies, including, but not limited to, risks related to the successful discovery and development of drug candidates, raising additional capital, development of competing drugs and therapies, protection of proprietary technology and market acceptance of the Company's products. As a result of these and other factors and the related uncertainties, there can be no assurance of the Company's future success.

## Cash and Cash Equivalents

Cash and cash equivalents consist of cash, institutional bank money market accounts, and commercial paper with original maturities of three months or less when acquired and are stated at cost, which approximates fair value.

## Short-term Investments

The Company has classified its investments in debt securities with readily determinable fair value as available-for-sale securities. These securities are carried at estimated fair value with the aggregate unrealized gains and losses related to these investments reflected as a part of "Accumulated other comprehensive income (loss)" within stockholders' equity.

The fair value of the investments is based on the specific quoted market price of the securities or comparable securities at the balance sheet dates. Investments in debt securities are considered to be impaired when a decline in fair value is judged to be other than temporary because the Company either intends to sell or it is more-likely-than not that it will have to sell the impaired security before recovery. Once a decline in fair value is determined to be other than temporary, an impairment charge is recorded and a new cost basis in the investment is established.

#### Concentration of Credit Risk

Financial instruments, which potentially subject the Company to concentrations of credit risk, consist principally of cash and cash equivalents and foreign exchange derivatives not designated as hedging. The Company places its cash and cash equivalents with a limited number of high quality financial institutions and at times may exceed the amount of insurance provided on such deposits.

#### Accrued Research and Development Costs

The Company records the costs associated with research, nonclinical studies, clinical trials, and manufacturing development as incurred. These costs are a significant component of the Company's research and development expenses, with a substantial portion of the Company's on-going research and development activities conducted by third-party service providers, including contract research and manufacturing organizations.

The Company accrues for expenses resulting from obligations under agreements with contract research organizations (“CROs”), contract manufacturing organizations (“CMOs”), and other outside service providers for which payment flows do not match the periods over which materials or services are provided to the Company. Accruals are recorded based on estimates of services received and efforts expended pursuant to agreements established with CROs, CMOs, and other outside service providers. These estimates are typically based on contracted amounts applied to the proportion of work performed and determined through analysis with internal personnel and external service providers as to the progress or stage of completion of the services. The Company makes significant judgments and estimates in determining the accrual balance in each reporting period. In the event advance payments are made to a CRO, CMO, or outside service provider, the payments will be recorded as a prepaid asset which will be amortized or expensed as the contracted services are performed. As actual costs become known, the Company adjusts its prepaids and accruals. Inputs, such as the services performed, the number of patients enrolled, or the study duration, may vary from the Company’s estimates resulting in adjustments to research and development expense in future periods. Changes in these estimates that result in material changes to the Company’s accruals could materially affect the Company’s results of operations. To date, the Company has not experienced any material deviations between accrued and actual research and development expenses.

#### Business Combinations

Assets acquired and liabilities assumed as part of a business acquisition are recorded at their estimated fair value at the date of acquisition. The excess of purchase price over the fair value of assets acquired and liabilities assumed is recorded as goodwill. Determining fair value of identifiable assets, particularly intangibles, and liabilities acquired also requires management to make estimates, which are based on all available information and, in some cases, assumptions with respect to the timing and amount of future revenue and expenses associated with an asset.

#### Goodwill, Acquired In-Process Research and Development (IPR&D) and Deferred Tax Liability

Goodwill and acquired IPR&D are not amortized but are tested annually for impairment or more frequently if impairment indicators exist. The Company adopted accounting guidance related to annual and interim goodwill and acquired IPR&D impairment tests which allows the Company to first assess qualitative factors before performing a quantitative assessment of the fair value of a reporting unit. If it is determined on the basis of qualitative factors that the fair value of the reporting unit is more likely than not less than the carrying amount, a quantitative impairment test is required. During the three months ended March 31, 2018, the Company experienced a \$0.1 million and \$0.3 million increase in the carrying value of goodwill and IPR&D, respectively, related to its acquisition of Savara ApS on July 15, 2016, which was due to foreign currency translation. In addition, during the three months ended March 31, 2018, the Company recorded \$21.7 million of impairment charges and a corresponding decrease to the carrying value of IPR&D related to the Aironite drug candidate assumed in the Merger as further described in Note 6 due to the unfavorable results from a Phase 2 study that demonstrated a failure of Aironite to meet the endpoints of the study and limited effectiveness of the compound in patients. As a result of the IPR&D impairment charges recorded in the first quarter of 2018, the Company reduced the associated deferred tax liability related to the acquired IPR&D from the Merger by \$4.6 million and recorded a tax benefit.

#### Tax Refund Receivable

The Company has recorded a Danish tax credit earned by its subsidiary, Savara ApS, for the three months ended March 31, 2018. Under Danish tax law, Denmark remits a research and development tax credit equal to 22% of

qualified research and development expenditures, not to exceed established thresholds. As of March 31, 2018, credits totaling \$1.6 million had been generated but not yet received. This Danish tax credit of approximately \$1.6 million is composed of \$0.9 million related to research and development activities incurred during the year ended December 31, 2017 and is recorded as a receivable in prepaid expenses and other current assets as receipt is expected to occur in the fourth quarter of 2018. The remaining portion of the Danish tax credit of \$0.7 million which was generated during the three months ended March 31, 2018 is recorded in other non-current assets and is expected to be received in the fourth quarter of 2019.

The Company also recognized a tax benefit for the three months ended March 31, 2018 as provided by the Australian Taxation Office for qualified research and development expenditures incurred on the NTM program incurred through our subsidiary, Savara Australia Pty. Limited. Under Australian tax law, Australia remits a research and development tax credit equal to 43% of qualified research and development expenditures, not to exceed established thresholds. As of March 31, 2018, credits totaling \$0.1 million had been generated but not yet received. This Australian tax credit of approximately \$0.1 million is recorded in other non-current assets and is expected to be received in during the fourth quarter of 2019.

### Segment Reporting

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision maker, or decision-making group, in making decisions on how to allocate resources and assess performance. Our chief operating decision maker is the chief executive officer. We have one operating segment, specialty pharmaceuticals within the respiratory system.



## Fair Value of Financial Instruments

The accounting standard for fair value measurements provides a framework for measuring fair value and requires disclosures regarding fair value measurements. Fair value is defined as the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date, based on the Company's principal or, in absence of a principal, most advantageous market for the specific asset or liability.

The Company uses a three-tier fair value hierarchy to classify and disclose all assets and liabilities measured at fair value on a recurring basis, as well as assets and liabilities measured at fair value on a non-recurring basis, in periods subsequent to their initial measurement. The hierarchy requires the Company to use observable inputs when available, and to minimize the use of unobservable inputs, when determining fair value.

The three tiers are defined as follows:

- Level 1 – Observable inputs that reflect quoted market prices (unadjusted) for identical assets or liabilities in active markets;
- Level 2 – Observable inputs other than quoted prices in active markets that are observable either directly or indirectly in the marketplace for identical or similar assets and liabilities; and
  - Level 3 – Unobservable inputs that are supported by little or no market data, which require the Company to develop its own assumptions.

Financial instruments carried at fair value include cash and cash equivalents and contingent consideration related to the acquisition of Serendex (Note 8) for which any change is reflected in general and administrative expense, and foreign exchange derivatives, certain warrants previously classified as liabilities and embedded put options separated from the convertible promissory notes which were converted to equity or derecognized pursuant to the Merger.

Financial instruments not carried at fair value include accounts payable and accrued liabilities. The carrying amounts of these financial instruments approximate fair value due to the highly liquid nature of these short-term instruments.

## Net Loss per Share

Basic net loss attributable to common stockholders per share is calculated by dividing the net loss attributable to common stockholders by the weighted average number of shares of common stock, restricted stock and restricted stock units outstanding during the period without consideration of common stock equivalents. Since the Company was in a loss position for all periods presented, diluted net loss per share is the same as basic net loss per share for all periods presented as the inclusion of all potential dilutive securities would have been antidilutive.

## Stock-Based Compensation

The Company recognizes the cost of stock-based awards granted to employees based on the estimated grant-date fair value of the awards. The value of the portion of the award that is ultimately expected to vest is recognized as expense ratably over the requisite service period. The Company recognizes the compensation costs for awards that vest over several years on a straight-line basis over the vesting period (see Note 12). Forfeitures are recognized when they occur, which may result in the reversal of compensation costs in subsequent periods as the forfeitures arise. The Company recognizes the cost of stock-based awards granted to nonemployees at their then-current fair values as services are performed, and such awards are remeasured through the counterparty performance date.

## Manufacturing Commitments and Contingencies

The Company is subject to various manufacturing royalties and payments related to its product candidate, Molgradex. Under an agreement, as amended, with the Active Pharmaceutical Ingredients ("API") manufacturer, no signing fee or milestones are included in the royalty payments, and there is no minimum royalty. Upon the successful development,

registration and attainment of approval by the proper health authorities, such as the FDA, in any territory except Latin America, Central America and Mexico, the Company must pay a royalty of three percent (3%) on annual net sales to the manufacturer of its API. Additionally, Savara must make certain payments to the API manufacturer upon the achievement of the milestones outlined in the following table.

The Company is also subject to certain contingent milestone payments, disclosed in the following table, payable to the Company's manufacturer of its nebulizer used to administer Molgradex. In addition to these milestones, the Company will owe a royalty to the manufacturer of its nebulizer based on net sales. The royalty rate ranges from three and a half percent (3.5%) to five percent (5%) depending on the device technology used by the Company to administer the product.

## Manufacturing Contingent Milestone Payments (in thousands):

	March 31, 2018
Molgradex API manufacturer:	
Delivery of working and master cell banks	\$ 600
Achievement of certain milestones related to	
regulatory approval of Molgradex	2,000
Molgradex nebulizer manufacturer:	
Achievement of various development activities and	
regulatory approval of nebulizer utilized to administer	
Molgradex	8,628
Total manufacturing commitments	\$ 11,228

As of March 31, 2018 and December 31, 2017, none of the above milestones had been met.

## Income Taxes

The Company uses the asset and liability method of accounting for income taxes. Under this method, deferred tax assets and liabilities are recognized for the expected future tax consequences of temporary differences between the carrying amounts and the tax basis of assets and liabilities. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect of a change in tax rates on deferred tax assets and liabilities will be recognized in the period that includes the enactment date. A valuation allowance is established against the deferred tax assets to reduce their carrying value to an amount that is more likely than not to be realized.

## Recent Accounting Pronouncements

In May 2014, the FASB issued Accounting Standards Update (“ASU”) 2014-09, “Revenue from Contracts with Customers” and has subsequently issued several supplemental and/or clarifying ASUs, which comprise the new comprehensive revenue recognition standard that will replace all current GAAP guidance on this topic and eliminate all industry-specific guidance. The standard’s core principle is that a reporting entity will recognize revenue when it transfers control of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. We have performed an assessment of our contracts with third parties and determined that they would not have a material impact on our financial statements.

In February 2016, the FASB issued Accounting Standards Update 2016-02, “Leases” (“ASU 2016-02”). The update aims at making leasing activities more transparent and comparable and requires substantially all leases be recognized by lessees on their balance sheet as a right-of-use asset and a corresponding lease liability, including leases currently accounted for as operating leases. The update also requires improved disclosures to help users of financial statements better understand the amount, timing and uncertainty of cash flows arising from leases. ASU 2016-02 is effective for fiscal years beginning after December 15, 2018 with early adoption permitted. The Company is currently evaluating the impact of the adoption of ASU 2016-02 on its financial statements.

In August 2016, the FASB issued Accounting Standards Update 2016-15, “Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments” (“ASU 2016-15”), which intended to add or clarify guidance on the classification of certain cash receipts and payments on the statement of cash flows. The new guidance addresses cash flows related to the following: debt prepayment or extinguishment costs, settlement of zero coupon bonds, contingent consideration payments made after a business combination, proceeds from the settlement of insurance claims, proceeds from the settlement of corporate-owned life insurance policies and bank-owned life insurance policies, distributions received from equity method investees, beneficial interest in securitization transactions, and the application of predominance principle to separately identifiable cash flows. ASU 2016-15 is effective for the Company for annual periods beginning after December 15, 2017 with early adoption permitted. The Company’s adoption of this standard did not have a material impact on the Company’s financial statements.

In January 2017, the FASB issued Accounting Standards Update 2017-01, “Business Combinations (Topic 805): Clarifying the Definition of a Business” (“ASU 2017-01”), which intended to clarify the definition of a business with the objective of adding guidance to assist entities with evaluating whether transactions should be accounted for as acquisitions (or disposals) of assets or businesses. ASU 2017-01 is effective for the Company for annual periods beginning after December 15, 2017 with early adoption permitted. The Company’s adoption of this standard did not have a material impact on the Company’s financial statements.

## 3. Prepaid expenses and other current assets

Prepaid expenses, consisted of (in thousands):

	March 31,	December 31,
	2018	2017
R&D tax credit receivable	\$857	\$ 834
Prepaid clinical trial costs	1,245	2,129
VAT receivable	171	196
Prepaid insurance	123	158
Forward currency exchange derivative	—	40
Deposits and other	190	194
Total prepaid expenses and other current assets	\$2,586	\$ 3,551

## 4. Accrued expenses and other liabilities

Accrued expenses and other liabilities, consisted of (in thousands):

	March 31,	December 31,
	2018	2017
Accrued contracted research and development costs	\$1,477	\$ 1,308
Accrued general and administrative costs	187	323
Accrued compensation	227	1,328
Forward currency contract obligation	56	—
Other	—	7
Total accrued expenses and other liabilities	\$1,947	\$ 2,966

## 5. Short-term Investments

Short-term Investments in Available for Sale Securities

The Company's investment policy seeks to preserve capital and maintain sufficient liquidity to meet operational and other needs of the business. The following table summarizes, by major security type, the Company's investments as of March 31, 2018 (in thousands):

Edgar Filing: Savara Inc - Form 10-Q

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
<b>Short-term investments</b>				
U.S. government securities	\$ 11,914	\$ —	\$ (19 )	\$11,895
Asset backed securities	7,118	—	(16 )	7,102
Corporate securities	17,560	1	(35 )	17,526
Commercial paper	26,339	—	—	26,339
Total short-term investments	\$ 62,931	\$ 1	\$ (70 )	\$62,862

The Company has classified its investments as available-for-sale securities. These securities are carried at estimated fair value with the aggregate unrealized gains and losses related to these investments reflected as a part of “Accumulated other comprehensive income (loss)” in the Consolidated Balance Sheets. Classification as short-term or long-term is based upon whether the maturity of the debt securities is less than or greater than twelve months.

There were no significant realized gains or losses related to investments for the three months ended March 31, 2018 and March 31, 2017.

## 6. Acquisitions

### Mast

On April 27, 2017, Savara completed its business combination with Mast Therapeutics, Inc. ("Mast"), a publicly held company, in accordance with the terms of the Agreement and Plan of Merger and dated January 6, 2017 (the "Merger"). The Merger was accounted for as a reverse merger under the acquisition method of accounting whereby Savara was considered to have acquired Mast for financial reporting purposes because, immediately upon completion of the Merger, Savara stockholders held a majority of the voting interest of the combined company.

## Edgar Filing: Savara Inc - Form 10-Q

Pursuant to business combination accounting, the Company applied the acquisition method, which requires the assets acquired and liabilities assumed be recorded at fair value with limited exceptions. The Company used the Multi-Period Excess Earnings Model (MPEEM), a form of the income approach to value the in-process research and development intangible asset. Under the valuation method, the present value of future cash flows expected to be generated from the in-process research and development of the acquired product candidate, Aironite, was determined using a reasonable discount rate, and identified projected cash flows from Aironite were risk adjusted to take into consideration the probabilities of moving through the various clinical stages. The excess of the purchase price over the assets acquired and liabilities assumed represents goodwill. The goodwill is primarily attributable to the synergies expected to arise after the acquisition and is not expected to be deductible for tax purposes. All transaction costs associated with the Merger were incurred during the year ended December 31, 2017. The total purchase price for Mast was \$35.8 million based on the fair value of the outstanding Mast equity on the date of the Merger which was allocated as follows:

	(in thousands)
Purchase Consideration	
Fair value of Mast shares outstanding	\$ 33,117
Fair value of Mast equity	2,729
Fair value of total consideration	\$ 35,846
Assets acquired and liabilities assumed	
Cash and cash equivalents	\$ 3,442
Tangible assets	283
In-process research and development intangible assets	21,692
Liabilities	(2,396 )
Debt	(3,407 )
Deferred tax liability	(7,375 )
Total assets acquired and liabilities assumed	12,239
Goodwill	23,607
Total	\$ 35,846

The final allocation of the purchase price is dependent on the finalization of the valuation of the fair value of assets acquired and liabilities assumed and may differ from the amounts included in these financial statements.

As discussed in Note 2, during the three months ended March 31, 2018, the Company recorded a \$21.7 million impairment charge and corresponding decrease to the carrying value of IPR&D recorded with respect to the Merger to write the IPR&D asset off in full due to the failure of Aironite to meet its primary and secondary endpoints in the Phase 2 study. Following the negative outcome of the study, Savara does not plan to support any new development of Aironite. The decrease in the carrying value of IPR&D has been recognized as an expense to Impairment to acquired IPR&D included in the condensed consolidated statement of operations for the three months ended March 31 2018. As a result of the impairment charge recorded in the first quarter of 2018, the Company reduced the related deferred tax liability by \$4.6 million and recorded a tax benefit.

### 7. Debt Facility

On April 28, 2017, the Company entered into a loan and security agreement with Silicon Valley Bank (the “Loan Agreement”). During the year ended December 31, 2017, upon satisfaction of the conditions of the Loan Agreement, the Company executed two tranches totaling \$15.0 million, the maximum credit available pursuant to the debt facility. The capital was utilized for the repayment of \$3.7 million of principal debt and fees of Mast assumed in the Merger. The residual capital is being utilized to fund ongoing development programs of the Company and for general corporate purposes.

The Loan Agreement contains customary affirmative and negative covenants, including among others, covenants limiting our ability and that of our subsidiaries to dispose of assets, permit a change in control, merge or consolidate, make acquisitions, incur indebtedness, grant liens, make investments, make certain restricted payments and enter into transactions with affiliates, in each case subject to certain exceptions.

The Loan Agreement bears interest at the prime rate reported in The Wall Street Journal, plus a spread of 4.25%. Interest only payments are due through September 2018 followed by monthly payments of principal plus interest over the following 30 months. Since the second tranche was fully extended, the interest only period was extended for an additional 6 months, through March 2019 followed by monthly payments of principal plus interest over the following 24 months through the maturity date of March 1, 2021 under the Loan Agreement provisions. We were obligated to pay customary closing fees and are obligated to pay a final payment of 6.0% of the aggregate principal amount of term loans advanced under the facility. The end of term charge of \$0.9 million will be due on the scheduled maturity date and is being recognized as an increase to the principal with a corresponding charge to interest expense over the term of the facility using the effective interest method.



In connection with the Loan Agreement, we paid \$0.1 million in legal costs directly attributable to issuing the debt instrument. Such charges were accounted for as debt issuance costs and are being amortized to interest expense using the effective interest method through the scheduled maturity date.

Upon funding the first tranche of the Loan Agreement, the Company was obligated to issue warrants to purchase shares of the Company's common stock equal to 3.0% of the funded amount divided by the exercise price to be set based on the average price per share over the preceding 10 trading days prior to closing. The number of shares callable under the warrant agreement for the first tranche and exercise price were 24,725 shares of the Company's common stock at an exercise price of \$9.10 per share, with a ten year life, expiring April 28, 2027 ("April 2017 Warrants").

Upon funding the second tranche of the Loan Agreement, the Company was obligated to issue warrants to purchase shares of the Company's common stock equal to 3.0% of the funded amount divided by an exercise price to be set based on the average price per share over the preceding 10 trading days prior to funding or the price per share prior to the day of funding. As such, the Company issued additional warrants for 41,736 shares at an exercise price of \$5.39 with a ten year life, expiring June 15, 2027 ("June 2017 Warrants").

The April 2017 Warrants and June 2017 Warrants issued were valued using the Black-Scholes option pricing model with the following assumptions: volatility of 71.42% and 71.57%, respectively, expected term of ten years, risk-free interest rate of 2.33% and 2.16%, respectively, and a zero dividend yield. The collective warrant fair value of \$0.4 million has been recorded as a debt discount and is being amortized through interest expense using the effective interest method through the scheduled maturity date.

#### Summary of Carrying Value

The following table summarizes the components of the debt facility carrying value, which approximates the fair value (in thousands):

	As of March 31, 2018
	<del>Short-term</del>
Principal payments to lender and end of term charge	\$—\$ 15,216
Debt Issuance costs	— (73 )
Debt discount related to warrants	— (259 )
Carrying Value	\$—\$ 14,884

#### 8. Fair Value Measurements

The Company measures and reports certain financial instruments at fair value on a recurring basis and evaluates its financial instruments subject to fair value measurements on a recurring basis to determine the appropriate level in which to classify them in each reporting period.

The Company determined that certain investments in debt securities classified as available-for-sale securities were Level 1 financial instruments.

Additional investments in corporate debt securities and commercial paper are considered Level 2 financial instruments because the Company has access to quoted prices but does not have visibility to the volume and frequency of trading for all of these investments. For the Company's investments, a market approach is used for recurring fair value measurements and the valuation techniques use inputs that are observable, or can be corroborated by observable data, in an active marketplace.

Foreign exchange derivatives not designated as hedging instruments are considered Level 2 fair value measurements. The Company's foreign exchange derivative instruments are typically short-term in nature.

The Company also determined that the contingent consideration, described further below, was a Level 3 financial instrument.

Edgar Filing: Savara Inc - Form 10-Q

The fair value of these instruments as of March 31, 2018 and December 31, 2017 was as follows (in thousands):

	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
<b>As of March 31, 2018:</b>			
Cash equivalents:			
U.S. Treasury money market funds	\$10,777	\$ —	\$ —
Commercial paper	\$—	\$ 6,896	\$ —
Short-term investments:			
U.S. government securities	\$11,895	\$ —	\$ —
Asset backed securities		\$ 7,102	
Corporate securities	\$—	\$ 17,526	\$ —
Commercial paper	\$—	\$ 26,339	\$ —
Liabilities:			
Contingent consideration	\$—	\$ —	\$ 11,814
Foreign exchange derivatives not designated as hedging instruments	\$—	\$ 56	\$ —
<b>As of December 31, 2017:</b>			
Cash equivalents:			
U.S. Treasury money market funds	\$4,540	\$ —	\$ —
Commercial paper	\$—	\$ 1,029	\$ —
Repurchase agreements	\$—	\$ 2,500	\$ —
Short-term investments:			
U.S. government securities	\$11,885	\$ —	\$ —
Asset backed securities		\$ 8,383	
Corporate securities	\$—	\$ 22,082	\$ —
Commercial paper	\$—	\$ 29,842	\$ —
Other assets:			
Foreign exchange derivatives not designated as hedging instruments	\$—	\$ 40	\$ —
Liabilities:			
Contingent consideration	\$—	\$ —	\$ 11,948

Pursuant to the acquisition of certain assets, liabilities, and subsidiaries of Serendex A/S through its wholly-owned Danish subsidiary, Savara ApS on July 15, 2016, Savara agreed to pay the seller, in addition to a stipulated amount of

shares of Savara's common stock, (i) \$5.0 million upon receipt of marketing approval of Molgradex by the European Medicines Agency, (ii) \$15.0 million upon receipt of marketing approval of Molgradex by the FDA, and (iii) \$1.5 million upon receipt of marketing approval of Molgradex by the Japanese Pharmaceuticals and Medical Devices Agency (the "Contingent Milestone Payments"). The Company estimates the likelihood of approval in each region, separately, based on the product candidate's current phase of development and utilizing published studies of clinical development success rates for comparable non-oncology orphan drugs. The present value of the potential cash outflows from the probability weighted Contingent Milestone Payments is then estimated by taking into consideration that the Contingent Milestone Payments are similar to a business expense of the Company and would be senior to any Company debt obligations. The resulting weighted average present value factor is then applied to discount the probability adjusted Contingent Milestone Payments for each region to derive the fair value of the Contingent Milestone Payments.

Edgar Filing: Savara Inc - Form 10-Q

The following table sets forth a summary of the changes in the fair value of the Company's Level 3 financial instruments (in thousands) for the three months ended March 31, 2018 and year ended December 31, 2017:

	Warrant Liability	Put options convertible promissory notes	Contingent Consideration
As of December 31, 2016	\$ 303	\$ 979	\$ 9,708
Change in fair value	67	169	2,240
Put option at issuance of convertible promissory notes	—	828	—
Conversion of convertible promissory notes		(1,976 )	
Reclassification of warrant liability to common equity upon Merger	(370 )	—	—
Balance at December 31, 2017	\$ —	\$ —	\$ 11,948
Change in fair value	—	—	(134 )
Balance at March 31, 2018	\$ —	\$ —	\$ 11,814

The Company records changes in fair value of the contingent consideration in general and administrative expense.

In June 2017, the Company determined that there would be a change to the Molgradex program due to the FDA's guidance on the clinical program requirements for a New Drug Application submission in the U.S. related to the Molgradex product, which was issued in May 2017. Based on the FDA's guidance, the Company modified certain criteria of its Molgradex development program which the Company believes will accelerate the development timeline in the U.S. The Company accordingly accounted for this change in its valuation of the contingent consideration as of June 30, 2017. Additionally, in the first quarter of 2018, Savara received approval from the FDA of its expansion of the Molgradex Phase 3 Study for PAP into the U.S. to support its expedited U.S. development strategy for Molgradex. However, in order to achieve sufficient support for the study endpoints and outcome, the sample size of the study will be increased resulting in the extension of the patient enrollment completion dates, and hence, the approval dates of Molgradex, by approximately two calendar quarters. The Company likewise accounted for this change in its valuation of the contingent consideration as of March 31, 2018.

The Company also accounted for the time value of money related to the Contingent Milestone Payments from December 31, 2017 to March 31, 2018 in its assessment. Accordingly, the related contingent consideration liability was remeasured to \$11.8 million as of March 31, 2018 reflecting a change in fair value of \$0.1 million for the three months ended March 31, 2018.

The Company did not transfer any assets measured at fair value on a recurring basis to or from Level 1, Level 2 and Level 3 during the three months ended March 31, 2018 and year ended December 31, 2017.

## 9. Derivative Financial Instruments

In the normal course of business, the Company is exposed to the impact of foreign currency fluctuations. The Company seeks to limit these risks by following risk management policies and procedures, including the use of derivatives. The Company's derivative contracts, which are not designated as hedging instruments, principally address short-term foreign currency exchange. The estimated fair value of the derivative contracts was based upon the relative exchange rate as of the balance sheet date. Accordingly, any gains or losses resulting from variances between this exchange rate and the exchange rate at the contract inception date were recognized as other income or expense in the Condensed Consolidated Statements of Operations and Comprehensive Loss. As of March 31, 2018, there were approximately \$3.0 million of unsettled forward exchange contracts to purchase foreign currency and the net derivative financial instruments were recorded at their estimated fair value of fifty-six thousand dollars in accrued expenses.

## 10. Shareholders' Equity

### Common Stock Sales Agreement/At The Market (ATM)

On April 28, 2017, the Company entered into a Common Stock Sales Agreement (the "Sales Agreement") with H.C. Wainwright & Co., LLC, as sales agent ("Wainwright"), pursuant to which the Company may offer and sell, from time to time, through Wainwright, shares of Savara's common stock, par value \$0.001 per share (the "Shares"), having an aggregate offering price of not more than \$18.0 million. The Shares will be offered and sold pursuant to the Company's shelf registration statement on Form S-3. Subject to the terms and conditions of the Sales Agreement, Wainwright will use its commercially reasonable efforts to sell the Shares from time to time, based upon the Company's instructions. The Company has provided Wainwright with customary indemnification rights, and Wainwright will be entitled to a commission at a fixed commission rate equal to 3.0% of the gross proceeds per Share sold. Sales of the Shares, if any, under the Sales Agreement may be made in transactions that are deemed to be "at the market offerings" as defined

## Edgar Filing: Savara Inc - Form 10-Q

in Rule 415 under the Securities Act of 1933, as amended. The Company has no obligation to sell any of the Shares, and may at any time suspend sales under the Sales Agreement or terminate the Sales Agreement.

During the three months ended March 31, 2018, the Company sold 46,900 shares of common stock under the Sales Agreement, for net proceeds of approximately \$0.5 million.

### Common Stock

The Company's amended and restated certificate of incorporation, effective upon the completion of the Merger, authorizes the Company to issue 501 million shares of common and preferred stock, consisting of 500 million shares of common stock with \$0.001 par value and one million shares of preferred stock with \$0.001 par value. The following is a summary of the Company's common stock at March 31, 2018 and December 31, 2017.

	March 31,	December 31,
	2018	2017
Common stock authorized	500,000,000	500,000,000
Common stock outstanding	30,604,366	30,509,522

The Company's shares of common stock reserved for issuance as of March 31, 2018 and December 31, 2017 were as follows:

	March,	December 31,
	2018	2017
Warrants from Mast acquired in Merger	1,152,231	1,152,231
Warrants Converted Pursuant to Merger	72,953	74,992
April 2017 SVB Warrants	24,725	24,725
June 2017 SVB Warrants	41,736	41,736
Prefunded warrants	775,000	775,000
Stock options outstanding	1,786,438	1,916,832
Issued and unvested RSU's	195,625	86,875
Total shares reserved	4,048,708	4,072,391

### Warrants

The following table summarizes the outstanding warrants for the Company's common stock as of March 31, 2018:

Shares	Exercise	Expiration Date
Underlying	Price	

Edgar Filing: Savara Inc - Form 10-Q

Outstanding

Warrants

401,391	\$ 45.50	June 2018
314,446	\$ 52.50	November 2019
32,467	\$ 7.00	August 2020
403,927	\$ 29.40	February 2021
72,953	\$ 8.98	June 2021
24,725	\$ 9.10	April 2027
41,736	\$ 5.39	June 2027
775,000	\$ 0.01	October 2024
2,066,645		



## 11. Commitments

### Operating Leases

We are obligated under operating leases for office space. We lease an office space in Copenhagen, Denmark with a lease effective on June 1, 2014 and expiring on November 30, 2019. Our monthly rent is approximately five thousand dollars. On March 23, 2017, we sublet office space located in San Diego, California with rentable office space of approximately 13,707 square feet, which previously served as Mast's corporate headquarters, to a third party. However, as a result of the Merger, the Company no longer had an ongoing need for these facilities. The term of the sub-sublease commenced on July 1, 2017 and expires on May 31, 2020, coterminous with a sublease agreement dated June 19, 2014 with the sublessor. Monthly base rent under the sub-sublease is approximately forty-four thousand dollars, subject to increases of 3.0% annually on the anniversary of the commencement date of the sub-sublease term. However, monthly base rent for calendar month two of the sub-sublease term was abated.

On November 29, 2017, we entered into a sublease agreement for a new office space for our corporate headquarters and subleased our previous office space with a lease expiring on November 30, 2019 with monthly rent of approximately five thousand dollars. The term of the sublease for the new space commenced on January 1, 2018 and will continue until July 31, 2021 and we agreed to make monthly rental payments of thirteen thousand dollars, subject to increases of approximately 2% annually on the anniversary of the commencement date of the sublease term. However, monthly base rent for calendar month one of the sublease term was abated.

We previously leased office space for our corporate headquarters, prior to our relocation on January 1, 2018, discussed herein, in Austin, Texas pursuant to an operating lease dated November 19, 2012, as amended May 22, 2015 under which we are obligated to remit monthly rental payments of approximately five thousand dollars for the period January 1, 2018 through January 31, 2019 and five thousand six hundred dollars thereafter through November 30, 2019. On November 29, 2017, we entered into a sublease agreement pursuant to which the sublessee will assume the office space and these rental payments effective January 1, 2018 except for the first month's rent on January 2018.

### Risk Management

The Company maintains various forms of insurance that the Company's management believes are adequate to reduce the exposure to these risks to an acceptable level.

### Employment Agreements

Certain executive officers are entitled to payments if they are terminated without cause or as a result of a change in control. Upon termination without cause, and not as a result of death or disability, each of such officers is entitled to receive a payment of base salary for three to twelve months following termination of employment and such officer will be entitled to continue to receive coverage under medical and dental benefit plans for three to twelve months or until such officer is covered under a separate plan from another employer. Upon a termination other than for cause or for good reason within twelve months following a change in control, each of such officers will be entitled to the same benefits as upon termination without cause and will also be entitled to certain acceleration of such officer's outstanding unvested options at the time of such termination.

## 12. Stock-Based Compensation

### A. 2008 Stock Option Plan

The Company adopted the Savara Inc. Stock Option Plan (the “2008 Plan”), pursuant to which the Company had reserved shares for issuance to employees, directors, and consultants. The 2008 Plan includes 1) the option grant program providing for both incentive and non-qualified stock options, as defined by the Internal Revenue Code, and 2) the stock issuance program providing for the issuance of awards that are valued based upon common stock, including restricted stock, dividend equivalents, stock appreciation rights, phantom stock, and performance units. The 2008 Plan also allows eligible persons to purchase shares of common stock at an amount determined by the Plan Administrator. Upon a participant’s termination, the Company retains the right to repurchase unvested shares issued in conjunction with the stock issuance program at the fair market value per share as of the date of termination.

Prior to the closing of the Merger, the Company had issued incentive and non-qualified options and restricted stock to employees and non-employees under the 2008 Plan. The terms of the stock options, including the exercise price per share and vesting provisions, were determined by the board of directors. Stock options were granted at exercise prices not less than the estimated fair market value of the Company’s common stock at the date of grant based upon objective and subjective factors including: third-party valuations, preferred stock transactions with third parties, current operating and financial performance, management estimates and future expectations. Stock option grants typically vest quarterly over three to four years and expire ten years from the grant date, and restricted stock grants vest on a quarterly basis over four years and expire ten years from the grant date. As of March 31, 2018, 562,596 shares of restricted stock had been issued excluding forfeited shares of restricted stock.

## Restricted Stock

The Company values stock-based compensation related to grants of its restricted stock, which were issued prior to the Merger date, based on the fair value of the Company's common stock as of the grant date and recognizes the expense over the requisite service period, usually four years. Forfeitures are recognized when they occur, which may result in the reversal of compensation costs in subsequent periods as the forfeitures arise. To determine the value of its common stock, the Company utilized the Option Pricing Method. The valuation methodology includes estimates and assumptions that require the Company's judgment. Inputs used to determine the estimated fair value of the Company's common stock include the equity value of the Company, expected timing to a liquidity event, a risk-free interest rate and the expected volatility. Generally, increases or decreases in these unobservable inputs would result in a directionally similar impact on the fair value measurement of the Company's common stock.

During the three months ended March 31, 2018 and 2017, the Company did not issue any shares of restricted stock to employees under the 2008 Plan.

## Stock Options

The Company values stock options using the Black-Scholes option-pricing model, which requires the input of subjective assumptions, including the risk-free interest rate, expected life, expected stock price volatility and dividend yield. The risk-free interest rate assumption is based upon observed interest rates for constant maturity U.S. Treasury securities consistent with the expected term of the Company's employee stock options. The expected life represents the period of time the stock options are expected to be outstanding and is based on the simplified method. The Company uses the simplified method due to the lack of sufficient historical exercise data to provide a reasonable basis upon which to otherwise estimate the expected life of the stock options. Expected volatility is based on historical volatilities for publicly traded stock of comparable companies over the estimated expected life of the stock options. The Company assumes no dividend yield because dividends are not expected to be paid in the near future, which is consistent with the Company's history of not paying dividends. The valuation of stock options is also impacted by the valuation of common stock. Refer to the section above for further information on the valuation methodology utilized by the Company to determine the value of its common stock.

## Changes in 2008 Plan

Subsequent to the Merger, the Company no longer issues stock based awards under the 2008 Plan.

## B. 2015 Omnibus Incentive Option Plan

The Company operates the 2015 Omnibus Incentive Plan (the "2015 Plan"), which was amended and approved by stockholders in June 2015. The 2015 Plan provides for the grant of incentive and non-statutory stock options, as well as share appreciation rights, restricted shares, restricted share units ("RSUs"), performance units, shares and other share-based awards. Share-based awards are subject to terms and conditions established by our board of directors or the compensation committee of our board of directors. As of March 31, 2018, the number of shares of our common stock available for grant under the 2015 Plan was 15,868 shares.

## C. Stock-Based Award Activity

The following table provides a summary of stock-based awards for the 2008 Plan and 2015 Plan (the “Plans”) for the three months ended March 31, 2018 and 2017:

	Three months ended March 31, 2018			Three months ended March 31, 2017		
	Stock Options	RSUs	Total	Stock Options	RSUs	Total
Outstanding as of December 31	1,916,832	86,875	2,003,707	2,129,844	—	2,129,844
Granted	10,000	120,000	130,000	—	72,588	72,588
Exercised	(37,894 )	(11,250 )	(49,144 )	—	—	-
Forfeited	(102,500 )	-	(102,500 )	(128,906 )	(227 )	(129,133 )
Outstanding as of March 31	1,786,438	195,625	1,982,063	2,000,938	72,361	2,073,299

## D. Stock-Based Compensation

Stock-based compensation expense is included in the following line items in the accompanying statements of operations and comprehensive loss for the three months ended March 31, 2018 and 2017 (in thousands):

	Three Months Ended	
	March 31, 2018	March 31, 2017
Research and development	\$228	\$ 36
General and administrative	184	45
Total stock-based compensation	\$412	\$ 81

## 13. Net Loss per Share

Basic net loss per share is computed by dividing the net loss by the weighted-average number of common shares outstanding. Diluted net loss per share is computed similarly to basic net loss per share except that the denominator is increased to include the number of additional common shares that would have been outstanding if the potential common shares had been issued and if the additional common shares were dilutive. Diluted net loss per share is the same as basic net loss per common share, since the effects of potentially dilutive securities are antidilutive.

As of March 31, 2018 and 2017, potentially dilutive securities include:

	Three Months Ended	
	March 31, 2018	March 31, 2017
Awards under equity incentive plan	1,786,438	1,715,026
Unvested restricted shares	207,768	73,095
Securities converted into common stock upon Merger	—	7,832,102
Warrants to purchase common stock	2,066,645	74,512
Total	4,060,851	9,694,735

The following table reconciles basic earnings per share of common stock to diluted earnings per share of common stock for the three months ended March 31, 2018 and 2017.

Edgar Filing: Savara Inc - Form 10-Q

	Three Months Ended	
	March 31	March 31,
	2018	2017
Net loss	\$(26,849 )	\$(4,974 )
Accretion of convertible redeemable preferred stock	—	(24 )
Net loss attributable to common stockholders	(26,849 )	(4,998 )
Undistributed earnings and net loss attributable to		
common stockholders, basic and diluted	(26,849 )	(4,998 )
Weighted average common shares outstanding, basic		
and diluted	30,543,746	3,029,223
Basic and diluted EPS	\$(0.88 )	\$(1.65 )

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

This Quarterly Report on Form 10-Q contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Any statements contained herein that involve risks and uncertainties, such as Savara's plans, objectives, expectations, intentions and beliefs should be considered forward-looking statements. Savara's actual results could differ materially from those discussed in these forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified below and those discussed in the section entitled "Risk Factors" in this Quarterly Report on pages 26 through 47.

Overview

We are an orphan lung disease company. Our current pipeline comprises Molgradex, an inhaled granulocyte-macrophage colony-stimulating factor, or GM-CSF, in Phase 3 development for pulmonary alveolar proteinosis ("PAP"), and in Phase 2a development for nontuberculous mycobacterial ("NTM") lung infection, and AeroVanc, a Phase 3 stage inhaled vancomycin for treatment of persistent methicillin-resistant *Staphylococcus aureus* ("MRSA") lung infection in individuals living with cystic fibrosis ("CF"). Our strategy involves expanding our pipeline of potentially best-in-class products through indication expansion, strategic development partnerships and product acquisitions, with the goal of becoming a leading company in our field. Our management team has significant experience in orphan drug development and pulmonary medicine, identifying unmet needs, developing and acquiring new product candidates, and effectively advancing them to approvals and commercialization.

Together with our wholly owned subsidiaries, we operate in one segment and have our principal offices in Austin, Texas. Since inception, we have devoted substantially all of our efforts and resources to identifying and developing our product candidates, recruiting personnel, and raising capital. We have incurred operating losses and negative cash flow from operations and have no material product revenue from inception to date as we have not yet commenced commercial operations. From our inception, as a private company prior to the Merger, to March 31, 2018, we have raised net cash proceeds of approximately \$154.1 million, from public offerings of common stock and private placements of convertible preferred stock, note financings and debt financings.

We have never been profitable and have incurred operating losses in each year since inception. Our net losses were \$26.8 million for the three months ended March 31, 2018, which included an impairment charge of \$21.7 million on certain acquired IPR&D, and \$29.8 million for the year ended December 31, 2017. As of March 31, 2018, we had an accumulated deficit of \$95.1 million. Substantially all of our operating losses resulted from expenses incurred in connection with our research and development programs and from general and administrative costs associated with our operations.

We have chosen to operate by outsourcing our manufacturing and most of our clinical operations. We expect to incur significant additional expenses and increasing operating losses for at least the next several years as we initiate and continue the clinical development of, and seek regulatory approval for, our product candidates and add personnel necessary to operate as a public company with an advanced clinical candidate pipeline of products. In addition, operating as a publicly traded company, following the Merger, will involve the hiring of additional financial and other personnel, upgrading financial information systems and incurring costs associated with public company operations. We expect that our operating losses will fluctuate significantly from quarter to quarter and year to year due to timing of clinical development programs and efforts to achieve regulatory approval.

As of March 31, 2018, we had cash of \$22.1 million and short-term investments of \$62.9 million. We will continue to require substantial additional capital to continue our clinical development and potential commercialization activities. Accordingly, we will need to raise substantial additional capital to continue to fund our operations. The amount and timing of our future funding requirements will depend on many factors, including the pace and results of our clinical development efforts. Failure to raise capital as and when needed, on favorable terms or at all, would have a negative impact on our financial condition and our ability to develop our product candidates.

#### Recent Events

##### Merger with Mast

On April 27, 2017, Savara completed its business combination with Mast Therapeutics, Inc. ("Mast"), a publicly held company, in accordance with the terms of the Agreement and Plan of Merger and Reorganization (the "Merger Agreement"), dated January 6, 2017 (the "Merger"). In connection with and immediately prior to the effective time of the Merger, Mast implemented a reverse stock split at a ratio of one new share for every 70 shares of its common stock outstanding. Under the terms of the Merger Agreement, each outstanding share of Savara common stock was then converted into Mast common stock at a ratio of approximately .5860 of a Savara share (the "Exchange Ratio"). As a result of the Merger, the Mast equity holders owned approximately 23% of the combined company, and Savara's pre-existing equity holders owned approximately 77%.



## Expansion of Molgradex into NTM

On October 24, 2017, we announced our plans to expand the development of our lead product candidate Molgradex to include the treatment of NTM lung infection. We initiated a Phase 2a open-label clinical trial in subjects with antibiotic-resistant NTM lung infection in early 2018. The clinical trial will investigate the efficacy of Molgradex on NTM sputum culture conversion to negative, reduction of NTM bacterial load in sputum, exercise capacity as well as its effect on patient reported outcomes, and safety.

## Public Offerings

On June 7, 2017, we completed an underwritten public offering consisting of 9,034,210 shares of our common stock which included 613,157 shares upon the partial exercise of the underwriters' option to purchase additional shares of Savara common stock at the public offering price, less the underwriting discounts and commissions. The underwriters' option to purchase the remaining balance of additional shares expired as of June 30, 2017. The net proceeds from the offering, after deducting the underwriting discounts and commissions and offering expenses, were approximately \$39.5 million.

On October 27, 2017, we completed an underwritten public offering consisting of 5,250,000 shares of our common stock, in addition to pre-funded warrants to purchase 775,000 shares of our common stock. Under the offering, the underwriter was granted an option to purchase 787,500 additional shares of our common stock (the "Optional Shares") at the public offering price, less the underwriting discounts and commissions. On November 2, 2017, the underwriters exercised their option to purchase the Optional Shares, and such transaction closed on November 6, 2017, resulting in net proceeds of approximately \$5.8 million. The net proceeds from the offering, including the Optional Shares, after deducting the underwriting discounts and commissions and offering expenses, were approximately \$50.0 million.

We are using the net proceeds, totaling \$89.6 million from both the June 2017 offering and October 2017 offering, for working capital and general corporate purposes, which include, but are not limited to, the funding of clinical development of and pursuing regulatory approval for our product candidates, including the indication expansion for Molgradex for the treatment of NTM lung infection, and general and administrative expenses. The June 2017 and October 2017 offerings were executed under an existing shelf registration statement as previously file with the U.S. Securities exchange Commission ("SEC") on August 12, 2015 and declared effective on August 19, 2015.

## IND Approval - Molgradex

In February 2018, the U.S. Food and Drug Administration ("FDA") approved our Investigational New Drug ("IND") application for Molgradex for the treatment of PAP, allowing for the expansion of our Phase 3 study into the U.S, which will support our expedited U.S. development strategy for Molgradex.

## Impairment of IPR&D Acquired in Merger

During the three months ended March 31, 2018, we recorded \$21.7 million of impairment charges and a corresponding decrease to the carrying value of IPR&D related to the Aironite drug candidate assumed in the Merger due to the unfavorable results received in February 2018 from a Phase 2 study that demonstrated a failure of Aironite to meet the endpoints of the study and limited effectiveness of the compound in patients. Due to these results, we are no longer supporting the Aironite program. As a result of this IPR&D impairment, we fully wrote off the corresponding deferred tax liability related to the acquired IPR&D from the Merger in the amount of \$4.6 million and recorded a tax benefit.

## Financial Operations Overview

### Research and Development Expenses

## Edgar Filing: Savara Inc - Form 10-Q

Research and development expenses represent costs incurred to conduct research and development, such as the development of our product candidates. We recognize all research and development costs as they are incurred. Research and development expenses consist primarily of the following:

- expenses incurred under agreements with consultants and clinical trial sites that conduct research and development activities on our behalf;
- laboratory and vendor expenses related to the execution of clinical trials;
- contract manufacturing expenses, primarily for the production of clinical supplies; and
- internal costs that are associated with activities performed by our research and development organization and generally benefit multiple programs.

20

---

Where appropriate, these costs are allocated by product candidate. Any unallocated internal research and development costs consist primarily of:

- personnel costs, which include salaries, benefits and stock-based compensation expense;
- allocated facilities and other expenses, which include expenses for rent and maintenance of facilities and depreciation expense; and
- regulatory expenses and technology license fees related to development activities.

The largest component of our operating expenses has historically been our investment in research and development activities. The following table shows our research and development expenses for the three months ended March 31, 2018 and 2017:

	Three Months Ended	
	March 31, 2018	2017
	(in thousands)	
<b>Product candidates:</b>		
AeroVanc	\$3,039	\$942
Molgradex	4,781	2,006
Other	719	—
<b>Total research and development expenses</b>	<b>\$8,539</b>	<b>\$2,948</b>

We expect research and development expenses will increase in the future as we advance our product candidates into and through clinical trials and pursue regulatory approvals, which will require a significant increased investment in regulatory support and contract manufacturing and inventory build-up related costs. In addition, we continue to evaluate opportunities to acquire or in-license other product candidates and technologies, which may result in higher research and development expenses due to license fee and/or milestone payments.

The process of conducting clinical trials necessary to obtain regulatory approval is costly and time consuming. We may never succeed in timely developing and achieving regulatory approval for our product candidates. The probability of success of our product candidates may be affected by numerous factors, including clinical data, competition, intellectual property rights, manufacturing capability and commercial viability. As a result, we are unable to accurately determine the duration and completion costs of our development projects or when and to what extent we will generate revenue from the commercialization and sale of any of our product candidates.

#### General and Administrative Expenses

General and administrative expenses consist of personnel costs, facility expenses, expenses for outside professional services, including legal, audit and accounting services, and changes in the fair value of certain contingent consideration. Personnel costs consist of salaries, benefits and stock-based compensation. Facility expenses consist of rent, other related costs and other supplies. We have incurred additional expenses as a result of becoming a public company following the Merger, including expenses related to compliance with the rules and regulations of the SEC and Nasdaq, additional insurance, investor relations, and other administrative expenses and professional services.

#### Critical Accounting Policies and Estimates

Our management's discussion and analysis of financial condition and results of operations is based on our condensed consolidated financial statements, which have been prepared in accordance with GAAP. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities and expenses. On an ongoing basis, we evaluate these estimates and judgments. We base our estimates on historical experience and on various assumptions that we believe to be reasonable under the circumstances. These estimates and assumptions form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. Actual results may differ materially from these estimates. We believe that the accounting policies discussed below are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving management's judgments and estimates.

#### Accrued Research and Development Expenses

We record accrued expenses for estimated costs of our research and development activities conducted by external service providers, which include the conduct of clinical trial and contract formulation and manufacturing activities. We record the estimated costs of development activities based upon the estimated amount of services provided but not yet invoiced and include these costs in accrued liabilities in the consolidated balance sheet and within development expense in the consolidated statement of operations and comprehensive loss. These costs are a significant component of our research and development expenses. We record accrued expenses for these costs based on the estimated amount of work completed and in accordance with agreements established with these external service providers.

We estimate the amount of work completed through discussions with internal personnel and external service providers as to the progress or stage of completion of the services and the agreed-upon fee to be paid for such services. We make significant judgments and estimates in determining the accrued balance in each reporting period. As actual costs become known, we adjust our accrued estimates.

### Stock-based Compensation

We recognize the cost of stock-based awards granted to employees based on the estimated grant-date fair value of the awards. The value of the portion of the award ultimately expected to vest is recognized as expensed ratably over the requisite service period. We recognize the compensation costs for awards that vest over several years on a straight-line basis over the vesting period. Forfeitures are recognized when they occur and may result in the reversal of compensation costs in subsequent periods as the forfeitures arise. We recognize the cost of stock-based awards granted to nonemployees at their then-current fair values as services are performed, and such awards are remeasured through the counterparty performance date.

We estimate the grant date fair value of a stock option award using the Black-Scholes option-pricing model. In determining the grant date fair value of a stock option award under the Black-Scholes model, we must make a number of assumptions, including the term of the award, the volatility of the price of our common stock over the term of the award, and the risk-free interest rate. Changes in these or other assumptions could have a material impact on the compensation expense we recognize.

### Results of Operations — Comparison of Three Months Ended March 31, 2018 and 2017

	Three Months Ended		
	March 31, 2018	2017	Dollar Change (in thousands)
Operating expenses:			
Research and development	\$8,539	\$2,948	\$5,591
General and administrative	1,769	1,736	33
Impairment of IPR&D	21,692	—	21,692
Depreciation	107	90	17
Total operating expenses	32,107	4,774	27,333
Loss from operations	(32,107)	(4,774)	(27,333)
Other income (expense)	(221 )	(437 )	216
Net loss before income taxes	(32,328)	(5,211)	(27,117)
Income tax benefit	5,479	237	5,242
Net loss	\$(26,849)	\$(4,974)	\$(21,875)

### Research and development

Research and development expenses increased by \$5.6 million, or 189.7%, to \$8.5 million for the three months ended March 31, 2018 from \$2.9 million for the three months ended March 31, 2017. The increase was primarily due to \$2.7 million in increased development costs associated with the development of Molgradex including the expansion of the PAP study in the U.S. and the commencement of the NTM study, an increase of \$2.2 million in AeroVanc study costs related to Phase 3 activities, and \$0.7 million related to milestone and development costs of the Aironite study

acquired in the Merger, which was not a part of our product pipeline in the first quarter of 2017.

#### General and administrative

General and administrative expenses in total for the three months ended March 31, 2018 and 2017 were consistent.

#### Impairment of IPR&D

During the three months ended March 31, 2018, we recognized a \$21.7 million impairment charge to the carrying value of IPR&D related to the Aironite drug candidate assumed in the Merger due to the unfavorable results from a Phase 2 study that demonstrated a failure of Aironite to meet the endpoints of the study and limited effectiveness of the compound in patients. We do not intend to further support or pursue the Aironite drug candidate.

## Other expense

Other expense decreased by \$0.2 million for the three months ended March 31, 2018. The decrease was primarily due to accrued interest on certain convertible notes and the change in fair value of financial instruments held by us in the first quarter of 2017 which were subsequently extinguished or converted to common stock as part of the Merger.

## Income tax benefit

We reported a \$4.6 million tax benefit for the first quarter of 2018 related to the reversal of a deferred tax liability resulting from the impairment of IPR&D acquired in the Merger.

The income tax benefit provided by the Danish government in the form of a refundable research credit associated with research and development expenditures of Molgradex for the treatment of PAP through our subsidiary, Savara ApS, for the three months ended 2018 in the amount of \$0.7 million exceeded the benefit recognized for the three months ended March 31, 2017 of \$0.2 million due to an increase in qualified research and development costs incurred in the first quarter of 2018.

We also recognized a tax benefit for the three months ended March 31, 2018 in the amount of \$0.1 million as provided by the Australian Taxation Office for qualified research and development expenditures incurred on the NTM program incurred through our subsidiary, Savara Australia Pty. Limited.

## Liquidity and Capital Resources

As of March 31, 2018, we had \$22.1 million in cash, \$62.9 million in short-term investments and an accumulated deficit of \$95.1 million. We entered into a loan and security agreement with Silicon Valley Bank during the year ended December 31, 2017 and have executed two tranches totaling \$15 million, the maximum credit available pursuant to the debt facility, to date. We expect that our research and development and general and administrative expenses will increase, and, as a result, we anticipate that we will continue to incur losses in the foreseeable future. Therefore, we will need to raise additional capital to fund our operations, which may be through the issuance of additional equity, and potentially through borrowings.

## Cash Flows

The following table summarizes our cash flows for the periods indicated:

	Three Months Ended March 31, 2018      2017 (in thousands)	
Cash used in operating activities	\$ (9,671)	\$ (2,844)
Cash provided by / (used in) investing activities	9,390	(59 )
Cash provided by financing activities	271	—
Effect of exchange rate changes	11	(6 )
Net increase / (decrease) in cash	\$ 1	\$ (2,909)

## Cash flows from operating activities

Cash used in operating activities for the three months ended March 31, 2018 was \$9.7 million, consisting of a net loss of \$26.8 million, which was partially offset by noncash charges of \$17.7 million, mainly comprised of impairment of IPR&D, depreciation, noncash interest, fair value changes, provision for deferred taxes, accretion on discount to short-term investments, amortization of debt issuance costs, and stock-based compensation, and increased by a net decrease in assets and liabilities of \$0.5 million. The change in our net operating assets and liabilities was primarily due to an increase in accrued liabilities mostly related to research and development costs for both AeroVanc and Molgradex.

Cash used in operating activities for the three months ended March 31, 2017 was \$2.8 million, consisting mainly of a net loss of \$5.0 million, which was partially offset by noncash charges of \$0.6 million, mainly comprised of depreciation, noncash interest, fair value changes, loss on debt extinguishment, accretion of discount to convertible promissory notes, and stock-based compensation, and by a net increase in assets and liabilities of \$1.6 million.

#### Cash flows from investing activities

Cash provided by investing activities for the three months ended March 31, 2018 was primarily the result of the cash generated from the maturity of short-term investments net of cash used to purchase available-for-sale securities.



### Cash flows from financing activities

Cash provided by financing activities for the three months ended March 31, 2018 was primarily related to proceeds of \$0.5 million from the “at the market offerings” under the Sales Agreement as partially offset by \$0.2 million in principal payments on our capital lease obligation.

### Future Funding Requirements

We have not generated any revenue from product sales. We do not know when, or if, we will generate any revenue from product sales. We do not expect to generate any revenue from product sales unless and until we obtain regulatory approval for and commercialize any of our product candidates. At the same time, we expect our expenses to increase in connection with our ongoing development and manufacturing activities, particularly as we continue the research, development, manufacture and clinical trials of, and seek regulatory approval for, our product candidates. We expect to continue to incur additional costs associated with operating as a public company. In addition, subject to obtaining regulatory approval of any of our product candidates, we anticipate that we will need additional funding in connection with our continuing operations.

As of March 31, 2018, we had cash of \$22.1 million and short-term investments of \$62.9 million. We will continue to require additional capital to continue our clinical development and potential commercialization activities. Accordingly, we will need to raise additional capital to continue to fund our operations. The amount and timing of our future funding requirements will depend on many factors, including the pace and results of our clinical development efforts. Failure to raise capital as and when needed, on favorable terms or at all, would have a negative impact on our financial condition.

Until we can generate a sufficient amount of product revenue to finance our cash requirements, we expect to finance our future cash needs primarily through the issuance of additional equity and potentially through borrowings, grants and strategic alliances with partner companies. To the extent that we raise additional capital through the issuance of additional equity or convertible debt securities, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of existing stockholders. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through marketing and distribution arrangements or other 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 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 commercialization efforts or grant rights to develop and market product candidates to third parties that we would otherwise prefer to develop and market ourselves.

### Contractual Obligations

There were no material changes outside of the ordinary course of business in our contractual obligations during the three months ended March 31, 2018 from those disclosed in Item 7, “Management’s Discussion and Analysis of Financial Condition and Results of Operations - Contractual Obligations and Other Commitments” of our Annual Report on Form 10-K for the year ended December 31, 2017 filed with the SEC on March 14, 2018.

### Manufacturing Commitments and Contingencies

We are subject to various manufacturing royalties and payments related to our product candidate, Molgradex. Under an agreement, as amended, with the Active Pharmaceutical Ingredients (“API”) manufacturer, no signing fee or milestones are included in the royalty payments, and there is no minimum royalty. Upon the successful development, registration and attainment of approval by the proper health authorities, such as the FDA, in any territory except Latin America, Central America and Mexico, we must pay a royalty of three percent (3%) on annual net sales to the manufacturer of our API. Additionally, we must make certain payments to the API manufacturer upon the achievement of the milestones.

We are also subject to certain contingent milestone payments payable to the manufacturer of our nebulizer used to administer Molgradex. In addition to these milestones, we will owe a royalty to the manufacturer of our nebulizer based on net sales. The royalty rate ranges from three and a half percent (3.5%) to five percent (5%) depending on the device technology used by us to administer the product.

For a summary of the contingent milestone payments, refer to Note 2 “Summary of Significant Accounting Policies - Manufacturing Commitments and Contingencies” of the Condensed Consolidated Financial Statements in this report.

#### Other Contracts

We enter into contracts in the normal course of business with various third parties for research studies, clinical trials, testing and other services. These contracts generally provide for termination upon notice, and therefore we believe that our non-cancelable obligations under these agreements are not material except for certain obligations under our agreement for our capitalized lease asset.

### Off-Balance Sheet Arrangements

We have not entered into any off-balance sheet arrangements and do not have any holdings in variable interest entities.

### Recent Accounting Pronouncements

See Note 2, “Summary of Significant Accounting Policies – Recent Accounting Pronouncements,” of the Condensed Consolidated Financial Statements in this report for a discussion of recent accounting pronouncements and their effect, if any, on us.

### Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We have market risk exposure related to our cash, cash equivalents and short-term investment securities. Such interest-earning instruments carry a degree of interest rate risk; however, we have not been exposed nor do we anticipate being exposed to material risks due to changes in interest rates. A hypothetical 1% change in interest rates during any of the periods presented would not have had a material impact on our consolidated financial statements. Additionally, our investment securities are fixed income instruments denominated and payable in U.S. dollars and have short-term maturities, typically less than twelve months, and typically carry credit ratings of “A” at a minimum by two of three Nationally Recognized Statistical Rating Organizations, specifically Moody’s, Standard & Poor’s or Fitch. As such, we do not believe that our cash, cash equivalents and short-term investment securities have significant risk of default or illiquidity.

We have ongoing operations in Denmark as a result of our acquisition of Serendex and pay those vendors in local currency (Danish Krone) or Euros. We seek to limit the impact of foreign currency fluctuations through the use of derivative instruments, short-term foreign currency forward exchange contracts not designated as hedging instruments. We also have ongoing operations in Australia as a result of the expansion of Molgradex for the treatment of NTM lung infection and pay our respective vendors in Australian Dollars. We did not recognize any significant exchange rate losses during the three months ended March 31, 2018 and 2017. A 10% change in the Krone-to-dollar, Euro-to-dollar, Australian dollar-to-dollar, or Krone-to-Australian dollar exchange rate on March 31, 2018 would not have had a material effect on our results of operations or financial condition.

We also have interest rate exposure as a result of our loan and security agreement with Silicon Valley Bank. As of March 31, 2018, the outstanding gross principal amount of the secured term loan was \$15.0 million. The loan agreement bears interest at the prime rate reported in The Wall Street Journal, plus a spread of 4.25%. Changes in the prime rate may therefore affect our interest expense associated with our secured term loan.

If a 10% change in interest rates from the interest rates on March 31, 2018 were to have occurred, this change would not have had a material effect on the value of our investment portfolio or on our interest expense obligations with respect to outstanding borrowed amounts.

### Item 4. Controls and Procedures.

#### Evaluation of Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to provide reasonable assurance that information required to be disclosed by us in the reports we file or submit under the Securities Exchange Act of 1934, as amended, (the “Exchange Act”) is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms, and that such information is accumulated and communicated to our management, including our

principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, management recognized that any controls and procedures, no matter how well designed and operated, can only provide reasonable assurance of achieving the desired control objectives, and in reaching a reasonable level of assurance, management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, we have evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of March 31, 2018. Based on that evaluation, our principal executive officer and principal financial officer have concluded that as of March 31, 2018 these disclosure controls and procedures were effective at the reasonable assurance level.

#### Changes in Internal Control over Financial Reporting

There was no change in our internal control over financial reporting identified in connection with the evaluation required by Rules 13a-15(d) and 15d-15(d) under the Exchange Act that occurred during the quarterly period covered by this report that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

## PART II—OTHER INFORMATION

### Item 1. Legal Proceedings.

From time to time, we may become involved in various claims and legal proceedings. Regardless of outcome, litigation and other legal and administrative proceedings can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors. We are not currently a party to any material pending litigation or other material legal proceeding.

### Item 1A. Risk Factors.

Investment in our common stock involves a high degree of risk and uncertainty. Our business, operating results, growth prospects and financial condition are subject to various risks, many of which are not exclusively within our control, that may cause actual performance to differ materially from historical or projected future performance. We urge investors to consider carefully the risks described below, together with all of the information in this report and our other public filings, before making investment decisions regarding our securities. Each of these risk factors, as well as additional risks not presently known to us or that we currently deem immaterial, could adversely affect our business, operating results, growth prospects or financial condition, as well as the trading price of our common stock, in which case you may lose all or part of your investment.

#### Risks Related to Our Capital Requirements and Financial Condition

We have a limited operating history and have incurred significant losses since inception and expect that we will continue to incur losses for the foreseeable future, which makes it difficult to assess our future viability.

We are a clinical development-stage biopharmaceutical company with a limited operating history upon which to evaluate our business and prospects. We have not been profitable since we commenced operations and may not ever achieve profitability. In addition, we have limited history as an organization and have not yet demonstrated an ability to successfully overcome many of the risks and uncertainties frequently encountered by companies in new and rapidly evolving fields, particularly in the biopharmaceutical industry. Drug development is a highly speculative undertaking and involves a substantial degree of risk. To date, we have not obtained any regulatory approvals for any of our product candidates, commercialized any of our product candidates or generated any product revenue. We have devoted significant resources to research and development and other expenses related to our ongoing clinical trials and operations, in addition to acquiring product candidates.

For the three months ended March 31, 2018, we incurred a net loss of \$26.8 million, and net cash used in operating activities was \$9.7 million. At March 31, 2018, our cash, cash equivalents and short-term investment securities were \$85.0 million, and working capital was \$82.2 million. At March 31, 2018, we had an accumulated deficit of \$95.1 million. We expect to continue to incur substantial operating losses for the next several years as we seek to advance our product candidates through clinical development, global regulatory approvals, and commercialization. No revenue from operations will likely be available until, and unless, one of our product candidates is approved by the FDA or another regulatory agency and successfully marketed, or we enter into an arrangement that provides for licensing revenue or other partnering-related funding, outcomes which we may not achieve.

We will require additional financing to obtain regulatory approval for Molgradex and AeroVanc, and a failure to obtain this necessary capital when needed on acceptable terms, or at all, could force us to delay, limit, reduce or terminate our product development efforts or other operations.

Since our Aravas subsidiary was formed in 2007, most of our resources have been dedicated to the development and acquisition of our product candidates, Molgradex and AeroVanc. Under our current operating plan, we believe that our existing capital resources will be sufficient to fund our planned operations into 2020. However, we may raise additional capital, including through our “at the market” offering program to fund new studies, programs or acquisitions, or to address changes in our existing development programs. We cannot estimate with reasonable certainty the actual amounts necessary to successfully complete the development and commercialization of our product candidates and there is no certainty that we will be able to raise the necessary capital on reasonable terms or at all.

Our capital requirements for the foreseeable future will depend in large part on, and could increase significantly as a result of, our expenditures on our development programs. Future expenditures on our development programs are subject to many uncertainties, and will depend on, and could increase significantly as a result of, many factors, including:

- the number, size, complexity, results and timing of our drug development programs;
- the timing and terms of any collaborative or other strategic arrangement that we may establish;

26

---

- the number of clinical and nonclinical studies necessary to demonstrate acceptable evidence of the safety and efficacy of our product candidates;
- changes in standards of care which could increase the size and complexity of our clinical studies;
- the number of patients who participate, the rate of enrollment, and the ratio of randomized to evaluable patients in each clinical study;
- the ability to locate patients to participate in a study given the limited number of patients available for orphan or ultra-orphan indications;
- the number and location of sites and the rate of site initiation in each study;
- the duration of patient treatment and follow-up;
- the potential for additional safety monitoring or other post-marketing studies that may be requested by regulatory agencies;
- the time and cost to manufacture clinical trial material and commercial product, including process development and scale-up activities, and to conduct stability studies, which can last several years;
- the degree of difficulty and cost involved in securing alternate manufacturers or suppliers of drug product, components or delivery devices, as necessary to meet FDA requirements and/or commercial demand;
- the costs, requirements, timing of, and the ability to, secure regulatory approvals;
- the extent to which we increase our workforce and the costs involved in recruiting, training and incentivizing new employees;
- the costs related to developing, acquiring and/or contracting for sales, marketing and distribution capabilities, supply chain management capabilities, and regulatory compliance capabilities, if we obtain regulatory approval for a product candidate and commercialize it without a partner;
- the costs involved in evaluating competing technologies and market developments or the loss in sales in case of such competition; and
- the costs involved in establishing, enforcing or defending patent claims and other proprietary rights.

Additional capital may not be available when we need it, on terms that are acceptable to us or at all. If adequate funds are not available to us on a timely basis, we will be required to delay, limit, reduce or terminate our establishment of sales and marketing, manufacturing or distribution capabilities, development activities or other activities that may be necessary to commercialize our product candidates, conduct preclinical or clinical studies, or other development activities.

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. If we raise additional capital through public or private equity offerings, the ownership interest of our stockholders will be diluted and the terms of any new equity securities may have preferential rights over our common stock. If we raise additional capital through debt financing, it may be subject to covenants limiting or restricting our ability to take specific actions, such as incurring additional debt or making capital expenditures, or subject to specified financial ratios, any of which could restrict our ability to develop and commercialize our product candidates or operate as a business.

Our loan agreement contains covenants which may adversely impact our business; the failure to comply with such covenants could cause our outstanding debt to become immediately payable.

On April 28, 2017, we entered into a Loan and Security Agreement between us and Aravas, as co-borrowers, and Silicon Valley Bank, which we refer to as the Loan Agreement. The Loan Agreement includes a number of restrictive covenants, including restrictions on incurring additional debt, making investments, granting liens, disposing of assets, paying dividends and redeeming or repurchasing capital stock, subject to certain exceptions. Collectively, these covenants could constrain our ability to grow our business through acquisitions or engage in other transactions. In addition, the Loan Agreement includes covenants requiring, among other things, that we provide financial statements, comply with all laws, pay all taxes and maintain insurance. If we are not able to comply with these covenants, the loans under the Loan Agreement could become immediately due and payable and would have a material adverse

effect on our liquidity, financial condition, operating results, business, and prospects and cause the price of our common stock to decline.

27

---



We have significant goodwill and IPR&D and impairment of goodwill and IPR&D may have a significant adverse impact on our future financial condition and results of operations.

As of March 31, 2018, we had goodwill and IPR&D of approximately \$39.4 million. These intangible assets are subject to an impairment analysis whenever an event or change in circumstances indicates the carrying amount of such an asset may not be recoverable. We test our goodwill and IPR&D for impairment annually, or more frequently if an event or change in circumstances indicates that the asset may be impaired. If an impairment is identified, we would be required to record an impairment charge with respect to the impaired asset to our consolidated statements of operations and comprehensive loss. A significant impairment charge could have a material negative impact on our financial condition and results of operations. For example, during the three months ended March 31, 2018, we recorded \$21.7 million of impairment charges and a corresponding decrease to the carrying value of IPR&D related to the Aironite drug candidate due to the unfavorable results from a Phase 2 study that demonstrated a failure of Aironite to meet the endpoints of the study and limited effectiveness of the compound in patients. We will cease supporting or advancing the development of the Aironite drug candidate.

We will continue to evaluate our intangible assets for potential impairment in accordance with our accounting policies. If additional impairments are identified, we would be required to record an impairment charge with respect to the impaired asset to our consolidated statements of operations and comprehensive loss. A significant impairment charge could have a material negative impact on our financial condition and results of operations.

Events giving rise to impairment are difficult to predict and are an inherent risk in the pharmaceutical industry. Some of the potential risks that could result in impairment of our goodwill and IPR&D include negative clinical study results, adverse regulatory developments, delay or failure to obtain regulatory approval, additional development costs, changes in the manner of our use or development of our product candidates, competition, earlier than expected loss of exclusivity, pricing pressures, higher operating costs, changes in tax laws, prices that third parties are willing to pay for our IPR&D or similar assets in an arm's-length transaction being less than the carrying value of our IPR&D, and other market and economic environment changes or trends. Events or changes in circumstances may lead to significant impairment charges on our goodwill and/or IPR&D in the future, which could materially adversely affect our financial condition and results of operations.

#### Risks Related to Our Business Strategy and Operations

We are substantially dependent upon the clinical, regulatory and commercial success of our product candidates, Molgradex and AeroVanc. Clinical drug development involves a lengthy and expensive process with an uncertain outcome, results of earlier studies and trials may not be predictive of future trial results, and our clinical trials may fail to adequately demonstrate to the satisfaction of regulatory authorities the safety and efficacy of our product candidates.

The success of our business is dependent on our ability to advance the clinical development of Molgradex for the treatment of patients with pulmonary alveolar proteinosis, or PAP, and its expansion into nontuberculous mycobacterial, or NTM, lung infection, and AeroVanc for the treatment of persistent methicillin-resistant *Staphylococcus aureus*, or MRSA, infections in the lungs of CF patients. The Molgradex Phase 3 clinical study, designated as IMPALA, is ongoing in Europe, Japan, and the U.S. We expect to announce top-line results from the Phase 3 study of Molgradex in the second quarter of 2019. The AeroVanc Phase 3 study started in the U.S. and Canada in the third quarter of 2017.

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. A failure of one or more of our clinical trials 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. There is a high failure rate for drugs proceeding through clinical trials, and product candidates in later stages of clinical trials may fail to show the required safety and efficacy despite having progressed through preclinical studies

and initial clinical trials. A number of companies in the pharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier clinical trials, and we cannot be certain that we will not face similar setbacks. Even if our clinical trials are completed, the results may not be sufficient to obtain regulatory approval for our product candidates.

Given the developmental nature of our product candidates, we are subject to risks associated with initiating, completing and achieving positive outcomes from our current and future clinical trials, including:

- slow implementation, enrollment and completion of the clinical trials;
  - inability to enroll enough patients in the clinical trials;
- low patient compliance and adherence to dosing and reporting requirements, for example incomplete reporting of patient reported outcomes in the clinical trials or missed doses;
- lack of safety and efficacy in the clinical trials;

28

---

- delays in manufacture of supplies for both drug and device components due to delays in formulation, process development, or manufacturing activities;
- requirements for additional nonclinical or clinical studies based on changes to formulation and/or changes to regulatory requirements; and
- requirements for additional clinical studies based on inconclusive clinical results or changes in market, standard of care, and/or regulatory requirements.

If we successfully complete the necessary clinical trials for our product candidates, our success will be subject to the risks associated with obtaining regulatory approvals, product launch, and commercialization, including:

- FDA rejection of our New Drug Application (“NDA”) submissions for our product candidates;
- regulatory rejection in the EU, Japan, and other markets;
- delays during regulatory review and/or requirements for additional Chemistry, Manufacturing, and Controls (“CMC”), nonclinical, or clinical studies, resulting in increased costs and/or delays in marketing approval and subsequent commercialization of the product candidates in the United States and other markets;
- inability to consistently manufacture commercial supplies of drug and delivery devices resulting in slowed market development and lower revenue;
- poor commercial sales due to:
  - the ability of our future sales organization or our potential commercialization partners to effectively sell the product candidates;
  - our lack of success in educating physicians and patients about the benefits, administration and use of our product candidates;
  - the availability, perceived advantages, relative cost, relative safety and relative efficacy of other products or treatments for the targeted indications of the product candidates;
  - low patient demand for the product candidates; and
  - poor prescription coverage and inadequate reimbursement for our product candidates;
- our inability to enforce our intellectual property rights in our product candidates; and
- reduction in the safety profile of our product candidates following approval.

Many of these clinical, regulatory and commercial matters are beyond our control and are subject to other risks described elsewhere in this “Risk Factors” section. Accordingly, we cannot assure that we will be able to advance our product candidates further through final clinical development, or obtain regulatory approval of, commercialize or generate significant revenue from them. If we cannot do so, or are significantly delayed in doing so, our business will be materially harmed.

If we fail to attract and retain senior management and key scientific personnel, we may be unable to successfully develop and commercialize our product candidates.

We have historically operated with a limited number of employees that manage third-parties for most development activities. Institutional knowledge is concentrated within a small number of employees. Our success depends on our continued ability to attract, retain and motivate highly qualified management, clinical and scientific personnel. Our future success is highly dependent upon the contributions of our senior management, as well as our senior scientists and other members of our senior management team. The loss of services of any of these individuals, who all have at-will employment arrangements with us, could delay or prevent the successful development of our product pipeline, completion of our planned clinical trials or the commercialization of our product candidates.

Replacing key employees may be a difficult, costly and protracted process, and we may not have other personnel with the capacity to assume all the responsibilities of a key employee upon his/her departure. Transition periods can be difficult to manage and may cause disruption to our business. In addition, there may be intense competition from other companies and organizations for qualified personnel. Other companies and organizations with which we compete for personnel may have greater financial and other resources and different risk profiles than us, and a history of successful development and commercialization of our product candidates. If we cannot attract and retain skilled personnel, as needed, we may not achieve our development and other goals.



In addition, the success of our business will depend on our ability to develop and maintain relationships with respected service providers and industry-leading consultants and advisers. If we cannot develop and maintain such relationships, as needed, the rate and success at which we can develop and commercialize product candidates may be limited. In addition, our outsourcing strategy, which has included engaging consultants that spend considerable time to manage key functional areas, may subject us to scrutiny under labor laws and regulations, which may divert management time and attention and have an adverse effect on our business and financial condition.

We do not have, and do not have plans to establish manufacturing facilities. We completely rely on third parties for the manufacture and supply of our clinical trial drug and delivery device supplies and, if approved, commercial product materials. The loss of any of these vendors or a vendor's failure to provide us with an adequate supply of clinical trial or commercial product material in a timely manner and on commercially acceptable terms, or at all, could harm our business.

We outsource the manufacture of our product candidates and do not plan to establish our own manufacturing facilities. To manufacture our product candidates, we have made numerous custom modifications at CMOs, making us highly dependent on these CMOs. For clinical and commercial supplies, if approved, we have supply agreements with third party CMOs for drug substance, finished drug product, drug delivery devices and other necessary components of our product candidates. While we have secured long-term commercial supply agreements with many of the third party CMOs, we would need to negotiate agreements for commercial supply with several important CMOs, and we may not be able to reach agreement on acceptable terms. In addition, we rely on these third parties to conduct or assist us in key manufacturing development activities, including qualification of equipment, developing and validating methods, defining critical process parameters, releasing component materials and conducting stability testing, among other things. If these third parties are unable to perform their tasks successfully in a timely manner, whether for technical, financial or other reasons, we may be unable to secure clinical trial material, or commercial supply material if approved, which likely would delay the initiation, conduct or completion of our clinical studies or prevent us from having enough commercial supply material for sale, which would have a material and adverse effect on our business.

All manufacturers of our clinical trial material and, if approved, commercial product, including drug substance manufacturers, must comply with current good manufacturing practices ("cGMP") requirements enforced by the FDA through its facilities inspection program and applicable requirements of foreign regulatory authorities. These requirements include quality control, quality assurance and the maintenance of records and documentation. Manufacturers of our clinical trial material may be unable to comply with these cGMP requirements and with other FDA, state and foreign regulatory requirements. While we and our representatives generally monitor and audit our manufacturers' systems, we do not have full control over their ongoing compliance with these regulations. And while the responsibility to maintain cGMP compliance is shared between us and the third-party manufacturer, we bear ultimately responsibility for our supply chain and compliance with regulatory standards. Failure to comply with these requirements may result in fines and civil penalties, suspension of production, suspension or delay or failure to obtain product approval, product seizure or recall, or withdrawal of product approval.

Currently, we do not have alternative vendors to back up our primary vendors of clinical trial material or, if approved, commercial supply material. Identification of and discussions with other vendors may be protracted and/or unsuccessful, or these new vendors may be unsuccessful in producing the same results as the current primary vendors producing the material. Therefore, if our primary vendors become unable or unwilling to perform their required activities, we could experience protracted delays or interruptions in the supply of clinical trial material and, ultimately, product for commercial sale, which would materially and adversely affect our development programs, commercial activities, operating results and financial condition. In addition, the FDA or regulatory authorities outside of the United States may require that we have an alternate manufacturer of a drug product before approving it for marketing and sale in the United States or abroad and securing such alternate manufacturer before approval of an NDA could result in considerable additional time and cost prior to NDA approval.

Any new manufacturer or supplier of finished drug product or its component materials, including drug substance and delivery devices, would be required to qualify under applicable regulatory requirements and would need to have sufficient rights under applicable intellectual property laws to the method of manufacturing of such product or ingredients required by us. The FDA or foreign regulatory agency may require us to conduct additional clinical studies, collect stability data and provide additional information concerning any new supplier, or change in a validated manufacturing process, including scaling-up production, before we could distribute products from that manufacturer or supplier or revised process. For example, if we were to engage a third party other than our current CMOs to supply the drug substance or drug product for future clinical trial, or commercial product, the FDA or regulatory authorities outside of the United States may require us to conduct additional clinical and nonclinical studies to ensure comparability of the drug substance or drug product manufactured by our current CMOs to that manufactured by the new supplier. Changing of suppliers or equipment is particularly challenging for companies like us, with inhalation products, because any change could alter the performance of the drug product. The manufacturing of the drug substance of Molgradex, molgramostim, a biological drug substance, as well as the drug product, Molgradex, is currently being transferred to a new manufacturing site. Producing Molgradex, a biological product, is challenging, and therefore, may require more time and resources than previously anticipated. The transfer of the manufacturing to the new site may also cause regulatory agencies, including the FDA, to require additional nonclinical or clinical studies, which may cause delay or failure to obtain regulatory approval, and incur substantial additional cost.

The manufacture of pharmaceutical products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling-up initial production. These problems include difficulties with production costs and yields, quality control, including stability of the product candidate and quality assurance testing, and shortages of qualified personnel. Some of our product candidates have not been manufactured at the scale we believe will be necessary to maximize its commercial value and, accordingly, we may encounter difficulties in attempting to scale-up production and may not succeed in that effort on a timely basis or at all. In addition, the FDA or other regulatory authorities may impose additional requirements as we scale up initial production capabilities, which may delay our scale-up activities and/or add expense.

If our manufacturers encounter any of the aforementioned difficulties or otherwise fail to comply with their contractual obligations or there are delays entering commercial supply agreements due to capital constraints, we may have insufficient quantities of material to support ongoing and/or planned clinical studies or to meet commercial demand, if approved. In addition, any delay or interruption in the supply of materials necessary or useful to manufacture our product candidates could delay the completion of our clinical studies, increase the costs associated with our development programs and, depending upon the period of delay, require us to commence new clinical studies at significant additional expense or terminate the studies completely. Delays or interruptions in the supply of commercial product could result in increased cost of goods sold and lost sales. We cannot provide assurance that manufacturing or quality control problems will not arise in connection with the manufacture of our clinical trial material or commercial product, if approved, or that third-party manufacturers will be able to maintain the necessary governmental licenses and approvals to continue manufacturing such clinical trial material or commercial product, as applicable. In addition, Molgradex and AeroVanc are currently manufactured entirely or partially outside the United States and, as a result, we may experience interruptions in supply due to shipping or customs difficulties or regional instability. Furthermore, changes in currency fluctuations, shipping costs, or import tariffs could adversely affect cost of goods sold. Any of the above factors could cause us to delay or suspend anticipated or ongoing trials, regulatory submissions or commercialization of our product candidates, entail higher costs or result in being unable to effectively commercialize our products. Our dependence upon third parties for the manufacture of our clinical trial material may adversely affect our future costs and our ability to develop and commercialize our product candidates on a timely and competitive basis.

We rely significantly on third parties to conduct our nonclinical testing and clinical studies and other aspects of our development programs and if those third parties do not satisfactorily perform their contractual obligations or meet anticipated deadlines, the development of our product candidates could be adversely affected.

We do not employ personnel or possess the facilities necessary to conduct many of the activities associated with our programs. We engage consultants, advisors, CROs, CMOs, and others to assist in the design and conduct of nonclinical and clinical studies of our product candidates, with interpretation of the results of those studies and with regulatory activities, and we expect to continue to outsource all or a significant amount of such activities. As a result, many important aspects of our development programs are and will continue to be outside our direct control, and our third-party service providers may not perform their activities as required or expected including the maintenance of good clinical practice (“GCP”), good laboratories practice (“GLP”) and good manufacturing practices (“GMP”) compliance, which are ultimately our responsibility to ensure. Further, such third parties may not be as committed to the success of our programs as our own employees and, therefore, may not devote the same time, thoughtfulness or creativity to completing projects or problem-solving as our own employees would. To the extent we are unable to successfully manage the performance of third-party service providers, our business may be adversely affected.

The CROs that we engage to execute our clinical studies play a significant role in the conduct of the studies, including the collection and analysis of study data, and we likely will depend on CROs and clinical investigators to conduct future clinical studies and to assist in analyzing data from completed studies and developing regulatory strategies for our product candidates. Individuals working at the CROs with which we contract, as well as investigators at the sites at which our studies are conducted, are not our employees, and we have limited control over the amount or timing of

resources that they devote to their programs. If our CROs, study investigators, and/or third-party sponsors fail to devote sufficient time and resources to studies of our product candidates, if we and/or our CROs do not comply with all GLP and GCP regulatory and contractual requirements, or if their performance is substandard, we may delay commencement and/or completion of these studies, submission of applications for regulatory approval, regulatory approval, and commercialization of our product candidates. Failure of CROs to meet their obligations to us could adversely affect development of our product candidates.

In addition, CROs we engage may have relationships with other commercial entities, some of which may compete with us. Through intentional or unintentional means, our competitors may benefit from lessons learned on our projects that could ultimately harm our competitive position. Moreover, if a CRO fails to properly, or at all, perform our activities during a clinical study, we may not be able to enter into arrangements with alternative CROs on acceptable terms or in a timely manner, or at all. Switching CROs may increase costs and divert management time and attention. In addition, there likely would be a transition period before a new CRO commences work. These challenges could result in delays in the commencement or completion of our clinical studies, which could materially impact our ability to meet our desired and/or announced development timelines and have a material adverse impact on our business and financial condition.



We currently have limited marketing capabilities and no sales organization. If we are unable to establish sales and marketing capabilities on our own or through third parties, we will be unable to successfully commercialize our products, if approved, or generate product revenue.

To commercialize our products, if approved, in the United States and other jurisdictions we seek to enter, we must build our marketing, sales, managerial and other non-technical capabilities or make arrangements with third parties to perform these services, and we may not be successful in doing so. If our products receive regulatory approval, we expect to market such products in the United States through a focused, specialized sales force, which will be costly and time consuming. Institutionally, we have no prior experience in the marketing and sale of pharmaceutical products and there are significant risks involved in building and managing a sales organization, including our ability to hire, retain and incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel and effectively manage a geographically dispersed sales and marketing team. Outside of the United States, we may consider collaboration arrangements. If we are unable to enter into such arrangements on acceptable terms or at all, we may not be able to successfully commercialize our products in certain markets. Any failure or delay in the development of our internal sales, marketing and distribution capabilities would adversely impact the commercialization of our products. If we are not successful in commercializing our products, either on our own or through collaborations with one or more third parties, our future product revenue will suffer and we would incur significant additional losses.

Any future acquisitions that we make could disrupt our business and harm our financial condition.

We expect to evaluate, from time to time, potential strategic acquisitions of complementary businesses, products or technologies. In addition, we expect to evaluate joint ventures, licensing and other collaborative projects. We may not be able to identify appropriate acquisition candidates or strategic partners, or successfully negotiate, finance or integrate acquisitions of any businesses, products or technologies. Furthermore, the integration of any acquisition and management of any collaborative project may divert our management's time and resources from our core business and disrupt our operations. Any cash acquisition we pursue would diminish the funds otherwise available to us for other uses. Any stock acquisition would dilute our stockholders' ownership interests.

To establish a sales and marketing infrastructure and expand our manufacturing capabilities, we will need to increase the size of our organization, and we may experience difficulties in managing this growth.

As of May 1, 2018, we had 23 full-time employees, including 14 employees engaged in research and development. As we advance our product candidates through the development process and to commercialization, we will need to continue to expand our development, regulatory, quality, managerial, sales and marketing, operational, finance and other resources to manage our operations and clinical trials, continue our development activities and commercialize our product candidates, if approved. As our operations expand, we expect that we will need to manage additional relationships with various manufacturers and collaborative partners, suppliers and other organizations.

Due to our limited financial resources and our limited experience in managing a company with anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. In addition, the physical expansion of our operations may lead to significant costs and may divert our management attention and resources. Any inability to manage growth could delay the execution of our development and strategic objectives, or disrupt our operations, which could materially impact our business, revenue and operating results.

Our product candidates may cause undesirable side effects or adverse events, or have other properties that could delay or prevent our clinical development, regulatory approval or commercialization.

Undesirable side effects or adverse events caused by our product candidates could interrupt, delay or halt clinical studies and could result in the denial of regulatory approval by the FDA or other regulatory authorities for any or all

indications, and in turn prevent us from commercializing our product candidates. A significant challenge in clinical development is that the patient population in early studies, where small numbers of patients are required, is different from the patient population observed in later stage studies, where larger groups of patients are required. For example, patients in earlier stage studies may be more sick, compliant, or otherwise motivated than patients in larger studies. As such, efficacy or safety results may differ significantly between studies. Side-effects seen at high doses in earlier studies of AeroVanc, such as bronchoconstriction or other airway irritation, may be seen in significant numbers at the lower doses selected for later studies. Also, for AeroVanc, while not observed in the Phase 2 clinical study, the emergence of vancomycin-resistant MRSA could occur during the longer dosing period of AeroVanc that is currently implemented in the Phase 3 clinical study. If this or other undesirable side effects occur, they could possibly prevent approval, which would have a material and adverse effect on our business.

If any of our product candidates receive marketing approval and we or others later identify undesirable side effects caused by the product:

- regulatory authorities may require the addition of labeling statements, such as a “black box” warning or a contraindication;
- regulatory authorities may withdraw their approval of the product;
- we may be required to change the way the product is administered, conduct additional clinical studies or change the labeling of the product; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product or could substantially increase the costs and expenses of commercializing the product, which in turn could delay or prevent us from generating significant revenue from its sale.

We may not achieve our projected development goals in the time frames we have announced.

We have set goals for accomplishing certain objectives material to the successful development of our product candidates. The actual timing of these events may vary due to many factors, including delays or failures in our nonclinical testing, clinical studies and manufacturing and regulatory activities and the uncertainties inherent in the regulatory approval process. From time to time we create estimates for the completion of enrollment of or announcement of data from clinical studies of our product candidates. However, predicting the rate of enrollment or the time from completion of enrollment to announcement of data for any clinical study requires us to make significant assumptions that may prove to be incorrect. Our estimated enrollment rates and the actual rates may differ materially and the time required to complete enrollment of any clinical study may be considerably longer than we estimate. Such delays may adversely affect our financial condition and results of operations.

Even if we complete a clinical study with successful results, we may not achieve our projected development goals in the time frames we initially anticipate or announce. If a development plan for a product candidate becomes more extensive and costly than anticipated, we may determine that the associated time and cost are not financially justifiable and, as a result, may discontinue development in a particular indication or of the product candidate as a whole. In addition, even if a study did complete with successful results, changes may occur in regulatory requirements or policy during the period of product development and/or regulatory review of an NDA that relate to the data required to be included in NDAs which may require additional studies that may be costly and time consuming. Any of these actions may be viewed negatively, which could adversely impact our financial condition.

Further, throughout development, we must provide adequate assurance to the FDA and other regulatory authorities that we can consistently develop and produce our product candidates in conformance with GLP, GCP, cGMP, and other regulatory standards. As discussed above, we rely on CMOs for the manufacture of clinical, and future commercial, quantities of our product candidates. If future FDA or other regulatory authority inspections identify cGMP compliance deficiencies at these third-party facilities, production of our clinical trial material or, in the future, commercial product, could be disrupted, causing potentially substantial delay in or failure of development or commercialization of our product candidates.

Our employees, independent contractors and consultants, principal investigators, CROs, CMOs and other vendors, and any future commercial partners may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are exposed to the risk that our employees, independent contractors and consultants, principal investigators, CROs, CMOs and other vendors, and any future commercial partners may engage in fraudulent conduct or other misconduct, including intentional failures to comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, to provide accurate information to the FDA or comparable foreign regulatory authorities, to comply with manufacturing standards required by cGMP or our standards, to comply with federal and

state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable foreign regulatory authorities, and to report financial information or data accurately or disclose unauthorized activities to them. The misconduct of our employees and other service providers could involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. Although we have adopted a code of business conduct and ethics, it is not always possible to identify and deter such misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against them, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant fines or other sanctions. For example, if one of our manufacturing partners was placed under a consent decree, we may be hampered in our ability to manufacture clinical or commercial supplies.

Our business and operations would suffer in the event of third-party computer system failures, cyber-attacks on third-party systems or deficiency in our cyber security.

We rely on information technology systems, including third-party “cloud based” service providers, to keep financial records, maintain laboratory, clinical data and corporate records, communicate with staff and external parties and operate other critical functions. This includes critical systems such as email, other communication tools, electronic document repositories, and archives. If any of these third-party information technology (“IT”) providers are compromised due to computer viruses, unauthorized access, malware, natural disasters, fire, terrorism, war and telecommunication failures, electrical failures, cyber-attacks or cyber-intrusions over the internet, then sensitive emails or documents could be exposed or deleted. Similarly, we could incur business disruption if our access to the internet is compromised and we are unable to connect with third-party IT providers. The risk of a security breach or disruption, particularly through cyber-attacks or cyber intrusion, including by computer hackers, foreign governments, and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. In addition, we rely on those third parties to safeguard important confidential personal data regarding our employees and patients enrolled in our clinical trials. If a disruption event were to occur and cause interruptions in a third-party IT provider’s operations, it could result in a disruption of our drug development programs. For example, the loss of clinical trial data from completed, ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and development of our product candidates could be delayed, or could fail. We have experienced and may continue to experience attempts to breach our security and attempts to introduce malicious software into our information technology systems; however, to date, such attacks have not resulted in any material damage to us.

We are continually working to maintain reliable systems to control costs and improve our operations. Our efforts include, but are not limited to the following: firewalls, antivirus protection, patches, log monitors, routine backups with offsite retention of storage media, system audits, data partitioning and routine password modifications. Our internal information technology systems environment continues to evolve and our business policies and internal security controls may not keep pace as new threats emerge. No assurance can be given that our efforts to continue to enhance our systems will be successful.

Our operations might be interrupted by the occurrence of a natural disaster or other catastrophic event.

Our corporate headquarters is located in a single commercial facility in Austin, Texas, USA. We maintain a second office in a single commercial facility in Denmark where many of our product development staff are located. Important documents and records, including copies of our regulatory documents and other records for our product candidates, are located both at a secure offsite document storage facility as well at our own facilities and we depend on our facilities for the continued operation of our business. Natural disasters and other catastrophic events, such as wildfires and other fires, earthquakes and extended power interruptions and terrorist attacks or severe weather conditions, could significantly disrupt our operations and result in additional, unplanned expense. As a small company with limited resources, we have not prepared or implemented a formal business continuity or disaster recovery plan and any natural disaster or catastrophic event could disrupt our business operations and result in setbacks to our development programs. Even though we believe we carry commercially reasonable insurance, we might suffer losses that are not covered by or exceed the coverage available under these insurance policies.

#### Risks Related to Drug Development and Commercialization

We depend on the successful completion of clinical studies of our product candidates, and any positive results in prior clinical studies do not ensure that ongoing or future clinical studies will be successful.

Pharmaceutical products are subject to stringent regulatory requirements covering quality, safety, and efficacy. The burden of proof is on the manufacturer, such as Savara, to show with substantial clinical data that the risk/benefit profile for any new drug is favorable. Only after successfully completing extensive pharmaceutical development, nonclinical testing, and clinical studies may a product be considered for regulatory approval.

Clinical studies are expensive, difficult to design and implement, they can take many years to complete, and outcomes are inherently uncertain. A drug product may fail to demonstrate positive results at any stage of testing despite having progressed satisfactorily through nonclinical testing and initial clinical studies. There is significant risk in clinical development where later stage clinical studies are designed and powered based on the analysis of data from earlier studies, with these earlier studies involving a smaller number of patients, and the results of the earlier studies being driven primarily by a subset of responsive patients. In addition, interim results of a clinical study do not necessarily predict final results. Further, clinical study data frequently are susceptible to varying interpretations. Medical professionals and/or regulatory authorities may analyze or weigh study data differently than the sponsor company, resulting in delay or failure to obtain marketing approval for a product candidate. Additionally, the possible lack of standardization across multiple investigative sites may induce variability in the results which can interfere with the evaluation of treatment effects.

If we license rights to develop our product candidates to independent third parties or otherwise permit such third parties to evaluate our product candidates in clinical studies, we may have limited control over those clinical studies. Any safety or efficacy concern identified in a third-party sponsored study could adversely affect our or another licensee's development of our product candidate and prospects for its regulatory approval, even if the data from that study are subject to varying interpretations and analyses.

There is significant risk that ongoing and future clinical studies of our product candidates are unsuccessful. Negative or inconclusive results could cause the FDA and other regulatory authorities to require us to repeat or conduct additional clinical studies, which could significantly increase the time and expense associated with development of that product candidate or cause us to elect to discontinue one or more clinical programs. Failure to complete a clinical study of a product candidate or an unsuccessful result of a clinical study could have a material adverse effect on our business.

Molgradex and AeroVanc have received Orphan Drug Designation by the FDA and Molgradex has received Orphan Drug Designation also in Europe. While orphan designation provides certain benefits there are also associated risks.

Molgradex has received Orphan Drug Designation in the United States by the FDA and in Europe by the European Medicines Agency for the treatment of PAP, and AeroVanc has been granted Orphan Drug Designation in the United States by the FDA for the treatment of MRSA lung infection in patients with CF. Orphan Drug Designation will not shorten the regulatory review or reduce the clinical data requirements needed to obtain approval. If approval is received to market either Molgradex or AeroVanc for the respective indications, the FDA will not approve a similar product, with the same active ingredient, to Molgradex or AeroVanc for seven years and the European Medicines Agency will not approve a similar product to Molgradex for ten years, unless we are unable to produce enough supply to meet demand in the marketplace or another similar product, with the same active ingredient, is deemed clinically superior. Similar product candidates, with the same active ingredient and route of delivery, may be granted Orphan Drug Designation during the development of the respective products, but the Orphan Drug exclusivity is granted only to the first of such products approved, which means there is risk that a competitor product candidate may receive approval and Orphan Drug exclusivity before us, thus preventing us from marketing one or more of our product candidates until the exclusivity of the competing product expires. Also, the Orphan Drug status will not prevent a competitor with a different active ingredient from competing with our product candidates. If we are prevented from marketing one or more product candidates due to a competitor's Orphan Drug exclusivity, this would have a material adverse effect on our business.

Delays in commencement and completion of clinical studies are common and have many causes. Delays in clinical studies of our product candidates would likely increase overall development costs and jeopardize our ability to obtain regulatory approval and successfully commercialize any approved products.

Clinical testing typically is expensive, can take many years to complete, and its outcome is inherently uncertain. Clinical studies may not commence on time or be completed on schedule, if at all. The commencement and completion of clinical studies can be delayed for a variety of reasons, including:

- inability to raise sufficient funding to initiate or continue a clinical study;
- delays in obtaining regulatory approval to commence a clinical study;
- delays in identifying and reaching agreement on acceptable terms with prospective CROs, clinical study sites and investigators, which agreements can be subject to extensive negotiation and may vary significantly among study sites;
- delays in obtaining regulatory approval in a prospective country;
- delays in obtaining ethics committee approval to conduct a clinical study at a prospective site;
- delays in reaching agreements on acceptable terms with prospective CMOs or other vendors for the production and supply of clinical trial material and, if necessary, drug administration devices, which agreements can be subject to extensive negotiation;

- delays in the production or delivery of sufficient quantities of clinical trial material or drug delivery devices from our CMOs and other vendors to initiate or continue a clinical study;
- delays due to product candidate recalls as result of stability failure, excessive product complaints or other failures of the product candidate during its use or testing;
- invalidation of clinical data caused by premature unblinding or integrity issues;
- invalidation of clinical data caused by mixing up of the active drug and placebo through randomization or manufacturing errors;

35

---



- delays on the part of our CROs, CMOs, and other third-party contractors in developing procedures and protocols or otherwise conducting activities in accordance with applicable policies and procedures and in accordance with agreed upon timelines;
- delays in identifying and hiring or engaging, as applicable, additional employees or consultants to assist in managing clinical study-related activities;
- delays in recruiting and enrolling individuals to participate in a clinical study, which historically can be challenging in orphan diseases;
- delays caused by patients dropping out of a clinical study due to side effects, concurrent disorders, difficulties in adhering to the study protocol, unknown issues related to different patient profiles than in previous studies, such as the reduced age limit required for inclusion into the AeroVanc Phase 3 study, or otherwise;
- delays in having patients complete participation in a clinical study, including returning for post-treatment follow-up;
  - delays resulting from study sites dropping out of a trial, providing inadequate staff support for the study, problems with shipment of study supplies to clinical sites or focusing its staff's efforts on enrolling studies that compete for the same patient population;
- suspension of enrollment at a study site or the imposition of a clinical hold by the FDA or other regulatory authority following an inspection of clinical study operations at study sites or finding of a drug-related serious adverse event; and
- delays in quality control/quality assurance procedures necessary for study database lock and analysis of unblinded data.

Patient enrollment, a critical component to successful completion of a clinical study, is affected by many factors, including the size and nature of the study population, the proximity of patients to clinical sites, the eligibility criteria for the study, the design of the clinical study, ongoing studies competing for the same patient population and clinicians, patients' perceptions as to the potential advantages of the drug being studied in relation to available alternatives, including therapies being investigated by other companies which may be viewed as more beneficial or important to study, fear of being randomized to the placebo arm, and changes in standard of care. Challenges to complete enrollment can be exacerbated in orphan indications, like those being pursued by us, with a limited number of qualifying patients and the lack of clinical sites with the necessary expertise and experience to conduct our studies. Further, completion of a clinical study and/or its results may be adversely affected by failure to retain patients who enroll in a study but withdraw due to adverse side effects, perceived lack of efficacy, belief that they are on placebo, improvement in condition before treatment has been completed, or for personal reasons, or without reason, or by patients who fail to return for or complete post-treatment follow-up.

Clinical studies may not begin on time or be completed in the time frames we anticipate and may be costlier than we anticipate for a variety of reasons, including one or more of those described above. The length of time necessary to successfully complete clinical studies varies significantly and is difficult to predict accurately. We may make statements regarding anticipated timing for completion of enrollment in and/or availability of results from our clinical studies, but such predictions are subject to a number of significant assumptions and actual timing may differ materially for a variety of reasons, including patient enrollment rates, length of time needed to prepare raw study data for analysis and then to review and analyze it, and other factors described above. If we experience delays in the completion of a clinical study, if a clinical study is terminated, or if failure to conduct a study in accordance with regulatory requirements or the study's protocol leads to deficient safety and/or efficacy data, the regulatory approval and/or commercial prospects for our product candidates may be harmed and our ability to generate product revenue will be delayed. In addition, any delays in completing our clinical studies likely will increase our development costs. Further, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical studies may ultimately lead to the denial of regulatory approval of a product candidate. Even if we ultimately commercialize our product candidates, the standard of care may have changed or other therapies for the same indications may have been introduced to the market in the interim and may establish a competitive threat to us or diminish the need for our products.

Clinical studies are very expensive, difficult to design and implement, often take many years to complete, and the outcome is inherently uncertain.

Clinical development of pharmaceutical products for humans is generally very expensive, takes many years to complete and failures can occur at any stage of clinical testing. We estimate that clinical development of our product candidates will take several additional years to complete, but because of the variety of factors that can affect the design, timing and outcome of clinical studies, we are unable to estimate the exact funds required to complete research and development, obtain regulatory approval and commercialize all of our product candidates. We will need significant additional capital to continue to advance our products as per current business plans.

Failure at any stage of clinical testing is not uncommon and we may encounter problems that would require additional, unplanned studies or cause us to abandon a clinical development program.

In addition, a clinical study may be suspended or terminated by us, an Independent Review Board (“IRB”), a data safety monitoring board, the FDA or other regulatory authorities due to a number of factors, including:

- lack of adequate funding to continue the study;
- failure to conduct the study in accordance with regulatory requirements or the study’s protocol;
- inspection of clinical study operations or sites by the FDA or other regulatory authorities resulting in the imposition of a clinical hold;
- unforeseen safety issues, including adverse side effects; or
- changes in governmental regulations or administrative actions.

Changes in governmental regulations and guidance relating to clinical studies may occur and we may need to amend study protocols to reflect these changes, or we may amend study protocols for other reasons. Amendments may require us to resubmit protocols to IRBs for re-examination and approval or renegotiate terms with CROs, study sites and investigators, all of which may adversely impact the costs or timing of or our ability to successfully complete a trial.

There is significant uncertainty regarding the regulatory approval process for any investigational new drug, substantial further testing and validation of our product candidates and related manufacturing processes may be required, and regulatory approval may be conditioned, delayed or denied, any of which could delay or prevent us from successfully marketing our product candidates and substantially harm our business.

Pharmaceutical products generally are subject to rigorous nonclinical testing and clinical studies and other approval procedures mandated by the FDA and foreign regulatory authorities. Various federal and foreign statutes and regulations also govern or materially influence the manufacturing, safety, labeling, storage, record keeping and marketing of pharmaceutical products. The process of obtaining these approvals and the subsequent compliance with appropriate U.S. and foreign statutes and regulations is time-consuming and requires the expenditure of substantial resources.

Molgradex is currently undergoing a Phase 3 clinical study in the United States, Europe, and Japan. The product (formulation, process, packaging, and device) used in this Phase 3 study will be submitted in marketing applications to regulatory authorities unchanged. However, the product submitted may result in regulatory delays and/or non-acceptance for a variety of reasons including but not limited to: justification for inclusion of one or more excipients; safety qualification of one or more excipients; acceptability of commercial manufacturing site; ease of presenting the dose to the nebulizer; and, reproducibility of delivered dose from the nebulizer. Concurrently, we are exploring formulation, process, packaging, and device improvements that could simplify the composition of the drug product by eliminating one or more potentially unnecessary or harmful excipients, improve the ease of use of the product, and/or reduce the overall product variability. While we expect these changes to improve the product quality and possibly reduce other documentation requirements, the regulatory agencies may require additional clinical or nonclinical studies prior to approval of such changes, including conducting an additional Phase 3 clinical study that would significantly extend the timeline for clinical development of Molgradex in PAP.

The manufacturing process and site for the drug product may change post-Phase 3. Changes in the manufacturing process and site have a potential to result in untoward changes in drug product characteristics. If the commercial drug product differs significantly from the product studied in Phase 3, then regulatory agencies may require additional clinical or nonclinical studies prior to approval of such changes, including conducting an additional Phase 3 clinical study that would significantly extend the timeline for clinical development of Molgradex in PAP.

We recently received guidance from the FDA on the requirements to initiate clinical studies in the United States and on the clinical study requirements to achieve Biologics License Application (“BLA”) approval for Molgradex. Based on the guidance, we amended our ongoing Phase 3 clinical study to include more patients, and amended our endpoint hierarchy and statistical analyses to be used for U.S. approval purposes. Even if the clinical study meets all of its statistical goals and protocol end points, the FDA may not view the results to provide persuasive evidence of efficacy

across multiple clinical endpoints. Instead, they may require additional clinical studies and/or other costly studies, including an additional Phase 3 study, which would require us to expend substantial additional resources and would significantly extend the timeline for clinical development prior to market approval, or result in failure to complete the clinical development of Molgradex.

We have commenced the Phase 3 trial of AeroVanc, the success of which will be needed for FDA approval to market AeroVanc in the United States to treat persistent MRSA lung infection in individuals living with CF. While significant communication with the FDA on the Phase 3 study design has occurred, even if the Phase 3 clinical study meets all of its statistical goals and protocol end points, the FDA may not view the results as robust and convincing. They may require additional clinical studies and/or other costly studies, which could require us to expend substantial additional resources and could significantly extend the timeline for clinical development prior to market approval. Additionally, we are conducting a two-year nonclinical carcinogenicity study on the AeroVanc powder, required by the FDA. The results of this study will not be known until a short time prior to potential submission of an NDA for AeroVanc. If the carcinogenicity study cannot be completed for technical or other reasons, or provides results that the FDA determines to be concerning, this may cause a delay or failure in obtaining approval for AeroVanc.

Significant uncertainty exists with respect to the regulatory approval process for any investigational new drug, including Molgradex, and AeroVanc. Regardless of any guidance the FDA or foreign regulatory agencies may provide a drug's sponsor during its development, the FDA or foreign regulatory agencies retain complete discretion in deciding whether to accept an NDA or BLA or the equivalent foreign regulatory approval submission for filing or, if accepted, approve an NDA or BLA. There are many components to an NDA or BLA or marketing authorization application submission in addition to clinical study data. For example, the FDA or foreign regulatory agencies will review the sponsor's internal systems and processes, as well as those of its CROs, CMOs and other vendors, related to development of its product candidates, including those pertaining to its clinical studies and manufacturing processes. Before accepting an NDA for review or before approving the NDA or BLA, the FDA or foreign regulatory agencies may request that we provide additional information that may require significant resources and time to generate and there is no guarantee that its product candidates will be approved for any indication for which we may apply. The FDA or foreign regulatory agencies may choose not to approve an NDA or BLA for any of a variety of reasons, including a decision related to the safety or efficacy data, manufacturing controls or systems, or for any other issues that the agency may identify related to the development of its product candidates. Even if one or more Phase 3 clinical studies are successful in providing statistically significant evidence of the efficacy and safety of the investigational drug, the FDA or foreign regulatory agencies may not consider efficacy and safety data from the submitted studies adequate scientific support for a conclusion of effectiveness and/or safety and may require one or more additional Phase 3 or other studies prior to granting marketing approval. If this were to occur, the overall development cost for the product candidate would be substantially greater and competitors may bring products to market before us, which could impair our ability to generate revenues from the product candidates, or even seek approval, if blocked by a competitor's Orphan Drug exclusivity, which would have a material adverse effect on our business, financial condition and results of operations.

Further, development of our product candidates and/or regulatory approval may be delayed for reasons beyond our control. For example, U.S. federal government shut-down or budget sequestration, such as one that occurred during January 2018, may result in significant reductions to the FDA's budget, employees and operations, which may lead to slower response times and longer review periods, potentially affecting our ability to progress development of our product candidates or obtain regulatory approval for our product candidates.

Even if the FDA or foreign regulatory agencies grant approvals for our product candidates, the conditions or scope of the approval(s) may limit successful commercialization of the product candidates and impair our ability to generate substantial sales revenue. For example, the FDA may approve label claims for AeroVanc with age restrictions and/or treatment duration limitations, or Molgradex with restrictions for use only by patients unresponsive to the current standard of care. They may limit the label of Molgradex or AeroVanc to a subset of patients based on a review of which patient groups had the greatest efficacious response in clinical studies. Such label restriction may be undesirable and may limit successful commercialization. The FDA or foreign regulatory agencies may also only grant marketing approval contingent on the performance of costly post-approval nonclinical or clinical studies, or subject to warnings or contraindications that limit commercialization. Additionally, even after granting approval, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion and recordkeeping for our products will be subject to extensive and ongoing regulatory requirements. These

requirements include submissions of safety and other post-marketing information and reports, registration, and continued compliance with cGMP, GCP, international conference on harmonization regulations and GLP, which are regulations and guidelines that are enforced by the FDA or foreign regulatory agencies for all of its clinical development and for any clinical studies that we conduct post-approval. The FDA or foreign regulatory agencies may decide to withdraw approval, add warnings or narrow the approved indications in the product label, or establish risk management programs that could restrict distribution of our products. These actions could result from, among other things, safety concerns, including unexpected side effects or drug interaction problems, or concerns over misuse of a product. If any of these actions were to occur following approval, we may have to discontinue commercialization of the product, limit our sales and marketing efforts, implement risk minimization procedures, and/or conduct post-approval studies, which in turn could result in significant expense and delay or limit our ability to generate sales revenues.

Regulations may be changed prior to submission of a marketing application that require higher hurdles than currently anticipated. These may occur as a result of drug scandals, recalls, or a political environment unrelated to our products.

Even if we receive regulatory approval for a product candidate, we may face regulatory difficulties that could materially and adversely affect our business, financial condition and results of operations.

Even if initial regulatory approval is obtained, as a condition to the initial approval, the FDA or a foreign regulatory agency may impose significant restrictions on a product's indicated uses or marketing or impose ongoing requirements for potentially costly post-approval studies or marketing surveillance programs, any of which would limit the commercial potential of the product. Our product candidates also will be subject to ongoing FDA requirements related to the manufacturing processes, labeling, packaging, storage, distribution, advertising, promotion, record-keeping and submission of safety and other post-market information regarding the product. For instance, the FDA may require changes to approved drug labels, require post-approval clinical studies and impose distribution and use restrictions on certain drug products. In addition, approved products, manufacturers and manufacturers' facilities are subject to continuing regulatory review and periodic inspections. If previously unknown problems with a product are discovered, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, the FDA may impose restrictions on that product or us, including requiring withdrawal of the product from the market. If we or a CMO of ours fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters or untitled letters;
- impose civil or criminal penalties;
- suspend or withdraw regulatory approval;
- suspend or terminate any ongoing clinical studies;
- refuse to approve pending applications or supplements to approved applications;
- exclude our product from reimbursement under government healthcare programs, including Medicaid or Medicare;
- impose restrictions or affirmative obligations on our or our CMO's operations, including costly new manufacturing requirements;
- close the facilities of a CMO; or
- seize or detain products or require a product recall.

If any of our product candidates for which we receive regulatory approval fails to achieve significant market acceptance among the medical community, patients or third-party payers, the revenue we generate from its sales will be limited and our business may never achieve profitability.

Our success will depend in substantial part on the extent to which our product candidates, if approved, are accepted by the medical community and patients and reimbursed by third-party payers, including government payers. The degree of market acceptance with respect to each of our approved products, if any, will depend upon a number of factors, including:

- the safety and efficacy of our products as demonstrated in clinical studies;
- acceptance in the medical and patient communities of our products as a safe and effective treatment;
- the product's taste, ease of use, or features associated with the delivery device;
- the perceived advantages of our product over alternative treatments, including with respect to the incidence and severity of any adverse side effects and the cost of treatment;
- the indications for which our product is approved;
  - claims or other information (including limitations or warnings) in a product's approved labeling;
- reimbursement and coverage policies of government and other third-party payers;
- pricing and cost-effectiveness of our product relative to alternative treatments;
- availability of alternative treatments;
-

smaller than expected market size due to lack of disease awareness of a rare disease, or the patient population with a specific rare disease being smaller than anticipated;  
• inappropriate diagnostic efforts due to limited knowledge and/or resources among clinicians;

39

---



- the prevalence of off-label substitution of chemically equivalent products or alternative treatments; and
- the resources we devote to marketing our product and restrictions on promotional claims we can make with respect to the product.

We cannot predict with reasonable accuracy whether physicians, patients, healthcare insurers or health maintenance organizations, or the medical community in general, will accept or utilize any of our products, if approved. If our product candidates are approved but do not achieve an adequate level of acceptance by these parties, we may not generate sufficient revenue to become or remain profitable. In addition, our efforts to educate the medical community and third-party payers regarding benefits of our products may require significant resources and may never be successful.

If we determine that a product candidate may not achieve adequate market acceptance or that the potential market size does not justify additional expenditures on the program, we may reduce our expenditures on the development and/or the process of seeking regulatory approval of the product candidate while we evaluate whether and on what timeline to move the program forward.

Even if we receive regulatory approval to market one or more of our product candidates in the United States, we may never receive approval or commercialize our products outside of the United States, which would limit our ability to realize the full commercial potential of our product candidates.

In order to market products outside of the United States, we must establish and comply with the numerous and varying regulatory requirements of other countries regarding safety and efficacy. Approval procedures vary among countries and can involve additional product testing and validation and additional administrative review periods. The time required to obtain approval in other countries generally differs from that required to obtain FDA approval. The regulatory approval process in other countries may include all of the risks detailed above regarding FDA approval in the United States, as well as other risks. Regulatory approval in one country does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in others. Failure to obtain regulatory approval in other countries or any delay or setback in obtaining such approval could have the same adverse effects detailed above regarding FDA approval in the United States. As described above, such effects include the risks that our product candidates may not be approved for all indications requested, which could limit the uses of our product candidates and have an adverse effect on product sales, and that such approval may be subject to limitations on the indicated uses for which the product may be marketed or require costly, post-marketing follow-up studies. Conversely, if the product candidates do receive approval outside the United States in the future, we may not meet the FDA requirements in the United States for approval.

We must comply with the U.S. Foreign Corrupt Practices Act and similar foreign anti-corruption laws.

The U.S. Foreign Corrupt Practices Act, to which we are subject, prohibits corporations and individuals from engaging in certain activities to obtain or retain business or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any foreign government official, government staff member, political party or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity. Other countries, such as the U.K., have similar laws with which we must comply. We face the risk that an employee or agent could be accused of violating one or more of these laws, particularly in geographies where significant overlap exists between local government and healthcare industries. Such an accusation, even if unwarranted, could prove disruptive to our developmental and commercialization efforts.

#### Risks Related to Our Intellectual Property

Our success will depend on obtaining and maintaining effective patent and other intellectual property protection for our product candidates and proprietary technology.

AeroVanc has received a U.S. Patent Notice of Allowance for its formulation in the United States, AeroVanc's primary market. AeroVanc has either been issued patents or is prosecuting patent applications in numerous countries outside the United States. We have no patent protection for Molgradex for the treatment of PAP, and primarily rely on the Orphan Drug exclusivity as our primary barrier to competition. Both Molgradex and AeroVanc utilize proprietary delivery devices with exclusive supply agreements. Molgradex is eligible for protection via a proprietary cell bank used in the production of the drug substance.

Our success will depend on our ability to:

- obtain and maintain patent and other exclusivity rights with respect to our products and their uses;
- prevent third parties from infringing upon our proprietary rights;

40

---

- maintain proprietary know-how and trade secrets;
- operate without infringing upon the patents and proprietary rights of others; and
- obtain appropriate licenses to patents or proprietary rights held by third parties if infringement would otherwise occur or if necessary to secure exclusive rights to them, both in the United States and in foreign countries.

The patent and intellectual property positions of biopharmaceutical companies generally are highly uncertain, involve complex legal and factual questions, and have been and continue to be the subject of much litigation. There is no guarantee that we have or will develop or obtain the rights to products or processes that are patentable, that patents will issue from any pending applications or that claims allowed will be sufficient to protect the technology we develop or have developed or that is used by us, our CMOs or our other service providers. In addition, any patents that are issued to us may be limited in scope or challenged, invalidated, infringed or circumvented, including by our competitors, and rights we have under issued patents may not provide competitive advantages to us. If competitors can develop and commercialize technology and products similar to ours, our ability to successfully commercialize our technology and products may be impaired.

Patent applications in the United States are confidential for a period of time until they are published, and publication of discoveries in scientific or patent literature typically lags actual discoveries by several months. As a result, we cannot be certain that the inventors listed in any patent or patent application owned by us were the first to conceive of the inventions covered by such patents and patent applications (for U.S. patent applications filed before March 16, 2013), or that such inventors were the first to file patent applications for such inventions outside the United States and, after March 15, 2013, in the United States. In addition, changes in or different interpretations of patent laws in the United States and foreign countries may affect our patent rights and limit the number of patents we can obtain, which could permit others to use our discoveries or to develop and commercialize our technology and products without any compensation to us.

Our AeroVanc patent is specific to the formulation of the AeroVanc powder. While this may prevent identical products from entering the market, it may not preclude someone skilled in the art from inventing an alternate formulation approach with comparable or improved characteristics.

We also rely on unpatented know-how and trade secrets and continuing technological innovation to develop and maintain our competitive position, which we seek to protect, in part, through confidentiality agreements with employees, consultants, collaborators and others. We also have invention or patent assignment agreements with our employees and certain consultants. The steps we have taken to protect our proprietary rights, however, may not be adequate to preclude misappropriation of or otherwise protect our proprietary information or prevent infringement of our intellectual property rights, and we may not have adequate remedies for any such misappropriation or infringement. In addition, it is possible that inventions relevant to our business could be developed by a person not bound by an invention assignment agreement with us or independently discovered by a competitor.

We also intend to rely on regulatory exclusivity for protection of our product candidates, if approved for commercial sale. Implementation and enforcement of regulatory exclusivity, which may consist of regulatory data protection and market protection, varies widely from country to country. Failure to qualify for regulatory exclusivity, or failure to obtain or maintain the extent or duration of such protections that we expect for our product candidates, if approved, could affect our decision on whether to market the products in a particular country or countries or could otherwise have an adverse impact on our revenue or results of operations. For Molgradex, which is administered via nebulization, we may rely on regulatory exclusivity for the combination of Molgradex and its delivery system. However, there is no assurance that our Molgradex product and its delivery system, if approved, will benefit from this type of market protection.

We may rely on trademarks, trade names and brand names to distinguish our products, if approved for commercial sale, from the products of our competitors. We intend to seek approval for new names for AeroVanc and Molgradex that meet the FDA's and foreign regulatory requirements. However, our trademark applications may not be approved. Third parties may also oppose our trademark applications or otherwise challenge our use of the trademarks in which

case we may expend substantial resources to defend our proposed or approved trademarks and may enter into agreements with third parties that may limit our use of our trademarks. In the event that our trademarks are successfully challenged, we could be forced to rebrand our product, which could result in loss of brand recognition and could require us to devote significant resources to advertising and marketing these new brands. For example, we filed a trademark for the name “Savara” and were challenged. We decided to terminate its application, but we may revisit such filings at a future date. Further, our competitors may infringe our trademarks or we may not have adequate resources to enforce our trademarks.

Our success depends on our ability to prevent competitors from duplicating or developing and commercializing equivalent versions of our product candidates, but patent protection may be difficult to obtain and any issued claims may be limited.

We have filed for patent protection in the United States and other countries to cover the formulation of AeroVanc and were granted a notice of allowance in the United States, its primary market. However, this patent may not provide us with significant competitive advantages, because the validity or enforceability of the patents may be challenged and, if instituted, one or more of the challenges may be successful. Patents may be challenged in the United States under post-grant review proceedings, inter partes re-examination, ex parte re-examination, or challenges in district court. Any patents issued in foreign jurisdictions may be subjected to comparable proceedings lodged in various foreign patent offices, or courts. These proceedings could result in either loss of the patent or loss or reduction in the scope of one or more of the claims of the patent. Even if a patent issues, and is held valid and enforceable, competitors may be able to design around our patents, such as by using pre-existing or newly developed technology, in which case competitors may not infringe our issued claims and may be able to market and sell products that compete directly with us before and after our patents expire.

We have filed for patent protection in the U.S. and other countries to cover various methods of therapeutic use of our product candidates, including the use of Molgradex for treating NTM lung infection. The potential use and potential therapeutic benefits of systemically administered GM-CSF for systemic NTM disease have been described in case reports in the literature, and therefore the use of an inhaled form of GM-CSF may be considered to lack novelty and an inventive step, and thereby to be unpatentable.

The patent prosecution process is expensive and time-consuming. We and any future licensors and licensees may not apply for or prosecute patents on certain aspects of our product candidates at a reasonable cost, in a timely fashion, or at all. We may not have the right to control the preparation, filing and prosecution of some patent applications related to our product candidates or technologies. As a result, these patents and patent applications may not be prosecuted and enforced in a manner consistent with our best interests. It is also possible that we or any future licensors or licensees will fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. Further, it is possible that defects of form in the preparation or filing of our patent applications may exist, or may arise in the future, such as with respect to proper priority claims, inventorship, assignment, or claim scope. If there are material defects in the form or preparation of our patents or patent applications, such patents or applications may be invalid or unenforceable. In addition, one or more parties may independently develop similar technologies or methods, duplicate our technologies or methods, or design around the patented aspects of our products, technologies or methods. Any of these circumstances could impair our ability to protect our products, if approved, in ways which may have an adverse impact on our business, financial condition and operating results.

Furthermore, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in and outside of the United States. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability use our patents to stop others from using or commercializing similar or identical products or technology, or limit the duration of the patent protection of our technology and drugs. Given the amount of time required for the development, testing and regulatory review of new drug 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 drugs similar or identical ours once Orphan Drug and Qualified Infectious Disease Product exclusivities have expired. See the section entitled “Risks Related to Our Industry” for further description of Orphan Drug and Qualified Infectious Disease Product exclusivities.

Enforcement of intellectual property rights in certain countries outside the United States has been limited or non-existent. Future enforcement of patents and proprietary rights in many other countries will likely be problematic

or unpredictable. Moreover, the issuance of a patent in one country does not assure the issuance of a similar patent in another country. Claim interpretation and infringement laws vary by nation, so the extent of any patent protection is uncertain and may vary in different jurisdictions.

Obtaining and maintaining 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, renewal fees, annuity fees and various other governmental fees on patents and applications are required to be paid to the United States Patent and Trademark Office, or USPTO, and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and applications. The USPTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process and after a patent has issued. There are situations in which non-compliance can result in decreased patent term adjustment or in abandonment or lapse of the patent or patent application, leading to partial or complete loss of patent rights in the relevant jurisdiction.

Third parties may claim that our products, if approved, infringe on their proprietary rights and may challenge the approved use or uses of a product or its patent rights through litigation or administrative proceedings, and defending such actions may be costly and time consuming, divert management attention away from our business, and result in an unfavorable outcome that could have an adverse effect on our business.

Our commercial success depends on our ability and the ability of our CMOs and component suppliers to develop, manufacture, market and sell our products and product candidates and use our proprietary technologies without infringing the proprietary rights of third parties. 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 or may be developing products. Because patent applications can take many years to publish and issue, there currently may be pending applications, unknown to us, that may later result in issued patents that our products, product candidates or technologies infringe, or that the process of manufacturing our products or any of our respective component materials, or the component materials themselves, infringe, or that the use of our products, product candidates or technologies infringe.

We or our CMOs or component material suppliers may be exposed to, or threatened with, litigation by a third party alleging that our products, product candidates and/or technologies infringe its patents and/or other intellectual property rights, or that one or more of the processes for manufacturing our products or any of our respective component materials, or the component materials themselves, or the use of our products, product candidates or technologies, infringe its patents and/or other intellectual property rights. If a third-party patent or other intellectual property right is found to cover our products, product candidates, technologies or our uses, or any of the underlying manufacturing processes or components, we could be required to pay damages and could be unable to commercialize our products or use our technologies or methods unless we are able to obtain a license to the patent or intellectual property right. A license may not be available to us in a timely manner or on acceptable terms, or at all. In addition, during litigation, the third-party alleging infringement could obtain a preliminary injunction or other equitable remedy that could prohibit us from making, using, selling or importing our products, technologies or methods.

There generally is a substantial amount of litigation involving patent and other intellectual property rights in the industries in which we operate and the cost of such litigation may be considerable. We can provide no assurance that our product candidates or technologies will not infringe patents or rights owned by others, licenses to which might not be available to us in a timely manner or on acceptable terms, or at all. If a third party claims that we or our CMOs or component material suppliers infringe its intellectual property rights, we may face a number of issues, including, but not limited to:

- infringement and other intellectual property claims which, with or without merit, may be expensive and time consuming to litigate and may divert management's time and attention from our business;
- substantial damages for infringement, including the potential for treble damages and attorneys' fees, which we may have to pay if it is determined that the product and/or its use at issue infringes or violates the third party's rights;
- a court prohibiting us from selling or licensing the product unless the third-party licenses its intellectual property rights to us, which it may not be required to do;
- if a license is available from the third party, we may have to pay substantial royalties, fees and/or grant cross-licenses to the third party; and
- redesigning our products or processes so they do not infringe, which may not be possible or may require substantial expense and time.

No assurance can be given that patents do not exist, have not been filed, or could not be filed or issued, which contain claims covering our products, product candidates or technology or those of our CMOs or component material suppliers or the use of our products, product candidates or technologies. Because of the large number of patents issued and patent applications filed in the industries in which we operate, there is a risk that third parties may allege they have patent rights encompassing our products, product candidates or technologies, or those of our CMOs or component material suppliers, or uses of our products, product candidates or technologies.

In the future, it may be necessary for us to enforce our proprietary rights, or to determine the scope, validity and unenforceability of other parties' proprietary rights, through litigation or other dispute proceedings, which may be costly, and to the extent we are unsuccessful, adversely affect our rights. In these proceedings, a court or administrative body could determine that our claims, including those related to enforcing patent rights, are not valid or that an alleged infringer has not infringed our rights. The uncertainty resulting from the mere institution and continuation of any patent or other proprietary rights-related litigation or interference proceeding could have a material and adverse effect on our business prospects, operating results and financial condition.



## Risks Related to Our Industry

We expect competition in the marketplace for our product candidates, should any of them receive regulatory approval.

Molgradex and AeroVanc have received Orphan Drug Designation from the FDA and Molgradex has received Orphan Drug Designation from the European Medicines Agency. Orphan Drug Designation will provide market exclusivity in the U.S. for 7 years and 10 years in Europe, but only if (1) Molgradex and AeroVanc receive market approval before a competitor using the same active compound for the same indication, (2) we are able to produce sufficient supply to meet demand in the marketplace, and (3) another product with the same active ingredient is not deemed clinically superior. AeroVanc has also received Qualified Infectious Disease Product (“QIDP”) status extending market exclusivity by an additional five years in addition to any other exclusivity obtained in the United States.

The industries in which we operate (biopharmaceutical, specialty pharmaceutical, biotechnology and pharmaceutical) are highly competitive and subject to rapid and significant change. Developments by others may render potential application of any of our product candidates in a particular indication obsolete or noncompetitive, even prior to completion of its development and approval for that indication. If successfully developed and approved, we expect our product candidates will face competition. We may not be able to compete successfully against organizations with competitive products, particularly large pharmaceutical companies. Many of our potential competitors have significantly greater financial, technical and human resources than us, and may be better equipped to develop, manufacture, market and distribute products. Many of these companies operate large, well-funded research, development and commercialization programs, have extensive experience in nonclinical and clinical studies, obtaining FDA and other regulatory approvals and manufacturing and marketing products, and have multiple products that have been approved or are in late-stage development. These advantages may enable them to receive approval from the FDA or any foreign regulatory agency before us and prevent us from competing due to their orphan drug protections. Smaller companies may also prove to be significant competitors, particularly through collaborative arrangements with large pharmaceutical and biotechnology companies. Furthermore, heightened awareness on the part of academic institutions, government agencies and other public and private research organizations of the potential commercial value of their inventions have led them to actively seek to commercialize the technologies they develop, which increases competition for investment in our programs. Competitive products may be more effective, easier to dose, or more effectively marketed and sold, than ours, which would have a material adverse effect on our ability to generate revenue.

We are subject to uncertainty relating to healthcare reform measures and reimbursement policies that, if not favorable to our products, could hinder or prevent our products’ commercial success, if any of our product candidates are approved.

The unavailability or inadequacy of third-party payer coverage and reimbursement could negatively affect the market acceptance of our product candidates and the future revenues we may expect to receive from those products. The commercial success of our product candidates, if approved, will depend on the extent to which the costs of such products will be covered by third-party payers, such as government health programs, commercial insurance and other organizations. Third-party payers are increasingly challenging the prices and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. These challenges to prices may be problematic to us since our products are targeted for a small number of patients (those suffering from orphan diseases) thus requiring us to charge very high prices in order to recover development costs and achieve a profit on our revenue. If these third-party payers do not consider our products to be cost-effective compared to other therapies, we may not obtain coverage for our products after approval as a benefit under the third-party payers’ plans or, even if we do, the level of coverage or payment may not be sufficient to allow us to sell our products on a profitable basis.

Significant uncertainty exists as to the reimbursement status for newly approved drug products, including coding, coverage and payment. There is no uniform policy requirement for coverage and reimbursement for drug products among third-party payers in the United States, therefore coverage and reimbursement for drug products can differ

significantly from payer to payer. The coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payer separately, with no assurance that coverage and adequate payment will be applied consistently or obtained. The process for determining whether a payer will cover and how much it will reimburse a product may be separate from the process of seeking approval of the product or for setting the price of the product. Even if reimbursement is provided, market acceptance of our products may be adversely affected if the amount of payment for our products proves to be unprofitable for healthcare providers or less profitable than alternative treatments or if administrative burdens make our products less desirable to use. Third-party payer reimbursement to providers of our products, if approved, may be subject to a bundled payment that also includes the procedure of administering our products or third-party payers may require providers to perform additional patient testing to justify the use of our products. To the extent there is no separate payment for our product(s), there may be further uncertainty as to the adequacy of reimbursement amounts.

The continuing efforts of governments, private insurance companies, and other organizations to contain or reduce costs of healthcare may adversely affect:

- our ability to set an appropriate price for our products;
- the rate and scope of adoption of our products by healthcare providers;
- our ability to generate revenue or achieve or maintain profitability;
  - the future revenue and profitability of our potential customers, suppliers and collaborators; and
- our access to additional capital.

Our ability to successfully commercialize our products will depend on the extent to which governmental authorities, private health insurers and other organizations establish what we believe are appropriate coverage and reimbursement for our products. The containment of healthcare costs has become a priority of federal and state governments worldwide and the prices of drug products have been a focus in this effort. For example, there have been several recent U.S. Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs and President Trump has stated that reducing drug pricing is a priority for his administration. We expect that federal, state and local governments in the United States, as well as in other countries, will continue to consider legislation directed at lowering the total cost of healthcare. In addition, in certain foreign markets, the pricing of drug products is subject to government control and reimbursement may in some cases be unavailable or insufficient. It is uncertain whether and how future legislation, whether domestic or abroad, could affect prospects for our product candidates or what actions federal, state, or private payers for healthcare treatment and services may take in response to any such healthcare reform proposals or legislation. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures may prevent or limit our ability to generate revenue, attain profitability or commercialize our product candidates, especially in light of our plans to price our product candidates at a high level.

Furthermore, we expect that healthcare reform measures that may be adopted in the future are unpredictable, and the potential impact on our operations and financial position is uncertain, but may result in more rigorous coverage criteria, lower reimbursement, and additional downward pressure on the price we may receive for approved product. Any reduction in reimbursement from Medicare or other government-funded programs may result in a similar reduction in payments from private payers. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our products, if approved.

We face potential product liability exposure and, if successful claims are brought against us, we may incur substantial liability for a product or product candidate and may have to limit its commercialization. In the future, we anticipate that we will need to obtain additional or increased product liability insurance coverage and it is uncertain whether such increased or additional insurance coverage can be obtained on commercially reasonable terms, if at all.

Our business (in particular, the use of our product candidates in clinical studies and the sale of any products for which we obtain marketing approval) will expose us to product liability risks. Product liability claims might be brought against us by patients, healthcare providers, pharmaceutical companies or others selling or involved in the use of our products. If we cannot successfully defend ourselves against any such claims, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for our products and loss of revenue;
- impairment of our business reputation;
- delays in enrolling patients to participate in our clinical studies;
- withdrawal of clinical study participants;
- a “clinical hold,” suspension or termination of a clinical study or amendments to a study design;
- significant costs of related litigation;

- substantial monetary awards to patients or other claimants; and
- the inability to commercialize our products and product candidates.

We maintain limited product liability insurance for our clinical studies, but our insurance coverage may not reimburse us or may not be sufficient to reimburse us for all expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive and, in the future, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses.

We expect that we will expand our insurance coverage to include the sale of commercial products if we obtain marketing approval for any of our product candidates, but we may be unable to obtain product liability insurance on commercially acceptable terms or may not be able to maintain such insurance at a reasonable cost or in sufficient amounts to protect us against potential losses. Large judgments have been awarded in class action lawsuits based on drug products that had unanticipated side effects. A successful product liability claim or series of claims brought against us, if judgments exceed our insurance coverage, could consume a significant portion of our cash and adversely affect our business.

#### Risks Related to our Common Stock

Our stock price is expected to continue to be volatile.

The market price of our common stock has been and is expected to continue to be subject to significant fluctuations. Market prices for securities of early-stage pharmaceutical, biotechnology and other life sciences companies have historically been particularly volatile. Some of the factors that may cause the market price of our common stock to fluctuate include:

- our ability to obtain regulatory approvals for our product candidates, and delays or failures to obtain such approvals;
- failure to meet or exceed any financial and development projections that we may provide to the public;
- failure to meet or exceed the financial and development projections of the investment community;
- failure of any of our product candidates, if approved, to achieve commercial success;
- failure to maintain our existing third party license and supply agreements;
- failure by us or our licensors to prosecute, maintain, or enforce our intellectual property rights;
- changes in laws or regulations applicable to our product candidates;
- any inability to obtain adequate supply of our product candidates or the inability to do so at acceptable prices;
- adverse regulatory authority decisions;
- introduction of new products, services, or technologies by our competitors;
  - if securities or industry analysts do not publish research or reports about our business, or if they issue an adverse or misleading opinions regarding our business and stock;
- failure to obtain sufficient capital to fund our business objectives;
- sales of our common stock by us or our stockholders in the future;
- trading volume of our common stock;
- the perception of the pharmaceutical industry by the public, legislatures, regulators, and the investment community;
- announcements of significant acquisitions, strategic partnerships, joint ventures, or capital commitments by us or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters, and our ability to obtain patent protection for our technologies;
- additions or departures of key personnel;
- significant lawsuits, including patent or stockholder litigation;
- changes in the market valuations of similar companies;
- general market or macroeconomic conditions;
- announcements by commercial partners or competitors of new commercial products, clinical progress or the lack thereof, significant contracts, commercial relationships or capital commitments;
- adverse publicity relating to the CF, PAP, or NTM markets generally, including with respect to other products and potential products in such markets;
- the introduction of technological innovations or new therapies that compete with potential products of the combined organization;
- changes in the structure of health care payment systems; and
- period-to-period fluctuations in our financial results.

Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies. These broad market fluctuations may also adversely affect the trading price of our common stock.

In the past, following periods of volatility in the market price of a company's securities, stockholders have often instituted class action securities litigation against those companies. Such litigation, if instituted, could result in substantial costs and diversion of management attention and resources, which could significantly harm our profitability and reputation.

We will incur costs and demands upon management as a result of complying with the laws and regulations affecting public companies.

We will incur significant legal, accounting and other expenses that we did not incur as a private company prior to the Merger, including costs associated with public company reporting requirements. We will also incur costs associated with corporate governance requirements, including requirements under the Sarbanes-Oxley Act, as well as rules implemented by the SEC and Nasdaq. These rules and regulations are expected to increase our legal and financial compliance costs and to make some activities more time-consuming and costly. For example, our management team consists of certain officers prior to the Merger whom have not previously managed and operated a public company. These officers and other personnel will need to devote substantial time to gaining expertise regarding operations as a public company and compliance with applicable laws and regulations. These rules and regulations may also make it difficult and expensive for us to obtain directors' and officers' liability insurance. As a result, it may be more difficult for us to attract and retain qualified individuals to serve on our board of directors or as executive officers, which may adversely affect investor confidence in us and cause our business or stock price to suffer.

We do not expect to pay any cash dividends in the foreseeable future.

We expect to retain any future earnings to fund the development and growth of our business. As a result, capital appreciation, if any, of our common stock will be stockholders' sole source of gain, if any, for the foreseeable future.

Because the Merger likely has resulted in an ownership change under Section 382 of the Internal Revenue Code, our pre-merger net operating loss carryforwards and certain other tax attributes will be subject to limitation. The net operating loss carryforwards and certain other tax attributes may also be subject to limitations as a result of ownership changes.

If a corporation undergoes an "ownership change" within the meaning of Sections 381, 382, and 383 of the Internal Revenue Code, the corporation's net operating loss carryforwards and certain other tax attributes arising from before the ownership change are subject to limitations on use after the ownership change. In general, an ownership change occurs if there is a cumulative change in the corporation's equity ownership by certain stockholders that exceeds fifty percentage points over a rolling three-year period. Similar rules may apply under state tax laws. The Merger likely resulted in an ownership change for us and, accordingly, our net operating loss carryforwards and certain other tax attributes with respect to the pre-closing period will be subject to limitations on use. Additional ownership changes in the future could result in additional limitations on our net operating loss carryforwards. Consequently, even if we achieve profitability, we may not be able to utilize a material portion of our net operating loss carryforwards and other tax attributes, which could have a material adverse effect on cash flow and results of operations.

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

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

None.

Item 6. Exhibits.

An Exhibit Index has been attached as part of this report and is incorporated by reference.

47

---

Exhibit Index

Exhibit

Number	Description
31.1	<u>Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</u>
31.2	<u>Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</u>
32.1	<u>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.</u>
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document



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.

Company Name

Date: May 9, 2018 By: /s/ Dave Lowrance  
Dave Lowrance  
Chief Financial Officer

(Principal Financial and Accounting Officer)

Date: May 9, 2018 By: /s/ Robert Neville  
Robert Neville  
Chief Executive Officer

(Principal Executive Officer)