

Axovant Sciences Ltd.
Form 10-Q
February 14, 2017
Table of Contents

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 December 31, 2016

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 001-37418

Axovant Sciences Ltd.
(Exact name of registrant as specified in its charter)

Bermuda 98-1333697
(State or other jurisdiction of (I.R.S. Employer
incorporation or organization) Identification No.)

Clarendon House - 2 Church Street
Hamilton HM 11
Bermuda Not Applicable

(Address of principal executive offices) (Zip Code)
Registrant's telephone number, including area code: +1 (441) 824-8100
(former name, former address and former fiscal year, if changed since last report)

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, or a smaller reporting company. See the definitions of "large accelerated filer", "accelerated filer", and "smaller reporting company" in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer Accelerated filer

Edgar Filing: Axovant Sciences Ltd. - Form 10-Q

Non-accelerated filer (Do not check if a smaller reporting company) Smaller reporting company

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

The number of shares outstanding of the Registrant's common shares, \$0.00001 par value per share, on February 13, 2017, was 99,161,719.

Table of Contents

AXOVANT SCIENCES LTD.

QUARTERLY REPORT ON FORM 10-Q
FOR THE QUARTER ENDED DECEMBER 31, 2016

TABLE OF CONTENTS

	Page
<u>PART I. FINANCIAL INFORMATION</u>	
<u>Item 1. Financial Statements (Unaudited):</u>	
<u>Condensed Consolidated Balance Sheets as of December 31, 2016 and March 31, 2016</u>	<u>3</u>
<u>Condensed Consolidated Statements of Operations and Comprehensive Loss for the Three and Nine Months Ended December 31, 2016 and December 31, 2015</u>	<u>4</u>
<u>Condensed Consolidated Statements of Shareholders' Equity for the Nine Months Ended December 31, 2016</u>	<u>5</u>
<u>Condensed Consolidated Statements of Cash Flows for the Nine Months Ended December 31, 2016 and December 31, 2015</u>	<u>6</u>
<u>Notes to Condensed Consolidated Financial Statements</u>	<u>7</u>
<u>Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations</u>	<u>14</u>
<u>Item 3. Quantitative and Qualitative Disclosures About Market Risk</u>	<u>27</u>
<u>Item 4. Controls and Procedures</u>	<u>27</u>
<u>PART II. OTHER INFORMATION</u>	
<u>Item 1. Legal Proceedings</u>	<u>28</u>
<u>Item 1A. Risk Factors</u>	<u>29</u>
<u>Item 2. Unregistered Sales of Equity Securities and Use of Proceeds</u>	<u>58</u>
<u>Item 3. Defaults Upon Senior Securities</u>	<u>59</u>
<u>Item 4. Mine Safety Disclosures</u>	<u>59</u>
<u>Item 5. Other Information</u>	<u>59</u>
<u>Item 6. Exhibits</u>	<u>59</u>
<u>SIGNATURES</u>	<u>60</u>

Table of Contents

PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

AXOVANT SCIENCES LTD.

Condensed Consolidated Balance Sheets

(Unaudited, in thousands, except share and per share data)

	December 31, 2016	March 31, 2016
Assets		
Current assets:		
Cash	\$ 200,405	\$ 276,251
Prepaid expenses and other current assets	4,234	4,865
Income tax receivable	938	970
Total current assets	205,577	282,086
Property and equipment, net	159	89
Deferred tax assets	323	323
Total assets	\$ 206,059	\$ 282,498
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	\$ 9,251	\$ 622
Due to Roivant Sciences Ltd. and Roivant Sciences, Inc.	4,947	1,814
Accrued expenses	25,397	8,319
Contingent payment liability	—	5,000
Total current liabilities	39,595	15,755
Total liabilities	39,595	15,755
Commitments and contingencies (Note 8)		
Shareholders' equity:		
Common shares, par value \$0.00001 per share, 1,000,000,000 shares authorized, 99,161,719 ¹ and 99,150,000 issued and outstanding at December 31, 2016 and March 31, 2016	1	1
Additional paid-in capital	448,773	420,934
Accumulated deficit	(282,310)	(154,192)
Total shareholders' equity	166,464	266,743
Total liabilities and shareholders' equity	\$ 206,059	\$ 282,498

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

Table of Contents

AXOVANT SCIENCES LTD.

Condensed Consolidated Statements of Operations and Comprehensive Loss

(Unaudited, in thousands, except share and per share amounts)

	Three Months Ended December 31,		Nine Months Ended December 31,	
	2016	2015	2016	2015
Operating expenses:				
Research and development expenses (includes share-based compensation expense of \$4,592 and \$14,599 for the three months ended December 31, 2016 and 2015 and \$14,029 and \$24,421 for the nine months ended December 31, 2016 and 2015, respectively)	\$36,630	\$34,324	\$93,980	\$53,209
General and administrative expenses (includes share-based compensation expense of \$3,739 and \$24,457 for the three months ended December 31, 2016 and 2015 and \$13,800 and \$39,537 for the nine months ended December 31, 2016 and 2015, respectively)	11,342	28,230	33,422	49,364
Total operating expenses	47,972	62,554	127,402	102,573
Loss before provision for income tax	(47,972)	(62,554)	(127,402)	(102,573)
Income tax (benefit) expense	(161)	802	716	901
Net loss and comprehensive loss	\$(47,811)	\$(63,356)	\$(128,118)	\$(103,474)
Net loss per common share — basic and diluted	\$(0.48)	\$(0.64)	\$(1.29)	\$(1.11)
Weighted average common shares outstanding — basic and diluted	99,161,719	99,150,000	99,157,415	92,914,909

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

Table of Contents

AXOVANT SCIENCES LTD.

Condensed Consolidated Statements of Shareholders' Equity

(Unaudited, in thousands, except share data)

	Common Shares		Additional Paid	Accumulated	Total
	Shares	Amount	in Capital	Deficit	Shareholders'
					Equity
Balance at March 31, 2016	99,150,000	\$ 1	\$ 420,934	\$(154,192)	\$ 266,743
Exercise of stock options	11,719	—	10	—	10
Share-based compensation expense	—	—	19,718	—	19,718
Capital contribution —share-based compensation expense	—	—	8,111	—	8,111
Net loss	—	—	—	(128,118)	(128,118)
Balance at December 31, 2016	99,161,719	\$ 1	\$ 448,773	\$(282,310)	\$ 166,464

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

Table of Contents

AXOVANT SCIENCES LTD.

Condensed Consolidated Statements of Cash Flows
(Unaudited, in thousands)

	Nine Months Ended December 31,	
	2016	2015
Cash flows from operating activities:		
Net loss	\$(128,118)	\$(103,474)
Adjustments to reconcile net loss to net cash used in operating activities:		
In-process research and development expenses	—	5,252
Share-based compensation	27,829	63,958
Depreciation and amortization	35	8
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	631	(3,814)
Accounts payable	8,629	3,153
Due to Roivant Sciences Ltd. and Roivant Sciences, Inc.	3,133	(781)
Accrued liabilities	17,078	5,469
Income tax receivable	32	98
Net cash used in operating activities	(70,751)	(30,131)
Cash flows from investing activities:		
Purchase of in-process research and development	—	(4,756)
Purchase of furniture and equipment	(105)	(59)
Net cash used in investing activities	(105)	(4,815)
Cash flows from financing activities:		
Cash proceeds from issuance of common shares in initial public offering, net of underwriting discount	—	336,893
Initial public offering costs paid	—	(2,351)
Cash capital contribution from Roivant Sciences Ltd.	—	750
Repayment of amounts due to Roivant Sciences Ltd. and Roivant Sciences, Inc. for amounts paid on behalf of the Company	—	(627)
Payment of contingent liability	(5,000)	—
Exercise of stock options	10	—
Due to Roivant Sciences Ltd. and Roivant Sciences, Inc. for amounts paid on behalf of the Company	—	279
Net cash (used in) provided by financing activities	(4,990)	334,944
Net change in cash	(75,846)	299,998
Cash—beginning of period	276,251	—
Cash—end of period	\$200,405	\$299,998
Supplemental disclosure of cash paid:		
Income Taxes	\$684	\$804

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

Table of Contents

AXOVANT SCIENCES LTD.

Notes to Condensed Consolidated Financial Statements (Unaudited)

Note 1—Description of Business

Axovant Sciences Ltd., inclusive of its wholly-owned subsidiaries (the “Company”) is a clinical-stage biopharmaceutical company focused on acquiring, developing and commercializing novel therapeutics for the treatment of dementia. The Company intends to develop a pipeline of product candidates to comprehensively address the cognitive, functional and behavioral aspects of dementia and related neurological disorders. The Company was founded on October 31, 2014 as a Bermuda Exempted Limited Company and a wholly-owned subsidiary of Roivant Sciences Ltd. (“RSL”), under the name Roivant Neurosciences Ltd. The Company changed its name to Axovant Sciences Ltd. in March 2015. On February 24, 2015, Axovant Sciences, Inc. (“ASI”) was formed, and on March 7, 2015 it became a wholly-owned subsidiary of the Company based in the United States of America. In August 2016, the Company incorporated as its wholly-owned subsidiaries Axovant Holdings Limited (“AHL”), a private limited company incorporated under the laws of England and Wales, and Axovant Sciences GmbH (“ASG”), a company with limited liability formed under the laws of Switzerland. ASG holds the Company’s intellectual property rights and will be the principal operating company for conducting its business.

From its inception, the Company has devoted substantially all of its efforts to organizing and staffing the Company, raising capital, acquiring product candidates and preparing for and advancing its lead product candidates, intepirdine, previously referred to as RVT-101, and nelotanserin into clinical development for patients with Alzheimer’s disease or Lewy body dementia. In addition, the Company has the rights to develop RVT-103, a combination of donepezil and a peripheral muscarinic receptor antagonist, and RVT-104, a combination of rivastigmine and a peripheral muscarinic receptor antagonist, and intends to develop these product candidates alone and in combination with intepirdine as potential treatments for patients with Alzheimer’s disease or Lewy body dementia. The Company has determined that it has one operating and reporting segment.

Note 2—Summary of Significant Accounting Policies

(A) Basis of Presentation:

The Company’s fiscal year ends on March 31, and its fiscal quarters end on June 30, September 30, and December 31.

The accompanying interim unaudited condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States (“U.S. GAAP”) for interim financial information and with the instructions to Form 10-Q and Article 10 of Regulation S-X. Accordingly, they do not include all of the information and disclosures required by U.S. GAAP for complete financial statements. These interim unaudited condensed consolidated financial statements should be read in conjunction with the Company’s audited consolidated financial statements included in the Company’s Annual Report on Form 10-K for the fiscal year ended March 31, 2016, filed with the Securities and Exchange Commission (“SEC”) on June 6, 2016. In the opinion of management, all adjustments (consisting of normal recurring adjustments) considered necessary to present fairly the financial position of the Company and its results of operations and cash flows for the interim periods presented have been included. Operating results for the three and nine months ended December 31, 2016 are not necessarily indicative of the results that may be expected for the year ending March 31, 2017, for any other interim period, or for any other future year.

Any reference in these notes to applicable guidance is meant to refer to the authoritative U.S. GAAP as found in the Accounting Standards Codification (“ASC”) and Accounting Standards Update (“ASU”) of the Financial Accounting Standards Board (“FASB”). The condensed consolidated financial statements include the accounts of the Company and ASI, AHL and ASG, its wholly-owned subsidiaries. The Company has no unconsolidated subsidiaries. All

intercompany balances and transactions have been eliminated in consolidation.

There have been no significant changes in the Company's accounting policies from those disclosed in its Annual Report on Form 10-K for the fiscal year ended March 31, 2016, filed with the SEC on June 6, 2016.

Table of Contents

(B) Use of Estimates:

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. The Company regularly evaluates estimates and assumptions related to assets, liabilities, costs and expenses, including compensation expense allocated to the Company under its services agreement with Roivant Sciences, Inc. ("RSI"), a wholly-owned subsidiary of RSL as well as contingent liabilities, share-based compensation and research and development costs. The Company bases its estimates and assumptions on historical experience and on various other factors that it believes to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results could differ from those estimates.

(C) Net Loss per Common Share:

Basic net loss per common share is computed by dividing net loss applicable to common shareholders by the weighted-average number of common shares outstanding during the period. Diluted net loss per common share is computed by dividing the net loss applicable to common shareholders by the diluted weighted-average number of common shares outstanding during the period calculated in accordance with the treasury stock method. Stock options to purchase 7.5 million common shares were not included in the calculation of diluted weighted-average common shares outstanding for the three and nine months ended December 31, 2016 because they were anti-dilutive. Stock options to purchase 5.7 million common shares were not included in the calculation of diluted weighted-average common shares outstanding for the three and nine months ended December 31, 2015 because they were anti-dilutive.

(D) Financial Instruments:

The Company utilizes fair value measurement guidance prescribed by accounting standards to value its financial instruments. The guidance establishes a fair value hierarchy for instruments measured at fair value that distinguishes between assumptions based on market data (observable inputs) and the Company's own assumptions (unobservable inputs). Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability, and are developed based on the best information available in the circumstances.

Fair value is identified as the exchange price, or exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As a basis for considering market participant assumptions in fair value measurements, the guidance establishes a three-tier fair value hierarchy that distinguishes among the following:

- Level 1-Valuations are based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access.
- Level 2-Valuations are based on quoted prices for similar assets or liabilities in active markets, quoted prices for identical or similar assets or liabilities in markets that are not active and models for which all significant inputs are observable, either directly or indirectly.
- Level 3-Valuations are based on inputs that are unobservable (supported by little or no market activity) and significant to the overall fair value measurement.

To the extent the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the

fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement. The Company's financial instruments consist of cash and accounts payable. These financial instruments are stated at their respective historical carrying amounts, which approximate fair value due to their short-term nature.

8

Table of Contents

(E) Recent Accounting Pronouncements:

In February 2016, the FASB issued ASU No. 2016-02, “Leases (Topic 842)” (ASU No. 2016-02), which is a comprehensive new lease standard that amends various aspects of existing accounting guidance for leases. The core principle of ASU No. 2016-02 will require lessees to present the assets and liabilities that arise from leases on their balance sheets. ASU No. 2016-02 is effective for annual periods beginning after December 15, 2018, and interim periods within fiscal years beginning after December 15, 2018. Early adoption is permitted. The Company is currently evaluating the new standard and its impact on the Company's consolidated financial position and results of operations.

In March 2016, the FASB issued ASU No. 2016-09, “Compensation - Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting” (ASU No. 2016-09). This ASU makes several modifications to Topic 718 related to the accounting for forfeitures, employer tax withholding on share-based compensation, and the financial statement presentation of excess tax benefits or deficiencies. ASU No. 2016-09 also clarifies the statement of cash flows presentation for certain components of share-based awards. The standard is effective for interim and annual reporting periods beginning after December 15, 2016, with early adoption permitted. The Company expects to adopt this guidance when effective and is currently evaluating the effect that the updated standard will have on its consolidated financial statements and related disclosures.

Note 3—Accrued Expenses

As of December 31, 2016 and March 31, 2016 the Company's accrued expenses consisted of the following (in thousands):

	December 31, 2016	March 31, 2016
Research and development expenses	\$ 20,532	\$ 5,659
Salaries, bonuses, and other compensation	2,246	1,893
Legal expenses	1,153	183
Other expenses	1,466	584
Total accrued expenses	\$ 25,397	\$ 8,319

Note 4 - License Agreement

In August 2016, the Company entered into an exclusive license agreement with Qaam Pharmaceuticals, LLC (“Qaam”) for intellectual property covering the combination of cholinesterase inhibitors with peripheral muscarinic receptor antagonists. The Company expects to develop and, if successful, commercialize products covered by the licensed intellectual property. The Company will initially develop RVT-103, a combination of a peripheral muscarinic receptor antagonist and donepezil. In addition, the Company expects to develop RVT-104, a combination of a peripheral muscarinic receptor antagonist and high-dose rivastigmine. The Company paid an initial license fee and expense reimbursement totaling \$0.6 million, which was recorded as research and development expense in the accompanying condensed consolidated statements of operations and comprehensive loss.

Note 5—Related Party Transactions

(A) Services Agreement:

During 2015, the Company and ASI entered into a services agreement with RSI (the “Services Agreement”) under which RSI has agreed to provide certain administrative and research and development services to the Company. The Company and ASI amended and restated the Services Agreement with RSI on October 13, 2015 effective for the fiscal year commencing April 1, 2015. Under the Services Agreement, as amended and restated, the Company pays or reimburses RSI for any expenses it, or third parties acting on its behalf, incurs for the Company. For any general and administrative and research and development activities performed by RSI employees, RSI charges back the employee compensation expense plus a pre-determined mark-up. Employee compensation expense, inclusive of base salary and fringe benefits, is determined based upon the relative percentage of time utilized on Company matters. All other costs are billed back at cost. The accompanying interim unaudited condensed consolidated financial statements include third-party expenses that have been paid by RSI and RSL.

Table of Contents

Under the Services Agreement, for the three months ended December 31, 2016 and 2015, the Company incurred expenses of \$1.9 million and \$2.1 million respectively, inclusive of the mark-up. For the nine months ended December 31, 2016 and 2015, the Company incurred expenses of \$5.3 million and \$5.4 million, respectively.

In February 2017, the Company and ASI amended and restated the Services Agreement, effective as of December 13, 2016, to add ASG as a services recipient. In addition, in February 2017, ASG entered into a separate services agreement with Roivant Sciences GmbH ("RSG"), a wholly-owned subsidiary of RSL, effective as of December 13, 2016, for the provision of services by RSG to ASG in relation to the identification of potential product candidates and project management of clinical trials, as well as other services related to development, administrative and financial activities.

(B) Family Relationships:

Geetha Ramaswamy, MD, the Vice President, Medical Affairs for ASI, is the mother of Vivek Ramaswamy, the Chief Executive Officer of ASI and the Company's principal executive officer. Shankar Ramaswamy, MD, the Vice President of Medical and Scientific Communications of ASI, is the brother of Vivek Ramaswamy. Sarah Friedhoff, Senior Business Operations and Research and Development Specialist of ASI, is the daughter of Lawrence Friedhoff, the Company's Chief Development Officer.

Salary expenses were \$65,000 and \$62,500 for both Geetha Ramaswamy and Shankar Ramaswamy for the three months ended December 31, 2016 and 2015, respectively. Salary expenses were \$194,167 and \$187,500 for both Geetha Ramaswamy and Shankar Ramaswamy for the nine months ended December 31, 2016 and 2015, respectively. Salary expenses were \$18,750 and \$19,456 for Sarah Friedhoff for the three months ended December 31, 2016 and 2015, respectively. Salary expenses were \$55,417 and \$26,459 for Sarah Friedhoff for the nine months ended December 31, 2016 and 2015, respectively.

Note 6—Share-Based Compensation

In March 2015, the Company adopted its 2015 Equity Incentive Plan (the "2015 Plan"), under which 7.5 million of the Company's common shares were originally reserved for grant. In May 2015, the Company's Board of Directors amended the 2015 Plan to increase the number of common shares authorized for issuance thereunder to 9.5 million common shares. The amendment of the 2015 Plan became effective upon the execution of the underwriting agreement relating to the Company's initial public offering of common shares in June 2015. On April 1, 2016, the number of common shares authorized for issuance increased automatically to 12.5 million in accordance with the terms of the 2015 Plan. At December 31, 2016, a total of 5.0 million common shares were available for future grant under the 2015 Plan. At December 31, 2016 and 2015, there were 7.5 million and 5.7 million options outstanding, respectively, with a weighted average exercise price of \$7.23 and \$4.34, respectively. At December 31, 2016, there were 2.3 million vested options.

(A) Stock Options Granted to Employees and Directors:

During the nine months ended December 31, 2016 and 2015, the Company granted a total of 2.2 million and 1.5 million options, respectively, to its employees and directors, under the 2015 Plan. The Company recorded share-based compensation expense related to stock options issued to Company employees and directors of \$5.2 million and \$4.3 million, respectively, for the three months ended December 31, 2016 and 2015, and \$18.0 million and \$11.9 million, respectively, for the nine months ended December 31, 2016 and 2015. At December 31, 2016, total unrecognized compensation expense related to non-vested options was \$50.0 million and is expected to be recognized over the

remaining weighted-average service period of 2.67 years.

(B) Share-Based Compensation for Related Parties:

(1) Stock Options Granted to Non-Employees:

During the nine months ended December 31, 2016 and 2015, the Company granted stock options to purchase 83,500 and 215,000 shares, respectively, of the Company's common stock to employees of RSI as compensation for support services provided to the Company. The fair value of the stock options granted to RSI employees is accounted for by the Company in accordance with the authoritative guidance for non-employee equity awards and is remeasured on each valuation date until performance is complete using the Black-Scholes pricing model.

Table of Contents

Each award is subject to a specified vesting schedule. Compensation expense will be recognized by the Company over the required service period to earn each award. The Company recorded \$0.3 million and \$0.7 million of share-based compensation expense for the three months ended December 31, 2016 and 2015, respectively, and \$1.1 million and \$1.3 million of share-based compensation expense for the nine months ended December 31, 2016 and 2015, respectively. The share-based compensation was recorded as research and development and general and administrative expense in the accompanying condensed consolidated statements of operations and comprehensive loss. The total remaining unrecognized compensation cost related to the non-vested stock options amounted to \$4.0 million as of December 31, 2016, which will be recognized over the weighted-average remaining requisite service period of 2.42 years.

(2) Share-Based Compensation Allocated to the Company by RSL:

The Company incurs share-based compensation expense for RSL common share awards and RSL options issued by RSL to RSI employees. Share-based compensation expense is allocated to the Company by RSL based upon the relative percentage of time utilized by RSI employees on Company matters.

These share-based compensation amounts include compensation expense for BVC awards prior to the BVC Merger on December 4, 2015. On December 4, 2015, BVC Ltd. (“BVC”), a non-public entity, which held a non-controlling ownership interest in RSL, the parent of the Company, was merged with and into RSL (the “BVC Merger”), with RSL as the surviving entity, as disclosed in Note E(1) to the audited financial statements included in the Company's Annual Report on Form 10-K for the fiscal year ended March 31, 2016. Prior to the BVC Merger, the Company recorded share-based compensation expense, in relation to the share-based awards issued by BVC to RSI employees based on the changes in fair value of BVC share-based awards.

As these BVC share-based awards were not based on the Company's or RSL's shares, they were remeasured at each reporting period date until performance was completed. As a result of the BVC Merger, all outstanding BVC share-based awards were converted into RSL common share awards, with the same vesting and forfeiture terms as the original grant. The RSL common share awards are fair valued on the date of grant and that fair value is recognized over the requisite service period. On December 8, 2015 following the BVC Merger, RSL was recapitalized in conjunction with a private financing. The estimated fair value of these RSL common share awards was determined by the valuation of RSL in the December 8, 2015 private financing. As RSL is a non-public entity, the majority of the inputs used to estimate the fair value of the BVC awards prior to the BVC Merger and the RSL common share awards following the BVC Merger are classified as level 3 due to their unobservable nature. Significant judgment and estimates were used to estimate the fair value of these awards, as they are not publicly traded. RSL common share awards are subject to specified vesting schedules and requirements (a mix of time-based, performance-based and corporate event-based, including targets for RSL's post-IPO market capitalization and future financing events). The Company estimated the fair value of each RSL option on the date of grant using the Black-Scholes closed-form option-pricing model.

The Company recorded share-based compensation expense of \$2.6 million and \$33.6 million, respectively, for the three months ended December 31, 2016 and 2015 and \$8.1 million and \$49.5 million, respectively, for the nine months ended December 31, 2016 and 2015, in relation to the RSL common share awards and options issued by RSL to RSI employees.

Table of Contents

(3) Share-Based Compensation for Family Members:

The Company granted Geetha Ramaswamy, Shankar Ramaswamy and Sarah Friedhoff options to purchase 43,000 common shares, 43,000 common shares and 10,000 common shares, respectively, during the nine months ended December 31, 2016 as annual stock option grants in their capacities as employees of ASI. The Company recorded aggregate share-based compensation expense of \$0.9 million and \$0.9 million for the three months ended December 31, 2016 and 2015, respectively, and \$2.7 million and \$2.6 million for the nine months ended December 31, 2016 and 2015, respectively, in connection with these option grants.

Shankar Ramaswamy, while previously employed by RSI, was also granted restricted stock in BVC. Following the BVC Merger, this restricted stock in BVC was converted into RSL common shares, subject to vesting and forfeiture terms consistent with the original grant. (Refer to Note 6(B)(2)). The Company recorded share-based compensation expense of \$0.1 million and \$0.2 million, respectively, for the three months ended December 31, 2016 and 2015 and \$0.4 million and \$0.4 million, respectively, for the nine months ended December 31, 2016 and 2015 related to the RSL common share awards held by Shankar Ramaswamy (inclusive of the compensation expense noted above for BVC awards prior to the BVC Merger on December 4, 2015), which the Company has recorded as research and development expense in the accompanying condensed consolidated statements of operations and comprehensive loss. At December 31, 2016, total unrecognized compensation expense related to these non-vested RSL common share awards was \$0.7 million and is expected to be recognized over the remaining weighted-average service period of 1.52 years.

Note 7—Income Taxes

The Company's provision for income taxes is based on income taxes in the United States, Switzerland and the United Kingdom. The effective income tax rate for the Company for the three months ended December 31, 2016 and 2015 was 0.3% and (1.3)%, respectively. The effective tax rate for the Company for the nine months ended December 31, 2016 and 2015 was (0.6)% and (0.9)%, respectively, primarily due to the organization of the Company as a Bermuda Exempted Limited Company, for which there is no current tax regime, due to the U.S. permanent unfavorable tax differences, and a valuation allowance that effectively eliminates the Company's net deferred tax assets in the United States.

The Company assesses the realizability of its deferred tax assets at each balance sheet date based on available positive and negative evidence in order to determine the proper amount, if any, required for a valuation allowance. As a result of this assessment, a valuation allowance of \$16.2 million and \$6.9 million primarily related to share-based compensation has been recorded as of December 31, 2016 and March 31, 2016, respectively. The Company believes that it is more likely than not, given the weight of available evidence, that all other deferred tax assets will be realized.

As of December 31, 2016 and March 31, 2016, the Company had an unrecognized tax benefit of \$0.3 million and \$0.3 million, respectively, which if recognized would be reflected as an income tax benefit.

The Company files income tax returns in the United States for federal, state and local jurisdictions. The Company is subject to tax examinations for fiscal year 2015 and forward in all applicable tax jurisdictions.

Note 8—Commitments and Contingencies

The Company has entered into commitments under an asset purchase agreement with GlaxoSmithKline ("GSK"), a development, marketing, and supply agreement with Arena Pharmaceuticals, GmbH ("Arena"), amended its services agreement with RSI and entered into a separate service agreement with RSG (Refer to Note 5(A)) and a license agreement with Qaam Pharmaceuticals LLC (Refer to Note 4). Under the GSK agreement, the Company made a \$5.0

million milestone payment in June 2016, which had been recorded as a contingent payment liability as of March 31, 2016 in the accompanying condensed consolidated balance sheet. In addition, the Company has entered into services agreements with third parties for pharmaceutical research and manufacturing activities. The manufacturing agreements can be terminated by the Company with 30 days written notice. The Company expects to enter into other commitments as its business further develops.

Table of Contents

Note 9—Subsequent Events

On February 2, 2017, the Company and its subsidiaries, AHL, ASG and ASI entered into a Loan and Security Agreement (the “Loan Agreement”) with Hercules Capital, Inc., (“Hercules”), under which the Company, AHL and ASG (the “Borrowers”) borrowed an aggregate of \$55.0 million (the “Term Loan”). ASI has issued a guaranty of the Borrowers’ obligations under the Loan Agreement. At the closing of the Term Loan, the Borrowers paid Hercules a facility charge of \$550,000. The Term Loan bears interest at a variable per annum rate calculated for any day as the greater of either (i) the prime rate plus 6.80%, and (ii) 10.55%. Debt issuance fees paid to Hercules will be recorded as a discount on the debt and will be amortized to interest expense using the effective interest method over the life of the Loan Agreement. The Term Loan has a scheduled maturity date of March 1, 2021. The Borrowers are obligated to make monthly payments of accrued interest under the Loan Agreement until September 1, 2018, followed by monthly installments of principal and interest through March 1, 2021. The interest-only period may be extended until March 1, 2019 if the Company achieves certain clinical development milestones as set forth in the Loan Agreement. In connection with the Loan Agreement, the Borrowers and ASI, as guarantor, granted Hercules a first position lien on substantially all of their respective assets, excluding intellectual property. Prepayment of the Term Loan is subject to penalty.

In connection with the Loan Agreement, the Company issued a warrant to Hercules, exercisable for an aggregate of 274,086 of the Company’s common shares at an exercise price of \$12.04 per share (the “Warrant”). The Warrant may be exercised on a cashless basis, and is immediately exercisable through the earlier of (i) February 2, 2024 and (ii) the consummation of certain acquisition transactions involving the Company as set forth in the Warrant. The number of shares for which the Warrant is exercisable and the associated exercise price are subject to certain proportional adjustments as set forth in the Warrant.

The Loan Agreement includes customary affirmative and restrictive covenants and representations and warranties, including a minimum cash covenant that applies commencing on July 1, 2017 and ceases to apply if the Company achieves certain clinical development milestones, as set forth in the Loan Agreement, a covenant against the occurrence of a “change in control,” financial reporting obligations, and certain limitations on indebtedness, liens (including a negative pledge on intellectual property and other assets), investments, distributions (including dividends), collateral, transfers, mergers or acquisitions, taxes, corporate changes, and deposit accounts. The Loan Agreement also includes customary events of default, including payment defaults, breaches of covenants following any applicable cure period, the occurrence of certain events that could reasonably be expected to have a “material adverse effect” as set forth in the Loan Agreement, cross acceleration to the debt and certain events relating to bankruptcy or insolvency. Upon the occurrence of an event of default, a default interest rate of an additional 5.0% may be applied to the outstanding principal balance, and Hercules may declare all outstanding obligations immediately due and payable and take such other actions as set forth in the Loan Agreement.

In addition, for so long as the Term Loan remains outstanding, the Company shall be required to use its commercially reasonable efforts to afford Hercules the opportunity to participate in future underwritten equity offerings of the Company’s common shares up to a specified amount.

Table of Contents

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following discussion and analysis of our financial condition, results of operations and cash flows should be read in conjunction with (1) the unaudited interim condensed consolidated financial statements and the related notes thereto included elsewhere in this Quarterly Report on Form 10-Q, and (2) the audited consolidated financial statements and notes thereto and management's discussion and analysis of financial condition and results of operations for the fiscal year ended March 31, 2016 included in our Annual Report on Form 10-K, filed with the Securities and Exchange Commission, or the SEC, on June 6, 2016.

This Quarterly Report on Form 10-Q contains "forward-looking statements" within the meaning of Section 27A of the Securities Act and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. These statements are often identified by the use of words such as "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "project," "will," "would" or the negative or plural of these words or similar expressions or variations. Such forward-looking statements are subject to a number of risks, uncertainties, assumptions and other factors that could cause actual results and the timing of certain events to differ materially from future results expressed or implied by the forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified herein, and those discussed in the section titled "Risk Factors," set forth in Part II, Item 1A of this Quarterly Report on Form 10-Q and in our other filings with the SEC. You should not rely upon forward-looking statements as predictions of future events. Furthermore, such forward-looking statements speak only as of the date of this report. Except as required by law, we undertake no obligation to update any forward-looking statements to reflect events or circumstances after the date of such statements.

Overview

We are a clinical-stage biopharmaceutical company focused on acquiring, developing and commercializing novel therapeutics for the treatment of dementia. We intend to develop a pipeline of product candidates to comprehensively address the cognitive, functional and behavioral aspects of dementia and related neurological disorders. Our vision is to become the leading company focused on the treatment of dementia by addressing all forms and aspects of this condition.

Our near-term focus is to develop our lead product candidate, intepirdine, previously referred to as RVT-101, a selective 5-HT₆ receptor antagonist, for the treatment of Alzheimer's disease and dementia with Lewy bodies, or DLB, and to develop nelotanserin, our second product candidate, a potent and highly selective 5-HT_{2A} receptor inverse agonist, for the treatment of REM behavior disorder, or RBD, in DLB patients, and visual hallucinations in patients with Lewy body dementia or LBD. In addition, we have the rights to develop RVT-103, a combination of donepezil and a peripheral muscarinic receptor antagonist, and RVT -104, a combination of rivastigmine and a peripheral muscarinic receptor antagonist, and we intend to develop these product candidates alone and in combination with intepirdine as potential treatments for patients with Alzheimer's disease or LBD.

We were founded in October 2014 as a wholly-owned subsidiary of Roivant Sciences Ltd., or RSL, a company focused on the acquisition, development and commercialization of late-stage product candidates that are non-strategic, deprioritized or under-resourced at other biopharmaceutical companies, with the intent of reducing the time and cost of the drug development process. We have three subsidiaries. Axovant Holdings Limited, or AHL, a direct wholly-owned subsidiary of Axovant Sciences Ltd., was incorporated in England and Wales in August 2016; Axovant Sciences, Inc., or ASI, a direct wholly-owned subsidiary of AHL, was incorporated in Delaware in February 2015 and Axovant Sciences GmbH, or ASG, a direct wholly-owned subsidiary of AHL, was organized in Switzerland in August 2016. ASG holds our intellectual property rights and will be the principal operating company for conducting our business. We formed these additional entities in anticipation of conducting further research and development and global expansion during the commercialization phase, including building out the functions, personnel and facilities necessary for the commercialization of our drug candidates, if approved. Our operations to date have been limited to organizing and staffing our company, raising capital, acquiring our product candidates and preparing for and advancing our product candidates, intepirdine, nelotanserin, RVT-103 and RVT-104, as potential treatments for patients with Alzheimer's disease and LBD, into clinical development.

In June 2015, we completed our initial public offering, or IPO, from which we raised proceeds of \$334.5 million, net of underwriting discounts and issuance costs. We intend to use these proceeds to fund our planned clinical development programs. To date, we have not generated any revenue and we recorded net losses of \$47.8 million and \$63.4 million for the three months ended December 31, 2016 and 2015, respectively, and net losses of \$128.1 million and \$103.5 million for the nine months ended December 31, 2016 and 2015, respectively. We have determined that we have one operating and reporting segment.

Table of Contents

Our Product Pipeline:

The following table summarizes the status of our development programs:

Compound	Clinical Indication	Development Stage	Entity Holding Global Commercial Rights
Intepirdine			
	Mild-to-Moderate Alzheimer's disease	Phase 3 (MINDSET Study)	Axovant Sciences GmbH
	Dementia with Lewy Bodies (DLB)	Phase 2b (HEADWAY-DLB Study)	Axovant Sciences GmbH
	Gait and Balance in Alzheimer's disease, DLB and Parkinson's disease dementia	Phase 2	Axovant Sciences GmbH
Nelotanserin			
	Visual Hallucinations in LBD	Phase 2	Axovant Sciences GmbH
	REM Behavior Disorder (RBD) in DLB	Phase 2	Axovant Sciences GmbH
RVT-103	Alzheimer's disease	Proof of Concept Study	Axovant Sciences GmbH
RVT-104	Alzheimer's disease and DLB	Preparation for Proof of Concept Study	Axovant Sciences GmbH

Intepirdine

Overview

Our lead product candidate intepirdine is currently being developed for the treatment of mild-to-moderate Alzheimer's disease and DLB. We acquired the worldwide rights to intepirdine from Glaxo Group Limited and GlaxoSmithKline Intellectual Property Development Limited, collectively GSK, under an asset purchase agreement entered into in December 2014, or the GSK Agreement.

Intepirdine for the Treatment of Alzheimer's Disease

Alzheimer's disease, the most common form of dementia, is a progressive neurodegenerative disorder that results in significant impairments in cognition, function and behavior. According to the Alzheimer's Association, Alzheimer's disease affects approximately 5.3 million people in the United States. It is estimated that between 70% and 90% of Alzheimer's disease patients age 65 and older are classified as having mild-to-moderate Alzheimer's disease. No new chemical entity has been approved by the FDA for the treatment of Alzheimer's disease since 2003.

In October 2015, we commenced a global, multi-center, double-blind, placebo-controlled confirmatory Phase 3 clinical study of intepirdine, which we refer to as the MINDSET study, for the treatment of patients with mild-to-moderate Alzheimer's disease. The MINDSET study is evaluating the safety, tolerability and efficacy of intepirdine over a 24-week period and compares 35 mg, once-daily oral doses of intepirdine to placebo in approximately 1,150 patients with mild-to-moderate Alzheimer's disease on a background of stable donepezil therapy. The primary endpoints of the study are improvements in scores on the Alzheimer's Disease Assessment Scale-cognitive subscale, or ADAS-cog, and the Alzheimer's Disease Cooperative Study - Activities of Daily Living scale, or ADCS-ADL, which have been used as endpoints supporting regulatory approval of currently-marketed Alzheimer's disease treatments in the United States and Europe. Subjects completing the MINDSET study will be eligible to enroll in a 12-month, open-label extension in which other medications for the treatment of Alzheimer's disease, including memantine and other cholinesterase inhibitors, may be administered in combination with intepirdine. We have received a Special Protocol Assessment, or SPA, agreement from the FDA which states that the design and planned analysis of the MINDSET study adequately address the objectives necessary to support an application for marketing approval. The MINDSET study seeks to confirm results of a prior 684-subject Phase 2b adjunctive therapy study conducted by GSK. We have completed recruitment of patients for the MINDSET study and expect to report results in the third quarter of calendar year 2017. If the results of the MINDSET study are favorable, we plan to seek regulatory approval and commercialize intepirdine.

Table of Contents

Intepirdine for the Treatment of Dementia with Lewy Bodies

In addition to evaluating intepirdine in patients with mild-to-moderate Alzheimer's disease, we are also developing intepirdine to address other forms of dementia, such as dementia with Lewy bodies, or DLB. DLB, a subset of LBD, is a progressive neurodegenerative disorder pathologically characterized by the aggregation of alpha-synuclein and other proteins in the brain, known as Lewy bodies, causing disruption in cognition, function and behavior. DLB is the second most prevalent cause of neurodegenerative dementia in elderly patients. We believe that DLB affects approximately 1.1 million people in the United States. In addition to suffering from deficits and fluctuations in cognition, DLB patients often suffer from visual hallucinations, parkinsonism, sensitivity to neuroleptic (antipsychotic) medications and REM behavior disorder, or RBD, a condition in which patients physically act out their dreams.

In the first quarter of calendar year 2016, we began a Phase 2b clinical trial of intepirdine, called the HEADWAY-DLB study, in patients with DLB. In addition to the 35 mg dose of intepirdine that is being studied in the MINDSET study, we will evaluate a 70 mg dose of intepirdine in this trial, which we believe could have greater activity against the 5-HT_{2A} receptor to potentially address visual hallucinations and behavioral disturbances in this patient population. This decision is supported by a safety and food-effect study testing the 70 mg dose that we completed in 2015. In September 2016, we received Fast Track designation from the FDA for intepirdine for the treatment of DLB. We expect to report results from the HEADWAY-DLB trial in the fourth quarter of calendar year 2017. If the results of the HEADWAY-DLB study are favorable, we believe that it, in combination with data from our studies in Alzheimer's disease, could serve as the basis for seeking approval of intepirdine for DLB.

Intepirdine for Gait and Balance in Alzheimer's Disease, Dementia with Lewy Bodies and Parkinson's Disease Dementia

In addition to evaluating intepirdine in patients with mild-to-moderate Alzheimer's disease and DLB, we are also evaluating the effects of intepirdine on gait and balance in patients with Alzheimer's disease, DLB and Parkinson's disease dementia. In addition to cognitive deficits, these patients often present with a history of defined gait impairment. In September 2016, we initiated a double-blind, randomized, placebo-controlled Phase 2 crossover study of intepirdine to evaluate its effects on gait and balance in patients with Alzheimer's disease, DLB and Parkinson's disease dementia. We intend to enroll approximately 40 patients in this Phase 2 study and will seek to further explore the reduced rate of falls observed with intepirdine treatment in the prior 684-patient Phase 2b study in mild-to-moderate Alzheimer's disease in patients on background of stable donepezil therapy. We expect to report results from the Phase 2 gait and balance study in calendar year 2017.

Nelotanserin

Overview

In October 2015, we acquired from our parent company RSL the global rights to nelotanserin, a potent and highly selective inverse agonist of the 5-HT_{2A} receptor which has the effect of reducing the activity of the 5-HT_{2A} receptor. The 5-HT_{2A} receptor has been linked to neuropsychiatric disturbances including visual hallucinations and sleep disturbances. Initially, we intend to investigate and develop nelotanserin to address visual hallucinations in patients with LBD and RBD in patients with DLB. Nelotanserin has been evaluated in eight clinical studies to date with over 800 human subjects exposed to the drug candidate and has been observed to be well tolerated, with no drug-related serious adverse events reported.

Nelotanserin for Visual Hallucinations in Lewy Body Dementia

LBD includes two similar conditions, DLB and Parkinson's disease dementia, or PDD. There is significant overlap in the pathology and clinical presentation of both conditions; however, the primary difference generally depends on the timing of the onset of cognitive decline relative to the onset of movement-related symptoms. In DLB, the cognitive decline typically occurs before or within one year of the onset of movement disorder symptoms. In PDD, movement disorder symptoms typically precede cognitive decline by more than one year. The Lewy Body Dementia Association estimates that there are 1.4 million patients with LBD in the United States.

LBD patients suffer from frequent visual hallucinations, which are often treated with off-label atypical antipsychotic medications such as quetiapine. Use of atypical antipsychotic medications, which have activity against the dopamine D₂ receptor, can lead to increased or possibly irreversible parkinsonism in LBD patients and a life threatening

side-effect resembling neuroleptic malignant syndrome. We believe that there is a need for new therapeutic options that can reduce visual hallucinations in LBD patients without risk of these severe side effects.

Table of Contents

In January 2016, we initiated a double-blind, randomized, placebo-controlled, cross-over Phase 2 clinical study of nelotanserin in approximately 20 DLB and PDD patients suffering from visual hallucinations. In February 2017, we reported preliminary results from a planned interim analysis of the first 11 patients to complete this pilot study. Among these patients, we observed statistically significant improvements in the pre-specified primary endpoint of Unified Parkinson's Disease Rating Scale (UPDRS) Parts II+III during periods in which patients received nelotanserin, as compared to periods during which those same patients received placebo. Safety analysis included measurement of the incidence of adverse events. There were no drug-related serious adverse events, and there were no adverse events that led to discontinuation of the study drug. In the interim analysis, secondary endpoints did not demonstrate statistically significant differences for nelotanserin relative to placebo. Based on these preliminary results, Axovant plans to expand patient recruitment to confirm the treatment benefits observed in the interim results from this ongoing study. We expect the current study to complete in mid-2017.

Nelotanserin for REM Behavior Disorder (RBD) in Dementia with Lewy Bodies (DLB)

RBD is a common clinical feature of DLB, and is a condition in which patients physically act out their dreams, impacting their quality of life and endangering themselves and their bed partners. While off-label treatment of RBD with benzodiazepines is common, this class of drugs is associated with concerning side effects in patients with dementia, including sedation, worsening of cognition and increased risk of falls. We believe that there is a need for new therapeutic options that can reduce the frequency of RBD without sedating patients or worsening cognition in patients with dementia.

In March 2016, we initiated a double-blind, randomized, placebo-controlled Phase 2 study in patients with DLB suffering from RBD. This study will utilize objective measures of efficacy as assessed in a sleep-lab setting. We expect to receive results from this study in the second half of calendar year 2017.

RVT-103 and RVT-104

Overview

In August 2016, we and Qaam Pharmaceuticals LLC, or Qaam, entered into an exclusive license agreement under which we expect to develop and, if successful, commercialize products that combine cholinesterase inhibitors with peripherally acting quaternary amine muscarinic receptor antagonists such as glycopyrrolate. These combinations could provide a means to mitigate the known peripheral side effects of cholinesterase inhibitors and may also allow higher than currently approved doses of cholinesterase inhibitors such as rivastigmine, which may improve treatment of symptoms of neurodegenerative disorders such as Alzheimer's disease and LBD.

RVT-103 and RVT-104 in Alzheimer's disease and Lewy Body Dementia

We will initially develop RVT-103, a combination of a peripheral muscarinic receptor antagonist and donepezil, as a potential treatment for patients with Alzheimer's disease with the ultimate goal of creating a triple combination of intepirdine, a peripheral muscarinic receptor antagonist and donepezil. We are conducting a proof of concept clinical study in which subjects are randomized to treatment with donepezil and either placebo or a peripheral muscarinic receptor antagonist. We expect to report the initial results from this proof of concept study in the first half of calendar year 2017. We also expect to develop RVT-104, a combination of a peripheral muscarinic receptor antagonist and high-dose rivastigmine, as a potential treatment for patients with Alzheimer's disease and LBD.

Our Key Agreements

Asset Purchase Agreement with GlaxoSmithKline for Intepirdine

Under the GSK Agreement, we made an upfront payment of \$5.0 million and an additional \$5.0 million payment in June 2016, which was previously recorded as a contingent payment liability. We are also obligated to pay GSK \$35.0 million, \$25.0 million and \$10.0 million upon the receipt of marketing approval of intepirdine in the United States, the European Union and Japan, respectively, as well as an additional one-time payment of \$85.0 million for the first calendar year in which we achieve global net sales of \$1.2 billion for intepirdine.

Under the GSK Agreement we are also obligated to pay a fixed 12.5% royalty based on net sales of intepirdine, subject to reduction on account of expiration of patent and regulatory exclusivity or upon generic entry.

Table of Contents

Arena Development Agreement for Nelotanserin

In October 2015, we exercised an option to acquire global rights to nelotanserin from our parent company, RSL. In May 2015, RSL entered into a development, marketing and supply agreement for nelotanserin with Arena Pharmaceuticals GmbH, or Arena, and we entered into a Waiver and Option Agreement with RSL. Upon the exercise of our option, we assumed RSL's rights and obligations under the development, marketing and supply agreement with Arena, or the Arena Development Agreement. Under the Waiver and Option Agreement, we recorded \$5.3 million as research and development expense which was 110% of the payments made to Arena by RSL, and the costs incurred by RSL in connection with the development of nelotanserin. We will be responsible for future contingent payments under the Arena Development Agreement, including up to \$4.0 million in potential development milestone payments, up to \$37.5 million in potential regulatory milestone payments and up to \$60.0 million in potential commercial milestone payments. Under the Arena Development Agreement, we are also obligated to purchase finished drug product at a fixed price equal to 15% of net sales of nelotanserin.

Recent Developments

Services Agreements with Roivant Sciences, Inc. and Roivant Sciences GmbH

We and our wholly-owned subsidiary, ASI, have entered into a services agreement with Roivant Sciences Inc., or RSI, a wholly-owned subsidiary of RSL, pursuant to which RSI provides us with services in relation to the identification of potential product candidates and project management of clinical trials, as well as other services related to our development administrative and financial activities. We and ASI amended and restated our services agreement with RSI on October 13, 2015 for the fiscal year commencing April 1, 2015. In February 2017, in connection with the contribution and assignment of all of our intellectual property rights to ASG, we amended and restated this services agreement effective as of December 13, 2016, as a result of which ASG was added as a recipient of these services from RSI. In February 2017, ASG also entered into a separate services agreement with Roivant Sciences GmbH or RSG, a wholly-owned subsidiary of RSL, effective as of December 13, 2016, for the provision of services by RSG to ASG in relation to the identification of potential product candidates and project management of clinical trials, as well as other services related to development, administrative and financial activities. Under the terms of both services agreements (collectively the "Services Agreements"), we are obligated to pay or reimburse RSI and RSG for the costs they, or third parties acting on their behalf, incur in providing services to us, including administrative and support services as well as research and development services. In addition, we are obligated to pay to RSI and RSG a pre-determined mark-up on the costs incurred directly by RSI and RSG in connection with any general and administrative and support services as well as research and development services.

Under the services agreement in effect as of December 31, 2016, we incurred expenses of \$1.9 million and \$2.1 million for the three months ended December 31, 2016 and 2015, respectively, and \$5.3 million and \$5.4 million for the nine months ended December 31, 2016 and 2015, respectively, inclusive of the mark-up. We have recorded these charges as research and development expense and general and administrative expense in our condensed consolidated statement of operations.

Venture Debt Financing from Hercules Capital

On February 2, 2017, we, AHL, ASG and ASI entered into a Loan and Security Agreement, or the Loan Agreement, with Hercules Capital, Inc., or Hercules, under which we, AHL and ASG, or the Borrowers, borrowed an aggregate of \$55.0 million, or the Term Loan. ASI has issued a guaranty of the Borrowers' obligations under the Loan Agreement. At the closing of the Term Loan, the Borrowers paid Hercules a facility charge of \$550,000. The Term Loan bears interest at a variable per annum rate calculated for any day as the greater of either (i) the prime rate plus 6.80%, and (ii) 10.55%. Debt issuance fees paid to Hercules will be recorded as a discount on the debt and will be amortized to interest expense using the effective interest method over the life of the Loan Agreement. The Term Loan has a scheduled maturity date of March 1, 2021, or the Scheduled Maturity Date. The Borrowers are obligated to make monthly payments of accrued interest under the Loan Agreement until September 1, 2018, followed by monthly installments of principal and interest through the Scheduled Maturity Date. The interest-only period may be extended until March 1, 2019 if we achieve certain clinical development milestones as set forth in the Loan Agreement. In

connection with the Loan Agreement, the Borrowers and ASI, as guarantor, granted Hercules a first position lien on substantially all of their respective assets, excluding intellectual property. Prepayment of the Term Loan is subject to penalty.

Table of Contents

In connection with the entry into the Loan Agreement, we issued a warrant to Hercules, or the Warrant, which is exercisable for an aggregate of 274,086 of our common shares at an exercise price of \$12.04 per share. The Warrant may be exercised on a cashless basis, and is immediately exercisable through the earlier of (i) February 2, 2024 and (ii) the consummation of certain acquisition transactions involving us as set forth in the Warrant. The number of shares for which the Warrant is exercisable and the associated exercise price are subject to certain proportional adjustments as set forth in the Warrant.

The Loan Agreement includes customary affirmative and restrictive covenants and representations and warranties, including a minimum cash covenant that applies commencing on July 1, 2017 and ceases to apply if we achieve certain clinical development milestones as set forth in the Loan Agreement, a covenant against the occurrence of a “change in control,” financial reporting obligations, and certain limitations on indebtedness, liens (including a negative pledge on intellectual property and other assets), investments, distributions (including dividends), collateral, transfers, mergers or acquisitions, taxes, corporate changes, and deposit accounts. The Loan Agreement also includes customary events of default, including payment defaults, breaches of covenants following any applicable cure period, the occurrence of certain events that could reasonably be expected to have a “material adverse effect” as set forth in the Loan Agreement, cross acceleration to the debt and certain events relating to bankruptcy or insolvency. Upon the occurrence of an event of default, a default interest rate of an additional 5.0% may be applied to the outstanding principal balance, and Hercules may declare all outstanding obligations immediately due and payable and take such other actions as set forth in the Loan Agreement.

In addition, for so long as the Term Loan remains outstanding, we are required to use commercially reasonable efforts to afford Hercules the opportunity to participate in future underwritten equity offerings of our common shares up to a specified amount.

Financial Operations Overview

Revenue

We have not generated any revenue from the sale of any products, and we do not expect to generate any revenue unless and until we obtain regulatory approval of and begin to commercialize one of our product candidates in development.

Research and Development Expense

Since our inception, our operations have primarily been focused on organizing and staffing our company, raising capital, acquiring our product candidates and preparing for and advancing our product candidates, intepirdine, nelotanserin, RVT-103 and RVT-104, into clinical development. Our research and development expenses include: employee-related expenses, such as salaries, share-based compensation, benefits and travel expense for research and development personnel;

- costs allocated to us under the Services Agreements;

- expenses incurred under or in connection with agreements with contract research organizations, or CROs, as well as consultants who assist in the development of our product candidates;

- manufacturing costs in connection with producing materials for use in conducting nonclinical and clinical studies; costs for planning and developing clinical studies for Alzheimer’s disease and other forms of dementia including evaluating intepirdine for patients with DLB and evaluating intepirdine for gait and balance in patients with Alzheimer's disease, DLB and PDD;

- costs for planning and developing clinical studies for nelotanserin for patients with LBD;

-

costs for planning and developing clinical studies for product candidates that combine cholinesterase inhibitors with peripheral muscarinic receptor antagonists including RVT-103, a combination of a peripheral muscarinic receptor antagonist, donepezil and possibly intepirdine, and RVT-104, a combination of a peripheral muscarinic receptor antagonist and high-dose rivastigmine and possibly intepirdine;

• milestone payments and other costs that we incur under the GSK Agreement, the Arena Development Agreement, and our license agreement with Qaam;

• costs for sponsored research; and

Table of Contents

depreciation expense for assets used in research and development activities.

Research and development activities will continue to be central to our business model. We expect our research and development expense to increase significantly primarily as a result of our ongoing Phase 3 MINDSET study for intepirdine, our ongoing intepirdine HEADWAY-DLB study in patients with DLB and our ongoing development program for nelotanserin in LBD. We expect our share-based compensation expense attributable to RSL common share awards to become less variable because of the December 2015 merger of BVC Ltd., or BVC, with and into RSL, a transaction we refer to in this report as the BVC Merger. Refer to Note 5, "Related Party Transactions", in the accompanying notes to the consolidated financial statements included in this Quarterly Report on Form 10-Q. Product candidates in later stages of clinical development, such as intepirdine and nelotanserin, generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials.

The duration, costs and timing of clinical trials of our products in development and any other product candidates will depend on a variety of factors that include, but are not limited to:

- the number of trials required for approval;
- the per patient trial costs;
- the number of patients who participate in the trials;
- the number of sites included in the trials;
- the countries in which the trials are conducted;
- the length of time required to enroll eligible patients;
- the number of doses that patients receive;
- the drop-out or discontinuation rates of patients;
- the potential additional safety monitoring or other studies requested by regulatory agencies;
- the duration of patient follow-up;
- the timing and receipt of regulatory approvals; and
- the efficacy and safety profile of the product candidates.

In addition, the probability of success of our products in development and any other product candidate will depend on numerous factors, including competition, manufacturing capability and commercial viability. We may never succeed in achieving regulatory approval of our product candidates for any indication in any country. As a result of the uncertainties discussed above, we are unable to determine the duration and completion costs of any clinical trial we conduct, or when and to what extent we will generate revenue from the commercialization and sale of our products in development or other product candidates, if at all.

General and Administrative Expense

General and administrative expenses consist primarily of share-based compensation, legal and accounting fees, consulting services, services received under the Services Agreements and employee salaries and related benefits for general and administrative personnel.

We anticipate that our general and administrative expenses will increase in the future to support our growth and our operations as a public company. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants, lawyers and accountants, among other expenses. We also expect to incur additional expenses associated with maintaining compliance with NYSE rules and SEC requirements, insurance, and investor relations costs. In addition, we expect to incur expenses associated with building a sales, commercial and marketing team before our products in development obtain regulatory approval for marketing. We also expect our share-based compensation expense attributable to RSL common share awards to become less variable because of the BVC Merger.

Table of Contents

Results of Operations for the Three and Nine Months Ended December 31, 2016 and 2015

The following table summarizes our results of operations for the three and nine months ended December 31, 2016 and 2015 (in thousands):

	Three Months Ended			Nine Months Ended		
	December 31, 2016	2015	Change	December 31, 2016	2015	Change
Operating expenses:						
Research and development expenses (includes share-based compensation expense of \$4,592 and \$14,599 for the three months ended December 31, 2016 and 2015, and \$14,029 and \$24,421 for the nine months ended December 31, 2016 and 2015, respectively)	\$36,630	\$34,324	\$2,306	\$93,980	\$53,209	\$40,771
General and administrative expenses (includes share-based compensation expense of \$3,739 and \$24,457 for the three months ended December 31, 2016 and 2015, and \$13,800 and \$39,537 for the nine months ended December 31, 2016 and 2015, respectively)	11,342	28,230	(16,888)	33,422	49,364	(15,942)
Total operating expenses	\$47,972	\$62,554	\$(14,582)	\$127,402	\$102,573	\$24,829

Research and Development Expenses

Research and development expenses increased by \$2.3 million, to \$36.6 million, in the three months ended December 31, 2016 compared to the three months ended December 31, 2015, primarily due to increases in expenses for the ongoing MINDSET study. Research and development expenses for the three months ended December 31, 2016 consisted primarily of CRO fees of \$21.2 million, share-based compensation expense of \$4.6 million and CMO fees of \$2.3 million, as well as employee salaries and benefits and payments made to consultants and other third-party vendors engaged in the development of our product candidates. The share-based compensation expense for the three months ended December 31, 2016 included \$2.1 million related to the RSL common share awards and RSL options issued by RSL to RSI employees.

Research and development expenses were \$34.3 million for the three months ended December 31, 2015, and consisted primarily of share-based compensation of \$14.6 million, CRO fees of \$8.0 million and expenses of \$5.3 million for the acquisition of nelotanserin, as well as CMO fees, employee salaries and benefits and payments made to consultants and other third party vendors engaged in the development of our product candidates. The share-based compensation expense included \$11.4 million related to the RSL common share awards issued by RSL to RSI employees.

Research and development expenses increased by \$40.8 million, to \$94.0 million, in the nine months ended December 31, 2016 compared to the nine months ended December 31, 2015, primarily due to increases in expenses for the ongoing MINDSET study. Research and development expenses for the nine months ended December 31, 2016 consisted primarily of CRO fees of \$52.4 million, share-based compensation expense of \$14.0 million and CMO fees of \$6.4 million as well as employee salaries and benefits and payments made to consultants and other third party vendors engaged in the development of our product candidates. The share-based compensation expense included \$6.8 million related to the RSL common share awards and RSL options issued by RSL to RSI employees.

Research and development expenses were \$53.2 million for the nine months ended December 31, 2015, and consisted primarily of share-based compensation of \$24.4 million, CRO fees of \$10.8 million and expenses of \$5.3 million for the acquisition of nelotanserin as well as employee salaries and benefits, payments to CMOs, consultants and other

third party vendors engaged in the development of our product candidates. The share-based compensation expense included \$15.9 million for RSL common share awards issued to RSI employees. These compensation amounts include share-based compensation expense for BVC awards issued to RSI employees as described below.

Table of Contents

On December 4, 2015, BVC, a non-public entity, which held a non-controlling ownership interest in RSL, our parent company, was merged with and into RSL (“BVC Merger”), with RSL as the surviving entity. The compensation amounts for the three and nine months ended December 31, 2016 includes \$2.1 million and \$6.8 million, respectively, for share-based compensation expense for BVC awards issued to RSI employees prior to the BVC Merger. Prior to the BVC Merger, we recorded share-based compensation expense in relation to the share-based awards issued by BVC to RSI employees based on the changes in fair value of BVC share-based awards. As these BVC share based awards were not based on our or RSL’s shares, they were remeasured at each reporting period date until performance was completed.

As a result of the BVC Merger, all outstanding BVC share-based awards were converted into RSL common share awards, with the same vesting and forfeiture terms as the original grant. The RSL common share awards are fair valued on the date of grant and that fair value is recognized over the requisite service period. At the time of the BVC Merger on December 4, 2015, the unvested BVC awards that were converted into common shares of RSL were remeasured at the estimated fair value of RSL and that fair value is being recognized over the remaining requisite service period.

General and Administrative Expenses

General and administrative expenses decreased by \$16.9 million, to \$11.3 million, in the three months ended December 31, 2016 compared to the three months ended December 31, 2015, primarily due to a \$20.7 million decrease in share-based compensation expense, partially offset by higher employee salaries and benefits resulting from increased headcount. General and administrative expenses for the three months ended December 31, 2016 consisted primarily of share-based compensation expense of \$3.7 million and employee salaries and related benefits, legal and professional fees, and direct and indirect costs allocated to us under the Services Agreement. The share-based compensation expense for the three months ended December 31, 2016 included share-based compensation expense of \$0.5 million for RSL common share awards issued to RSI employees. These compensation amounts include share-based compensation expense for BVC awards issued to RSI employees prior to the BVC Merger. Prior to the BVC Merger we recorded share-based compensation expense in relation to the share-based awards issued by BVC to RSI employees based on the changes in fair value of share-based awards which were remeasured at each reporting period date until performance was completed as described above.

General and administrative expenses were \$28.2 million for the three months ended December 31, 2015, and consisted primarily of share-based compensation expense of \$24.5 million. The remainder consisted of employee salaries and related benefits, legal and professional fees, and direct and indirect costs allocated to us under the Services Agreement. The share-based compensation expense for the three months ended December 31, 2015 includes \$22.2 million for RSL common share awards issued to RSI employees. These compensation amounts include share-based compensation for BVC awards issued to RSI employees prior to the BVC merger. Prior to the BVC merger, we recorded share-based expense in relation to the share-based awards issued by BVC to RSI employees based on the changes in fair value of share-based awards which were remeasured at each reporting period date until performance was completed as described above.

General and administrative expenses decreased by \$15.9 million, to \$33.4 million, in the nine months ended December 31, 2016 compared to the nine months ended December 31, 2015, primarily due to a \$25.7 million decrease in share-based compensation expense, partially offset by higher employee salaries and benefits resulting from increased headcount. General and administrative expenses for the nine months ended December 31, 2016 consisted primarily of share-based compensation expense of \$13.8 million and employee salaries and related benefits, legal and professional fees and direct and indirect costs allocated to us under the Services Agreement. The share-based compensation includes \$1.3 million for RSL common share awards issued to RSI employees.

General and administrative expenses were \$49.4 million for the nine months ended December 31, 2015, and consisted primarily of share-based compensation expense of \$39.5 million. The remainder consisted of employee salaries and

related benefits, legal and professional fees, and direct and indirect costs allocated to us under the Services Agreement. The share-based compensation expense for the nine months ended December 31, 2015 includes \$33.5 million for RSL common share awards issued to RSI employees. These compensation amounts include share-based compensation expense for BVC awards issued to RSI employees as described above.

Contractual Obligations

Our long-term contractual obligations include commitments and estimated purchase obligations entered into in the normal course of business. We have entered into commitments under the GSK Agreement, the Arena Development Agreement, the license agreement with Qaam and the Services Agreements with RSI and RSG and subleases with RSI for office space. In addition, we have entered into various services agreements with third parties for pharmaceutical manufacturing and research activities. The manufacturing agreements can be terminated by us with 30 days written notice. We expect to enter into other commitments as our business further develops.

Table of Contents

During the quarter ended December 31, 2016 there were no material changes outside the ordinary course of business to our specified contractual obligations from those disclosed in our contractual obligations section included in our Annual Report on Form 10-K for the year ended March 31, 2016. As described above under "Recent Developments", subsequent to December 31, 2016, we entered into the Loan Agreement with Hercules.

Liquidity and Capital Resources

Sources of Liquidity

We completed our IPO in June 2015, in which we sold 24,150,000 common shares at a price of \$15.00 per share, including 3,150,000 common shares sold pursuant to the exercise in full of the underwriters' option to purchase additional shares, for gross proceeds of \$362.3 million. We received net proceeds of \$334.5 million, after deducting underwriting discounts and commissions and offering expenses of \$27.7 million. As of December 31, 2016, our principal source of liquidity was our cash balance totaling \$200.4 million. As described above under "Recent Developments," on February 2, 2017, we and our subsidiaries, AHL, ASG and ASI, entered into the Loan Agreement with Hercules, as agent and lender, under which we, AHL and ASG, as co-borrowers, borrowed an aggregate of \$55.0 million on the closing date. ASI has issued a guaranty of the co-borrowers' obligations under the Loan Agreement. We intend to use the proceeds of the Term Loan to fund our ongoing and planned clinical trials and for general corporate purposes.

Funding Requirements

For the nine months ended December 31, 2016, we used \$70.8 million of cash in our operating activities and also made a milestone payment of \$5.0 million under the terms of the GSK Agreement. We have incurred and expect to continue to incur significant and increasing operating losses at least for the next several years. We do not expect to generate revenue unless and until after we successfully complete development and obtain regulatory approval for one of our products in development. Our cash utilization may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our planned clinical trials and our expenditures on other research and development activities and activities related to potential commercialization. We anticipate that our expenses will increase substantially as we:

- continue our Phase 3 MINDSET trial of intepirdine for the treatment of mild-to-moderate Alzheimer's disease designed to support regulatory approval in the United States and Europe, and initiate additional registrational studies to support regulatory approval in Japan;
- continue our twelve month open-label extension study of intepirdine for patients completing the MINDSET study;
- continue the intepirdine HEADWAY-DLB study for the development of intepirdine for DLB;
- continue extension studies for patients completing the HEADWAY-DLB study;
- continue studies of intepirdine for gait and balance in patients with Alzheimer's disease, DLB and PDD;
- continue clinical studies for product candidates that combine cholinesterase inhibitors with peripheral muscarinic receptor antagonists including RVT-103, a combination of a peripheral muscarinic receptor antagonist and donepezil and potentially RVT-104, a combination of a peripheral muscarinic receptor antagonist and high-dose rivastigmine;
- potentially commence future studies of intepirdine for the treatment of severe Alzheimer's disease and other forms of dementia, such as PDD and vascular dementia;
- continue the development of nelotanserin for LBD and other indications;
- continue open-label extension studies for patients completing our nelotanserin phase 2 studies;
- seek to identify, acquire, develop and commercialize additional product candidates;
- integrate acquired technologies into a comprehensive regulatory and product development strategy;
- achieve milestones under our agreements with third parties that will require us to make substantial payments to those parties;
- maintain, expand and protect our intellectual property portfolio;
- hire scientific, clinical, regulatory, manufacturing, quality control, commercial and administrative personnel;

Table of Contents

- add operational, financial and management information systems and personnel, including personnel to support our drug development efforts;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- scale up external manufacturing capabilities to commercialize our product candidates;
- establish a sales, marketing and distribution infrastructure for drug candidates for which we may obtain regulatory approval; and
- operate as a public company.

Our primary use of cash is to fund the research and development of our product candidates. We expect that our existing cash, including net proceeds from our IPO and the Term Loan from Hercules, will be sufficient to fund our operating expenses and capital expenditure requirements into the calendar year 2018. However, we have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we expect. Our existing funds will not be sufficient to enable us to complete all necessary development and to commercially launch all of our products. Accordingly, we may be required to obtain further funding through other public or private offerings of our capital stock, debt financing, collaboration and licensing arrangements or other sources. Adequate additional funding may not be available to us on acceptable terms, or at all. If we are unable to raise capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of one or more of our products or potentially discontinue operations.

Until such time, if ever, as we can generate substantial revenue from sales of our products in development, we expect to finance our cash needs through a combination of equity offerings, debt financings and potential collaboration, license or development agreements. We do not currently have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our shareholders' ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our shareholders' rights. Debt financing and preferred equity 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.

For example, the Loan Agreement with Hercules includes customary affirmative and restrictive covenants and representations and warranties, including a minimum cash covenant that applies commencing on July 1, 2017 and ceases to apply if we achieve certain clinical development milestones as set forth in the Loan Agreement, a covenant against the occurrence of a "change in control," financial reporting obligations, and certain limitations on indebtedness, liens (including a negative pledge on intellectual property and other assets), investments, distributions (including dividends), collateral, transfers, mergers or acquisitions, taxes, corporate changes, and deposit accounts. The Loan Agreement also includes customary events of default, including payment defaults, breaches of covenants following any applicable cure period, the occurrence of certain events that could reasonably be expected to have a "material adverse effect" as set forth in the Loan Agreement, cross acceleration to the debt and certain events relating to bankruptcy or insolvency. Upon the occurrence of an event of default, a default interest rate of an additional 5.0% may be applied to the outstanding principal balance, and Hercules may declare all outstanding obligations immediately due and payable and take such other actions as set forth in the Loan Agreement. Our business, financial condition and results of operations could be materially adversely affected as a result of any of these events.

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

Table of Contents

Cash Flows

The following table sets forth a summary of our cash flows for the nine months ended December 31, 2016 and 2015 (in thousands):

	Nine Months Ended	
	December 31,	
	2016	2015
Net cash used in operating activities	\$(70,751)	\$(30,131)
Net cash used in investing activities	(105)	(4,815)
Net cash (used in) provided by financing activities	(4,990)	334,944

Operating Activities

Cash flows from operating activities consist of net loss adjusted for non-cash items, including depreciation and share-based compensation expense, as well as the effect of changes in working capital and other activities.

For the nine months ended December 31, 2016, net cash used in operating activities was \$70.8 million and was primarily attributable to a net loss of \$128.1 million which includes costs incurred for research and development activities, including CRO fees, manufacturing, regulatory and other clinical trial costs and our general and administrative expenses partially offset by \$27.8 million of non-cash share-based compensation expense and increases of \$17.1 million in accrued liabilities and \$8.6 million in accounts payable. For the nine months ended December 31, 2015, net cash used in operating activities was \$30.1 million and was primarily attributable to a net loss of \$103.5 million which includes costs incurred for research and development activities, including CRO fees, manufacturing, regulatory and other clinical trial costs, and our general and administrative expenses, partially offset by \$64.0 million of non-cash share-based compensation expense.

Investing Activities

For the nine months ended December 31, 2016, net cash used in investing activities was \$105,000 for purchases of computer equipment. For the nine months ended December 31, 2015, net cash used in investing activities was \$4.8 million for the payment made to RSL for our rights to nelotanserin.

Financing Activities

For the nine months ended December 31, 2016, net cash used in financing activities was \$5.0 million and reflects the deferred payment made to GSK under the terms of the GSK Agreement in June 2016. For the nine months ended December 31, 2015, net cash provided by financing activities was \$334.9 million, which was primarily attributable to the net proceeds of \$334.5 million, after deducting underwriting discounts and commissions and offering expenses of \$27.7 million, from our IPO of our common shares.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under the SEC's rules. Accordingly, our operating results, financial condition and cash flows are not subject to off-balance sheet risks.

Critical Accounting Policies and Significant Judgments and Estimates

Our condensed consolidated financial statements have been prepared in accordance with U.S. GAAP. The preparation of these financial statements requires us to make estimates, judgments and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities as of the dates of the balance sheets and the reported amounts of expenses during the reporting periods. In accordance with U.S. GAAP, we evaluate our estimates and judgments on an ongoing basis. Significant estimates include assumptions used in the determination of some of our costs incurred under the Services Agreements, which costs are charged to research and development and general and administrative expense, as well as assumptions used to estimate the fair value of our common shares. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

We define our critical accounting policies as those under U.S. GAAP that require us to make subjective estimates and judgments about matters that are uncertain and are likely to have a material impact on our financial condition and

results of operations, as well as the specific manner in which we apply those principles.

25

Table of Contents

We believe the estimates and judgments involved in our contingent payment liabilities, research and development accruals, share-based compensation and income taxes have the greatest potential impact on our condensed consolidated financial statements, and consider these to be our critical accounting policies and estimates.

Our significant accounting policies are more fully described in Note 2 to our unaudited condensed consolidated financial statements in this Quarterly Report on Form 10-Q and Note B to our consolidated financial statements in our Annual Report on Form 10-K for the year ended March 31, 2016. There have been no material changes to our critical accounting policies and significant judgments and estimates as compared to the critical accounting policies and significant judgments and estimates described in our Annual Report on Form 10-K for the year ended March 31, 2016.

Table of Contents

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Market risk is the potential loss arising from adverse changes in market rates and market prices such as interest rates, foreign currency exchange rates, and changes in the market value of equity instruments. We do not believe we are currently exposed to any material market risk. As of December 31, 2016, we had cash of \$200.4 million, consisting of non-interest bearing deposits denominated in the U.S. dollar.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, refers to controls and procedures that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Security and Exchange Commission’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that such information is accumulated and communicated to a company’s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

In designing and evaluating our disclosure controls and procedures, management recognizes that disclosure controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the disclosure controls and procedures are met. Additionally, in designing disclosure controls and procedures, our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible disclosure controls and procedures. The design of any system of controls also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions; over time, controls may become inadequate because of changes in conditions, or the degree of compliance with policies or procedures may deteriorate. Because of the inherent limitations in a control system, misstatements due to error or fraud may occur and not be detected.

Our management, with the participation of our Principal Executive Officer, our Principal Financial Officer and our Principal Accounting Officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2016, the end of the period covered by this Quarterly Report on Form 10-Q. Based on the evaluation of our disclosure controls and procedures as of December 31, 2016, our Principal Executive Officer and our Principal Financial Officer concluded that, as of such date, our 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 Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the period covered by this Quarterly Report on Form 10-Q that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Table of Contents

PART II: OTHER INFORMATION

Item 1. Legal Proceedings

From time to time, we may become involved in legal proceedings relating to claims arising from the ordinary course of business. We are not currently a party to any material legal proceedings, and we are not aware of any pending or threatened legal proceeding against us that we believe could have an adverse effect on our business, operating results or financial condition.

28

Table of Contents

Item 1A. Risk Factors

You should carefully consider the following risk factors, in addition to the other information contained in this Quarterly Report on Form 10-Q, including the section of this report titled “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and our condensed consolidated financial statements and related notes. If any of the events described in the following risk factors and the risks described elsewhere in this report occurs, our business, operating results and financial condition could be seriously harmed and the trading price of our common shares could decline. This Quarterly Report on Form 10-Q also contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those anticipated in the forward-looking statements as a result of factors that are described below and elsewhere in this report.

Risks Related to Our Business, Financial Position and Capital Requirements

We have a limited operating history and have never generated any product revenues.

We are a clinical-stage biopharmaceutical company with a limited operating history. We were formed in October 2014, and our operations to date have been organizing and staffing our company, raising capital, acquiring drug development programs and preparing for and advancing our product candidates, including intepirdine and nelotanserin, into clinical development. We have not yet demonstrated an ability to successfully complete a large-scale, pivotal clinical trial, obtain marketing approval, manufacture a commercial scale product, or arrange for a third-party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, we have no meaningful operations upon which to evaluate our business and predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing pharmaceutical products.

Our ability to generate revenue and become profitable depends upon our ability to successfully complete the development of our product candidates and other assets for the treatment of various forms of dementia and obtain the necessary regulatory approvals for their commercialization. We have never been profitable, have no products approved for commercial sale and to date have not generated any revenue from product sales.

Even if we receive regulatory approval for our product candidates, we do not know when those candidates will generate revenue, if at all. Our ability to generate product revenue depends on a number of factors, including our ability to:

- successfully complete clinical trials and obtain regulatory approval for the marketing of our product candidates;
- set an acceptable price for our product candidates and obtain coverage and adequate reimbursement from third-party payors;
- establish sales, marketing and distribution systems for our product candidates;
- add operational, financial and management information systems and personnel, including personnel to support our clinical, manufacturing and planned future commercialization efforts and operations as a public company;
- initiate and continue relationships with third-party manufacturers and have commercial quantities of our product candidates manufactured at acceptable cost levels;
- attract and retain an experienced management and advisory team;
- achieve broad market acceptance of our products in the medical community and with third party payors and consumers;
- launch commercial sales of our products, whether alone or in collaboration with others; and
- maintain, expand and protect our intellectual property portfolio.

Because of the numerous risks and uncertainties associated with product development, we are unable to predict the timing or amount of increased expenses, or when, or if, we will be able to achieve or maintain profitability. Our expenses could increase beyond expectations if we are required by the FDA, European Medicines Agency, or EMA, Japan’s Pharmaceutical and Medical Devices Agency or PMDA, or comparable regulatory authorities in other countries, to perform studies or clinical trials in addition to those that we currently anticipate. Even if our product candidates are approved for commercial sale, we anticipate incurring significant costs associated with their commercial launch. If we cannot successfully execute any one of the foregoing, our business may not succeed and your investment will be adversely affected.

Table of Contents

We expect to incur significant losses for the foreseeable future and may never achieve or maintain profitability. Investment in pharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that a product candidate will fail to gain regulatory approval or become commercially viable. We have never generated any revenues, and we cannot estimate with precision the extent of our future losses. We do not currently have any products that are available for commercial sale and we may never generate revenue from selling products or achieve profitability. We expect to continue to incur substantial and increasing losses through the projected commercialization of our product candidates. Our product candidates have not been approved for marketing in the United States or any other jurisdiction, and we may never receive any such approvals. As a result, we are uncertain when or if we will achieve profitability and, if so, whether we will be able to sustain it. Our ability to produce revenue and achieve profitability is dependent on our ability to complete the development of our product candidates, obtain necessary regulatory approvals, and have our product candidates manufactured and successfully marketed and commercialized. We cannot assure you that we will be profitable even if we successfully commercialize our product candidates. If we do successfully obtain regulatory approval to market our product candidates, our revenues will be dependent, in part, upon, among other things, the size of the markets in the territories for which we gain regulatory approval, the number of competitors in such markets, the accepted price for our product candidates and whether we own the commercial rights for that territory. If the indication approved by regulatory authorities is narrower than we expect, or the treatment population is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of our product candidates, even if approved. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Failure to become and remain profitable may adversely affect the market price of our common shares and our ability to raise capital and continue operations.

We expect our research and development expenses to be significant in connection with our Phase 3 MINDSET trial of intepirdine in patients with mild-to-moderate Alzheimer's disease, and continue to increase as we conduct clinical trials of intepirdine for DLB, clinical trials of our second product candidate, nelotanserin, for the treatment of multiple aspects of LBD, and additional clinical development for RVT-103 and RVT-104. In addition, if we obtain regulatory approval for intepirdine, we expect to incur increased sales and marketing expenses. As a result, we expect to continue to incur significant and increasing operating losses and negative cash flows for the foreseeable future. These losses have had and will continue to have an adverse effect on our financial position and working capital.

We are heavily dependent on the success of intepirdine and nelotanserin, our key product candidates, which are still in clinical development, and if either of these product candidates does not receive regulatory approval or is not successfully commercialized, our business may be harmed.

We currently have no products that are approved for commercial sale and may never be able to develop marketable drug products. We expect that a substantial portion of our efforts and expenditures over the next few years will be devoted to intepirdine and nelotanserin. Accordingly, our business currently depends heavily on the successful development, regulatory approval and commercialization of these product candidates. We cannot be certain that our product candidates will receive regulatory approval or be successfully commercialized even if we receive regulatory approval. The research, testing, manufacturing, labeling, approval, sale, marketing and distribution of drug products are and will remain subject to extensive regulation by the FDA, the EMA, the PMDA and other comparable regulatory authorities that each have differing regulations. We are not permitted to market our product candidates in the United States or in any foreign countries until they receive the requisite approvals from the FDA or comparable regulatory authorities in other countries. We have not submitted marketing applications to the FDA or foreign regulatory authorities and do not expect to be in a position to do so for the foreseeable future. Obtaining marketing approval is an extensive, lengthy, expensive and inherently uncertain process, and regulatory authorities, may delay, limit or deny approval of our product candidates for many reasons, including:

- we may not be able to demonstrate that a product candidate is safe and effective as a treatment for our targeted indications to the satisfaction of the applicable regulatory authorities;
- the regulatory authorities may require additional nonclinical studies or registrational studies of the product candidate in mild-to-moderate Alzheimer's disease, which would increase our costs and prolong our development;
-

the results of our clinical trials may not meet the level of statistical or clinical significance required for marketing approval;

- the regulatory authorities may disagree with the number, design, size, conduct or implementation of our clinical trials;

- the contract research organizations, or CROs, that we retain to conduct clinical trials may take actions outside of our control that materially adversely impact our clinical trials;

30

Table of Contents

- the regulatory authorities may not find the data from nonclinical studies and clinical trials sufficient to demonstrate that the clinical and other benefits of the product candidate outweigh its safety risks;
- the regulatory authorities may disagree with our interpretation of data from our nonclinical studies and clinical trials or may require that we conduct additional studies;
- the regulatory authorities may not accept data generated at our clinical trial sites;
- the regulatory authorities may require, as a condition of approval, limitations on approved labeling or distribution and use restrictions;
- in the United States, the FDA may require development of a risk evaluation and mitigation strategy, or REMS, as a condition of approval;
- the regulatory authorities may identify deficiencies in the manufacturing processes or facilities of our third-party manufacturers; or
- the regulatory authorities may change their approval policies or adopt new regulations.

Our operating activities may be restricted as a result of restrictive covenants in the agreement governing our outstanding indebtedness, and we may be required to repay the outstanding indebtedness in an event of default.

In February 2017, we and our subsidiaries entered into a loan and security agreement, or the Loan Agreement, with Hercules Capital, Inc., or Hercules, for a term loan of \$55.0 million or the Term Loan, the full amount of which was funded upon execution of the Loan Agreement.

The Loan Agreement is secured by substantially all of our property and that of our subsidiaries that are parties to the Loan Agreement, other than intellectual property.

The Loan Agreement subjects us and our subsidiaries to various affirmative and restrictive covenants, including a minimum cash covenant that applies commencing on July 1, 2017 and ceases to apply if we achieve certain clinical development milestones as set forth in the Loan Agreement, a covenant against the occurrence of a “change in control,” financial reporting obligations, and certain limitations on indebtedness, liens (including a negative pledge on intellectual property and other assets), investments, distributions (including dividends), collateral, transfers, mergers or acquisitions, taxes, corporate changes, and deposit accounts. Compliance with these covenants may limit our flexibility in operating our business and our ability to take actions that might be advantageous to us and our stockholders. Our business may be adversely affected by these restrictions on our ability to operate our business. Additionally, we may be required to repay the entire amount of outstanding indebtedness under the Term Loan in cash if we fail to stay in compliance with our covenants or suffer some other event of default under the Loan Agreement. Under the Loan Agreement, an event of default will occur if, among other things: we fail to make payments under the Loan Agreement; we breach any of our covenants under the Loan Agreement, subject to specified cure periods with respect to certain breaches; there occurs an event that has a material adverse effect on (i) our business, operations, properties, assets or financial condition, (ii) our ability to perform or satisfy our obligations under the Loan Agreement as they become due or Hercules’s ability to enforce its rights or remedies with respect to our obligations under the Loan Agreement, or (iii) the collateral or liens securing our obligations under the Loan Agreement; we or our assets become subject to certain legal proceedings, such as bankruptcy proceedings; we are unable to pay our debts as they become due; or we default on contracts with third parties which would permit Hercules to accelerate the maturity of such indebtedness or that could have a material adverse effect on us. We may not have enough available cash or be able to raise additional funds through equity or debt financings to repay such indebtedness at the time any such event of default occurs. In that case, we may be required to delay, limit, reduce or terminate our clinical development efforts or grant to others rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. Hercules could also exercise its rights as collateral agent to take possession and dispose of the collateral securing the Term Loan for its benefit, which collateral includes all of our property other than our intellectual property. Our business, financial condition and results of operations could be substantially harmed as a result of any of these events.

Table of Contents

We may require additional capital to fund our operations, and if we fail to obtain necessary financing, we may not be able to complete the development and commercialization of our product candidates.

We expect to spend substantial amounts to complete the development of, seek regulatory approvals for and commercialize our product candidates. These expenditures will include costs to GSK under the GSK Agreement, and costs to Arena under the Arena Development Agreement. Under the terms of these agreements, we are obligated to make significant cash payments upon the achievement of specified development, regulatory and sales performance milestones, as well as payments in connection with the sale of resulting products.

Even with the net proceeds from our IPO in June 2015, we will require additional capital to complete the development and potential commercialization of our product candidates. If we are unable to raise capital when needed or on acceptable terms, we could be forced to delay, reduce or eliminate our development program or any future commercialization efforts. In addition, attempting to secure additional financing may divert the time and attention of our management from day-to-day activities and harm our product candidate development efforts.

Based upon our current operating plan, we believe that the net proceeds from our IPO and the Term Loan from Hercules will be sufficient to fund our operating expenses and capital expenditure requirements into the calendar year 2018. This estimate is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Because the length of time and activities associated with successful development of our product candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and any approved marketing and commercialization activities. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the initiation, progress, timing, costs and results of our planned clinical trials for our product candidates;
- the outcome, timing and cost of meeting regulatory requirements established by the FDA, the EMA, or the PMDA, and other comparable foreign regulatory authorities;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights;
- the cost of defending potential intellectual property disputes, including patent infringement actions brought by third parties against us or our product candidates or any future product candidates;
- the effect of competing technological and market developments;
- the cost and timing of completion of commercial-scale manufacturing activities;
- the cost of establishing sales, marketing and distribution capabilities for our product candidates in regions where we choose to commercialize our products on our own; and
- the initiation, progress, timing and results of our commercialization of our product candidates, if approved for commercial sale.

We cannot be certain that additional funding will be available on acceptable terms, or at all. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of our product candidates or potentially discontinue operations.

We may be required to make significant payments to third parties under the agreements pursuant to which we acquired our product candidates.

In December 2014, we acquired the rights to intepirdine under the GSK Agreement; in October 2015, we acquired the rights to nelotanserin and assumed the obligations under the Arena Development Agreement; and in August 2016, we entered into a license agreement with Qaam for RVT-103 and RVT-104. Under these agreements, we are subject to significant obligations, including payment obligations upon achievement of specified milestones and payments based on product sales, as well as other material obligations. If these payments become due under the terms of the agreements, we may not have sufficient funds available to meet our obligations and in which case our development efforts would be substantially harmed.

Table of Contents

Raising additional funds by issuing securities may cause dilution to existing shareholders, and raising funds through lending and licensing arrangements may restrict our operations or require us to relinquish proprietary rights. We expect that significant additional capital will be needed in the future to continue our planned operations. Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, strategic alliances and license and development agreements in connection with any collaborations. We do not have any committed external source of funds. To the extent that we raise additional capital by issuing equity securities, our existing shareholders' ownership may experience substantial dilution, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common shareholder. Debt financing and preferred equity 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 collaborations, strategic alliances or marketing, distribution 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. Any debt financing we enter into may involve covenants that restrict our operations. These restrictive covenants may include limitations on additional borrowing and specific restrictions on the use of our assets as well as prohibitions on our ability to create liens, pay dividends, redeem our shares or make investments. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise develop and market ourselves.

We currently have a limited number of employees who are employed by our wholly-owned subsidiary, Axovant Sciences, Inc. ("ASI"), and we rely on Roivant Sciences, Inc. ("RSI") and Roivant Sciences GmbH ("RSG") to provide various administrative, research and development and other services.

As of December 31, 2016, we and our wholly-owned subsidiary, ASI, had 57 employees. We rely in part on the administrative support and research and development services provided by our affiliates, RSI and RSG, wholly-owned subsidiaries of RSL, pursuant to our Services Agreements with RSI and RSG. Personnel and support staff that provide services to us under the Services Agreements are not required to, and we do not expect that they will, have as their primary responsibility the management and administration of our business or act exclusively for us. Under the Services Agreements, RSI and RSG have the discretion to determine which of their employees will perform services under the agreements. Further, Vivek Ramaswamy, our Principal Executive Officer, Lawrence T. Friedhoff, M.D., Ph.D., our Chief Development Officer, and Michael Adaszczik, our Principal Accounting Officer, are employees of RSI, and Marianne L. Romeo, our Head of Global Transactions and Risk Management, is an employee of RSL. As a result, these officers are unlikely to allocate all of their time and resources to us.

RSI and RSG have limited financing and accounting and other resources. If RSI or RSG fail to perform their obligations in accordance with the terms of the Services Agreements, it could be difficult for us to operate our business. In addition, the termination of our relationship with RSI or RSG, and any delay in appointing or finding a suitable replacement provider, if one exists, could make it difficult for us to operate our business. Any failure by RSI or RSG to effectively manage our administrative, research and development or other services could harm our business, financial condition and results of operations.

Table of Contents

We will need to expand our organization, and we may experience difficulties in managing this growth, which could disrupt our operations.

We expect to hire, either directly or through ASI or Axovant Sciences GmbH, additional employees for our managerial, clinical, scientific and engineering, operational, sales and marketing teams. We may have operational difficulties in connection with identifying, hiring and integrating new personnel. Future growth would impose significant additional responsibilities on our management, including the need to identify, recruit, maintain, motivate and integrate additional employees, consultants and contractors. Also, our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations across our entities, which may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and grow revenues could be reduced, and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize our product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth. Many of the other pharmaceutical companies that we compete against for qualified personnel and consultants have greater financial and other resources, different risk profiles and a longer history in the industry than we do. They also may provide more diverse opportunities and better chances for career advancement. Some of these characteristics may be more appealing to high-quality candidates and consultants than what we have to offer. If we are unable to continue to attract and retain high-quality personnel and consultants, the rate and success at which we can discover and develop product candidates and our business will be limited.

Our employees, independent contractors, principal investigators, consultants, commercial collaborators, service providers and other vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could have an adverse effect on our results of operations.

We are exposed to the risk that our employees and contractors, including principal investigators, consultants, commercial collaborators, service providers and other vendors may engage in fraudulent or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or other unauthorized activities that violate laws and regulations, including those of the FDA and other similar regulatory bodies that require the reporting of true, complete and accurate information; manufacturing standards; federal and state healthcare fraud and abuse and health regulatory laws and other similar foreign fraudulent misconduct laws; or laws that require the true, complete and accurate reporting of financial information or data. Activities subject to these laws also involve the improper use or misrepresentation of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter third-party 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 us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and financial results, including the imposition of significant criminal, civil and administrative penalties, damages, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Our business and operations would suffer in the event of system failures.

Our computer systems, as well as those of ASI, ASG, RSI, RSG and our CROs and other contractors, consultants, and law and accounting firms, may sustain damage from computer viruses, unauthorized access, cybercriminals, natural disasters, terrorism, war and telecommunication and electrical failures. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption, including to our drug development programs. For example, the loss of nonclinical or clinical trial data from completed, ongoing or planned 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 were to result in a loss of or damage to our data or applications, or inappropriate disclosure of personal, confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed.

Table of Contents

Potential product liability lawsuits against us could cause us to incur substantial liabilities and limit commercialization of any products that we may develop.

The use of our product candidates in clinical trials and the sale of any products for which we obtain marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, health care providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. On occasion, large judgments have been awarded in class action lawsuits based on drugs that had unanticipated adverse effects. If we are not successful in defending ourselves against product liability claims, we could incur substantial liability and costs. In addition, regardless of merit or eventual outcome, product liability claims may result in:

- impairment of our business reputation and significant negative media attention;
- withdrawal of participants from our clinical trials;
- significant costs to defend related litigation;
- distraction of management's attention from our primary business;
- substantial monetary awards to patients or other claimants;
- inability to commercialize our product candidates or any future product candidate;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- decreased demand for our product candidates or any future product candidate, if approved for commercial sale; and
- loss of revenue.

The product liability insurance we currently carry, and any additional product liability insurance coverage we acquire in the future, may not be sufficient to reimburse us for any 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 due to liability. If we obtain marketing approval for our product candidates, we intend to acquire insurance coverage to include the sale of commercial products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. A successful product liability claim or series of claims brought against us could cause our share price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business, including preventing or limiting the commercialization of any product candidates we develop.

Risks Related to Clinical Development, Regulatory Approval and Commercialization

Clinical trials are very expensive, time-consuming, difficult to design and implement and involve an uncertain outcome.

Our product candidates are still in development and will require extensive clinical testing before we are prepared to submit an application for marketing approval to regulatory authorities. We cannot predict with any certainty if or when we might submit any such application for regulatory approval for our product candidates or whether any such application will be approved by the applicable regulatory authority in our target markets. Human clinical trials are very expensive and difficult to design and implement, in part because they are subject to rigorous regulatory requirements. For instance, regulatory authorities may not agree with our proposed endpoints for any clinical trials of our product candidates, which may delay the commencement of our clinical trials. The clinical trial process is also time-consuming. We estimate that clinical trials of our product candidates will take at least several years to complete. Furthermore, failure can occur at any stage of the trials, and we could encounter problems that cause us to abandon or repeat clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through nonclinical studies and initial clinical trials, and the results of early clinical trials therefore may not be predictive of the results of later-stage clinical programs. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials.

The commencement and completion of clinical trials may be delayed by several factors, including:

- failure to obtain regulatory approval to commence a trial;
- unforeseen safety issues;
- determination of dosing issues;

Table of Contents

- lack of effectiveness during clinical trials;
- inability to reach agreement on acceptable terms with prospective CROs and clinical trial sites;
- slower than expected rates of patient recruitment or failure to recruit suitable patients to participate in a trial;
- failure to manufacture sufficient quantities of a drug candidate or placebo or failure to obtain sufficient quantities of concomitant medication for use in clinical trials;
- inability to monitor patients adequately during or after treatment;
- inability or unwillingness of medical investigators to follow our clinical protocols;
- adding a sufficient number of clinical trial sites; or
- clinical sites deviating from trial protocol or dropping out of a trial.

Further, by way of example, we, the FDA or an institutional review board, or IRB, at a clinical trial site may suspend our clinical trials at any time if it appears that we or our collaborators are failing to conduct a trial in accordance with regulatory requirements, including the FDA's current Good Clinical Practice, or GCP, regulations, that we are exposing participants to unacceptable health risks, or if the FDA finds deficiencies in our IND submissions or the conduct of these trials. Therefore, we cannot predict with any certainty the schedule for commencement and completion of clinical trials. If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our product candidates could be harmed, and our ability to generate revenues may be delayed. In addition, any delays in our clinical trials could increase our costs, slow down the approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, financial condition and results of operations. Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA. The FDA may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA and may ultimately lead to the denial of marketing approval of one or more of our product candidates.

In addition, we acquired worldwide rights to our product candidates and were not involved in their development prior to such acquisitions. Any difficulties we experience in transitioning and integrating such product candidates into our operations may result in delays in clinical trials as well as problems in our development efforts and regulatory filings, particularly if we do not receive all of the necessary drug products, information, reports and data from third parties in a timely manner. More particularly, we have had no involvement with or control over the nonclinical and clinical development of either of our product candidates prior to acquiring the rights to them. We are dependent on predecessors including GSK and Arena, as applicable, having conducted such research and development in accordance with the applicable protocols, legal, regulatory and scientific standards, having accurately reported the results of all clinical trials and other research conducted prior to our acquisition of the product candidates, having correctly collected and interpreted the data from these trials and other research and having supplied us with complete information, data sets and reports required to adequately demonstrate the results reported through the date of our acquisition of these assets. Problems related to predecessors including GSK and Arena could result in increased costs and delays in the development of our product candidates, which could adversely affect our ability to generate future revenues.

The results of our clinical trials may not demonstrate that our product candidates are safe and effective. Even if our clinical trials are completed as planned, we cannot be certain that their results will support the safety and effectiveness of our product candidates for the particular indications for which they are being developed. Success in nonclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and we cannot be sure that the results of later clinical trials will replicate the results of prior clinical trials and nonclinical testing. Likewise, promising results in interim analyses or other preliminary analyses do not ensure that the clinical

trial as a whole will be successful. Failure of a clinical trial to meet its predetermined endpoints would likely cause us to abandon a product candidate and may delay development of any other product candidates. Any delay in, or termination of, our clinical trials will delay the submission of our applications for marketing approval and, ultimately, our ability to commercialize our product candidates and generate product revenues.

Table of Contents

Enrollment and retention of patients in clinical trials is an expensive and time-consuming process and could be made more difficult or rendered impossible by multiple factors outside our control.

We may encounter delays in enrolling, or be unable to enroll, a sufficient number of patients to complete any of our clinical trials, and even once enrolled we may be unable to retain a sufficient number of patients to complete any of our trials. Patient enrollment and retention in clinical trials depends on many factors, including the size of the patient population, the nature of the trial protocol, the existing body of safety and efficacy data with respect to the study drug, the number and nature of competing treatments and ongoing clinical trials of competing drugs for the same indication, the proximity of patients to clinical sites and the eligibility criteria for the study. Furthermore, any negative results we may report in clinical trials of any of our product candidates may make it difficult or impossible to recruit and retain patients in other clinical trials of those product candidates. Delays or failures in planned patient enrollment or retention may result in increased costs, program delays or both, which could have a harmful effect on our ability to develop our product candidates, or could render further development impossible. In addition, we expect to rely on CROs and clinical trial sites to ensure proper and timely conduct of our future clinical trials and, while we intend to enter into agreements governing their services, we will be limited in our ability to compel their actual performance. A Fast Track designation by the FDA may not actually lead to a faster development or regulatory review or approval process and does not increase the likelihood that our product candidates will receive marketing approval.

If a product is intended for the treatment of a serious or life-threatening condition and the product demonstrates the potential to address unmet medical needs for this condition, the sponsor may apply for Fast Track designation in the United States from the FDA. The FDA has broad discretion whether or not to grant this designation. We have applied for and received Fast Track designation from the FDA for intepirdine in the treatment of dementia with Lewy bodies, and we may apply for that designation for some or all of our other product candidates in the future. The Fast Track designation we received, as is the case for any other Fast Track designation we may obtain in the future, does not necessarily lead to a faster development pathway or regulatory review process, and does not increase the likelihood of regulatory approval. The FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development programs.

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

Drug development is highly competitive and subject to rapid and significant technological advancements. As a significant unmet medical need exists for the treatment of Alzheimer's disease and other dementias, there are several large and small pharmaceutical companies focused on delivering therapeutics for the treatment of these diseases. Further, it is likely that additional drugs will become available in the future for the treatment of our target indications.

We are aware of several companies that are working to develop drugs that would compete against our product candidates, including intepirdine and nelotanserin. Many of our existing or potential competitors have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, as well as in obtaining regulatory approvals of those product candidates in the United States and in foreign countries. Our current and potential future competitors also have significantly more experience commercializing drugs that have been approved for marketing. Mergers and acquisitions in the pharmaceutical and biotechnology industries could result in even more resources being concentrated among a small number of our competitors.

Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors may succeed in developing, acquiring or licensing, on an exclusive basis, drugs that are more effective or less costly than any product candidate that we may develop.

We will face competition from other drugs or from other non-drug products currently approved or that will be approved in the future for the treatment of Alzheimer's disease and other dementias, including Lewy body dementia.

Therefore, our ability to compete successfully will depend largely on our ability to:

- develop and commercialize drugs that are superior to other products in the market;

demonstrate through our clinical trials that our product candidates are differentiated from existing and future therapies;

• attract qualified scientific, product development and commercial personnel;

• obtain patent or other proprietary protection for our medicines;

• obtain required regulatory approvals;

• obtain coverage and adequate reimbursement from, and negotiate competitive pricing with, third-party payors; and

• successfully collaborate with pharmaceutical companies in the discovery, development and commercialization of new medicines.

Table of Contents

The availability of our competitors' products could limit the demand, and the price we are able to charge, for any product candidate we develop. The inability to compete with existing or subsequently introduced drugs would have an adverse impact on our business, financial condition and prospects.

Established pharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make our product candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, discovering, developing, receiving regulatory and marketing approval for or commercializing drugs before we do, which would have an adverse impact on our business and results of operations.

If we are not able to obtain required regulatory approvals, we will not be able to commercialize our product candidates, and our ability to generate revenue will be materially impaired.

The activities associated with the development and commercialization of our product candidates, including their design, research, testing, manufacture, safety, efficacy, recordkeeping, labeling, packaging, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by the EMA, the PMDA and similar regulatory authorities outside the United States. Failure to obtain marketing approval for our product candidates will prevent us from commercializing them. We have not received approval from regulatory authorities to market any product candidate in any jurisdiction, and we will need to complete pivotal clinical trials successfully for our product candidates before we can submit any application for regulatory approval. It is possible that our product candidates in the future will never obtain the appropriate regulatory approvals necessary for us to commence product sales.

We expect to rely on third-party CROs and consultants to assist us in filing and supporting the applications necessary to gain marketing approvals. Securing marketing approval requires the submission of extensive nonclinical and clinical data and supporting information for our product candidates to regulatory authorities for each therapeutic indication to establish safety and efficacy of the product candidate for that indication. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. Delays in the submission of applications for marketing approval and issues, included those related to gathering the appropriate data and the inspection process, may ultimately delay or affect our ability to obtain regulatory approval, commercialize our product candidates and generate product revenues. Our product candidates may cause adverse effects or have other properties that could delay or prevent their regulatory approval or limit the scope of any approved label or market acceptance.

Adverse events caused by our product candidates could cause us, other reviewing entities, clinical trial sites or regulatory authorities to interrupt, delay or halt clinical trials and could result in the denial of regulatory approval. If an unacceptable frequency or severity of adverse events are reported in our clinical trials for our product candidates or any future product candidates, our ability to obtain regulatory approval for such product candidates may be negatively impacted. In addition, if the FDA determines that a drug candidate may present a risk of substance abuse, it can recommend to the Drug Enforcement Administration that the drug be scheduled under the Controlled Substances Act. The laws and regulations governing controlled substances could limit commercialization of our product candidates, and failure to comply with those laws and regulations could also result in adverse regulatory, legal, and operational consequences.

Furthermore, if any of our products are approved and then cause serious or unexpected side effects, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw their approval of the product or require a REMS to impose restrictions on its distribution or other risk management measures;
- regulatory authorities may require the addition of labeling statements, such as warnings or contraindications;
- we may be required to change the way the product is administered or to conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients;
- we could elect to discontinue the sale of our product; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product candidate and could substantially increase the costs of commercializing our product candidates.

Table of Contents

Even if we obtain FDA approval for our product candidates in the United States, we may never obtain approval for or commercialize them in any other jurisdiction, which would limit our ability to realize their full market potential. In order to market any products in any particular jurisdiction, we must establish and comply with numerous and varying regulatory requirements on a country-by-country basis regarding safety and efficacy. Approval by the FDA in the United States does not ensure approval by regulatory authorities in other countries or jurisdictions. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country. Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and costs for us and require additional nonclinical studies or clinical trials which could be costly and time consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. We do not have any product candidates approved for sale in any jurisdiction, including in international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of any product we develop will be unrealized.

Even if we obtain regulatory approval for our product candidates, we will still face extensive regulatory requirements and our products may face future development and regulatory difficulties.

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, packaging, distribution, adverse event reporting, storage, recordkeeping, export, import, advertising and promotional activities for such product, among other things, will be subject to extensive and ongoing requirements of and review by the FDA, the EMA, the PMDA and other comparable foreign regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, establishment registration and drug listing requirements, continued compliance with current Good Manufacturing Process, or cGMP regulations, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping and current GCP requirements for any clinical trials that we conduct post-approval. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, including any requirement to implement a REMS. If any of our product candidates receives marketing approval, the accompanying labels for such products may limit the approved use of the drug, which could limit sales.

Regulatory authorities may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product. These authorities closely regulate the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling. We will be subject to stringent restrictions on manufacturers' communications regarding off-label use and if we do not market our products for their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the Federal Food, Drug, and Cosmetic Act in the United States, and other comparable regulations in foreign jurisdictions, relating to the promotion of prescription drugs may lead to enforcement actions and investigations alleging violations of U.S. federal and state health care fraud and abuse laws, as well as state consumer protection laws and comparable laws in foreign jurisdictions.

In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on manufacturing such products;
- restrictions on the labeling or marketing of such products;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters;
- withdrawal of the products from the market;

recall of products;
fines, restitution or disgorgement of profits or revenues;
suspension or withdrawal of marketing approvals;
refusal to permit the import or export of such products;
product seizure; or

39

Table of Contents

injunctions or the imposition of civil or criminal penalties.

Government regulations may change and additional government regulations may be enacted, either of which could prevent, limit or delay regulatory approval of our product candidates or any future product candidate. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained.

Even if our product candidates receive marketing approval, they may fail to achieve market acceptance by physicians, patients, third-party payors or others in the medical community necessary for commercial success.

Even if our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If they do not achieve an adequate level of acceptance, we may not generate significant product revenues and become profitable. The degree of market acceptance for our product candidates, if approved for commercial sale, will depend on a number of factors, including but not limited to:

- the efficacy and potential advantages compared to alternative treatments;
- the effectiveness of sales and marketing efforts;
- the cost of treatment in relation to alternative treatments, including any similar generic treatments;
- our ability to offer our products for sale at competitive prices;
- the convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- the availability of third-party coverage and adequate reimbursement;
- the prevalence and severity of any side effects; and
- any restrictions on the use of our product together with other medications.

Because we expect sales of our product candidates, if approved, to generate substantially all of our product revenues for the foreseeable future, the failure of these products, especially of intepirdine, to find market acceptance would harm our business and could require us to seek additional financing.

If we are unable to establish sales, marketing and distribution capabilities either on our own or in collaboration with third-parties, we may not be successful in commercializing our product candidates, even if approved.

We do not have a full infrastructure for the sales, marketing or distribution of our products should they be approved, and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. In order to market any product that may be approved, we must build our sales, distribution, marketing, managerial and other non-technical capabilities or make arrangements with third parties to perform these services, and obtain requisite licenses. To achieve commercial success for any product for which we have obtained marketing approval, we will need a sales and marketing organization.

We plan to commercialize our product candidates in the United States, the European Union, Japan and other major markets. If our product candidates are approved for marketing, we may build a focused sales, distribution and marketing infrastructure to market them. There are significant expenses and risks involved with establishing our own sales, marketing and distribution capabilities, including our ability to hire, retain and appropriately 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. Any failure or delay in the development of our internal sales, marketing and distribution capabilities, and any failure to obtain and maintain the requisite licenses, could delay any product launch, which would adversely impact the commercialization of our product candidates. For example, if the commercial launch of our product candidates for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our products on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians or attain adequate numbers of physicians to prescribe any drugs; and

- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

40

Table of Contents

We do not anticipate having the resources in the foreseeable future to allocate to the sales and marketing of our product candidates in certain markets overseas. Therefore, our future success will depend, in part, on our ability to enter into and maintain collaborative relationships for such capabilities, the collaborator's strategic interest in the product and such collaborator's ability to successfully market and sell the product. To the extent that we depend on third parties for marketing and distribution, any revenues we receive will depend upon the efforts of such third parties, and there can be no assurance that such efforts will be successful or profitable.

If we are unable to build our own sales force or negotiate a collaborative relationship for the commercialization of our product candidates, we may be forced to delay the potential commercialization of such products or reduce the scope of our sales or marketing activities for our product candidates. If we elect to increase our expenditures to fund commercialization activities ourselves, we will need to obtain additional capital, which may not be available to us on acceptable terms, or at all. If we do not have sufficient funds, we will not be able to bring our product candidates to market or generate product revenue. We could enter into arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be ideal and we may be required to relinquish rights to one or more of our product candidates or otherwise agree to terms unfavorable to us, any of which may have an adverse effect on our business, operating results and prospects.

If we are unable to establish adequate sales, marketing and distribution capabilities, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates and may not become profitable. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

If we obtain approval to commercialize any products outside of the United States, a variety of risks associated with international operations could materially adversely affect our business.

If our product candidates are approved for commercialization, we may enter into agreements with third parties to market them in certain jurisdictions outside the United States. We expect that we will be subject to additional risks related to international operations or entering into international business relationships, including:

- different regulatory requirements for drug approvals and rules governing drug commercialization in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign reimbursement, pricing and insurance regimes;
- foreign taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential noncompliance with the U.S. Foreign Corrupt Practices Act, the U.K. Bribery Act 2010 and similar anti-bribery and anticorruption laws in other jurisdictions;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- and
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including earthquakes, typhoons, floods and fires.

Table of Contents

Our current and future relationships with investigators, health care professionals, consultants, third-party payors, and customers will be subject to applicable healthcare regulatory laws, which could expose us to penalties.

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors and customers, may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations. These laws may regulate the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute our products for which we obtain marketing approval. Such laws include:

the federal Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the federal Anti-Kickback Statute or specific intent to violate it to have committed a violation; in addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act;

the federal false claims laws, including the civil False Claims Act, impose criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for, among other things, executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it to have committed a violation;

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to the government information related to payments or other "transfers of value" made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, and requires applicable manufacturers and group purchasing organizations to report annually to the government ownership and investment interests held by the physicians described above and their immediate family members and payments or other "transfers of value" to such physician owners (covered manufacturers are required to submit reports to the government by the 90th day of each calendar year); and

analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state and foreign laws governing the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our current and future business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations, agency guidance or case law involving applicable healthcare laws. If our operations are found to be in violation of any of these or any other health regulatory laws that may apply to us, we may be subject to significant penalties, including the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgement, individual imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs,

contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. Defending against any such actions can be costly, time-consuming and may require significant financial and personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired.

42

Table of Contents

Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and affect the prices we may obtain.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any products for which we obtain marketing approval.

For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, collectively the Affordable Care Act, was enacted to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. Although it is too early to determine the full effect of the Affordable Care Act, the law has continued the downward pressure on pharmaceutical pricing, especially under the Medicare program, and increased the industry's regulatory burdens and operating costs. Among the provisions of the Affordable Care Act of importance to our potential drug candidates are the following:

- an annual, nondeductible fee payable by any entity that manufactures or imports specified branded prescription drugs and biologic agents;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries under their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D;
- extension of manufacturers' Medicaid rebate liability to individuals enrolled in Medicaid managed care organizations;
- expansion of eligibility criteria for Medicaid programs in certain states;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

We cannot predict the full impact of the Affordable Care Act on pharmaceutical companies, as many of the reforms require the promulgation of detailed regulations implementing the statutory provisions, some of which has not yet fully occurred. For example, in January 2016, the Centers for Medicare and Medicaid Services issued a final rule regarding the Medicaid Drug Rebate Program, effective April 1, 2016, that, among other things, revises the manner in which the "average manufacturer price" is to be calculated by manufacturers participating in the program and implements certain amendments to the Medicaid rebate statute created under the Affordable Care Act. Further, there have been judicial and Congressional challenges to certain aspects of the Affordable Care Act, and we expect there will be additional challenges and amendments to the Affordable Care Act in the future.

Other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. For example, in August 2011, the President signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee did not achieve a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, triggering the legislation's automatic reduction to several government programs. This included further reductions to Medicare payments to providers of 2% per fiscal year, which went into effect in April 2013 and, due to subsequent legislative amendments to the statute, will stay in effect through 2025 unless additional Congressional action is taken. Additionally, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers. Further, 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.

Table of Contents

Moreover, the Drug Supply Chain Security Act, which was enacted in 2012 as part of the Food and Drug Administration Safety and Innovation Act, imposes new obligations on manufacturers of pharmaceutical products related to product tracking and tracing. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We are not sure whether additional legislative changes will be enacted, or whether the current regulations, guidance or interpretations will be changed, or what the impact of such changes on our business, if any, may be.

We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

Coverage and adequate reimbursement may not be available for our product candidates, which could make it difficult for us to sell our products profitably.

Market acceptance and sales of any product candidates that we develop, will depend in part on the extent to which reimbursement for these products and related treatments will be available from third-party payors, including government health administration authorities and private health insurers. Third-party payors decide which drugs they will pay for and at which reimbursement levels. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own coverage and reimbursement policies. However, decisions regarding the extent of coverage and amount of reimbursement to be provided for any product candidates that we develop will be made on a plan-by-plan basis. One payor's determination to provide coverage for a product does not assure that other payors will also provide coverage, and adequate reimbursement, for the product. Additionally, a third-party payor's decision to provide coverage for a drug does not imply that an adequate reimbursement rate will be approved. Each plan determines whether or not it will provide coverage for a drug, what amount it will pay the manufacturer for the drug, and on what tier of its formulary the drug will be placed. The position of a drug on a formulary generally determines the co-payment that a patient will need to make to obtain the drug and can strongly influence the adoption of a drug by patients and physicians. Patients who are prescribed treatments for their conditions and providers prescribing such services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Patients are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our products.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. We cannot be sure that coverage and reimbursement will be available for any product that we commercialize and, if reimbursement is available, what the level of reimbursement will be. Inadequate coverage and reimbursement may impact the demand for, or the price of, any product for which we obtain marketing approval. If coverage and adequate reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize any product candidates that we develop.

Additionally, there have been a number of legislative and regulatory proposals to change the healthcare system in the United States and in some foreign jurisdictions that could affect our ability to sell any future drugs profitably. These legislative and regulatory changes may negatively impact the reimbursement for any future drugs, following approval.

Risks Related to Our Dependence on Third Parties

We do not have our own manufacturing capabilities and will rely on third parties to produce clinical and commercial supplies of our product candidates.

We have no experience in drug formulation or manufacturing and do not own or operate, and we do not expect to own or operate, facilities for product manufacturing, storage and distribution, or testing. While intepirdine was being developed by GSK, it was also being manufactured by GSK. We expect that the drug substance transferred from GSK under the GSK Agreement will be sufficient for us to complete our planned Phase 3 pivotal program, and we have contracted with third parties to fill, finish, supply, store and distribute intepirdine for this program. We also will rely on third-party manufacturers to supply us with sufficient quantities of intepirdine to be used, if approved, for the commercialization of intepirdine.

Under the Arena Development Agreement, subject to specified exceptions, Arena remains the sole and exclusive manufacturer of nelotanserin, and we will depend on Arena to manufacture sufficient quantities of nelotanserin for our planned clinical trials. Arena is reliant on its own third-party supplier for the active pharmaceutical ingredient in nelotanserin, and Arena has notified us that it currently does not have an agreement in place for the supply of active pharmaceutical ingredient and is in the process of identifying a new supplier. If we are unable to initiate or continue our relationship with Arena or these other third-party contractors, or if Arena is unable to manufacture and supply nelotanserin to us, whether as a result of its own inability to obtain active pharmaceutical ingredient or otherwise, we could experience significant delays in our development efforts as new manufacturers for our product candidates are located and qualified.

Table of Contents

Further, our reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured product candidates ourselves, including:

- inability to meet our product specifications and quality requirements consistently;
- delay or inability to procure or expand sufficient manufacturing capacity;
- manufacturing and product quality issues related to scale-up of manufacturing;
- costs and validation of new equipment and facilities required for scale-up;
- failure to comply with cGMP and similar foreign standards;
- inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us;
- reliance on a limited number of sources, and in some cases, single sources for product components, such that if we are unable to secure a sufficient supply of these product components, we will be unable to manufacture and sell our product candidates or any future product candidate in a timely fashion, in sufficient quantities or under acceptable terms;
- lack of qualified backup suppliers for those components that are currently purchased from a sole or single source supplier;
- operations of our third-party manufacturers or suppliers could be disrupted by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier or regulatory sanctions related to the manufacture of our or other company's products;
- carrier disruptions or increased costs that are beyond our control; and
- failure to deliver our products under specified storage conditions and in a timely manner.

Any of these events could lead to clinical trial delays, failure to obtain regulatory approval or impact our ability to successfully commercialize our products. Some of these events could be the basis for FDA action, including injunction, recall, seizure, or total or partial suspension of production.

We intend to rely on third parties to conduct, supervise and monitor our non clinical studies and our clinical trials, and if those third parties perform in an unsatisfactory manner, it may harm our business.

We intend to rely on CROs and non clinical and clinical trial sites to ensure the proper and timely conduct of our non clinical studies and our clinical trials, and we expect to have limited influence over their actual performance.

We intend to rely upon CROs to monitor and manage data for our clinical programs, as well as the execution of future nonclinical studies. We expect to control only certain aspects of our CROs' activities. Nevertheless, we will be responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards and our reliance on the CROs does not relieve us of our regulatory responsibilities. We and our CROs will be required to comply with Good laboratory practices or GLPs and GCPs, which are regulations and guidelines enforced by the FDA and are also required by the competent authorities of the member states of the European Economic Area and comparable foreign regulatory authorities in the form of International Conference on Harmonization guidelines for any of our product candidates that are in nonclinical and clinical development. The regulatory authorities enforce GCPs through periodic inspections of trial sponsors, principal investigators and clinical trial sites. If we or our CROs fail to comply with GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. Accordingly, if we or our CROs fail to comply with these regulations or fail to recruit a sufficient number of subjects, we may be required to repeat clinical trials, which would delay the relevant regulatory approval process.

Table of Contents

Our CROs will not be our employees, and we will not control whether or not they devote sufficient time and resources to our clinical and nonclinical programs. These CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials, or other drug development activities which could harm our competitive position. We face the risk of potential unauthorized disclosure or misappropriation of our intellectual property by CROs, which may reduce our trade secret and intellectual property protection and allow our potential competitors to access and exploit our proprietary technology. If our CROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for any other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize, any product candidate that we develop. As a result, our financial results and the commercial prospects for any product candidate that we develop could be harmed, our costs could increase, and our ability to generate revenues could be delayed.

If our relationships with these CROs terminate, we may not be able to enter into arrangements with alternative CROs or do so on commercially reasonable terms. Switching or adding additional CROs involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we intend to carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have an adverse impact on our business, financial condition and prospects.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain patent protection for our technology and products or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our drug development programs and product candidates. Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries for our product candidates and any future product candidates. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our development programs and product candidates. The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our current product candidates or any future product candidate in the United States or in other foreign countries.

There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents do successfully issue and even if such patents cover our product candidates, third parties may challenge their validity, enforceability or scope, which may result in such patents being narrowed, invalidated, or held unenforceable. Any successful opposition to these patents or any other patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of any product candidates or companion diagnostic that we may develop. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate and companion diagnostic under patent protection could be reduced.

If the patent applications we hold or have in-licensed with respect to our development programs and product candidates fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our product candidates, it could dissuade companies from collaborating with us to develop product candidates, and threaten our ability to commercialize, future drugs. Any such outcome could have a materially adverse effect on our business.

Table of Contents

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does. Publications of discoveries in scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or whether we were the first to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to United States patent law. These include provisions that affect the way patent applications are prosecuted and may also affect patent litigation. The USPTO recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have an adverse effect on our business and financial condition.

Moreover, we may be subject to a third party pre-issuance submission of prior art to the USPTO, or become involved in opposition, derivation, reexamination, inter partes review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly against us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates. 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 the United States and abroad. 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 to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Moreover, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Without patent protection for our current or future product candidates, we may be open to competition from generic versions of such products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

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

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and other foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign national or international patent

agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of patent rights include, but are not limited to, failure to timely file national and regional stage patent applications based on an international patent application, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, our competitors might be able to enter the market, which would have an adverse effect on our business.

Table of Contents

Third party claims or litigation alleging infringement of patents or other proprietary rights or seeking to invalidate patents or other proprietary rights, may delay or prevent the development and commercialization of our product candidates.

Our commercial success depends in part on our avoiding infringement and other violations of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, derivation and administrative law proceedings, inter partes review, and post-grant review before the USPTO, as well as oppositions and similar processes in foreign jurisdictions. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we and our collaborators are developing product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, and as we gain greater visibility and market exposure as a public company, the risk increases that our product candidates or other business activities may be subject to claims of infringement of the patent and other proprietary rights of third parties. Third parties may assert that we are infringing their patents or employing their proprietary technology without authorization. We have conducted searches for information in support of patent protection and otherwise evaluated the patent landscape for our product candidates, and, based on these searches and evaluations to date, we do not believe that there are valid patents which contain granted claims that could be asserted with respect to our product. However, we may be incorrect.

There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire. Similarly, if any third-party patent were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patent may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all. In addition, we may be subject to claims that we are infringing other intellectual property rights, such as trademarks or copyrights, or misappropriating the trade secrets of others, and to the extent that our employees, consultants or contractors use intellectual property or proprietary information owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful infringement or other intellectual property claim against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our affected products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates, and we have done so from time to time. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize one or more of our product candidates, which could harm our business significantly. We cannot provide any assurances that third-party patents do not exist which might be enforced against our drugs or product candidates, resulting in either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties.

Table of Contents

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

Competitors may infringe or otherwise violate our patents, the patents of our licensors or our other intellectual property rights. To counter infringement or unauthorized use, we may be required to file legal claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing. The initiation of a claim against a third party may also cause the third party to bring counter claims against us such as claims asserting that our patents are invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, non-enablement or lack of statutory subject matter. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant material information from the USPTO, or made a materially misleading statement, during prosecution. Third parties may also raise similar validity claims before the USPTO in post-grant proceedings such as ex parte reexaminations, inter partes review, or post-grant review, or oppositions or similar proceedings outside the United States, in parallel with litigation or even outside the context of litigation. The outcome following legal assertions of invalidity and unenforceability is unpredictable. We cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. For the patents and patent applications that we have licensed, we may have limited or no right to participate in the defense of any licensed patents against challenge by a third party. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of any future patent protection on our current or future product candidates. Such a loss of patent protection could harm our business.

We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States. Our business could be harmed if in litigation the prevailing party does not offer us a license on commercially reasonable terms. Any litigation or other proceedings to enforce our intellectual property rights may fail, and even if successful, may result in substantial costs and distract our management and other employees.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have an adverse effect on the price of our common shares.

Changes in U.S. patent law or the patent law of other countries could diminish the value of patents in general, thereby impairing our ability to protect our products.

The United States has recently enacted and implemented wide-ranging patent reform legislation. The U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce patents that we have licensed or that we might obtain in the future. Similarly, changes in patent law and regulations in other countries or jurisdictions or changes in the governmental bodies that enforce them or changes in how the relevant governmental authority enforces patent laws or regulations may weaken our ability to obtain new patents or to enforce patents that we have licensed or that we may obtain in the future.

We may not be able to protect our intellectual property rights throughout the world, which could impair our business.

Filing, prosecuting and defending patents covering our product candidates throughout the world would be prohibitively expensive. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we may obtain patent protection, but where patent enforcement is not as strong as that in the United States. These products may compete with our products in jurisdictions where we do not have any issued or licensed patents and any future patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing.

Table of Contents

Our reliance on third parties requires us to share our trade secrets and other proprietary information, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed. Because we expect to rely on third parties to manufacture our product candidates, and we expect to collaborate with third parties on the development of our product candidates, we must, at times, share trade secrets and other proprietary information with them. We also conduct joint research and development programs that may require us to share trade secrets and other proprietary information under the terms of our research and development partnerships or similar agreements. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such proprietary information becomes known by our competitors, is inadvertently incorporated into the technology of others, or is disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have an adverse effect on our business and results of operations.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets, although our agreements may contain certain limited publication rights. Despite our efforts to protect our confidential information, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any of our third-party collaborators. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of their former employers or other third parties.

We employ individuals who were previously employed at other biotechnology or pharmaceutical companies. Although we seek to protect our ownership of intellectual property rights by ensuring that our agreements with our employees, collaborators and other third parties with whom we do business include provisions requiring such parties to assign rights in inventions to us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed confidential information of our employees', consultants' or independent contractors' former employers, clients, or other third parties. We may also be subject to claims that former employers or other third parties have an ownership interest in our patents. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and if we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Even if we are successful, litigation could result in substantial cost and be a distraction to our management and other employees.

Table of Contents

Risks Related to Our Common Shares

An active trading market for our common shares may not be sustained.

Prior to our IPO, there was no public market for our common shares. Although our common shares are listed on the NYSE, we cannot assure you that an active trading market for our common shares will continue to develop or be sustained. As a result of RSL owning approximately 75.6% of our common shares, trading in our common shares may be less liquid than the shares of companies with broader public ownership. If an active market for our common shares is not sustained, you may not be able to sell your shares quickly or at the market price. An inactive market may also impair our ability to raise capital to continue to fund operations by selling common shares and may impair our ability to acquire other companies or technologies by using our common shares as consideration.

The market price of our common shares is likely to be highly volatile, and you may lose some or all of your investment.

The market price of our common shares is likely to be highly volatile and may be subject to wide fluctuations in response to a variety of factors, including the following:

- any delay in the commencement, enrollment and ultimate completion of clinical trials;
- results of clinical trials of our product candidates or those of our competitors;
- any delay in filing applications for marketing approval and any adverse development or perceived adverse development with respect to applicable regulatory authorities' review of those applications;
- failure to successfully develop and commercialize our current product candidates or any future product candidate;
- inability to obtain additional funding;
- regulatory or legal developments in the United States and other countries applicable to our product candidates;
- adverse regulatory decisions;
- changes in the structure of healthcare payment systems;
- inability to obtain adequate product supply for our current product candidates or any future product candidate, or the inability to do so at acceptable prices;
- introduction of new products, services or technologies by our competitors;
- failure to meet or exceed financial projections we provide to the public;
- failure to meet or exceed the estimates and projections of the investment community;
- changes in the market valuations of similar companies;
- market conditions in the pharmaceutical and biotechnology sectors, and the issuance of new or changed securities analysts' reports or recommendations;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- significant lawsuits, including patent or shareholder litigation, and disputes or other developments relating to our proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- additions or departures of key scientific or management personnel;
- sales of our common shares by us or our shareholders in the future;
- trading volume of our common shares;
- general economic, industry and market conditions; and
- the other factors described in this "Risk Factors" section.

Table of Contents

In addition, the stock markets have experienced extreme price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many companies. These fluctuations have often been unrelated or disproportionate to the operating performance of those companies. Broad market and industry factors, as well as general economic, political, regulatory and market conditions, may negatively affect the market price of our common shares, regardless of our actual operating performance.

Volatility in our share price could subject us to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because pharmaceutical companies have experienced significant share price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

We are a "controlled company" within the meaning of the applicable rules of the New York Stock Exchange and, as a result, qualify for exemptions from certain corporate governance requirements. If we rely on these exemptions, you will not have the same protections afforded to shareholders of companies that are subject to such requirements. RSL controls a majority of the voting power of our outstanding common shares. As a result, we are a "controlled company" within the meaning of the New York Stock Exchange, or NYSE, corporate governance requirements. Under these rules, a company of which more than 50% of the voting power for the election of directors is held by an individual, group or another company is a "controlled company" and may elect not to comply with certain corporate governance requirements, including the requirements:

- that a majority of its board of directors consists of independent directors;
- for an annual performance evaluation of the nominating and corporate governance and compensation committees;
- to have a nominating and corporate governance committee that is composed entirely of independent directors with a written charter addressing the committee's purpose and responsibilities; and
- to have a compensation committee that is composed entirely of independent directors with a written charter addressing the committee's purpose and responsibility.

We have elected to use certain of these exemptions and we may continue to use all or some of these exemptions in the future. As a result, you may not have the same protections afforded to shareholders of companies that are subject to all of the NYSE corporate governance requirements.

RSL owns a significant percentage of our common shares and is able to exert significant control over matters subject to shareholder approval.

Based on common shares outstanding as of December 31, 2016, RSL beneficially owns approximately 75.6% of the voting power of our outstanding common shares and has the ability to substantially influence us through this ownership position. For example, RSL may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. RSL's interests may not always coincide with our corporate interests or the interests of other shareholders, and it may act in a manner with which you may not agree or that may not be in the best interests of our other shareholders. So long as RSL continues to own a significant amount of our equity, it will continue to be able to strongly influence or effectively control our decisions.

If securities or industry analysts do not publish research or reports about our business, or publish negative reports about our business, our share price and trading volume could decline.

The trading market for our common shares will depend, in part, on the research and reports that securities or industry analysts publish about us or our business. We do not have any control over these analysts. If our financial performance fails to meet analyst estimates or one or more of the analysts who cover us downgrade our common shares or change their opinion of our common shares, our share price would likely decline. If one or more of these analysts cease coverage of our company or fail to regularly publish reports on us, we could lose visibility in the financial markets, which could cause our share price or trading volume to decline.

Table of Contents

Because we do not anticipate paying any cash dividends on our common shares in the foreseeable future, capital appreciation, if any, would be your sole source of gain.

We have never declared or paid any cash dividends on our common shares. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. We are also subject to Bermuda legal constraints that may affect our ability to pay dividends on our common shares and make other payments. Additionally, our ability to pay dividends is also currently restricted by the terms of our Loan Agreement with Hercules. As a result, capital appreciation, if any, of our common shares would be your sole source of gain on an investment in our common shares for the foreseeable future.

Future sales of our common shares, or the perception that such sales may occur, could depress our share price, even if our business is doing well.

Sales of a substantial number of our common shares in the public market, or the perception by investors that our stockholders intend to sell substantial amounts of our common stock in the public market, could depress the market price of our common shares, even if our business is doing well. Such a decrease in our share price could in turn impair our ability to raise capital through the sale of additional equity securities.

All of the shares sold in our IPO, as well as shares issued upon the exercise of options granted to persons other than our officers and directors, are freely transferable without restrictions or further registration under the Securities Act. 75,000,000 shares outstanding, representing a majority of our common stock, are held by RSL. If RSL were to sell a substantial portion of these shares, or if the market perceived that RSL intends to sell these shares, it could negatively affect our share price. Prior to RSL's corporate reorganization and recapitalization in December 2015, any decision by RSL to sell or otherwise dispose of our shares required the unanimous agreement of all of the directors of RSL, including Vivek Ramaswamy, our principal executive officer. Subsequent to RSL's corporate reorganization and recapitalization in December 2015, any such decision no longer requires a unanimous vote of RSL's directors, meaning that all or a portion of the shares of our common stock held by RSL may be sold without Vivek Ramaswamy's consent. However, any such sales must still be made in compliance with the Securities Act and the rules and regulations thereunder, which could limit the number of our shares that RSL could sell in any 90-day period.

We have filed registration statements on Form S-8 under the Securities Act to register the common shares that may be issued under our equity incentive plans from time to time. Shares registered under these registration statements are available for sale in the public market subject to vesting arrangements and exercise of options, as well as Rule 144 in the case of our affiliates. We also filed a "shelf" registration statement on Form S-3 under the Securities Act in December 2016, allowing us, from time to time, to offer up to \$750,000,000 of any combination of registered common shares, preferred shares, debt securities and warrants.

We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to compliance with our public company responsibilities and corporate governance practices. As a public company, and particularly after we are no longer an emerging growth company, we will incur significant legal, accounting and other expenses. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the NYSE and other applicable securities rules and regulations impose various requirements on public companies. Our management and other personnel expect to devote a substantial amount of time to compliance with these requirements. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain directors' and officers' liability insurance, which could make it more difficult for us to attract and retain qualified members of our Board of Directors.

We previously identified a material weakness in our internal control over financial reporting. Although we believe this material weakness has since been remediated, we may identify additional material weaknesses in the future or otherwise fail to maintain an effective system of internal control over financial reporting, which may result in material misstatements of our financial statements or cause us to fail to meet our reporting obligations.

In connection with the preparation of our financial statements as of and for the period from October 31, 2014 (date of inception) to March 31, 2015, we and our independent registered public accounting firm identified a material weakness in our internal control over financial reporting, as defined in the standards established by the Public Company Accounting Oversight Board of the United States. A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected on a timely basis.

Table of Contents

We did not design or maintain an effective control environment because we did not maintain a sufficient complement of personnel with an appropriate level of knowledge of accounting, experience and training commensurate with our financial reporting requirements. This material weakness resulted in material audit adjustments related to the affiliate charge for share-based compensation. Our limited personnel also resulted in our inability to consistently establish appropriate authorities and responsibilities in pursuit of our financial reporting objectives, as demonstrated by, among other things, our insufficient segregation of duties in our finance and accounting functions. During this time, we relied on RSI for information systems and financial and accounting support. RSI had limited staff and performed nearly all aspects of our financial reporting process, including, but not limited to, accessing the underlying accounting records and systems, posting and recording journal entries and taking responsibility for the preparation of the financial statements.

During the year ended March 31, 2016, we implemented processes and procedures intended to mitigate the identified material weakness. This included the expansion of our staff by hiring a full-time principal financial officer and principal accounting officer. We have also hired additional finance and accounting personnel with appropriate training to build our financial management and reporting infrastructure. We formalized and implemented our accounting policies and internal controls and the related documentation, including for share-based compensation. We believe that, as a result, we had fully remediated the material weakness discussed above as of March 31, 2016. However, we cannot assure you that the measures we have taken to date, or any measures we may take in the future, will be sufficient to identify or prevent future material weaknesses. If other material weaknesses or other deficiencies occur, our ability to accurately and timely report our financial position could be impaired, which could result in late filings of our annual and quarterly reports under the Exchange Act, restatements of our consolidated financial statements, a decline in our stock price, suspension or delisting of our common stock from the NYSE, and could adversely affect our reputation, results of operations and financial condition.

As a public company, we will be obligated to develop and maintain proper and effective internal controls over financial reporting, and any failure to maintain the adequacy of these internal controls may adversely affect investor confidence in our company and, as a result, the value of our common shares.

The Sarbanes-Oxley Act of 2002, or Sarbanes-Oxley, requires, among other things, that we assess the effectiveness of our internal control over financial reporting annually and disclosure controls and procedures quarterly. In particular, beginning with the fiscal year ending on March 31, 2017, we must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting, as required by Section 404 of Sarbanes-Oxley, or Section 404. Section 404 of Sarbanes-Oxley also generally requires an attestation from our independent registered public accounting firm on the effectiveness of our internal control over financial reporting. However, for as long as we remain an “emerging growth company” as defined in the JOBS Act, we intend to utilize the provision exempting us from the requirement that our independent registered public accounting firm provide an attestation on the effectiveness of our internal control over financial reporting.

We are beginning the costly and challenging process of compiling the system and processing documentation necessary to perform the evaluation needed to comply with Section 404, and we may not be able to complete our evaluation, testing and any required remediation in a timely fashion. Our compliance with Section 404 will require that we incur substantial accounting expense and expend significant management efforts. We have only recently engaged a third-party service provider for our internal audit function, and we may hire additional accounting and financial staff with appropriate public company experience and technical accounting knowledge to assist with compiling the system and process documentation necessary for our management to perform the evaluation needed to comply with Section 404.

During the evaluation and testing process, if we identify one or more material weaknesses in our internal control over financial reporting, we will be unable to assert that our internal control over financial reporting is effective. We cannot assure you that there will not be material weaknesses or other deficiencies in our internal control over financial reporting in the future. Any failure to maintain internal control over financial reporting could severely inhibit our ability to accurately report our financial condition or results of operations. If we are unable to conclude that our internal control over financial reporting is effective, or if our independent registered public accounting firm determines we have a material weakness or other deficiency in our internal control over financial reporting once that firm begins its Section 404 audits of internal control over financial reporting, investors could lose confidence in the accuracy and completeness of our financial reports, the market price of our common shares could decline, and we could be subject to sanctions or investigations by the NYSE, the SEC or other regulatory authorities. Failure to remedy any material weakness in our internal control over financial reporting, or to implement or maintain other effective control systems required of public companies, could also restrict our future access to the capital markets.

Table of Contents

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

We are an emerging growth company, as defined in the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including exemption from compliance with the auditor attestation requirements of Section 404, reduced disclosure obligations regarding executive compensation and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved. We will remain an emerging growth company until the earlier of (1) March 31, 2021, (2) the last day of the fiscal year in which we have total annual gross revenue of at least \$1.0 billion, (3) the date on which we are deemed to be a large accelerated filer, which means the market value of our common shares that are held by non-affiliates exceeds \$700.0 million as of the prior September 30, the end of our second fiscal quarter, and (4) the date on which we have issued more than \$1.0 billion in non-convertible debt during the prior three-year period.

In addition, under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Even after we no longer qualify as an emerging growth company, we may still qualify as a “smaller reporting company” which would allow us to take advantage of many of the same exemptions from disclosure requirements including exemption from compliance with the auditor attestation requirements of Section 404 and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements.

We cannot predict if investors will find our common shares less attractive because we may rely on these exemptions. If some investors find our common shares less attractive as a result, there may be a less active trading market for our common shares and our share price may be more volatile.

We are a Bermuda company and it may be difficult for you to enforce judgments against us or our directors and executive officers.

We are a Bermuda exempted company. As a result, the rights of our shareholders are governed by Bermuda law and our memorandum of association and bye-laws. The rights of shareholders under Bermuda law may differ from the rights of shareholders of companies incorporated in another jurisdiction. It may be difficult for investors to enforce in the United States judgments obtained in U.S. courts against us based on the civil liability provisions of the U.S. securities laws. It is doubtful whether courts in Bermuda will enforce judgments obtained in other jurisdictions, including the United States, against us or our directors or officers under the securities laws of those jurisdictions or entertain actions in Bermuda against us or our directors or officers under the securities laws of other jurisdictions. Bermuda law differs from the laws in effect in the United States and may afford less protection to our shareholders. We are organized under the laws of Bermuda. As a result, our corporate affairs are governed by the Bermuda Companies Act 1981, as amended, or the Companies Act, which differs in some material respects from laws typically applicable to U.S. corporations and shareholders, including the provisions relating to interested directors, amalgamations, mergers and acquisitions, takeovers, shareholder lawsuits and indemnification of directors. Generally, the duties of directors and officers of a Bermuda company are owed to the company only. Shareholders of Bermuda companies typically do not have rights to take action against directors or officers of the company and may only do so in limited circumstances. Shareholder class actions are not available under Bermuda law. The circumstances in which shareholder derivative actions may be available under Bermuda law are substantially more proscribed and less clear than they would be to shareholders of U.S. corporations. The Bermuda courts, however, would ordinarily be expected to permit a shareholder to commence an action in the name of a company to remedy a wrong to the company where the act complained of is alleged to be beyond the corporate power of the company or illegal, or would result in the violation of the company’s memorandum of association or bye-laws. Furthermore, consideration would be given by a Bermuda court to acts that are alleged to constitute a fraud against the minority shareholders or, for instance, where an act requires the approval of a greater percentage of the company’s shareholders than those who actually approved it.

Table of Contents

When the affairs of a company are being conducted in a manner that is oppressive or prejudicial to the interests of some shareholders, one or more shareholders may apply to the Supreme Court of Bermuda, which may make such order as it sees fit, including an order regulating the conduct of the company's affairs in the future or ordering the purchase of the shares of any shareholders by other shareholders or by the company. Additionally, under our bye-laws and as permitted by Bermuda law, each shareholder has waived any claim or right of action against our directors or officers for any action taken by directors or officers in the performance of their duties, except for actions involving fraud or dishonesty. In addition, the rights of our shareholders and the fiduciary responsibilities of our directors under Bermuda law are not as clearly established as under statutes or judicial precedent in existence in jurisdictions in the United States, particularly the State of Delaware. Therefore, our shareholders may have more difficulty protecting their interests than would shareholders of a corporation incorporated in a jurisdiction within the United States.

There are regulatory limitations on the ownership and transfer of our common shares.

Common shares may be offered or sold in Bermuda only in compliance with the provisions of the Companies Act and the Bermuda Investment Business Act 2003, which regulates the sale of securities in Bermuda. In addition, the Bermuda Monetary Authority must approve all issues and transfers of shares of a Bermuda exempted company. However, the Bermuda Monetary Authority has, pursuant to its statement of June 1, 2005, given its general permission under the Exchange Control Act 1972 and related regulations for the issue and free transfer of our common shares to and among persons who are non-residents of Bermuda for exchange control purposes as long as the shares are listed on an appointed stock exchange, which includes the NYSE. This general permission would cease to apply if we were to cease to be listed on the NYSE.

We have anti-takeover provisions in our bye-laws that may discourage a change of control.

Our bye-laws contain provisions that could make it more difficult for a third party to acquire us without the consent of our Board of Directors. These provisions provide for:

- a classified Board of Directors with staggered three-year terms;
- directors only to be removed for cause;
- an affirmative vote of 66 2/3% of our voting shares for certain "business combination" transactions that have not been approved by our Board of Directors;
- restrictions on the time period in which directors may be nominated; and
- our Board of Directors to determine the powers, preferences and rights of our preference shares and to issue the preference shares without shareholder approval.

These anti-takeover defenses could discourage, delay or prevent a transaction involving a change in control of our company and may prevent our shareholders from receiving the benefit from any premium to the market price of our common shares offered by a bidder in a takeover context. Even in the absence of a takeover attempt, the existence of these provisions may adversely affect the prevailing market price of our common shares if the provisions are viewed as discouraging takeover attempts in the future. These provisions could also discourage proxy contests, make it more difficult for you and other shareholders to elect directors of your choosing and cause us to take corporate actions other than those you desire.

We may reduce the voting power of your common shares without your consent.

Under our amended and restated bye-laws, in the event that any U.S. person holds, directly, indirectly or constructively, 9.5% or more of the total voting power of our issued share capital, excluding any U.S. person that held, directly, indirectly or constructively, 9.5% or more of the total voting power of issued share capital immediately prior to the closing of our IPO, the aggregate votes conferred by the common shares held by such person (or by any person through which such U.S. person indirectly or constructively holds shares) will be reduced by our Board of Directors to the extent necessary such that the common shares held, directly, indirectly or constructively, by such U.S. person will constitute less than 9.5% of the voting power of all issued and outstanding shares. RSL, certain of its affiliates, and Vivek Ramaswamy, our principal executive officer, will not be subject to these provisions. Further, our Board of Directors may determine that shares shall carry different or no voting rights as it reasonably determines, based on the advice of counsel, to be appropriate to (1) avoid the existence of any U.S. person who holds 9.5% or more of the total voting power of our issued share capital or (2) avoid adverse tax, legal or regulatory consequences to us, any subsidiary of ours or any holder of our common shares or its affiliates.

Table of Contents

These provisions may discourage potential investors from acquiring a stake or making a significant investment in our company as well as discourage a takeover attempt, which may prevent our shareholders from receiving the benefit of any such transactions as well as adversely affect the prevailing market price of our common shares if viewed as discouraging takeover attempts in the future.

We may become subject to unanticipated tax liabilities and higher effective tax rates.

We are incorporated under the laws of Bermuda, where we are not subject to any income or withholding taxes. We may, however, become subject to income, withholding or other taxes in certain jurisdictions by reason of our activities and operations, and it is also possible that taxing authorities in any such jurisdictions could assert that we are subject to greater taxation than we currently anticipate. Any such non-Bermudan tax liability could materially adversely affect our results of operations. For example, Axovant Sciences GmbH will be the principal operating company for conducting our business and is the entity that holds our intellectual property rights, including for intepirdine and nelotanserin. The establishment of this Swiss entity as our principal operating company and the transfer of our intellectual property rights to this entity may result in a higher overall effective tax rate.

The intended tax effects of our corporate structure and intercompany arrangements depend on the application of the tax laws of various jurisdictions and on how we operate our business.

We and RSL, our principal shareholder, are based in Bermuda, and we currently have subsidiaries in the United Kingdom, Switzerland and the United States. If we succeed in growing our business, we expect to conduct increased operations through our subsidiaries in various countries and tax jurisdictions, in part through intercompany service agreements between us, our parent company and our subsidiaries. In that case, our corporate structure and intercompany transactions, including the manner in which we develop and use our intellectual property, will be organized so that we can achieve our business objectives in a tax-efficient manner and in compliance with applicable transfer pricing rules and regulations. If two or more affiliated companies are located in different countries or tax jurisdictions, the tax laws and regulations of each country generally will require that transfer prices be the same as those between unrelated companies dealing at arms' length and that appropriate documentation is maintained to support the transfer prices. While we believe that we operate in compliance with applicable transfer pricing laws and intend to continue to do so, our transfer pricing procedures are not binding on applicable tax authorities.

Significant judgment is required in evaluating our tax positions and determining our provision for income taxes. During the ordinary course of business, there are many transactions and calculations for which the ultimate tax determination is uncertain. For example, our effective tax rates could be adversely affected by changes in foreign currency exchange rates or by changes in the relevant tax, accounting and other laws, regulations, principles and interpretations. As we intend to operate in numerous countries and taxing jurisdictions, the application of tax laws can be subject to diverging and sometimes conflicting interpretations by tax authorities of those jurisdictions. It is not uncommon for taxing authorities in different countries to have conflicting views, for instance, with respect to, among other things, the manner in which the arm's length standard is applied for transfer pricing purposes, or with respect to the valuation of intellectual property. In addition, tax laws are dynamic and subject to change as new laws are passed and new interpretations of the law are issued or applied. In particular, there is uncertainty as to any future U.S. tax legislation on corporate tax rates but also the U.S. tax consequences of income derived from intellectual property held overseas in low tax jurisdictions.

If tax authorities in any of these countries were to successfully challenge our transfer prices as not reflecting arms' length transactions, they could require us to adjust our transfer prices and thereby reallocate our income to reflect these revised transfer prices, which could result in a higher tax liability to us. In addition, if the country from which the income is reallocated does not agree with the reallocation, both countries could tax the same income, resulting in potentially double taxation. If tax authorities were to allocate income to a higher tax jurisdiction, subject our income to double taxation or assess interest and penalties, it would increase our consolidated tax liability, which could adversely affect our financial condition, results of operations and cash flows.

Changes in our effective tax rate may reduce our net income in future periods.

Our tax position could be adversely impacted by changes in tax rates, tax laws, tax practice, tax treaties or tax regulations or changes in the interpretation thereof by the tax authorities in Europe, the United States, Bermuda and other jurisdictions as well as being affected by certain changes currently proposed by the OECD and their action plan

on Base Erosion and Profit Shifting. Such changes may become more likely as a result of recent economic trends in the jurisdictions in which we operate, particularly if such trends continue. If such a situation were to arise, it could adversely impact our tax position and our effective tax rate. Failure to manage the risks associated with such changes, or misinterpretation of the laws providing such changes, could result in costly audits, interest, penalties and reputational damage, which could adversely affect our business, results of operations and financial condition.

Table of Contents

Our actual effective tax rate may vary from our expectation and that variance may be material. A number of factors may increase our future effective tax rates, including: (1) the jurisdictions in which profits are determined to be earned and taxed; (2) the resolution of issues arising from any future tax audits with various tax authorities; (3) changes in the valuation of our deferred tax assets and liabilities; (4) increases in expenses not deductible for tax purposes, including transaction costs and impairments of goodwill in connection with acquisitions; (5) changes in the taxation of share-based compensation; (6) changes in tax laws or the interpretation of such tax laws, and changes in generally accepted accounting principles; and (7) challenges to the transfer pricing policies related to our structure.

U.S. holders of our common shares may suffer adverse tax consequences if we are characterized as a passive foreign investment company.

Generally, if, for any taxable year, at least 75% of our gross income is passive income, or at least 50% of the value of our assets is attributable to assets that produce passive income or are held for the production of passive income, including cash, we would be characterized as a passive foreign investment company, or PFIC, for U.S. federal income tax purposes. For purposes of these tests, passive income includes dividends, interest, and gains from the sale or exchange of investment property and rents and royalties other than rents and royalties which are received from unrelated parties in connection with the active conduct of a trade or business. If we are characterized as a PFIC, U.S. holders of our common shares may suffer adverse tax consequences, including having gains realized on the sale of our common shares treated as ordinary income rather than capital gain, the loss of the preferential tax rate applicable to dividends received on our common shares by individuals who are U.S. holders, and having interest charges apply to distributions by us and the proceeds of sales of our common shares.

Our status as a PFIC will depend on the composition of our income and the composition and value of our assets (assuming we are not a “controlled foreign corporation,” or a CFC, under Section 957(a) of the Code for the year being tested which may be determined in large part by reference to the market value of our common shares, which may be volatile) from time to time. Our status may also depend, in part, on how quickly we utilize the cash proceeds from our IPO in our business. We believe that we were not a CFC prior to our IPO and were not a CFC at any point after our IPO in the taxable year that ended on March 31, 2016. Based on this belief, with respect to the taxable year that ended on March 31, 2016 and foreseeable future taxable years, we believe that we were not a PFIC and presently do not anticipate that we will be a PFIC based upon the expected value of our assets, including any goodwill, and the expected composition of our income and assets. However, our status as a PFIC is a fact-intensive determination made on an annual basis and we cannot provide any assurances regarding our PFIC status for the current or future taxable years.

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

(a) Recent Sales of Unregistered Equity Securities

None.

(b) Use of Proceeds

On June 16, 2015, we closed our initial public offering, or IPO, in which we issued and sold 24,150,000 common shares at a public offering price of \$15.00 per share, including 3,150,000 common shares sold pursuant to the exercise in full of the underwriters’ option to purchase additional shares, for gross proceeds of \$362.2 million. All of the common shares issued and sold in our IPO were registered under the Securities Act pursuant to a registration statement on Form S-1 (Registration No. 333-204073), which was declared effective by the SEC on June 10, 2015. Jefferies LLC, Evercore Group L.L.C., RBC Capital Markets LLC, JMP Securities LLC and Robert W. Baird & Co. acted as underwriters. The net proceeds to us were approximately \$334.5 million, after deducting underwriting discounts and commissions and offering expenses payable by us. Substantially all of the cash proceeds are currently deposited in three banking institutions and are substantially all in excess of insured levels.

No offering expenses were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning ten percent or more of any class of our equity securities or to any other affiliates.

As of December 31, 2016, we have used \$134.1 million of the net proceeds from the IPO primarily to fund the nonclinical and clinical development of intepirdine and nelotanserin, to expand our internal research and development capabilities, and for general corporate purposes.

Such uses are consistent with the planned use of proceeds described in our prospectus dated June 10, 2015 filed with the SEC on June 11, 2015 pursuant to Rule 424(b) under the Securities Act.

Table of Contents

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

On February 14, 2017, in connection with the contribution and assignment of all of our intellectual property rights to ASG, we and our wholly-owned subsidiaries, ASI and ASG, entered into an amended and restated services agreement with RSI, effective as of December 13, 2016. As a result of the amended and restated agreement, ASG was added as a recipient of these services from RSI. On February 14, 2017, ASG also entered into a separate services agreement with RSG, effective as of December 13, 2016, for the provision of services by RSG to ASG in relation to the identification of potential product candidates and project management of clinical trials, as well as other services related to development, administrative and financial activities. Under the terms of both services agreements, we are obligated to pay or reimburse RSI and RSG for the costs they, or third parties acting on their behalf, incur in providing services to us, including administrative and support services as well as research and development services. In addition, we are obligated to pay to RSI and RSG a pre-determined mark-up on the costs incurred directly by RSI and RSG in connection with any general and administrative and research and development services.

Item 6. Exhibits.

The exhibits listed in the Exhibit Index to this Quarterly Report on Form 10-Q are incorporated herein by reference.

Table of Contents

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.

AXOVANT SCIENCES LTD.

By: /s/ Gregory Weinhoff

Gregory Weinhoff

Date February 14, 2017

(Duly Authorized Officer and Principal Financial Officer)

Table of Contents

Exhibit Index

Exhibit Number	Description of Document
3.1	Certificate of Incorporation. (1)
3.2	Memorandum of Association. (2)
3.3	Amended and Restated Bye-laws. (3)
4.1	Warrant Agreement, dated February 2, 2017, issued to Hercules Capital, Inc. (4)
10.1	Amended and Restated Services Agreement, dated February 14, 2017, by and among Roivant Sciences, Inc., Axovant Sciences Ltd., Axovant Sciences, Inc. and Axovant Sciences GmbH
10.2	Services Agreement, dated February 14, 2017, by and among Roivant Sciences GmbH and Axovant Sciences GmbH.
31.1	Certification of Principal Executive Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of Principal Financial Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1*	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2*	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS XBRL	Instance Document
101.SCH XBRL	Taxonomy Extension Schema
101.CAL XBRL	Taxonomy Extension Calculation Linkbase
101.DEF XBRL	Taxonomy Extension Definition Linkbase
101.LAB XBRL	Taxonomy Extension Label Linkbase
101.PRE XBRL	Taxonomy Extension Presentation Linkbase

(1) Incorporated herein by reference to Exhibit 3.1 to the Registrant's Registration Statement on Form S-1 (File No. 333-204073), filed with the Commission on May 11, 2015.

(2) Incorporated herein by reference to Exhibit 3.2 to the Registrant's Registration Statement on Form S-1 (File No. 333-204073), filed with the Commission on May 11, 2015.

(3) Incorporated herein by reference to Exhibit 3.4 to Amendment No. 2 to the Registrant's Registration Statement on Form S-1 (File No. 333-204073), filed with the Commission on June 1, 2015.

(4) Incorporated herein by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K (File No. 001-37418), filed with the Commission on February 3, 2017.

Table of Contents

* These certifications are being furnished solely to accompany this Quarterly Report on Form 10-Q pursuant to 18 U.S.C. Section 1350, and are not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and are not to be incorporated by reference into any filing of the Registrant, whether made before or after the date hereof, regardless of any general incorporation language in such filing.