

Axovant Sciences Ltd.  
Form 10-Q  
August 07, 2017

---

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 June 30, 2017

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.)

Suite 1, 3rd Floor  
11-12 St. James's Square Not Applicable  
London SW1Y 4LB, United Kingdom  
(Address of principal executive offices) (Zip Code)  
Registrant's telephone number, including area code: +44 203 318 9708  
20-22 Bedford Row  
London, United Kingdom  
WC1R 4JS  
(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, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer", "accelerated

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

filer”, “smaller reporting company,” and “emerging growth company” in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer  Accelerated filer

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

Emerging growth company

---

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

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

The number of shares outstanding of the Registrant's common shares, \$0.00001 par value per share, on August 3, 2017, was 107,525,925.

---

---

AXOVANT SCIENCES LTD.  
QUARTERLY REPORT ON FORM 10-Q  
FOR THE QUARTER ENDED JUNE 30, 2017

TABLE OF CONTENTS

	Page
<u>PART I. FINANCIAL INFORMATION</u>	
<u>Item 1. Financial Statements (Unaudited):</u>	
<u>Condensed Consolidated Balance Sheets as of June 30, 2017 and March 31, 2017</u>	<u>4</u>
<u>Condensed Consolidated Statements of Operations for the Three Months Ended June 30, 2017 and 2016</u>	<u>5</u>
<u>Condensed Consolidated Statements of Comprehensive Loss for the Three Months Ended June 30, 2017 and 2016</u>	<u>5</u>
<u>Condensed Consolidated Statement of Shareholders' Equity for the Three Months Ended June 30, 2017</u>	<u>7</u>
<u>Condensed Consolidated Statements of Cash Flows for the Three Months Ended June 30, 2017 and 2016</u>	<u>8</u>
<u>Notes to Condensed Consolidated Financial Statements</u>	<u>9</u>
<u>Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations</u>	<u>17</u>
<u>Item 3. Quantitative and Qualitative Disclosures About Market Risk</u>	<u>31</u>
<u>Item 4. Controls and Procedures</u>	<u>31</u>
<u>PART II. OTHER INFORMATION</u>	
<u>Item 1. Legal Proceedings</u>	<u>32</u>
<u>Item 1A. Risk Factors</u>	<u>33</u>
<u>Item 2. Unregistered Sales of Equity Securities and Use of Proceeds</u>	<u>67</u>
<u>Item 3. Defaults Upon Senior Securities</u>	<u>68</u>
<u>Item 4. Mine Safety Disclosures</u>	<u>68</u>
<u>Item 5. Other Information</u>	<u>68</u>
<u>Item 6. Exhibits</u>	<u>68</u>
<u>SIGNATURES</u>	<u>69</u>

## PART I. FINANCIAL INFORMATION

## Item 1. Financial Statements (Unaudited)

## AXOVANT SCIENCES LTD.

## Condensed Consolidated Balance Sheets

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

	June 30, 2017	March 31, 2017
Assets		
Current assets:		
Cash	\$297,858	\$212,573
Prepaid expenses and other current assets	7,627	6,457
Income tax receivable	1,224	658
Total current assets	306,709	219,688
Property and equipment, net	2,294	142
Deferred tax assets	—	2,709
Total assets	\$309,003	\$222,539
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	\$8,547	\$8,551
Due to Roivant Sciences Ltd. and Roivant Sciences, Inc.	4,466	2,919
Accrued expenses	38,041	34,796
Total current liabilities	51,054	46,266
Long term debt	51,752	51,436
Total liabilities	102,806	97,702
Commitments and contingencies (Note 9)		
Shareholders' equity:		
Common shares, par value \$0.00001 per share, 1,000,000,000 shares authorized, 107,473,925 and 99,163,919 issued and outstanding at June 30, 2017 and March 31, 2017, respectively	1	1
Additional paid-in capital	610,811	459,601
Accumulated deficit	(404,644 )	(335,143 )
Accumulated other comprehensive income	29	378
Total shareholders' equity	206,197	124,837
Total liabilities and shareholders' equity	\$309,003	\$222,539

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



## AXOVANT SCIENCES LTD.

## Condensed Consolidated Statements of Operations

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

	Three Months Ended June 30,	
	2017	2016
Operating expenses:		
Research and development expenses (includes share-based compensation expense of \$6,256 and \$4,964 for the three months ended June 30, 2017 and 2016, respectively)	\$43,712	\$25,276
General and administrative expenses (includes share-based compensation expense of \$9,344 and \$6,597 for the three months ended June 30, 2017 and 2016, respectively)	21,518	12,631
Total operating expenses	65,230	37,907
Other (income) expenses:		
Interest expense	1,874	—
Other income	(357 )	—
Loss before provision for income taxes	(66,747 )	(37,907 )
Income tax expense	2,519	148
Net loss	\$(69,266)	\$(38,055 )
Net loss per common share — basic and diluted	\$(0.65 )	\$(0.38 )
Weighted average common shares outstanding — basic and diluted	106,400,919	99,150,000

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

AXOVANT SCIENCES LTD.

Condensed Consolidated Statements of Comprehensive Loss  
(Unaudited, in thousands)

	Three Months Ended June 30,	
	2017	2016
Net loss	\$(69,266)	\$(38,055)
Other comprehensive loss:		
Foreign currency translation adjustment	(349)	—
Total other comprehensive loss	(349)	—
Comprehensive loss	\$(69,615)	\$(38,055)

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



## AXOVANT SCIENCES LTD.

Condensed Consolidated Statement of Shareholders' Equity  
(Unaudited, in thousands, except share data)

	Common Shares		Additional	Accumulated	Accumulated	Total
	Shares	Amount	Paid-in Capital	Deficit	Other Comprehensive Income	Shareholders' Equity
Balance at March 31, 2017	99,163,919	\$ 1	\$ 459,601	\$(335,143 )	\$ 378	\$ 124,837
Adjustment to adopt ASU 2016-09	—	—	235	(235 )	—	—
Exercise of stock options	556,501	—	747	—	—	747
Stock issued for equity financing, net of underwriting discounts and commissions and offering expenses of \$9.1 million	7,753,505	—	134,628	—	—	134,628
Share-based compensation expense	—	—	13,468	—	—	13,468
Capital contribution —share-based compensation expense	—	—	2,132	—	—	2,132
Foreign currency translation adjustment	—	—	—	—	(349 )	(349 )
Net loss	—	—	—	(69,266 )	—	(69,266 )
Balance at June 30, 2017	107,473,925	\$ 1	\$ 610,811	\$(404,644 )	\$ 29	\$ 206,197

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

## AXOVANT SCIENCES LTD.

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

	Three Months Ended June 30,	
	2017	2016
Cash flows from operating activities:		
Net loss	\$(69,266 )	\$(38,055 )
Adjustments to reconcile net loss to net cash used in operating activities:		
Unrealized currency translation gain	(349 )	—
Share-based compensation	15,600	11,561
Depreciation and amortization	335	11
Deferred tax assets	2,709	—
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(1,170 )	1,565
Accounts payable	(4 )	2,221
Due to Roivant Sciences Ltd. and Roivant Sciences, Inc.	1,547	404
Accrued expenses	3,245	3,553
Income tax receivable	(566 )	174
Net cash used in operating activities	(47,919 )	(18,566 )
Cash flows from investing activities:		
Purchase of property and equipment	(2,171 )	(28 )
Net cash used in investing activities	(2,171 )	(28 )
Cash flows from financing activities:		
Payment of contingent liability	—	(5,000 )
Exercise of stock options	747	—
Cash proceeds from issuance of common shares, net of costs	134,628	—
Net cash provided by (used in) financing activities	135,375	(5,000 )
Net change in cash	85,285	(23,594 )
Cash—beginning of period	212,573	276,251
Cash—end of period	\$297,858	\$252,657
Supplemental disclosure of cash paid:		
Income taxes	\$375	\$4
Interest expense	\$1,512	\$—

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

AXOVANT SCIENCES LTD.

Notes to Condensed Consolidated Financial Statements (Unaudited)

Note 1—Description of Business

Axovant Sciences Ltd., together with its wholly owned subsidiaries (the “Company”), is a clinical-stage biopharmaceutical company focused on developing and commercializing innovative medicines to broadly address multiple forms of dementia and related neurological disorders. The Company is developing a pipeline of late- and early-stage product candidates that focuses on the cognitive, functional and behavioral aspects of debilitating conditions such as Alzheimer’s and Lewy body dementia and other neurological disorders.

The Company is an exempted limited company incorporated under the laws of Bermuda in October 2014 under the name Roivant Neurosciences Ltd. The Company changed its name to Axovant Sciences Ltd. in March 2015. The Company has four wholly owned subsidiaries. Axovant Holdings Limited (“AHL”), a direct wholly owned subsidiary of Axovant Sciences Ltd., was incorporated in England and Wales in August 2016; Axovant Sciences, Inc. (“ASI”), a direct wholly owned subsidiary of AHL, was incorporated in Delaware in February 2015; Axovant Sciences GmbH (“ASG”), a direct wholly owned subsidiary of AHL, was organized in Switzerland in August 2016; and Axovant Sciences America, Inc. (“ASA”), a direct wholly owned subsidiary of AHL, was incorporated in Delaware in July 2017. ASG holds the Company’s intellectual property rights and is the principal operating company for conducting the Company’s 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 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 dementia with Lewy bodies. The Company has determined that it has one operating and reporting segment as it allocates resources and assesses financial performance on a consolidated basis. The Company does not expect to generate revenue unless and until it successfully completes development and obtains regulatory approval for one of its product candidates. The Company believes it currently has sufficient funds to meet its financial needs for at least the next 12 months if it makes significant reductions in spending that would otherwise be required to commercialize intepirdine. The Company may be required to obtain further funding through other public or private offerings of its share capital, debt financing, collaboration and licensing arrangements or other sources. Adequate additional funding may not be available to the Company on acceptable terms, or at all.

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,

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

2017, filed with the Securities and Exchange Commission (“SEC”) on June 13, 2017. 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 months ended June 30, 2017 are not necessarily indicative of the results that may be expected for the year ending March 31, 2018, 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 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, 2017, filed with the SEC on June 13, 2017.

(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, expenses and compensation expense allocated to the Company under its services agreements with Roivant Sciences, Inc. (“RSI”) and Roivant Sciences GmbH (“RSG”), each a wholly owned subsidiary of the Company’s parent company, Roivant Sciences Ltd., 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. An aggregate of 14.5 million common shares, issuable upon exercise of outstanding stock options and a warrant, were not included in the calculation of diluted weighted-average common shares outstanding for the three months ended June 30, 2017 because they were anti-dilutive. Stock options to purchase 7.3 million common shares were not included in the calculation of diluted weighted-average common shares outstanding for the three months ended June 30, 2016 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 include 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. The estimated fair value of the Company's long term debt was \$51.8 million as of June 30, 2017 and was based on current interest rates for similar types of borrowings and is in Level 2 of the fair value hierarchy. See Note 4 for the actual book carrying value of the Company's long term debt at June 30, 2017.

(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 Company has adopted this guidance as of April 1, 2017, using a modified retrospective transition method. As a result of the adoption of this standard, the Company elected to change its policy from estimating forfeitures to recognizing forfeitures when they occur and, as a result, recorded an adjustment of \$235 thousand to accumulated deficit with a corresponding offset to additional paid-in-capital as of April 1, 2017. The other amended requirements of ASU No. 2016-09 did not have a material impact on the Company's condensed consolidated financial statements and related disclosures.

Note 3—Accrued Expenses

As of June 30, 2017 and March 31, 2017, the Company's accrued expenses consisted of the following (in thousands):

	June 30, 2017	March 31, 2017
Research and development expenses	\$29,945	\$27,667
Salaries, bonuses, and other compensation expenses	4,419	3,497
Legal expenses	671	1,271
Other expenses	3,006	2,361
Total accrued expenses	\$38,041	\$34,796

## Note 4 - Long Term Debt

On February 2, 2017, the Company and its subsidiaries, AHL, ASG and ASI entered into a loan and security agreement (as amended on May 24, 2017, the "Hercules 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"). Pursuant to the Hercules Loan Agreement, ASI has issued a guaranty of the Borrowers' obligations under the Hercules Loan Agreement. 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%. 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 Hercules Loan Agreement until September 1, 2018, followed by monthly installments of principal and interest beginning October 1, 2018 through March 1, 2021. The interest-only period may be extended until either March 2019 or September 2019 if, in each case, the Company achieves certain clinical development milestones, as set forth in the Hercules Loan Agreement and if such were to occur, monthly principal and interest payments would commence on April 1, 2019 or October 1, 2019, respectively. In connection with the Hercules 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 April 2017, the Company completed a follow-on public offering of our common shares, from which it raised net proceeds of \$134.6 million, after deducting underwriting discounts and commissions and offering expenses payable by the Company. On May 24, 2017, the Hercules Loan Agreement was amended such that the required minimum amount of unrestricted cash applies commencing on July 1, 2017 and is equal to the lesser of (i) \$35.0 million (the "Applicable Amount") plus certain aged accounts payable amounts (as further defined in the Hercules Loan Agreement) and (ii) the outstanding amount of debt under the Hercules Loan Agreement plus certain aged accounts payable (as further defined in the Hercules Loan Agreement), provided that the Applicable Amount may be lowered to \$30 million upon the achievement of certain clinical and/or financial milestones, and further lowered to \$25 million upon the achievement of certain other clinical and/or financial milestones, each as set forth in the Hercules Loan Agreement. This unrestricted cash covenant would still cease to apply if the Company achieves certain clinical development milestones, as set forth in the Hercules Loan Agreement.

The Hercules Loan Agreement also includes customary events of default. 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 Hercules 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.

In connection with the Hercules 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 Company has accounted for this warrant as an equity instrument since the Warrant is indexed to the Company's common shares and meets the criteria for classification in shareholders' equity. The relative fair value of the Warrant on the date of issuance was approximately \$2.3 million and is treated as a discount to the debt. This amount will be amortized to interest expense under the effective interest method over the life of the Term Loan, which is a period of 48 months. The Company estimated the value of the Warrant using the Black-Scholes model. The key assumptions used to value the Warrant are as follows:

Exercise price	\$ 12.04
Share price on date of issuance	\$ 11.96
Volatility	77.6 %



Risk-free interest rate	2.27	%
Expected dividend yield	—	%
Contractual term (in years)	7	

In addition, at the closing of the Term Loan, the Company paid transaction costs of \$1.5 million, which were recorded as a discount on the debt and will be amortized to interest expense using the effective interest method over the life of the Term Loan, which is a period of 48 months.

Outstanding debt obligations are as follows (in thousands):

	June 30, 2017
Principal amount	\$55,000
Less: unamortized discount and debt issuance costs	(3,248 )
Loan payable less unamortized discount and debt issuance costs	51,752
Less: current maturities	—
Long-term loan payable, net of current maturities	\$51,752

#### Note 5—Related Party Transactions

##### (A) Services Agreements:

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.

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 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 Services Agreements, for the three months ended June 30, 2017 and 2016, the Company incurred expenses of \$2.7 million and \$2.0 million, respectively, inclusive of a mark-up.

##### (B) Family Relationships:

Geetha Ramaswamy, MD, the Vice President, Medical and Scientific Strategy for ASI, is the mother of Vivek Ramaswamy, a director of ASL and the chief executive officer of RSL. Shankar Ramaswamy, MD, the Vice President of Global Medical Affairs 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 \$66,950 and \$65,000 for both Geetha Ramaswamy and Shankar Ramaswamy for the three months ended June 30, 2017 and 2016, respectively. Salary expenses were \$19,313 and \$17,917 for Sarah Friedhoff for the three months ended June 30, 2017 and 2016, respectively.

#### Note 6—Shareholders' Equity

In April 2017, we issued and sold 7,753,505 common shares, including 1,011,326 common shares sold pursuant to the exercise in full of the underwriters' option to purchase additional shares, at an offering price of \$18.54 per common share for gross proceeds of \$143.7 million. The net proceeds to us were approximately \$134.6 million, after deducting underwriting discounts and commissions and offering expenses paid by us.

Note 7—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. In April 2017, the number of common shares authorized for issuance increased automatically to approximately 16.5 million in accordance with the terms of the 2015 Plan. At June 30, 2017, a total of 1.6 million common shares were available for future grant under the 2015 Plan and options to purchase approximately 14.3 million common shares were outstanding under the Plan with a weighted average exercise price of \$13.04.

(A) Stock Options Granted to Employees and Directors:

During the three months ended June 30, 2017 and 2016, the Company granted options to purchase a total of 7.1 million and 2.0 million common shares, 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 \$12.2 million and \$7.9 million, respectively, for the three months ended June 30, 2017 and 2016. At June 30, 2017, total unrecognized compensation expense related to non-vested options was \$123.5 million, which is expected to be recognized over the remaining weighted-average service period of 3.22 years.

During the three months ended June 30, 2017, the Company granted two options, each to purchase 2,000,000 common shares (or an aggregate of 4,000,000 common shares) of the Company, to our Principal Executive Officer, Dr. Hung. The grant date fair value of one of the options to purchase 2,000,000 common shares was estimated using the Black-Scholes option pricing model and the fair value is recognized over the requisite service period. The other option to purchase 2,000,000 common shares is a performance-based award (“Second Option”) which vests over a period of five years, contingent on the achievement of pre-determined performance-based milestones that are related to product development or the achievement of specified market targets. The grant date fair value for this Second Option was estimated using a Monte Carlo valuation model and is recognized using the accelerated method over the requisite service period.

(B) Share-Based Compensation for Related Parties:

(1) Stock Options Granted to Non-Employees:

During the three months ended June 30, 2017 and 2016, the Company granted options to purchase a total of 209,600 and 83,500 common shares, respectively, 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.

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 \$1.0 million and \$0.4 million of share-based compensation expense for the three months ended June 30, 2017 and 2016, respectively. The share-based compensation was recorded as research and development and general and administrative expense in the accompanying condensed consolidated statements of operations. The total remaining unrecognized compensation cost related to the non-vested stock options amounted to \$7.8 million as of June 30, 2017, which is expected to be recognized over the remaining weighted-average service period of 2.26 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.

The RSL common share awards are fair valued on the date of grant and that fair value is recognized over the requisite service period. 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 \$2.1 million and \$3.1 million, respectively, for the three months ended June 30, 2017 and 2016 in relation to the RSL common share awards and options issued by RSL to RSI employees.

(3) Share-Based Compensation for Family Members:

The Company granted Geetha Ramaswamy, Shankar Ramaswamy and Sarah Friedhoff options to purchase 37,500 common shares, 37,500 common shares and 12,500 common shares, respectively, during the three months ended June 30, 2017 as annual stock option grants in their capacities as employees of ASI. The Company recorded aggregate share-based compensation expense of \$1.4 million and \$0.9 million for the three months ended June 30, 2017 and 2016, respectively, in connection with the Company's option grants.

Shankar Ramaswamy, while previously employed by RSI, was also granted RSL common shares. The Company recorded share-based compensation expense of \$0.1 million and \$0.2 million, respectively, for the three months ended June 30, 2017 and 2016 related to the RSL common share awards held by Shankar Ramaswamy, which the Company has recorded as research and development expense in the accompanying condensed consolidated statements of operations. At June 30, 2017, total unrecognized compensation expense related to these non-vested RSL common share awards was \$0.5 million and is expected to be recognized over the remaining weighted-average service period of 1.02 years.

Note 8—Income Taxes

The Company is not subject to taxation under the laws of Bermuda since it was organized as a Bermuda Exempted Limited Company, for which there is no current tax regime. The Company's provision for income taxes is primarily federal, state and local income taxes in the United States. The Company's effective tax rate of (3.8)% and (0.4)% for the three months ended June 30, 2017 and 2016, respectively, differs from the Bermuda federal statutory rate of 0% primarily due to the U.S. permanent unfavorable tax differences, stock compensation windfalls 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 amount which is more likely than not to be realized and records a valuation allowance as necessary.

The Company files income tax returns in the United States for federal, state and local jurisdictions. ASI filed its initial U.S. federal, state and local income tax returns for the fiscal year ended March 31, 2015 in December 2015. The Company is subject to income tax examinations for fiscal year 2015 and forward in all applicable tax jurisdictions.



Note 9—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”), the Hercules Loan Agreement, an amended services agreement with RSI, a separate service agreement with RSG (Refer to Note 5(A)) and a license agreement with Qaam Pharmaceuticals LLC. In addition, the Company has entered into services agreements with third parties for pharmaceutical manufacturing and research activities. The Company expects to enter into other commitments as the business further develops.

During the quarter ended June 30, 2017, there were no other material changes outside the ordinary course of business to the specified contractual obligations set forth in the contractual obligations table included in our Annual Report on Form 10-K for the year ended March 31, 2017.



Item 2.  
of Operations

Management's Discussion and Analysis of Financial Condition and Results

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, 2017 included in our Annual Report on Form 10-K, filed with the Securities and Exchange Commission, or the SEC, on June 13, 2017. Unless the context requires otherwise, references in this report to "Axovant", the "Company," "we," "us," and "our" refer to Axovant Sciences Ltd. and its subsidiaries.

This Quarterly Report on Form 10-Q contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended, or 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 similar expressions or variations, although not all forward-looking statements contain these identifying words. We cannot assure you that the events and circumstances reflected in the forward-looking statements will be achieved or occur and actual results could differ materially from those projected in the forward-looking statements. The forward-looking statements in this Quarterly Report on Form 10-Q include, but are not limited to, statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things:

- the success and timing of our ongoing development programs for intepirdine, nelotanserin, RVT-103 and RVT-104;
- the success of our interactions with international regulatory authorities;
- our plans to develop and commercialize intepirdine, nelotanserin, RVT-103 and RVT-104;
- the anticipated start dates, durations and completion dates of our ongoing and future preclinical studies and clinical trials;
- the anticipated designs of our future clinical studies;
- anticipated future regulatory submissions and the timing of, and our ability to, obtain and maintain regulatory approval for our product candidates;
- our anticipated commercial launch of our key product candidates, intepirdine and nelotanserin;
- the rate and degree of market acceptance and clinical utility of any approved product candidate;
- our ability to quickly and efficiently identify and develop product candidates;
- our commercialization, marketing and manufacturing capabilities and strategy;
- continued service of our key scientific or management personnel;
- our ability to obtain, maintain and enforce intellectual property rights for our drug candidates;
- our anticipated future cash position;
- our estimates regarding our results of operations, financial condition, liquidity, capital requirements, prospects, growth and strategies;
- the success of competing drugs that are or may become available;
- our stated objective of becoming the leading biopharmaceutical company focused on neurology, with an initial emphasis on the treatment of dementia by addressing multiple forms and aspects of this condition.

We have based these forward-looking statements largely on our current expectations and projections about future events, including the responses we expect from the U.S. Food and Drug Administration, or FDA, and other regulatory authorities and financial trends that we believe may affect our financial condition, results of operations, business strategy, preclinical studies and clinical trials and financial needs. Such forward-looking statements are subject to a number of risks, uncertainties, assumptions and other factors known and unknown 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. These risks are not exhaustive. 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. New risk factors emerge from time to time and it is not possible for our management to predict all risk factors, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements. 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 developing and commercializing innovative medicines to broadly address multiple forms of dementia and related neurological disorders. We are developing a pipeline of late- and early-stage product candidates that focuses on the cognitive, functional and behavioral aspects of debilitating conditions such as Alzheimer's and Lewy body dementia and other neurological disorders. Our vision is to become the leading biopharmaceutical company focused on neurology, with an initial emphasis on the treatment of dementia by addressing multiple forms and aspects of this condition.

Our near-term focus is to develop our lead product candidate, intepirdine, a selective 5-HT<sub>6</sub> 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 highly selective 5-HT<sub>2A</sub> receptor inverse agonist, for the treatment of visual hallucinations in patients with Lewy body dementia, or LBD, movement disorder symptoms in patients with DLB and REM behavior disorder, or RBD, in patients with LBD. In addition, we are developing two other product candidates: 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, as potential treatments for patients with Alzheimer's disease or DLB. We intend to evaluate the safety and efficacy of RVT-103 and RVT-104 both alone and in combination with intepirdine.

We were founded in October 2014 and 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 lead product candidates, intepirdine and nelotanserin, into clinical development. In June 2015, we completed our initial public offering, or IPO, from which we raised net proceeds of \$334.5 million, after deducting underwriting discounts and commissions and offering expenses paid by us. In February 2017, we and our subsidiaries entered into a loan and security agreement with Hercules Capital, Inc., from which we raised net proceeds of \$53.5 million. In April 2017, we completed a follow-on public offering of our common shares, from which we raised net proceeds of approximately \$134.6 million, after deducting underwriting discounts and commissions and offering expenses paid by us.

To date, we have not generated any revenue and we recorded net losses of \$69.3 million during the three months ended June 30, 2017 and \$181.0 million for the year ended March 31, 2017. We have determined that we have one operating and reporting segment.

### Our Product Pipeline

The following table summarizes the status of our development programs to which Axovant Sciences GmbH, our wholly owned subsidiary, holds global commercial rights:

Compound	Clinical Indication	Development Stage
Intepirdine	Mild-to-moderate Alzheimer's disease	Phase 3 (MINDSET Study)
	Dementia with Lewy bodies (DLB)	Phase 2b (HEADWAY-DLB Study)
	Gait and balance in Alzheimer's disease, DLB and Parkinson's disease dementia	Phase 2
Nelotanserin	Visual hallucinations in Lewy body dementia (LBD)	Phase 2
	REM behavior disorder in LBD	Phase 2
RVT-103	Alzheimer's disease and DLB	Proof of Concept Study Completed
RVT-104	Alzheimer's disease and DLB	Preparation for Proof of Concept Study

## 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 5.5 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.

### Intepirdine MINDSET Phase 3 Study

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. Patient recruitment for the MINDSET study was completed in January 2017, with a total of 1,315 patients ultimately randomized. 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 patients with mild-to-moderate Alzheimer's disease on a background of stable donepezil therapy. The primary endpoints of the study are changes in scores on the Alzheimer's Disease Assessment Scale-cognitive, or ADAS-cog, and the Alzheimer's Disease Cooperative Study Activities of Daily Living, or ADCS-ADL scales, which have been used as endpoints supporting regulatory approval of currently-marketed Alzheimer's disease treatments in the United States and Europe. We have received a Special Protocol Assessment, or SPA, 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 the results of the prior 684-subject Phase 2b adjunctive therapy study conducted by GSK. We expect to report topline results of the MINDSET study in late September 2017.

### Intepirdine MINDSET Open-Label Extension Study

Subjects completing the MINDSET Phase 3 study are eligible to enter a 12-month open-label extension, or OLE, safety study. In the OLE study, other medications for the treatment of Alzheimer's disease, including memantine and other cholinesterase inhibitors, may be administered in combination with intepirdine. To date, we have observed approximately 93% rollover of eligible, completing patients from the MINDSET study into the OLE study.

### 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 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 estimate 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, a constellation of motor symptoms often found in patients with Parkinson's disease and related movement disorders, including tremor, bradykinesia, and stiffness; sensitivity to neuroleptic (antipsychotic) medications; and 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 on a background of stable standard of care therapy. In addition to the 35-mg dose of intepirdine that is being studied in the MINDSET study, we are evaluating a 70-mg dose of intepirdine in this trial, a higher dose which we believe could have greater activity against the 5-HT<sub>2A</sub> receptor to potentially address visual hallucinations and behavioral disturbances in this patient population. This decision was 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 completed recruitment for the Phase 2b HEADWAY-DLB study in April 2017 and 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.

Falls are a significant issue in the elderly, and 35% and 40% of community-dwelling generally healthy adults over age 65 fall each year. Falls can also impose a significant economic burden on the healthcare system in addition to the serious morbidity and mortality with which they are associated. It is estimated that the annual direct medical costs for fall injuries are approximately \$31 billion. Patients with dementia are more prone to falls due to impaired cognition and gait.

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 the fourth quarter of calendar year 2017.

#### Nelotanserin

##### Overview

In October 2015, we acquired from our parent company, Roivant Sciences Ltd., or RSL, the global rights to nelotanserin, a highly selective inverse agonist of the 5-HT<sub>2A</sub> receptor. Initially, we are investigating and developing nelotanserin to address visual hallucinations in patients with LBD and RBD in patients with LBD. 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.

##### Nelotanserin for Visual Hallucinations in Lewy Body Dementia

LBD includes two similar conditions, DLB and Parkinson's disease dementia. 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 Parkinson's disease dementia, 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.

In January 2016, we initiated a double-blind, randomized, placebo-controlled, cross-over Phase 2 clinical study of nelotanserin in approximately 20 patients with either DLB or Parkinson's disease dementia who had experienced frequent 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 a statistically significant 8.73 point difference in change from baseline at week 4 of a pre-specified primary endpoint of Unified Parkinson's Disease Rating Scale (UPDRS) Parts II+III (p-value = 0.012) during periods in which patients received nelotanserin, as compared to periods during which those same patients received placebo. On the UPDRS Part II, which measures activities of daily living, there was a 1.11 point difference from placebo (p-value = 0.434). On the UPDRS Part III, which measures motor function, there was a 7.92 point difference from placebo (p-value = 0.005). Safety analysis included measurement of the incidence of adverse events. In the interim analysis, secondary endpoints evaluating visual hallucinations did not demonstrate statistically significant differences for nelotanserin relative to placebo.

Based on these preliminary results, we plan to expand patient recruitment beyond the 20 patients already randomized, in order to confirm the treatment benefits observed in the interim results from this ongoing study. We expect to report

results from the expanded study in the fourth quarter of calendar year 2017. In June 2017, we received Fast Track designation from the FDA for nelotanserin for the treatment of visual hallucinations in DLB.

#### Nelotanserin for Parkinsonism in Dementia with Lewy Bodies

Parkinsonism is a core feature of LBD, which includes patients diagnosed with both Parkinson's disease dementia and DLB. Treatment with dopaminergic agents (such as L-DOPA and/or dopamine agonists) is common for patients with Parkinson's disease, but dopaminergic therapy can exacerbate neuropsychiatric symptoms in patients diagnosed with DLB. We believe that there is a need for new therapeutic options for DLB patients that can reduce the burden of motor symptoms without increasing the risk of neuropsychiatric side-effects.

If we are able to confirm the UPDRS improvements observed in DLB patients in the interim results of our ongoing Phase 2 clinical study of nelotanserin, we intend to discuss with the FDA the use of UPDRS as a potential registrational endpoint for the treatment of parkinsonism in patients with DLB. Depending on the FDA feedback, we would potentially pursue a Phase 3 program with nelotanserin in this indication.

#### Nelotanserin for REM Behavior Disorder in Lewy Body Dementia

RBD is a common clinical feature of LBD, and is a condition in which patients can 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 four-week double-blind, randomized, placebo-controlled Phase 2 study in patients with DLB and Parkinson's disease dementia 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 first quarter of calendar year 2018.

#### Nelotanserin Lewy Body Dementia Open-Label Extension Safety Study

Subjects completing either of the nelotanserin Phase 2 studies in LBD described above are eligible to enter a six-month OLE safety study. To date, three subjects in the OLE study exhibited elevated liver function test levels that led to discontinuation of the study drug. These subjects normalized their liver function test levels after they were taken off the study drug, and an independent Data Monitoring Committee, after reviewing their data, recommended continuing both of the nelotanserin Phase 2 studies and the OLE safety study with additional monitoring.

#### 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 in-licensed the rights to develop and commercialize RVT-103 and RVT-104, which are product candidates that combine cholinesterase inhibitors with peripherally acting quaternary amine muscarinic receptor antagonists such as glycopyrrolate or trospium. 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 Dementia with Lewy Bodies

Cholinesterase inhibitors are the standard of care in both Alzheimer's disease and DLB. Despite their widespread use, many patients cannot tolerate the cholinesterase inhibitors because of their cholinergic side effects such as nausea, vomiting and diarrhea. We believe that drugs that can mitigate these cholinergic side effects will allow more patients to receive optimal cholinesterase inhibitor therapy.

We are initially developing 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 conducted a proof-of-concept clinical study designed to characterize the pharmacokinetic profile, safety and tolerability of 5 mg donepezil plus placebo versus 10 mg of donepezil when given concomitantly with one of three different regimens including either glycopyrrolate or trospium. The study measured patient nausea in 48 healthy, elderly subjects using a Visual Analog Scale (VAS) at baseline and once every two hours for eight hours after dosing on Days 1, 3, 7 and 10. On Day 10, the last day of dosing, subjects in the arms receiving 10 mg of donepezil plus either glycopyrrolate or trospium had achieved a 67% to 95% percent reduction in nausea relative to the 5 mg donepezil plus placebo arm, as measured by the peak mean change from baseline on the VAS. Based on these results, we plan to meet with the FDA to discuss additional studies that could support registration of RVT-103. We also plan to initiate a pilot study in the second half of calendar year 2017 for RVT-104, a proposed combination of a higher than currently approved dose of rivastigmine and a peripheral muscarinic receptor antagonist as a potential treatment for patients with Alzheimer's disease and DLB.





## Our Key Agreements

### Asset Purchase Agreement with GlaxoSmithKline for Intepirdine

In December 2014, we acquired the worldwide rights to intepirdine from GSK. Under the GSK Agreement, we made an upfront payment of \$5.0 million and an additional \$5.0 million payment in June 2016. 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.

Our royalty obligations with respect to the GSK Agreement will end, on a product-by-product and country-by-country basis, on the latest of: (1) expiration of the last valid claim of the assigned patents covering the manufacture, use or composition of such product in such country; (2) expiration of regulatory exclusivity for such product in such country; or (3) 12 years from the first commercial sale of such product in such country, or if such country is one of the five major European countries listed in the GSK Agreement, then 12 years from the first commercial sale of such product in at least three such major European countries.

Our royalty payment obligations and milestone payment obligations under the GSK Agreement may be reduced by a portion of royalty payments, and in some cases other payments, made to third parties for rights to certain U.S. patents, in each case subject to a maximum reduction.

### Arena Development Agreement for Nelotanserin

In October 2015, we exercised an option to acquire global rights, title, interest and obligations in and 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 all commercial supplies of nelotanserin from Arena at a fixed price equal to 15% of net sales of nelotanserin. The Arena Development Agreement will remain in effect until terminated: (1) by the parties' mutual agreement; (2) for any reason by us upon 90 days' written notice to Arena; (3) by either party upon written notice for the other party's material breach or insolvency event if such party fails to cure such breach or the insolvency event is not dismissed within the specified cure period; or (4) by Arena if we or our affiliates participate in a challenge to certain Arena patents.

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

In October 2014, we and our wholly owned subsidiary, Axovant Sciences, Inc., or ASI, 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 functions. In February 2017, in connection with the contribution and assignment of all of our intellectual property rights to Axovant Sciences GmbH, or 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 services from RSI. In addition, 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, 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 or ASG, including administrative and support services as well as research and development services. In addition, we are obligated to pay to RSI and RSG at 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 provided directly by RSI and RSG.

Under the services agreement in effect as of December 31, 2016, we incurred expenses of \$2.7 million and \$2.0 million for the three months ended June 30, 2017 and 2016, 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 statements of operations.

#### Venture Debt Financing from Hercules Capital, Inc.

On February 2, 2017, we, AHL, ASG and ASI entered into a loan and security agreement, as amended on May 24, 2017, 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 were 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.

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 with RSI and RSG;
- expenses incurred under 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 preclinical and clinical studies;

costs for planning, developing and conducting 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 Parkinson's disease dementia;

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

costs for planning, developing and conducting clinical studies for product candidates that combine cholinesterase inhibitors with peripheral muscarinic receptor antagonists including RVT-103 and RVT-104;

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

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 other forms of dementia and our ongoing development program for nelotanserin in LBD. We expect our share-based compensation expense to increase as we continue to increase our number of employees and hire additional senior executives.

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 following:

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 with RSI and RSG 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.

### Results of Operations for the Three Months Ended June 30, 2017 and 2016

The following table summarizes our results of operations for the three months ended June 30, 2017 and 2016 (in thousands):

	Three Months Ended June 30,		
	2017	2016	Change
Operating expenses:			
Research and development expenses (includes share-based compensation expense of \$6,256 and \$4,964 for the three months ended June 30, 2017 and 2016, respectively)	\$43,712	\$25,276	\$18,436
General and administrative expenses (includes share-based compensation expense of \$9,344 and \$6,597 for the three months ended June 30, 2017 and 2016, respectively)	21,518	12,631	8,887
Total operating expenses	\$65,230	\$37,907	\$27,323

### Research and Development Expenses

Research and development expenses increased by \$18.4 million, to \$43.7 million, in the three months ended June 30, 2017 compared to the three months ended June 30, 2016, primarily due to increases in expenses for the ongoing MINDSET study. Research and development expenses for the three months ended June 30, 2017 consisted primarily of CRO fees of \$23.3 million, share-based compensation expense of \$6.3 million and CMO fees of \$4.2 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 June 30, 2017 included \$1.8 million related to the RSL common share awards and RSL options issued by RSL to RSI employees.

Research and development expenses were \$25.3 million for the three months ended June 30, 2016, and consisted primarily of share-based compensation of \$5.0 million, CRO fees of \$9.9 million, CMO fees, payments made to consultants and other third party vendors engaged in the development of our product candidates and employee salaries and benefits. The share-based compensation expense included \$2.7 million related to the RSL common share awards and RSL options issued by RSL to RSI employees.

### General and Administrative Expenses

General and administrative expenses increased by \$8.9 million, to \$21.5 million, in the three months ended June 30, 2017 compared to the three months ended June 30, 2016, primarily due to a \$2.7 million increase in share-based compensation expense and higher employee salaries and benefits resulting from increased headcount. General and administrative expenses for the three months ended June 30, 2017 consisted primarily of share-based compensation expense of \$9.3 million and employee salaries and related benefits, legal and professional fees, and direct and indirect costs allocated to us under the services agreements with RSI and RSG. The share-based compensation expense for the

three months ended June 30, 2017 included share-based compensation expense of \$0.3 million for RSL common share awards and RSL options issued to RSI employees.



General and administrative expenses were \$12.6 million for the three months ended June 30, 2016, and consisted primarily of share-based compensation expense of \$6.6 million and employee salaries and related benefits, legal and professional fees, and direct and indirect costs allocated to us under the services agreements with RSI and RSG. The share-based compensation expense for the three months ended June 30, 2016 includes \$0.4 million for RSL common share awards issued to RSI employees.

#### Interest Expense

Interest expense was \$1.9 million for the three months ended June 30, 2017, consisting of interest paid and the amortization of debt discount related to the Hercules Loan Agreement.

There was no interest expense for the three months ended June 30, 2016.

#### Liquidity and Capital Resources

##### Overview

As of June 30, 2017, we had cash totaling \$297.9 million. Subsequent to March 31, 2017, we raised net proceeds of approximately \$134.6 million, after deducting underwriting discounts and commissions and offering expenses payable by us, from the sale of 7,753,505 common shares in a follow-on public offering pursuant to a registration statement on Form S-3 that we filed with the SEC in December 2016. The registration statement permits for the offer and sale of up to \$750.0 million of any combination of registered common shares, preferred shares, debt securities and warrants.

##### Loan and Security Agreement with Hercules Capital, Inc.

On February 2, 2017, we and our wholly owned subsidiaries, Axovant Holdings Limited, or AHL, Axovant Sciences GmbH, or ASG, and Axovant Sciences, Inc., or ASI, entered into a loan and security agreement with Hercules Capital, Inc., or Hercules, as agent and lender. The loan and security agreement was amended on May 24, 2017 and is referred to herein, as amended, as the Hercules Loan Agreement. Pursuant to the Hercules Loan Agreement, we, AHL and ASG, as the borrowers, borrowed an aggregate of \$55.0 million, or the Term Loan, at the closing. ASI issued a guaranty of the borrowers' obligations under the Hercules Loan Agreement, and at the closing, we 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%. 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 Hercules 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 either March 2019 or September 2019 if, in each case, we achieve certain clinical development milestones, respectively, as set forth in the Hercules Loan Agreement. The borrowers' obligations under the Hercules Loan Agreement are secured by a first position lien on substantially all of their and ASI's respective assets, other than intellectual property. If we prepay the loan prior to the Scheduled Maturity Date, we will be obligated to pay Hercules a prepayment charge, based on a percentage of the then-outstanding principal balance, equal to 3.0% if the prepayment occurs within the first 18 months following February 2, 2017, 2.0% if the prepayment occurs after 18 months but prior to 36 months following February 2, 2017, and 1.0% if the prepayment occurs thereafter.

The Hercules 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 Hercules 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 Hercules 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 Hercules 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 Hercules Loan Agreement. In connection with the entry into the Hercules 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.

For the three months ended June 30, 2017, we used \$47.9 million and \$2.2 million of cash in our operating and investing activities, respectively. 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 potentially initiate additional registrational studies to support regulatory approval in Japan;
- continue our open-label extension study of intepirdine for patients completing the MINDSET study;
- continue the intepirdine HEADWAY-DLB study of intepirdine for the treatment of 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 Parkinson's disease dementia;
- 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 Parkinson's disease dementia 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;
- 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. If the MINDSET study is successful, our expenditures will increase significantly and we believe that our existing cash resources will be sufficient to enable us to file our NDA for intepirdine for the treatment of mild-to-moderate Alzheimer's disease and to fund our operating expenses and capital expenditure requirements into the first quarter of calendar year 2018. In the event the MINDSET study fails, we believe that our existing cash resources will be sufficient to enable us to fund our operating expenses and capital expenditure requirements into the fourth quarter of calendar 2018. We have based our estimates on assumptions that may prove to be incorrect, and we could use our available capital resources sooner than we currently 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 share capital, 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 product candidates 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. 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 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.

#### Cash Flows

The following table sets forth a summary of our cash flows for the three months ended June 30, 2017 and 2016 (in thousands):

	Three Months Ended	
	June 30,	
	2017	2016
Net cash used in operating activities	\$(47,919)	\$(18,566)
Net cash used in investing activities	(2,171 )	(28 )
Net cash provided by (used in) financing activities	135,375	(5,000 )

#### Operating Activities

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

For the three months ended June 30, 2017, net cash used in operating activities was \$47.9 million and was primarily attributable to a net loss of \$69.3 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 \$15.6 million of non-cash share-based compensation expense, an increase of \$3.2 million in accrued liabilities and a decrease of \$2.7 million in deferred tax assets. For the three months ended June 30, 2016, net cash used in operating activities was \$18.6 million and was primarily attributable to a net loss of \$38.1 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 \$11.6 million of non-cash share-based compensation expense.

Investing Activities

For the three months ended June 30, 2017 and 2016, net cash used in investing activities was \$2.2 million and \$28,000, respectively, in each case consisting of purchases of furniture and equipment.

#### Financing Activities

For the three months ended June 30, 2017, net cash provided by financing activities was \$135.4 million and consists primarily of net proceeds of \$134.6 million received from the sale of 7,753,505 common shares in a follow-on public offering. For the three months ended June 30, 2016, net cash used in financing activities was \$5.0 million, which represents the deferred payment made to GSK under the terms of the GSK Agreement in June 2016.

#### 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 Hercules Loan Agreement, an amended services agreement with RSI, a separate service agreement with RSG (Refer to Note 5(A)), and a license agreement with Qaam Pharmaceuticals LLC. In addition, the Company has entered into services agreements with third parties for pharmaceutical manufacturing and research activities. The Company expects to enter into other commitments as the business further develops.

As of June 30, 2017, the Company did not have any ongoing material financial commitments, other than pursuant to the GSK Agreement, Arena Development Agreement and Hercules Loan Agreement.

During the quarter ended June 30, 2017, 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, 2017.

#### 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 with RSI and RSG, 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.

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 2 to our consolidated financial statements in our Annual Report on Form 10-K for the year ended March 31, 2017. 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, 2017.

### 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. We are currently evaluating the new standard and its impact on our 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. We have adopted this guidance as of April 1, 2017, using a modified retrospective transition method. As a result of the adoption of this standard, we elected to change our policy from estimating forfeitures to recognizing forfeitures when they occur and, as a result, recorded an adjustment of \$235 thousand to accumulated deficit with a corresponding offset to additional paid-in-capital as of April 1, 2017. The other amended requirements of ASU No. 2016-09 did not have a material impact on our condensed consolidated financial statements and related disclosures.

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 June 30, 2017, we had cash of \$297.9 million, consisting of non-interest bearing deposits denominated in the U.S. dollar and Swiss franc.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Under the supervision of our principal executive officer and principal financial officer, we evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2017, the end of the period covered by this Quarterly Report on Form 10-Q. The term “disclosure controls and procedures” (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act), means controls and other procedures of a company that are designed to provide reasonable assurance that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to provide reasonable assurance that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow for timely decisions regarding required disclosure. Based on this evaluation, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures were effective as of June 30, 2017 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.

Inherent Limitations on Effectiveness of Controls

Our management, including our principal executive officer and principal financial officer, does not expect that our disclosure controls and procedures or our internal controls will prevent all error and all fraud. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, within Axovant Sciences Ltd. have been detected.



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.

32

---

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 limited to organizing and staffing our company, raising capital, acquiring drug development programs and preparing for and advancing our key product candidates, 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 to obtain the necessary regulatory approvals for their commercialization. We have never been profitable, have no products approved for commercial sale and 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 effective 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 and quality 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, 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.



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 ongoing Phase 3 MINDSET study of intepirdine in patients with mild-to-moderate Alzheimer's disease and our HEADWAY-DLB study of intepirdine in patients with DLB, and as we continue to develop our second key product candidate, nelotanserin, for the treatment of multiple aspects of LBD and our other product candidates, RVT-103 and RVT-104, for the potential treatment of Alzheimer's disease and DLB. 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;
- our NDA or other key regulatory filings may be delayed or rejected due to issues, including those related to the FDA's Pharmaceutical Quality/CMC guidance, timing of results from supporting studies, database lock, and data conversion,

cleaning, and transfer;

the regulatory authorities may require additional nonclinical studies or registrational studies of the product candidate in mild-to-moderate Alzheimer's disease or other indications, 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, or otherwise commit errors or breaches of protocols, that materially adversely impact our clinical trials;

- 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 FDA may withdraw Fast Track designation of intepirdine if it believes the designation is no longer supported by data from our clinical development programs;
- the regulatory authorities may require, as a condition of approval, limitations on approved labeling or distribution and use restrictions;
- 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, including Arena, our sole and exclusive supplier for nelotanserin, or any manufacturer that Arena may engage to manufacture nelotanserin on its behalf; or
- the regulatory authorities may change their approval policies or adopt new regulations.

The terms of our credit facility place restrictions on our operating and financial flexibility.

In February 2017, we and our subsidiaries entered into a loan and security agreement with Hercules Capital, Inc., or Hercules, for a term loan of \$55.0 million, or the Term Loan. We refer to this loan and security agreement, as amended on May 24, 2017, as the Hercules Loan Agreement.

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

The Hercules 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 Hercules 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 shareholders.

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 Hercules Loan Agreement. Under the Hercules Loan Agreement, an event of default will occur if, among other things: we fail to make payments under the Hercules Loan Agreement; we breach any of our covenants under the Hercules 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 Hercules Loan Agreement as they become due or Hercules’s ability to enforce its rights or remedies with respect to our obligations under the Hercules Loan Agreement, or (iii) the collateral or liens securing our obligations under the Hercules 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.

We will 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, and in the case of the Arena Development Agreement, in connection with the manufacture and supply of nelotanserin for clinical or commercial purposes.

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.

If the MINDSET study is successful, our expenditures will increase significantly and we believe that our existing cash resources will be sufficient to enable us to file our NDA for intepirdine for the treatment of mild-to-moderate Alzheimer's disease and to fund our operating expenses and capital expenditure requirement into the first quarter of calendar 2018. In the event the MINDSET study fails, we believe our existing cash resources, will be sufficient to enable us to fund our operating expenses and capital expenditure requirements into the fourth quarter of calendar 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 progress, timing, costs and results of our clinical trials of 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. Further, failure to make these payments or to meet our other material obligations may result in our counterparties pursuing remedies under those agreements that could adversely affect our operations.

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. Additional debt financing or preferred equity financing, if available, may involve agreements that include covenants further 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 additional debt financing we enter into may involve covenants that further 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 subsidiaries and we rely on RSI and RSG to provide various administrative, research and development and other services.

As of June 30, 2017, we had 95 employees. We also 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 these 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 these services agreements, RSI and RSG have the discretion to determine which of their employees will perform services for us. Further, Lawrence T. Friedhoff, M.D., Ph.D., our Chief Development Officer, and Michael Adasczik, our Principal Accounting Officer, are employees of RSI, and Marianne L. Romeo, our Head of 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 either RSI or RSG fails to perform its obligations in accordance with the terms of the services agreements or to effectively manage our administrative, research and development or other services, it could be difficult for us to operate our business and our business could be harmed. In the event of a default under or termination of the services agreements, we may be unable to contract with substitute service providers on similar terms in a timely fashion or at all, and the costs of substituting service providers may be substantial. In addition, in light of RSI's and RSG's familiarity with our assets, a substitute service provider may not be able to provide the same level of service due to lack of pre-existing knowledge or synergies. Any 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.

We may not be able to manage our business effectively if we or RSI or RSG are unable to attract and retain key personnel.

We may not be able to attract or retain qualified management and commercial, scientific and clinical personnel due to the intense competition for qualified personnel among biotechnology, pharmaceutical and other businesses. If we are not able to attract and retain necessary personnel to accomplish our business objectives, we may experience constraints that will significantly impede the achievement of our development objectives, our ability to raise additional capital and our ability to implement our business strategy.

Our industry has experienced a high rate of turnover of management personnel in recent years. We are highly dependent on the skills and leadership of our management team, as well as the key employees of RSI and RSG that provide services to us through our services agreements. Our senior management and key employees, as well as those of RSI and RSG, may terminate their position with us or their employment with RSI or RSG, respectively, at any time. Further, neither RSI nor RSG is required pursuant to the services agreements to maintain the employment of any of its key employees on our behalf or to cause those individuals to provide services to us. If we lose one or more members of our senior management team or key employees, or if RSI or RSG loses one or more members of their senior management teams or key employees, our ability to successfully implement our business strategy could be seriously harmed. Replacing these individuals may be difficult, cause disruption, and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain regulatory approval of and commercialize products successfully. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate additional key personnel. We do not maintain "key person" insurance for any of our executives or other employees.



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, ASG or ASA, 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, or those of our affiliates, 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, manufacturers, service providers and other vendors, or those of our affiliates, 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 the 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, state and foreign healthcare fraud and abuse laws and data privacy; or laws that require the true, complete and accurate reporting of financial information or data. In particular, sales, marketing and other business arrangements in the healthcare industry are subject to extensive laws intended to prevent fraud, kickbacks, self-dealing, bribery, corruption, antitrust violations, and other abusive practices. These laws may restrict or prohibit a wide range of business activities, including research, manufacturing, distribution, pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. 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 employee or 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. Additionally, we are subject to the risk that a person or government agency could allege such fraud or other misconduct, even if none occurred. If our employees, independent contractors, principal investigators, consultants, commercial collaborators, service providers or other vendors, or those of our affiliates, are found to be in violation of any such regulatory standards or requirements, it could have a significant impact on our business and financial results, including the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, FDA debarment, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and oversight if

we become subject to a corporate integrity agreement or similar agreement, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

We may not be successful in our efforts to identify and acquire additional product candidates.

Part of our strategy involves identifying and acquiring novel product candidates. The process by which we identify product candidates may fail to yield product candidates for clinical development for a number of reasons, including those discussed in these risk factors and also:

the process by which we identify and decide to acquire product candidates may not be successful

potential product candidates may, on further study, be shown to have harmful side effects or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance ; or potential product candidates may not be effective in treating their targeted diseases.

We may choose to focus our efforts and resources on a potential product candidate that ultimately proves to be unsuccessful. Further, time and resources spent searching for, identifying, acquiring, and developing potential product candidates may distract management's attention from our primary business or other development programs. If we are unable to identify and acquire suitable product candidates for clinical development, this would adversely impact our business strategy and our financial position and share price.

International expansion of our business exposes us to business, regulatory, political, operational, financial and economic risks associated with doing business in various jurisdictions globally.

Our business strategy incorporates international expansion, including establishing and maintaining operations and certain key functions in various jurisdictions around the world and establishing and maintaining relationships with distributors and manufacturers globally. Doing business internationally involves a number of risks, including: multiple, conflicting and changing laws and regulations such as tax laws, export and import restrictions, employment laws, anti-bribery and anti-corruption laws, regulatory requirements and other governmental approvals, permits and licenses;

failure by us or our distributors to obtain appropriate licenses or regulatory approvals for the sale or use of our product candidates, if approved, in various countries;

difficulties in managing foreign operations;

complexities associated with managing multiple payor-reimbursement regimes or self-pay systems;

financial risks, such as longer payment cycles, difficulty enforcing contracts and collecting accounts receivable and exposure to foreign currency exchange rate fluctuations;

reduced protection for intellectual property rights;

reduced protection of contractual rights in the event of bankruptcy or insolvency of the other contracting party;

natural disasters, political and economic instability, including wars, terrorism and political unrest, outbreak of disease, boycotts, curtailment of trade and other business restrictions; and

failure to comply with the Foreign Corrupt Practices Act, including its books and records provisions and its

anti-bribery provisions, including by failing to maintain accurate information and control over sales and distributors' activities.

Any of these risks, if encountered, could significantly harm our future international expansion and operations and, consequently, negatively impact our financial condition, results of operations and cash flows.

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

Our computer systems, including 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, data breaches, 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.

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 smaller preclinical or early clinical trials therefore may not be predictive of the results of large scale or later-stage clinical programs. Likewise, there can be no assurance that the results of studies conducted by collaborators or other third parties will be viewed favorably or are indicative of our own future study results. A number of companies in the biopharmaceutical industry, and especially in the neurology field, 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;
- 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;
- changes in or modifications to clinical trial design;
- failure to manufacture or obtain supply of sufficient quantities of a drug candidate or placebo or failure to obtain sufficient quantities of concomitant medication for use in clinical trials;



inability or unwillingness of medical investigators to follow our clinical and other applicable protocols;  
inability to monitor patients adequately during or after treatment;  
failure to establish sufficient number of clinical trial sites; or  
clinical sites or others deviating from trial protocol, inappropriately unblinding results, 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, cause a drop in our share price, 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.

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 effectiveness of our patient recruitment efforts, 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. Similarly, negative results reported by our competitors about their drug candidates may negatively affect patient recruitment in our clinical trials. 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 or Special Protocol Assessment with 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. A Special Protocol Assessment (SPA) agreement between a sponsor and FDA indicates the agency's concurrence with the adequacy and acceptability of the clinical trial design to support a submission for marketing approval, but neither modifies the standard of evidence required for nor guarantees marketing approval. We have applied for and received Fast Track designation from the FDA for intepirdine in the treatment of dementia with Lewy bodies, as well as for nelotanserin in the treatment of visual hallucinations in DLB, and we may apply for that designation for some or all of our other product candidates in the future. We also have in place a SPA agreement for the MINDSET study of intepirdine in patients with mild-to-moderate Alzheimer's disease, and may seek a SPA agreement for some or all of our other product candidates in the future. The Fast Track designations and SPA agreement we received, as is the case for any other Fast Track designation or SPA agreement we may obtain or enter into in the future, do not necessarily lead to a faster development pathway or regulatory review process, and do 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, and may rescind a SPA agreement if a substantial scientific issue essential to determining the safety or efficacy of the product has been identified after the trial has begun.

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 in the neurology field, particularly 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 in the neurology field, including for the treatment of Alzheimer's disease and other dementias such as LBD. 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.

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 or errors in the submission of applications for marketing approval or issues, including 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 or that of adjuncts 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.

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 cGMP, 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 marketing, 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  
injunctions or the imposition of civil or criminal penalties.

44

---

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.

45

---

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.

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:

46

---

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, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under a federal healthcare program such as 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, knowingly making, using or causing to be made or used, a false record or statement material to a false or fraudulent claim, or knowingly making, or causing to be made, 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, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or making false or fraudulent 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 on health plans, health care clearing houses, and most providers and their business associates, defined as independent contractors or agents of covered entities that create, receive or obtain protected health information in connection with providing a service for or on behalf of a covered entity;

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 our business practices, including but not limited to, research, distribution, sales, and marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; and 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, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement, and curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. Even the mere issuance of a subpoena or the fact of an investigation alone, regardless of the merit, may result in negative publicity, a drop in our share price, and other harm to our business, financial condition and 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.

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. 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. In January 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the Affordable Care Act to waive, defer, grant exemptions from, or delay the implementation of any provision of the Affordable Care Act that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. The U.S. House of Representatives passed legislation known as the American Health Care Act of 2017 in May 2017. More recently, the Senate Republicans released and then updated a bill to replace the Affordable Care Act known as the Better Care Reconciliation Act of 2017. The Senate Republicans also introduced legislation to repeal the Affordable Care Act without companion legislation to replace it, and a "skinny" version of the Better Care Reconciliation Act of 2017. Each of these measures was rejected by the full Senate. Congress will likely consider other legislation to replace elements of the Affordable Care Act. We continue to



evaluate the effect that the Affordable Care Act and its possible repeal and replacement has on our business.

48

---

Other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. For example, in August 2011, President Obama 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 and increased the statute of limitations period in which the government may recover overpayments to providers from three to five years. Further, there have been several recent U.S. Congressional inquiries and proposed federal and state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the out-of-pocket cost of prescription drugs and reform government program reimbursement methodologies for drugs. 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. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

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, if approved.

Market acceptance and sales of any approved product candidates that we develop, will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from third-party payors, including government health administration authorities and private health insurers. In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors. Third-party payors decide which drugs they will pay for and establish 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 through approval 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, on what tier of its formulary the drug will be placed, and whether to require step therapy. 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.

The process for determining whether a third-party payor will provide coverage for a product may be separate from the process for setting the price of a product or for establishing the reimbursement rate that such a payor will pay for the product. Even if we do obtain adequate levels of reimbursement, third-party payors, such as government or private healthcare insurers, carefully review and increasingly question the coverage of, and challenge the prices charged for, products. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Increasingly, third-party

payors are requiring that pharmaceutical companies provide them with predetermined discounts from list prices and are challenging the prices charged for products. We may also be required to conduct expensive pharmacoeconomic studies to justify the 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 or 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 to 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 are building teams with drug formulation and manufacturing expertise but do not own or operate, nor do we expect to own or operate, facilities for product manufacturing, storage and distribution, or testing. In addition to the technical challenges of drug product formulation and scale-up and environmental compliance aspects of chemical manufacturing, our vendors of manufacturing services will need to comply with U.S. and foreign regulatory authority licensure and GMP quality requirements. These obligations are enforced by periodic inspection and audit by regulatory authorities, and any adverse findings or violations discovered on such inspections could distract our vendors and be costly and time consuming to remediate, potentially impacting their supply of clinical and future commercial products to us.

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 (MINDSET), 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 and, if nelotanserin is approved, for commercial sale. Arena is reliant on its own third-party supplier for the active pharmaceutical ingredient in nelotanserin, and Arena currently does not have an agreement in place for the supply of active pharmaceutical ingredient and Arena is in the process of contracting with a new supplier. If we are unable to continue our relationship with Arena or they are unable to initiate a relationship with these other third-party contractors, or if Arena is unable to manufacture or otherwise supply nelotanserin to us, whether as a result of its own inability to obtain active pharmaceutical ingredient or otherwise, we could experience delays in our development efforts.

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

- failure to satisfy their contractual duties or obligations;
- 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 applicable laws, regulations and standards, including cGMP and similar foreign standards;
- deficient or improper record-keeping;
- contractual restrictions on our ability to engage additional or alternative manufacturers;
- 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 access or licenses to proprietary manufacturing methods used by third-party manufacturers to make our product candidates;

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 affecting our product candidates or those of adjuncts such as donepezil could lead to clinical trial delays, cost overruns, delay or failure to obtain regulatory approval or impact our ability to successfully commercialize our products, as well as potential product liability litigation, product recalls or product withdrawals. 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 nonclinical 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 nonclinical and clinical trial sites to ensure the proper and timely conduct of our nonclinical 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 Council for 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 reject our marketing applications or 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 other applicable laws, regulations or standards, 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. Failure by our CROs to properly execute study protocols in accordance with applicable law could also create product liability and healthcare regulatory risks for us as sponsors of those studies.

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 (or their own) 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 or in a timely manner. 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, trademarks, copyrights, trade secret protection and confidentiality agreements with employees, consultants, collaborators, advisors and other third parties to protect the intellectual property related to our drug development programs and product candidates. However, 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. We may also inadvertently make statements to regulatory agencies during the regulatory approval process that may be inconsistent with positions that have been taken during prosecution of our patents which may result in such patents being narrowed, invalidated, or held unenforceable.

The patent rights that we own or have licensed relating to our product candidates may be limited in ways that may affect our ability to exclude third parties from competing against us if we obtain regulatory approval to market these product candidates. For our product candidates for which we do not hold composition of matter patents, competitors who obtain the requisite regulatory approval can offer products with the same composition as our products so long as the competitors do not infringe any method of use patents that we may hold. For some of our product candidates, the principal patent protection that covers or that we expect will cover, our product candidate is a method of use patent.

This type of patent only protects the product when used or sold for the specified method. However, this type of patent does not limit a competitor from making and marketing a product that is identical to our product that is labeled for an indication that is outside of the patented method, or for which there is a substantial use in commerce outside the patented method.

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 that we may develop. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate 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. Our pending applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Any such outcome could have a materially adverse effect on our business.





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 in which the first inventor to file a patent application will be entitled to the patent, 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.

If we do not obtain protection under the Hatch-Waxman Amendments by extending the patent term and obtaining data exclusivity for our drug candidates, our business may be materially harmed.

Our commercial success will largely depend on our ability to obtain and maintain patent and other intellectual property in the United States and other countries with respect to our proprietary technology, drug candidates and our target indications. Given the amount of time required for the development, testing and regulatory review of new drug candidates, patents protecting our drug candidates might expire before or shortly after such candidates begin to be commercialized. We expect to seek extensions of patent terms in the United States and, if available, in other countries where we are prosecuting patents.

Depending upon the timing, duration and specifics of FDA marketing approval of our drug candidates, one or more of our U.S. patents may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years beyond the normal expiration of the patent as compensation for patent term lost during development and the FDA regulatory review process, which is limited to the approved indication (or any additional indications approved during the period of extension). This extension is limited to only one patent that covers the approved product. However, the applicable authorities, including the FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. We may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request.

If we are unable to extend the expiration date of our existing patents or obtain new patents with longer expiry dates, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data to obtain approval of competing products following our patent expiration and launch their product earlier than might otherwise be the case.

The validity, scope and enforceability of any patents listed in the Orange Book that cover our product candidates can be challenged by third parties.

If one of our product candidates is approved by the FDA, one or more third parties may challenge the current patents, or patents that may issue in the future, within our portfolio, including those covering intepirdine or nelotanserin, which could result in the invalidation of, or render unenforceable, some or all of the relevant patent claims or a finding of non-infringement. For example, if a third party files an Abbreviated New Drug Application, or ANDA, for a generic drug containing intepirdine, and relies in whole or in part on studies conducted by or for us, the third party will be required to certify to the FDA that either: (1) there is no patent information listed in the FDA's Orange Book with respect to our NDA for the applicable approved drug candidate; (2) the patents listed in the Orange Book have expired; (3) the listed patents have not expired, but will expire on a particular date and approval is sought after patent expiration; or (4) the listed patents are invalid or will not be infringed by the manufacture, use or sale of the third party's generic drug. A certification that the new drug will not infringe the Orange Book-listed patents for the applicable approved drug candidate, or that such patents are invalid, is called a paragraph IV certification. If the third party submits a paragraph IV certification to the FDA, a notice of the paragraph IV certification must also be sent to us once the third party's ANDA is accepted for filing by the FDA. We may then initiate a lawsuit to defend the patents identified in the notice. The filing of a patent infringement lawsuit within 45 days of receipt of the notice automatically prevents the FDA from approving the third party's ANDA until the earliest of 30 months or the date on which the patent expires, the lawsuit is settled, or the court reaches a decision in the infringement lawsuit in favor of the third party. If we do not file a patent infringement lawsuit within the required 45-day period, the third party's ANDA will not be subject to the 30-month stay of FDA approval.

Moreover, a third party may challenge the current patents, or patents that may issue in the future, within our portfolio, including those covering our product candidates, including intepirdine or nelotanserin, which could result in the invalidation of some or all of the patents that might otherwise be eligible for listing in the Orange Book for one of our products. If a third party successfully challenges all of the patents that might otherwise be eligible for listing in the Orange Book for one of our products, we will not be entitled to the 30-month stay of FDA approval upon the filing of an ANDA for a generic drug containing, for example, intepirdine, and relies in whole or in part on studies conducted by or for us.

Litigation or other proceedings to enforce or defend intellectual property rights are often very complex in nature, may be very expensive and time-consuming, may divert our management's attention from our core business, and may result in unfavorable results that could limit our ability to prevent third parties from competing with our drug candidates.

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.

We may need to license intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.

A third party may hold intellectual property, including patent rights that are important or necessary to the development of our drug candidates. It may be necessary for us to use the patented or proprietary technology of third parties to commercialize our drug candidates, in which case we would be required to obtain a license from these third parties on commercially reasonable terms, or our business could be harmed, possibly materially.

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. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

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.

We are aware of a third-party patent, as well as third-party patent applications, directed to administering a combination of cholinesterase inhibitor with a peripheral muscarinic receptor antagonist that could adversely affect the potential commercialization of RVT-104. While we do not believe that any such claims that would cover the potential commercialization of RVT-104 are valid or enforceable, we may be incorrect in this belief.

If we breach any of our license or collaboration agreements, it could compromise our development and commercialization efforts for our product candidates.

We have licensed rights to intellectual property from third parties in order to commercialize our product candidates, and, in the case of nelotanserin, have an exclusive supply agreement with Arena. If we materially breach or fail to perform any provision under these license and collaboration agreements, including failure to make payments to a licensor or collaborator when due for royalties and failure to use commercially reasonable efforts to develop and commercialize our product candidates, such as nelotanserin, such licensors and collaborators have the right to terminate our agreement, and upon the effective date of such termination, our right to practice the licensed patent rights and other intellectual property would end. Any uncured, material breach under the agreements could result in our loss of rights to practice the patent rights and other intellectual property licensed to us under the agreements.

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, lack of written description 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.

The United States federal government retains certain rights in inventions produced with its financial assistance under the Bayh-Dole Act. The federal government retains a “nonexclusive, nontransferable, irrevocable, paid-up license” for its own benefit. The Bayh-Dole Act also provides federal agencies with “march-in rights”. March-in rights allow the government, in specified circumstances, to require the contractor or successors in title to the patent to grant a “nonexclusive, partially exclusive, or exclusive license” to a “responsible applicant or applicants.” If the patent owner refuses to do so, the government may grant the license itself.

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 and our intellectual property rights outside of the United States can be less extensive than those in the United States. 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.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of some countries do not favor the enforcement of patents and other intellectual property protection, which could make it difficult for us to stop the infringement of our patents generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful.

Many countries, including European Union countries, India, Japan and China, have compulsory licensing laws under which a patent owner may be compelled under specified circumstances to grant licenses to third parties. In those

countries, we may have limited remedies if patents are infringed or if we are compelled to grant a license to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

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. Adequate remedies may not exist in the event of unauthorized use or disclosure. 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. Policing unauthorized use of our or our licensors' intellectual property is difficult, expensive and time-consuming, and we may be unable to determine the extent of any unauthorized use. 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 ensure that our employees and our licensors' employees and our consultants and independent contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed trade secrets, or other 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.

In addition, while it is our policy to require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own. Our and their assignment agreements may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we or our licensors fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we and our licensors are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.



Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common shares. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Some of our competitors are larger than we are and have substantially greater resources. They are, therefore, likely to be able to sustain the costs of complex patent litigation longer than we could. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing upon or misappropriating our intellectual property. Litigation could result in substantial costs and diversion of management resources, which could harm our business. In addition, the uncertainties associated with litigation could compromise our ability to raise the funds necessary to continue our clinical trials, continue our internal research programs, or in-license needed technology or other drug candidates. There could also be public announcements of the results of the hearing, motions, or other interim proceedings or developments. If securities analysts or investors perceive those results to be negative, it could cause the price of our common shares to decline. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace, including compromising our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development collaborations that would help us commercialize our drug candidates, if approved.

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

In addition to seeking patents for our drug candidates, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect our trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

Any trademarks we have obtained or may obtain may be infringed or successfully challenged, resulting in harm to our business.

We expect to rely on trademarks as one means to distinguish any of our drug candidates that are approved for marketing from the products of our competitors. Once we select new trademarks and apply to register them, our trademark applications may not be approved. Third parties may oppose or attempt to cancel our trademark applications or trademarks, or otherwise challenge our use of the trademarks. In the event that our trademarks are successfully challenged, we could be forced to rebrand our drugs, which could result in loss of brand recognition and

could require us to devote resources to advertising and marketing new brands. Our competitors may infringe our trademarks and we may not have adequate resources to enforce our trademarks.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative:

- others may be able to make formulations or compositions that are the same as or similar to our product candidates, but that are not covered by the claims of the patents that we own;

- others may be able to make product that is similar to product candidates we intend to commercialize that is not covered by the patents that we exclusively licensed and have the right to enforce;

- we, our licensor or any collaborators might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own;
- we or our licensor might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own may not provide us with any competitive advantages, or may be held invalid or unenforceable as a result of legal challenges;
- our competitors might conduct research and development activities in the United States and other countries that provide a safe harbor from patent infringement claims for certain research and development activities, as well as in countries where we do not have patent rights, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets; and
- we may not develop additional proprietary technologies that are patentable.

### Risks Related to Our Common Shares

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

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. In addition, as a result of RSL owning approximately 69.8% 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 has been and is likely to continue to be highly volatile, and you may lose some or all of your investment.

The market price of our common shares has been and is likely to continue 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 our clinical trials;
- results of clinical trials of our product candidates or those of our competitors;
- any delay in filing applications for marketing approval of intepirdine or nelotanserin, and any adverse development or perceived adverse development with respect to applicable regulatory authorities' review of those applications;
- failure to successfully develop and commercialize intepirdine or nelotanserin or any other of our current or 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 or statements;
- 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;
- short sales of our common shares;
- sales of our common shares by us or our shareholders in the future;
- negative coverage in the media or analyst reports, whether accurate or not;
- issuance of subpoenas or investigative demands, or the public fact of an investigation by a government agency, whether meritorious or not;

- trading volume of our common shares;
- general economic, industry and market conditions; and
- the other factors described in this “Risk Factors” section.

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 June 30, 2017, RSL beneficially owns approximately 69.8% of the voting power of our outstanding common shares and has the ability to substantially influence us through this ownership position. For example, RSL and its shareholders may be able to control elections of directors, issuance of equity, including to our employees under equity incentive plans, 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. Further, RSL is a privately-held company whose ownership and governance structure is not transparent to our other shareholders. There may be changes to the management or ownership of RSL that could impact RSL’s interests in a way that may not coincide with our corporate interests or the interests of 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.

Our organizational and ownership structure may create significant conflicts of interests.

Our organizational and ownership structure involves a number of relationships that may give rise to certain conflicts of interest between us and minority holders of our common shares, on the one hand, and RSL and its shareholders, on the other hand. Certain of our directors and employees have equity interests in RSL and, accordingly, their interests may be aligned with RSL's interests, which may not always coincide with our corporate interests or the interests of our other shareholders. Further, our other shareholders may not have visibility into the RSL ownership of any of our directors or officers, which may change at any time through acquisition, disposition, dilution, or otherwise. Any change in our directors' or officers' RSL ownership could impact the interests of those holders.

In addition, we are party to certain related party agreements with RSL, RSI and RSG. These entities and their shareholders, including certain of our directors and employees, may have interests which differ from our interests or those of the minority holders of our common shares. For example, we are party to an information sharing and cooperation agreement with RSL pursuant to which RSL has granted us a right of first review on any potential dementia-related product or investment opportunity that RSL may consider pursuing. It is possible that we could fail to pursue a product candidate under this agreement and that product candidate is then successfully developed and commercialized by RSL or one of its other subsidiaries or affiliates. Any material transaction between us and RSL, RSI or RSG is subject to our related party transaction policy, which requires prior approval of such transaction by our Audit Committee. To the extent we fail to appropriately deal with any such conflicts of interests, it could negatively impact our reputation and ability to raise additional funds and the willingness of counterparties to do business with us, all of which could have an adverse effect on our business, financial condition, results of operations and cash flows.

If securities or industry analysts cease to 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 depends, 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.

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 currently restricted by the terms of the Hercules Loan Agreement. 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 shareholders intend to sell substantial amounts of our common shares 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 and our recent follow-on offering, 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 of our outstanding common shares, representing a majority of our common shares, are held by RSL. If RSL or any of our executive officers or directors were to sell our common shares, or if the market perceived that RSL or any of our executive officers or directors intend to sell our common 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 director and former 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 million of any combination of registered common shares, preferred shares, debt securities and warrants. In April 2017, we offered and sold approximately \$134.6 million of our common shares, net of underwriting discounts and commissions and offering expenses, pursuant to this registration statement.

We have incurred and will continue to incur substantial costs as a result of operating as a public company, and our management has been and will be required to continue to devote substantial time to compliance with our public company responsibilities and corporate governance practices.

As a public company, we have incurred and will continue to 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 devote a substantial amount of time to compliance with these requirements. Moreover, changing rules and regulations may increase our legal and financial compliance costs and make some activities more time-consuming and costly. If, notwithstanding our efforts to comply with new or changing laws, regulations and standards, we fail to comply, regulatory authorities may initiate legal proceedings against us, and our business may be harmed.

Further, failure to comply with these laws, regulations and standards 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 or members of senior management.

If we are unable to maintain proper and effective internal controls over financial reporting, investor confidence in our company and, as a result, the value of our common shares, may be adversely affected.

Effective internal controls are necessary for us to provide reliable financial reports and to protect from fraudulent, illegal or unauthorized transactions. If we cannot provide effective controls and reliable financial reports, our business and operating results could be harmed. We have in the past discovered, and may in the future discover, areas of our internal controls that need improvement. We are required, pursuant to Section 404 of the Sarbanes-Oxley Act, to furnish a report by management on the effectiveness of our internal control over financial reporting as of March 31, 2017. Our independent registered public accounting firm will not be required to attest to the effectiveness of our internal control over financial reporting until our first annual report required to be filed with the SEC following the later of the date we are deemed to be a "large accelerated filer," as defined in the Exchange Act, or the date we are no longer an "emerging growth company," as defined in the JOBS Act.

If material weaknesses or control deficiencies occur in the future, we may be unable to report our financial results accurately on a timely basis, which could cause our reported financial results to be materially misstated and result in

the loss of investor confidence and cause the market price of our common shares to decline.

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.



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 or any other appointed stock exchange.

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 founder and former 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.

65

---

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 are centrally managed and controlled in the United Kingdom, and under current United Kingdom legislation, a company which is centrally managed and controlled in the United Kingdom is regarded as resident in the United Kingdom for taxation purposes. We may also 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 additional tax liability could materially adversely affect our results of operations. For example, Axovant Sciences GmbH is our 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.

RSL, our principal shareholder, is based in Bermuda. 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.

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, potentially resulting in 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 (including the United Kingdom), the United States, Bermuda and other jurisdictions as well as being affected by certain changes currently proposed by the Organisation for Economic Co-operation and Development 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 was 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 our operations and our financial condition.

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 Internal Revenue Code of 1986, as amended, 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 and subsequent financings 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 years that ended on March 31, 2016 and March 31, 2017. Based on this belief, with respect to the taxable year that ended on March 31, 2017 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 June 30, 2017, we have used \$224.8 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.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

None.

Item 6. Exhibits.

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

68

---

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 August 7, 2017

(Duly Authorized Officer and Principal Financial Officer)

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)
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.

\* 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.