

Axovant Sciences Ltd.
Form 10-Q
November 07, 2018

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

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2018

or

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

For the transition period from _____ to _____
Commission file number 001-37418

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

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

Suite 1, 3rd Floor
11-12 St. James's Square
London SW1Y 4LB, United Kingdom
(Address of principal executive offices) (Zip Code)
Not Applicable
Registrant's telephone number, including area code: +44 203 318 9708

(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 every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes No
Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer", "accelerated filer", "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange

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Act. (Check one):

Large accelerated filer Accelerated filer

Non-accelerated filer Smaller reporting company

Emerging growth company

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

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

The number of shares outstanding of the Registrant's common shares, \$0.00001 par value per share, on November 5, 2018, was 122,279,348.

AXOVANT SCIENCES LTD.
QUARTERLY REPORT ON FORM 10-Q
FOR THE QUARTER ENDED SEPTEMBER 30, 2018

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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)

	September 30, 2018	March 31, 2018
Assets		
Current assets:		
Cash	\$ 90,726	\$ 154,337
Prepaid expenses and other current assets	4,095	2,174
Income tax receivable	1,530	1,751
Total current assets	96,351	158,262
Other non-current assets	4,324	—
Property and equipment, net	1,513	2,524
Total assets	\$ 102,188	\$ 160,786
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	\$ 1,817	\$ 3,949
Due to RSL, RSI and RSG	2,859	1,011
Accrued expenses	24,315	31,862
Current portion of long-term debt	20,009	9,753
Total current liabilities	49,000	46,575
Long-term debt	33,309	42,925
Total liabilities	82,309	89,500
Commitments and contingencies (Note 11)		
Shareholders' equity:		
Common shares, par value \$0.00001 per share, 1,000,000,000 shares authorized, 122,175,480 and 107,788,074 issued and outstanding at September 30, 2018 and March 31, 2018, respectively	1	1
Additional paid-in capital	661,980	628,110
Accumulated deficit	(642,674) (556,951)
Accumulated other comprehensive income	572	126
Total shareholders' equity	19,879	71,286
Total liabilities and shareholders' equity	\$ 102,188	\$ 160,786

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

AXOVANT SCIENCES LTD.

Condensed Consolidated Statements of Operations

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

	Three Months Ended September 30,		Six Months Ended September 30,	
	2018	2017	2018	2017
Operating expenses:				
Research and development expenses ⁽¹⁾ (includes total share-based compensation expense (benefit) of \$(1,128) and \$5,916 for the three months ended September 30, 2018 and 2017 and \$1,389 and \$12,172 for the six months ended September 30, 2018 and 2017, respectively)	\$21,502	\$ 38,555	\$58,920	\$ 82,267
General and administrative expenses ⁽²⁾ (includes total share-based compensation expense of \$3,585 and \$9,424 for the three months ended September 30, 2018 and 2017 and \$6,927 and \$18,768 for the six months ended September 30, 2018 and 2017, respectively)	10,622	30,112	22,376	51,630
Total operating expenses	32,124	68,667	81,296	133,897
Other expenses:				
Interest expense	1,932	1,878	3,902	3,752
Other expense (income)	(315)	131	353	(226)
Loss before income tax expense	(33,741)	(70,676)	(85,551)	(137,423)
Income tax expense (benefit)	94	(1,590)	172	929
Net loss	\$(33,835)	\$(69,086)	\$(85,723)	\$(138,352)
Net loss per common share — basic and diluted	\$(0.28)	\$(0.64)	\$(0.75)	\$(1.29)
Weighted average common shares outstanding — basic and diluted	120,863,451	107,593,609	114,362,408	107,000,519

⁽¹⁾ Includes total costs allocated from RSL, RSI and RSG of \$(3,069) and \$2,257 for the three months ended September 30, 2018 and 2017, respectively, and \$(450) and \$5,258 for the six months ended September 30, 2018 and 2017, respectively.

⁽²⁾ Includes total costs allocated from RSL, RSI and RSG of \$772 and \$1,623 for the three months ended September 30, 2018 and 2017, respectively, and \$2,074 and \$3,496 for the six months ended September 30, 2018 and 2017, respectively.

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

AXOVANT SCIENCES LTD.

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

	Three Months Ended		Six Months Ended	
	September 30,		September 30,	
	2018	2017	2018	2017
Net loss	\$(33,835)	\$(69,086)	\$(85,723)	\$(138,352)
Other comprehensive income (loss):				
Foreign currency translation adjustment	(113)	99	446	(250)
Total other comprehensive income (loss)	(113)	99	446	(250)
Comprehensive loss	\$(33,948)	\$(68,987)	\$(85,277)	\$(138,602)

The accompanying notes are an integral part of these unaudited 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	Deficit	Other	Shareholders'
			Capital		Comprehensive	Equity
					Income	
Balance at March 31, 2018	107,788,074	\$ 1	\$628,110	\$(556,951)	\$ 126	\$ 71,286
Exercise of stock options	95,742	—	118	—	—	118
Shares issued for private placement offering	14,285,714	—	25,000	—	—	25,000
Shares sold under share sales agreement	5,950	—	14	—	—	14
Share-based compensation expense	—	—	11,004	—	—	11,004
Capital contribution — share-based compensation expense	—	—	(2,688)	—	—	(2,688)
Non-cash capital contribution received by ASG from RSI	—	—	422	—	—	422
Foreign currency translation adjustment	—	—	—	—	446	446
Net loss	—	—	—	(85,723)	—	(85,723)
Balance at September 30, 2018	122,175,480	\$ 1	\$661,980	\$(642,674)	\$ 572	\$ 19,879

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

AXOVANT SCIENCES LTD.

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

	Six Months Ended September 30,	
	2018	2017
Cash flows from operating activities:		
Net loss	\$(85,723)	\$(138,352)
Adjustments to reconcile net loss to net cash used in operating activities:		
Disposal of fixed assets	9	—
Foreign currency translation adjustment	446	(250)
Share-based compensation	8,316	30,940
Depreciation and non-cash amortization	1,647	1,050
Deferred tax assets	—	2,709
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(1,921)	1,870
Other non-current assets	(4,324)	—
Accounts payable	(2,132)	(5,691)
Due to RSL, RSI and RSG	2,283	1,323
Accrued expenses	(7,547)	(1,002)
Income tax receivable	221	(2,155)
Net cash used in operating activities	(88,725)	(109,558)
Cash flows from investing activities:		
Purchases of property and equipment	(18)	(3,643)
Net cash used in investing activities	(18)	(3,643)
Cash flows from financing activities:		
Exercise of stock options	118	1,486
Cash proceeds from issuance of common shares, net of costs	25,014	134,515
Net cash provided by financing activities	25,132	136,001
Net change in cash	(63,611)	22,800
Cash—beginning of period	154,337	212,573
Cash—end of period	\$90,726	\$235,373
Non-cash financing activities:		
Non-cash capital contribution received by ASG from RSI	\$422	\$—
Issuance of common stock upon exercise of warrant	—	2,594

The accompanying notes are an integral part of these unaudited 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 gene therapy company focused on developing a pipeline of innovative product candidates for debilitating neurological and neuromuscular diseases such as Parkinson's disease, oculopharyngeal muscular dystrophy ("OPMD"), amyotrophic lateral sclerosis ("ALS"), frontotemporal dementia, and other indications. The Company is also developing nelotanserin for the treatment of Lewy body dementia ("LBD") and potentially other neurology and psychiatry indications.

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 six 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; Axovant Sciences America, Inc. ("ASA"), a direct wholly owned subsidiary of AHL, was incorporated in Delaware in July 2017; and Axovant Treasury Holdings, Inc. ("ATH"), a direct wholly owned subsidiary of AHL and Axovant Treasury, Inc. ("ATI"), a direct wholly owned subsidiary of ATH, were each incorporated in Delaware in March 2018. ASG holds all of the Company's intellectual property rights and is the principal operating company for conducting the Company's business.

The Company's near-term focus is to develop its gene therapy product candidates AXO-Lenti-PD, a potential one-time treatment for Parkinson's disease, and AXO-AAV-OPMD, a potential one-time treatment for OPMD. The Company has initiated a clinical study of AXO-Lenti-PD in patients with Parkinson's disease and intends to initiate a clinical study of AXO-AAV-OPMD in patients with OPMD in the second half of 2019. Prior to the recent in-licensing of AXO-Lenti-PD in June 2018 and AXO-AAV-OPMD in July 2018, the Company's primary focus had been on developing nelotanserin, a selective inverse agonist of the 5-HT_{2A} receptor, and intepirdine, an antagonist of the 5-HT₆ receptor for which development had been terminated in January 2018. Topline data from the ongoing Phase 2 study of nelotanserin in REM Sleep Behavior Disorder ("RBD") in LBD patients is expected to be available in December 2018. The Company is evaluating the possibility of partnering or pursuing other strategic opportunities for nelotanserin. In October 2018, the Company discontinued its development plans for RVT-104 as a potential treatment for patients with Alzheimer's disease or dementia with Lewy bodies ("DLB"), which is a sub-type of LBD.

From its inception, the Company has devoted substantially all of its efforts to organizing and staffing the Company, raising capital, acquiring product candidates and advancing its product candidates into clinical development. 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 access to sufficient funds to meet its financial needs for at least the next 12 months. The Company will be required to obtain further funding through public or private offerings of its share capital, debt financing, collaboration and licensing arrangements or other sources as it advances its product candidates through preclinical and clinical development. 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 and notes thereto included in the Company's Annual Report on Form 10-K for the fiscal year ended March 31, 2018 the ("Annual Report"), filed with the Securities and Exchange Commission ("SEC") on June 11, 2018. In the opinion of management, all adjustments (consisting of normal recurring adjustments) considered necessary to present fairly the financial position of the

Company and its results of operations and cash flows for the interim periods presented have been included. Operating results for the three and six-months ended September 30, 2018 are not necessarily indicative of the results that may be expected for the year ending March 31, 2019, 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") issued by the Financial Accounting Standards Board ("FASB"). The unaudited 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.

(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 the assets, liabilities, costs and expenses (including 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, RSL, as well as the evaluation of the Company's ability to continue as a going concern, contingent liabilities, share-based compensation and research and development costs. Specifically, the Company estimates the grant date fair value of stock option awards with only time-based vesting requirements using a Black-Scholes valuation model and uses a Monte Carlo Simulation method under the income approach to estimate the grant date fair value of stock option awards with market-based performance conditions. 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 the net loss applicable to common shareholders by the weighted-average number of common shares outstanding during the period. Diluted net loss per common share is computed by dividing the net loss applicable to common shareholders by the diluted weighted-average number of common shares outstanding during the period calculated in accordance with the treasury stock method. Stock options to purchase approximately 15.8 million common shares were not included in the calculation of diluted weighted-average common shares outstanding for each of the three and six-months ended September 30, 2018 because they were anti-dilutive given the net loss of the Company. Stock options and a warrant which, combined, would enable the purchase of an aggregate of 5.4 million and 10.4 million common shares were not included in the calculation of diluted weighted-average common shares outstanding for the three and six-months ended September 30, 2017, respectively, because they were anti-dilutive given the net loss of the Company.

(D) Fair Value Measurements:

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 defined as the exchange price, or exit price, representing the amount that would be received from the sale of 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, accounts payable and long-term debt. Cash and accounts payable are stated at their respective historical carrying amounts, which approximate fair value due to their short-term nature. The carrying value of the Company's debt was \$53.3 million as of September 30, 2018 and approximates fair value based on current interest rates for similar types of borrowings and is in Level 2 of the fair value hierarchy. See Note 5 for the actual book carrying value of the Company's long-term debt at September 30, 2018.

(E) Recent Accounting Pronouncements:

In February 2016, the FASB issued ASU No. 2016-02, "Leases (Topic 842)" ("ASU No. 2016-02"), as well as ASU No. 2018-10, "Codification Improvements to Topic 842, Leases" and ASU No. 2018-11, "Leases (Topic 842): Targeted Improvements" in July 2018 (collectively, the "Lease Standards"), which relate to a comprehensive new lease standard that amends various aspects of existing accounting guidance for leases. The core principle of the Lease Standards will require lessees to present the assets and liabilities that arise from leases on their balance sheets. The Lease Standards are 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 has implemented a process to identify its outstanding lease portfolio and is currently evaluating its outstanding leases to determine the impact the Lease Standards will have on its consolidated financial statements.

In January 2017, the FASB issued ASU 2017-01, "Business Combinations (Topic 805): Clarifying the Definition of a Business" ("ASU No. 2017-01"), which clarifies the definition of a business with the objective of adding guidance to assist entities with evaluating whether transactions should be accounted for as acquisitions (or disposals) of assets or businesses. The Company adopted the provisions of ASU No. 2017-01 on April 1, 2018 on a prospective basis. The impact on the Company's consolidated financial statements and disclosures will depend on the facts and circumstances of any specific future transactions. See Note 3 for further information regarding the impact of the adoption of ASU No. 2017-01 on the license agreements executed during the three and six-months ended September 30, 2018.

In February 2018, the FASB issued ASU No. 2018-02, "Income Statement-Reporting Comprehensive Income (Topic 220): Reclassification of Certain Tax Effects from Accumulated Other Comprehensive Income" ("ASU No. 2018-02"). ASU No. 2018-02 allows companies to reclassify stranded tax effects resulting from the Tax Cuts and Jobs Act, from accumulated other comprehensive (loss) income to retained earnings. ASU No. 2018-02 is effective for interim and annual reporting periods beginning after December 15, 2018 and early adoption is permitted. The Company expects to adopt the provisions of ASU No. 2018-02 for the fiscal year beginning April 1, 2019. As the Company has not yet completed its final review of the impact of ASU No. 2018-02 but expects to by March 31, 2019, the Company has not determined whether the adoption of this guidance will have a material impact on its consolidated financial statements or disclosures.

In March 2018, the FASB issued ASU No. 2018-05, "Income Taxes (Topic 740): Amendments to SEC Paragraphs Pursuant to SEC Staff Accounting Bulletin No. 118," ("ASU No. 2018-05"). ASU No. 2018-05 amends certain SEC material in Topic 740 for the income tax accounting implications of the Tax Cuts and Jobs Act. ASU No. 2018-05 was effective immediately. The Company evaluated the impact of the Tax Cuts and Jobs Act as well as the guidance of Staff Accounting Bulletin 118 ("SAB 118") and incorporated the changes into the determination of a reasonable

estimate of deferred taxes and appropriate disclosures in the notes to the Company's consolidated financial statements. The Company will continue to evaluate the impact this tax reform legislation may have on our results of operations, financial position, cash flows and related disclosures.

In June 2018, the FASB issued ASU No. 2018-07, "Compensation - Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting," ("ASU No. 2018-07"). ASU No. 2018-07 requires equity-classified share-based payment awards issued to nonemployees to be measured on the grant date, rather than remeasuring the awards through the performance completion date as previously required. Additionally, for nonemployee awards with performance conditions, compensation cost associated with the award is to be recognized when achievement of the performance condition is probable, rather than upon achievement of the performance condition. Further, the requirement to reassess the liability or equity classification for nonemployee awards upon vesting is eliminated, except for awards in the form of convertible instruments. ASU No. 2018-07 also clarifies that any share-based payment awards issued to customers should be evaluated under ASC 606, Revenue from Contracts with Customers. ASU No. 2018-07 is effective for fiscal years beginning after December 15, 2018, including interim periods within that fiscal year, with early adoption permitted after the adoption of ASU No. 2014-09. The Company expects to adopt the provisions of ASU No. 2018-07 for the fiscal year beginning April 1, 2019. As the Company has not yet completed its final review of the impact of ASU No. 2018-07 but expects to by March 31, 2019, the Company has not determined whether the adoption of this guidance will have a material impact on its consolidated financial statements or disclosures.

In August 2018, the FASB issued ASU No. 2018-13, "Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement" ("ASU No. 2018-13"). ASU No. 2018-13 removes, modifies, and adds certain recurring and nonrecurring fair value measurement disclosures, including removing disclosures around the amount(s) of and reasons for transfers between Level 1 and Level 2 of the fair value hierarchy, the policy for timing of transfers between levels, and the valuation processes for Level 3 fair value measurements, among other things. ASU No. 2018-13 adds disclosure requirements around changes in unrealized gains and losses included in other comprehensive income for recurring Level 3 fair value measurements held at the end of the reporting period, the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements, and a narrative description of measurement uncertainty. The amendments in ASU No. 2018-13 are effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2019. The amendments on changes in unrealized gains and losses, the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements, and the narrative description of measurement uncertainty are to be applied prospectively for only the most recent interim or annual period presented in the initial fiscal year of adoption, with all other amendments applied retrospectively to all periods presented. Early adoption is permitted. The Company early adopted the provisions of ASU No. 2018-13 during the three months ended September 30, 2018, which did not have a material impact on its consolidated financial statements or disclosures because the Company does not currently have any Level 3 fair value measurements on a recurring or nonrecurring basis, and also has not had transfers between Level 1 and Level 2 of the fair value hierarchy.

Note 3—License and Collaboration Agreements

Oxford BioMedica License Agreement

On June 5, 2018, the Company, through its wholly owned subsidiary, ASG, entered into an exclusive license agreement (the "Oxford BioMedica Agreement") with Oxford BioMedica (UK) Ltd. ("Oxford BioMedica"), pursuant to which the Company received a worldwide, exclusive, royalty-bearing, sub-licensable license under certain patents and other intellectual property controlled by Oxford BioMedica to develop and commercialize AXO-Lenti-PD and related gene therapy products for all diseases and conditions. In June 2018, as consideration for the license, the Company made an upfront nonrefundable payment to Oxford BioMedica of \$30.0 million, \$5.0 million of which will be applied as a credit against the process development work and clinical supply that Oxford BioMedica will provide to the Company. Under the terms of the Oxford BioMedica Agreement, the Company could be obligated to make payments to Oxford BioMedica totaling up to \$55.0 million upon the achievement of specified development milestones and \$757.5 million upon the achievement of specified regulatory and sales milestones. The Company will

also be obligated to pay Oxford BioMedica a tiered royalty from 7% to 10%, based on yearly aggregate net sales of the underlying gene therapy products, subject to specified reductions upon the occurrence of certain events as set forth in the Oxford BioMedica Agreement. These royalties are required to be paid, on a product-by-product and country-by-country basis, until the latest to occur of the expiration of the last to expire valid claim of a licensed patent covering such product in such country, the expiration of regulatory exclusivity for such product in such country, or 10 years after the first commercial sale of such product in such country.

The Company is solely responsible, at its expense, for all activities related to the development and commercialization of the gene therapy products underlying the Oxford BioMedica Agreement. Pursuant to the Oxford BioMedica Agreement, the Company is required to use commercially reasonable efforts to develop, obtain regulatory approval of, and commercialize a gene therapy product underlying the Oxford BioMedica Agreement in the United States and at least one major market country in Europe. In addition, the Company is required to meet certain diligence milestones and to include at least one U.S.-based clinical trial site in a pivotal study of a gene therapy product underlying the Oxford BioMedica Agreement. If the Company fails to meet any of these specified development milestones, it may cure such failure by paying Oxford BioMedica certain fees, which range from \$0.5 million to \$1.0 million.

The Company has evaluated the Oxford BioMedica Agreement and has determined that the acquired set of assets and activities did not meet the definition of a business and thus the transaction was not considered a business combination. The Company determined that the in-process research and development ("IPR&D") had not reached technological feasibility and therefore has no alternative future use. Accordingly, \$25.0 million of the initial payment required under the license agreement was recorded as research and development expense in the Company's unaudited condensed consolidated statements of operations during the six months ended September 30, 2018. As the remaining \$5.0 million of the initial payment under the licensing agreement represents a nonrefundable payment for process development work and clinical supply that Oxford BioMedica will provide over the term of the license agreement, the Company fully capitalized this portion of the payment upon execution, with \$1.1 million remaining capitalized within prepaid expenses and other current assets and \$3.7 million remaining capitalized within other non-current assets in its unaudited condensed consolidated balance sheet as of September 30, 2018, which will be recorded to research and development expense as the process development work and clinical supply are provided by Oxford BioMedica. Additionally, the Company incurred \$1.2 million and \$1.3 million of AXO-Lenti-PD program-specific costs in its unaudited condensed consolidated statements of operations during the three and six-months ended September 30, 2018, respectively. During the three and six-months ended September 30, 2018, the Company paid a total of \$0.1 million and \$30.1 million, respectively, to Oxford BioMedica, including the upfront nonrefundable payment during the six months ended September 30, 2018.

Benitec Biopharma License and Collaboration Agreement

On July 8, 2018, ASG entered into a license and collaboration agreement (the "Benitec Agreement") with Benitec Biopharma Limited ("Benitec"). Pursuant to the Benitec Agreement, the Company received a worldwide, exclusive, royalty-bearing, sub-licensable license under certain patents and other intellectual property controlled by Benitec to develop and commercialize investigational gene therapy AXO-AAV-OPMD and related gene therapy products (collectively, the "AXO-AAV-OPMD Program") for all diseases and conditions.

Under the Benitec Agreement, the Company will also collaborate with Benitec on five additional research plans as part of the "Collaboration Programs" for other genetic neurological or neuromuscular disorders using Benitec technologies. The Company will receive a worldwide, exclusive, royalty-bearing, sub-licensable license under certain patents and other intellectual property controlled by Benitec to develop and commercialize products arising from each Collaboration Program.

The Company has evaluated the Benitec Agreement and has determined that the acquired set of assets and activities did not meet the definition of a business and thus the transaction was not considered a business combination. The Company determined that the IPR&D had not reached technological feasibility and therefore has no alternative future use. Accordingly, the \$10.0 million upfront nonrefundable payment required under the terms of the Benitec Agreement was recorded as research and development expense in the Company's unaudited condensed consolidated statements of operations during the three and six-months ended September 30, 2018. Additionally, the Company incurred \$1.7 million of AXO-AAV-OPMD program-specific costs in its unaudited condensed consolidated statements of operations during the three and six-months ended September 30, 2018. During the three and six-months

ended September 30, 2018, the Company paid a total of \$10.0 million to Benitec, including the upfront nonrefundable payment. Further, the Company will be obligated to make payments to Benitec totaling up to (i) for the AXO-AAV-OPMD Program, \$67.5 million upon the achievement of specified development and regulatory milestones and \$120.0 million upon the achievement of specified sales milestones, and (ii) for each Collaboration Program, \$33.5 million upon the achievement of specified development and regulatory milestones and \$60.0 million upon the achievement of specified sales milestones.

Benitec will receive 30% of net profits of world-wide sales of products from the AXO-AAV-OPMD Program, subject to an agreed minimum amount for such payments. This profit-sharing payment will be made for so long as the Company or its affiliates or sublicensees commercialize such products. The Company will also pay Benitec a tiered royalty based on yearly aggregate net sales of products arising from each Collaboration Program, subject to specified reductions upon the occurrence of certain events as set forth in the Benitec Agreement. These royalties are required to be paid, on a product-by-product and country-by-country basis, until the latest to occur of the expiration of the last to expire valid claim of a licensed patent covering such product in such country, the expiration of regulatory exclusivity for such product in such country, or ten years after the first commercial sale of such product in such country.

Under the Benitec Agreement, Benitec will perform certain development and manufacturing activities for the AXO-AAV-OPMD Program and research activities for each Collaboration Program, and the Company will reimburse Benitec for its costs incurred, in accordance with an agreed-upon research and development plan and budget. The Company is solely responsible, at its expense, for all other activities related to the research, development and commercialization of products from the AXO-AAV-OPMD Program and the Collaboration Programs.

Note 4—Accrued Expenses

As of September 30, 2018, and March 31, 2018, the Company's accrued expenses consisted of the following (in thousands):

	September 30, 2018	March 31, 2018
Research and development expenses	\$ 18,409	\$ 21,855
Salaries, bonuses, and other compensation expenses	3,169	7,718
Legal expenses	1,073	779
Other expenses	1,664	1,510
Total accrued expenses	\$ 24,315	\$ 31,862

Note 5—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 and September 22, 2017) (the "Loan Agreement") with Hercules Capital, Inc., ("Hercules"), under which the Company, AHL and ASG (the "Borrowers") borrowed an aggregate of \$55.0 million (the "Term Loan"). Subsequently, the Company added its subsidiary ASA as a Borrower in July 2017 and its subsidiaries ATH and ATI as Borrowers in April 2018. Pursuant to the Loan Agreement, ASI has issued a guaranty of the Borrowers' obligations under the 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 were obligated to make monthly payments of accrued interest under the Loan Agreement until September 1, 2018, followed by monthly installments of principal and interest beginning October 1, 2018 through March 1, 2021. 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.

On May 24, 2017, the Loan Agreement was amended such that, commencing July 1, 2017, the required minimum amount of unrestricted cash is equal to the lesser of (i) \$35.0 million (the "Applicable Amount") plus certain aged accounts payable amounts (as further defined in the Loan Agreement) and (ii) the outstanding amount of debt under the Loan Agreement plus certain aged accounts payable (as further defined in the Loan Agreement), provided that the Applicable Amount may be lowered to \$30 million upon the achievement of certain clinical milestones as set forth in

the Loan Agreement.

The 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 Loan Agreement. At no time has the Company been in default under the provisions of the Loan Agreement. In addition, for so long as the Term Loan remains outstanding, the Company shall be required to use its commercially reasonable efforts to afford Hercules the opportunity to participate in future underwritten equity offerings of the Company's common shares up to a total of \$3.0 million.

In connection with the Loan Agreement, the Company issued a warrant to Hercules, exercisable for an aggregate of 274,086 of the Company's common shares at an exercise price of \$12.04 per share (the "Warrant"). In August 2017, Hercules exercised the Warrant on a cashless basis and received a net issuance of 129,827 of the Company's common shares. The Company has accounted for the Warrant as an equity instrument since it was indexed to the Company's common shares and met 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 was 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 were 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):

	September 30, 2018	March 31, 2018
Principal amount	\$ 55,000	\$ 55,000
Less: unamortized discount and debt issuance costs	(1,682)	(2,322)
Loan payable less unamortized discount and debt issuance costs	53,318	52,678
Less: current portion of long-term debt	(20,009)	(9,753)
Long-term loan payable, net of current maturities	\$ 33,309	\$ 42,925

Note 6—Related Party Transactions

(A) Services Agreements:

In 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 predetermined 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, as well as share-based compensation expense allocated to the Company by RSL (see Note 8(B)(2)).

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, the Company incurred expenses of \$0.9 million and \$1.6 million for the three months ended September 30, 2018 and 2017, respectively, and \$4.3 million and \$4.4 million for the six months ended September 30, 2018 and 2017, respectively, inclusive of the predetermined mark-up.

(B) Family Relationships:

Geetha Ramaswamy, MD, the former Vice President, Medical and Scientific Strategy of ASI and an employee of RSI, is the mother of Vivek Ramaswamy, the Chief Executive Officer of RSI, former Chairman of the Company's Board of Directors and former Chief Executive Officer of the Company. Sarah Friedhoff, formerly Senior Business Operations and Research and Development Specialist of ASI, is the daughter of Lawrence Friedhoff, MD, PhD, formerly the Chief Development Officer of ASI and an officer of RSI. Shankar Ramaswamy, MD, the Senior Vice President, Business Development of ASI, and a former employee of RSI, is the brother of Vivek Ramaswamy. Lawrence Friedhoff, MD, PhD, Geetha Ramaswamy, MD and Sarah Friedhoff were no longer employed by ASI beginning in October 2017. The accompanying interim unaudited condensed consolidated financial statements include share-based compensation expense associated with family members Geetha Ramaswamy, MD, Shankar Ramaswamy, MD and Sarah Friedhoff (see Note 8(B)(3)).

Salary expenses for Shankar Ramaswamy, MD were \$75,000 and \$66,950 for the three months ended September 30, 2018 and 2017, respectively and \$150,000 and \$133,900 for the six months ended September 30, 2018 and 2017, respectively. Salary expenses for Geetha Ramaswamy, MD were \$66,950 and \$133,900 for the three and six-months ended September 30, 2017, respectively. Salary expenses for Sarah Friedhoff were \$19,312 and \$38,625 for the three and six-months ended September 30, 2017, respectively.

(C) RSL Private Placement Financing:

On July 9, 2018, the Company received \$25.0 million of net proceeds from RSL in exchange for the issuance and sale of 14,285,714 of the Company's common shares to RSL at a purchase price of \$1.75 per common share, which was the closing price per share of the Company's common shares on the Nasdaq Global Select Market on June 5, 2018, the date of the share purchase agreement (see Note 7).

Note 7—Shareholders' Equity

In April 2017, the Company 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 the Company were \$134.5 million, after deducting underwriting discounts and commissions and offering expenses paid by the Company.

During the three months ended March 31, 2018 and September 30, 2018, RSL incurred \$0.3 million and RSI incurred \$0.4 million, respectively, of expenses on behalf of the Company. These amounts were treated as capital contributions.

On June 5, 2018, the Company entered into a share purchase agreement with RSL, its majority shareholder, pursuant to which the Company agreed to issue and sell to RSL 14,285,714 of its common shares at a purchase price of \$1.75 per share, which was the closing price per share of the Company's common shares on the Nasdaq Global Select Market on June 5, 2018. On July 9, 2018, the Company received \$25.0 million of net proceeds from RSL upon the closing of this private placement (see Note 6 (C)).

On June 22, 2018, the Company entered into a sales agreement with Cowen and Company, LLC ("Cowen") to sell the Company's common shares having an aggregate offering price of up to \$75.0 million from time to time through an at-the-market equity offering program under which Cowen is acting as the Company's agent. Cowen is entitled to compensation for its services in an amount up to 3% of the gross proceeds of any of the Company's common shares sold under the sales agreement. As of September 30, 2018, approximately \$75.0 million of the Company's common shares remained available for sale under the sales agreement.

Note 8—Share-Based Compensation

In April 2017, the number of common shares authorized for issuance under the Company's 2015 Equity Incentive Plan increased automatically to an aggregate of approximately 16.5 million common shares in accordance with the terms of the 2015 Equity Incentive Plan. In June 2017, the Company's Board of Directors amended and restated the 2015 Equity Incentive Plan (the "2015 Plan") to, among other things, increase the number of common shares authorized for issuance thereunder to approximately 20.5 million common shares. The 2015 Plan became effective upon shareholder approval in August 2017. In April 2018, the number of common shares authorized for issuance under the 2015 Plan increased automatically to approximately 24.8 million common shares in accordance with the terms of the 2015 Plan. At September 30, 2018, a total of 8.2 million common shares were available for future grant under the 2015 Plan, and options to purchase approximately 15.8 million common shares were outstanding under the 2015 Plan, with a weighted average exercise price of \$4.66 per share.

(A) Stock Options Granted to Employees and Directors:

During the six months ended September 30, 2018 and 2017, the Company granted options to its employees and directors under the 2015 Plan to purchase a total of 2.1 million and 8.8 million common shares, respectively. The stock options granted during the six months ended September 30, 2018 include approximately 0.6 million common shares with market-based performance conditions to employees with a weighted average exercise price of \$2.98 per share, a contractual term of 10 years, and a corresponding estimated grant date fair value of \$1.1 million. As of September 30, 2018, stock options with market-based performance conditions to purchase 1.5 million common shares were outstanding with a weighted-average exercise price of \$2.02 per share. The market-based performance options vest based on exceeding certain closing prices of the Company's common shares. As of September 30, 2018, stock options with market-based performance conditions to purchase approximately 0.4 million common shares with a weighted-average exercise price of \$1.46 per share were vested, which occurred during the six months ended September 30, 2018.

The Company recorded total share-based compensation expense related to stock options issued to Company employees and directors of \$5.2 million and \$12.7 million, respectively, for the three months ended September 30, 2018 and 2017, and \$9.8 million and \$24.9 million for the six months ended September 30, 2018 and 2017. At September 30, 2018, total unrecognized compensation expense related to non-vested options was \$27.8 million, which is expected to be recognized over the remaining weighted-average service period of 2.3 years.

(B) Share-Based Compensation for Related Parties:

(1) Stock Options Granted to Non-Employees:

During the six months ended September 30, 2018 and 2017, the Company granted options to purchase a total of 1.0 million and 0.2 million common shares, respectively, to consultants as well as employees and consultants of RSI as compensation for support services provided to the Company. The fair value of the stock options granted to RSI employees and other consultants 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 \$(23) thousand and \$0.3 million of share-based compensation expense (benefit) for the three months ended September 30, 2018 and 2017, respectively, and \$0.4 million and \$1.3 million for the six months ended September 30, 2018 and 2017, respectively. The share-based compensation expense (benefit) was recorded within research and development and general and administrative expenses in the accompanying unaudited condensed consolidated statements of operations. The total remaining unrecognized compensation cost related to the non-vested stock options amounted to \$1.2 million as of September 30, 2018, which is expected to be recognized over the remaining weighted-average service period of 2.3 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 RSL, RSG and RSI employees. Share-based compensation expense is allocated to the Company by RSL based upon the relative percentage of time utilized by RSL, RSG and 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 (benefit) of \$(3.2) million and \$2.3 million for the three months ended September 30, 2018 and 2017, respectively, and \$(2.7) million and \$4.4 million for the six months ended September 30, 2018 and 2017, respectively, in relation to the RSL common share awards and options issued by RSL to RSG and RSI employees, net of forfeitures.

(3) Share-Based Compensation for Family Members:

The Company recorded aggregate share-based compensation expense of \$0.9 million and \$1.0 million for the three months ended September 30, 2018 and 2017, respectively, and \$1.7 million and \$2.4 million for the six months ended September 30, 2018 and 2017, respectively, in connection with options vesting for Geetha Ramaswamy, MD, Shankar Ramaswamy, MD and Sarah Friedhoff.

Shankar Ramaswamy, MD, while previously employed by RSI, was also granted RSL common shares. The Company recorded share-based compensation expense of \$7 thousand and \$0.1 million for the three months ended September 30, 2018 and 2017, respectively, and \$0.1 million and \$0.2 million for the six months ended September 30, 2018 and 2017, respectively, related to the RSL common share awards held by Shankar Ramaswamy, which the Company has recorded as research and development expense in the accompanying unaudited condensed consolidated statements of operations. At September 30, 2018, all compensation expense related to these RSL common share awards had been recognized.

Note 9—Restructuring

In October 2017, the Company initiated and committed to the first of two corporate realignments to focus its efforts and resources on the Company's ongoing and future programs that included a reduction in its workforce and a transfer of certain employees to affiliates. The second realignment was initiated and committed to in February 2018. The Company completed the reduction in headcount from these actions in the fourth quarter of fiscal 2018.

During the six months ended September 30, 2018, the Company made cash expenditures of approximately \$1.6 million for one-time severance and related costs in connection with the corporate realignments completed in the prior fiscal year.

The impacted employees are eligible to receive severance payments in specified amounts, health benefits and outplacement services. The Company has recorded these charges in research and development and general and administrative expenses in the accompanying condensed consolidated statements of operations based on responsibilities of the impacted employees.

The following sets forth information regarding the balances and activity associated with the Company's accrued employee severance and other personnel benefits (in thousands):

	Balance as of March 31, 2018	Expenses, net	Cash	Non-cash	Balance as of September 30, 2018
Employee severance and other personnel benefits	\$ 2,460	\$	—\$(1,573)	\$	—\$ 887

Note 10—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 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's effective tax rates of (0.3)% and 2.2% for the three months ended September 30, 2018 and 2017, respectively, and (0.2)% and

(0.7)% for the six months ended September 30, 2018 and 2017, respectively, differ from the Bermuda federal statutory rate of 0% primarily due to the U.S. permanent unfavorable tax differences, stock compensation deductions and a valuation allowance that effectively eliminates the Company's net deferred tax assets.

On December 22, 2017, the President of the United States signed into law an Act to provide for reconciliation pursuant to Titles II and V of the concurrent resolution on the budget for fiscal year 2018 (commonly known as the "Tax Cuts and Jobs Act"), which introduced a comprehensive set of tax reforms. The Tax Cuts and Jobs Act significantly revises U.S. tax law by, among other provisions, lowering the U.S. federal statutory income tax rate from 35% to 21% and eliminating or reducing certain income tax deductions.

The effects of changes in tax laws are required to be recognized in the period in which the legislation is enacted. However, due to the complexity and significance of the Tax Cuts and Jobs Act's provisions, the SEC staff issued SAB 118, which allows companies to record the tax effects of the Tax Cuts and Jobs Act on a provisional basis based on a reasonable estimate, and then, if necessary, subsequently adjust such amounts during a limited measurement period as more information becomes available. The measurement period ends when a company has obtained, prepared, and analyzed the information necessary to finalize its accounting, but cannot extend beyond one year from enactment.

The Tax Cuts and Jobs Act did not have a material impact on our financial statements since our deferred temporary differences are fully offset by a valuation allowance and the Company does not have any offshore earnings from which to record the mandatory transition tax. However, given the significant complexity of the Tax Cuts and Jobs Act, anticipated guidance from the U.S. Treasury about implementing the Tax Cuts and Jobs Act, and the potential for additional guidance from the SEC or the FASB related to the Tax Cuts and Jobs Act, these estimates may be adjusted during the measurement period. The Company's provisional amounts for income taxes were based on the Company's present interpretations of the Tax Cuts and Jobs Act and current available information, including assumptions and expectations about future events, such as its projected financial performance, and are subject to further refinement as additional information becomes available (including potential new or interpretative guidance issued by the FASB or the Internal Revenue Service and other tax agencies) and further analyses are completed. The Company continues to analyze the changes in certain income tax deductions and gather additional data to compute the full impacts on the Company's deferred and current tax assets and liabilities.

Note 11—Commitments and Contingencies

As of September 30, 2018, the Company had entered into commitments under a license agreement with Oxford BioMedica, a license and collaboration agreement with Benitec, a development, marketing, and supply agreement with Arena Pharmaceuticals GmbH ("Arena"), a Loan Agreement with Hercules, an amended services agreement with RSI, a separate service agreement with RSG (see Note 6(A)). In addition, the Company has entered into services agreements with third parties for pharmaceutical manufacturing and research activities. Expenditures to contract research organizations and contract manufacturing organizations represent significant costs in clinical development. Subject to required notice periods and the Company's obligations under binding purchase orders, the Company can elect to discontinue the work under these agreements at any time. The Company expects to enter into other commitments as the business further develops.

During the six months ended September 30, 2018, there were no material changes outside the ordinary course of business to the Company's specified contractual obligations set forth in the contractual obligations table included in the Annual Report, other than to the license agreement for 19,554 square feet of office space in New York, New York, which was originally set to expire in January 2019 and was extended to January 2021. For the three and six-months ended September 30, 2018, the Company incurred \$0.4 million and \$0.9 million, respectively, in rent expense associated with all contractual rent obligations. The following table provides information regarding remaining contractual rent obligations due within each respective year ending March 31, as of September 30, 2018 (in thousands):

	Total	2019	2020	2021
Rent obligations, net of prepayments	\$3,576	\$895	\$1,791	\$890

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, 2018 included in our Annual Report on Form 10-K, filed with the Securities and Exchange Commission (the "SEC") on June 11, 2018. 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 (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). These statements are often identified by the use of words such as "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "project," "will," "would" or the negative or plural of these words or similar expressions or variations, 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 appearing in a number of places 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 and commercialization of AXO-Lenti-PD, AXO-AAV-OPMD and nelotanserin;
- our relationships under our license agreements with Oxford BioMedica (UK) Ltd. and Benitec Biopharma Limited;
- the success of our interactions with international regulatory authorities;
- the anticipated start dates, durations and completion dates of our ongoing and future nonclinical 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;
- the rate and degree of market acceptance and clinical utility of any approved product candidate;
- our ability to identify and in-license or acquire additional 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 product 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;
 - and
- our stated objective of becoming the leading gene therapy company focused on developing a pipeline of innovative product candidates for debilitating neurological and neuromuscular diseases such as Parkinson's disease, oculopharyngeal muscular dystrophy ("OPMD"), amyotrophic lateral sclerosis ("ALS"), frontotemporal dementia, and other indications.

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 (the "FDA") and other regulatory authorities and financial trends that we believe may affect our financial condition, results of operations, business strategy, nonclinical 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 gene therapy company focused on developing a pipeline of innovative product candidates for debilitating neurological and neuromuscular diseases such as Parkinson's disease, OPMD, ALS, frontotemporal dementia, and other indications. We are also developing nelotanserin for the treatment of Lewy body dementia ("LBD") and potentially other neurology and psychiatry indications.

Our near-term focus is to develop our gene therapy product candidates AXO-Lenti-PD, a potential one-time treatment for Parkinson's disease, and AXO-AAV-OPMD, a potential one-time treatment for OPMD. In October 2018, we began a clinical study of AXO-Lenti-PD in patients with Parkinson's disease and intend to begin a clinical study of AXO-AAV-OPMD in patients with OPMD in the second half of 2019. Prior to the recent in-licensing of AXO-Lenti-PD in June 2018 and AXO-AAV-OPMD in July 2018, our primary focus had been on developing nelotanserin, a selective inverse agonist of the 5-HT_{2A} receptor, and intepirdine, an antagonist of the 5-HT₆ receptor, for which development was discontinued in January 2018. Topline data from the ongoing Phase 2 study of nelotanserin in REM Sleep Behavior Disorder ("RBD") in LBD patients is expected to be available in December 2018. We are evaluating the possibility of partnering or pursuing other strategic opportunities for nelotanserin. In October 2018, we discontinued our development plans for RVT-104 as a potential treatment for patients with Alzheimer's disease or dementia with Lewy bodies ("DLB"), which is a sub-type of LBD.

In January 2018, we announced the discontinuation of our development of intepirdine, an antagonist of the 5-HT₆ receptor, following our announcement that neither the Phase 2b HEADWAY clinical trial of intepirdine in patients with DLB nor the pilot Phase 2 Gait and Balance clinical trial of intepirdine in patients with dementia and gait impairment met their respective primary endpoints, and the September 2017 announcement that our Phase 3 MINDSET clinical trial of intepirdine in patients with mild-to-moderate Alzheimer's disease did not meet its co-primary efficacy endpoints. Following the announcement of Phase 3 MINDSET clinical trial results, we also discontinued further development of RVT-103, which had been intended for use in combination with intepirdine. We remain committed to identifying, developing and commercializing other novel gene therapy treatments for debilitating neurological and neuromuscular diseases. We are continuing to actively explore opportunities to acquire or in-license additional products, product candidates and technologies to further build our pipeline.

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 advancing our product candidates into clinical development. To date, we have not generated any revenue and we have financed our operations primarily through the public and private offerings of our equity securities and our venture debt financing. As of September 30, 2018, we had \$90.7 million of cash. In July 2018, we received \$25.0 million of net proceeds from the issuance and sale of our common shares in a private placement to RSL. We recorded net losses of \$33.8 million and \$69.1 million for the three months ended September 30, 2018 and 2017, respectively, \$85.7 million and \$138.4 million for the six months ended September 30, 2018 and 2017, respectively, and \$221.6 million for the year ended March 31, 2018. 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
Gene Therapy Programs		
AXO-Lenti-PD	Parkinson's disease	Clinical
AXO-AAV-OPMD	Oculopharyngeal muscular dystrophy	Preclinical
AXO-AAV-ALS	Amyotrophic lateral sclerosis	Research
AXO-AAV-FTD	Frontotemporal dementia	Research
Four additional AXO-AAV Collaboration Programs	Undisclosed	Research
Small Molecule Program		
Nelotanserin	Visual hallucinations in LBD REM sleep behavior disorder in LBD	Phase 2 Pilot Study Completed Phase 2 Ongoing

Gene Therapy Programs

AXO-Lenti-PD

Overview

AXO-Lenti-PD (also known as OXB-102) is an in vivo lentiviral gene therapy investigational product candidate currently being developed for the one-time treatment of Parkinson's disease. We licensed the worldwide development and commercialization rights to AXO-Lenti-PD and its predecessor product candidate ProSavin® from Oxford BioMedica (UK) Ltd. ("Oxford BioMedica"), under an exclusive license agreement (the "Oxford BioMedica Agreement") entered into in June 2018.

AXO-Lenti-PD delivers a construct of three genes that encode the critical enzymes required for the biochemical synthesis of dopamine from endogenous tyrosine. The three enzymes are: Tyrosine Hydroxylase (or TH, the enzyme that converts tyrosine to levodopa, or "L-dopa"), Cyclohydrolase 1 (or CH1, the rate-limiting enzyme for synthesis of Tetrahydrobiopterin, or BH4, a critical cofactor for production of L-dopa), and Aromatic L-Amino Acid Decarboxylase (or AADC, the enzyme that converts L-dopa to dopamine). AXO-Lenti-PD is delivered by a one-time MRI-guided stereotactic infusion into the putamen. We believe that delivery of all three of these genes will enable the continuous, tonic, endogenous synthesis of dopamine in this region of the brain that is suffering from loss of dopaminergic innervation. Dopamine deficiency plays a central role in Parkinson's disease and we believe that restoring dopamine synthesis capability in patients will offer lasting improvement in the symptoms of Parkinson's disease. Oxford BioMedica previously conducted a Phase 1/2 clinical study with ProSavin (also known as OXB-101), an earlier version of this product candidate. In this clinical trial, ProSavin was observed to have a favorable long-term safety profile and demonstrated effects on motor function, supporting proof-of-concept. AXO-Lenti-PD delivers a re-engineered transgene construct relative to ProSavin and has been demonstrated to increase dopamine production in nonclinical studies.

Parkinson's Disease Overview

Parkinson's disease is a chronic neurodegenerative disorder that primarily results in progressive and debilitating motor symptoms. It is estimated that up to one million people in the United States and 7 million to 10 million people worldwide suffer from Parkinson's disease. It typically develops between the ages of 55 and 65 years and affects approximately 1% of people over the age of 60 years. The underlying factors that result in the development of Parkinson's disease are largely unknown. However, Parkinson's disease is a neurodegenerative disease that results in reduced levels of the neurotransmitter dopamine in the striatum, a region in the brain. Dopamine is essential for movement, and low levels of dopamine in patients with Parkinson's disease are believed to result in the typical motor symptoms of the disease, including hypo- and bradykinesia, rigidity, tremor, and postural instability.

The treatment of Parkinson's disease is currently limited to symptomatic treatments, as no therapies have proven effective in altering the course of the disease or addressing the underlying pathophysiological processes. The mainstay of treatment typically involves the daily administration of oral L-dopa, the precursor to dopamine. While L-dopa is effective in controlling motor symptoms early in the disease, progressive loss of dopaminergic neurons and chronic L-dopa therapy are believed to contribute to the "wearing off" of L-dopa's efficacy in the more advanced stages of the disease. Patients become increasingly less responsive to oral L-dopa therapy and require higher doses to manage their symptoms. More advanced Parkinson's disease patients often begin to experience "on-off" motor fluctuations, characterized by unpredictable "OFF periods" of reduced mobility and increased rigidity and tremor. In addition, abnormal and involuntary movements known as dyskinesias may occur at higher L-dopa blood levels. Approximately 10% of patients per year develop "on-off" motor fluctuations after starting L-dopa therapy.

As Parkinson's disease progresses, other therapies can be given in combination with L-dopa and include dopamine receptor agonists and inhibitors of enzymes related to dopamine metabolism, such as monoamine oxidase B (MAO-B) and catechol O-methyl transferase (COMT). These therapies aim to further improve overall dopaminergic function. Patient-friendly treatment options for motor fluctuations in advanced Parkinson's disease are limited. Subcutaneous injections of the dopamine agonist apomorphine are used for the acute treatment of OFF episodes. Duopa/Duodopa is an enteral suspension of L-dopa and the peripheral AADC inhibitor carbidopa that is continuously administered over the course of the day through a surgically-placed percutaneous endoscopic gastrostomy with jejunal ("PEG-J") tube to reduce fluctuations in L-dopa blood levels. Deep-Brain Stimulation ("DBS"), a procedure in which electrodes are surgically placed in the basal ganglia, either in the subthalamic nucleus or internal globus pallidus, is another option in advanced Parkinson's disease. Through an impulse generator, electrical stimuli are delivered to the brain to modulate neural signals within these target regions. It remains unclear exactly how DBS improves the symptoms of Parkinson's disease. Both Duopa/Duodopa and DBS require indwelling hardware -- a PEG-J tube, or electrodes, leads and impulse generator -- respectively.

Predecessor Product Candidate: ProSavin (OXB-101)

ProSavin, the predecessor gene therapy candidate to AXO-Lenti-PD, delivered the same three genes (AADC, TH, and CH1) as AXO-Lenti-PD in the same lentiviral vector, but in a different payload configuration. AXO-Lenti-PD was the result of multifactorial experimentation to modify the payload configuration to improve endogenous dopamine production. The initial Phase 1/2 clinical trial of ProSavin was completed in 2012 and long-term follow-up is ongoing.

Nonclinical Studies for ProSavin

Nonclinical studies in non-human primate models of Parkinson's disease demonstrated that ProSavin can safely restore striatal dopamine production to approximately 50% and correct motor deficits without associated dyskinesias (p-value < 0.05). ProSavin was observed to improve Parkinson's disease symptoms and clinical disease severity in the same non-human primate model, with a durable response seen up to 12 months (p-value < 0.05 at all time points beyond week 4). One of the ProSavin treated non-human primates was continued on the study and exhibited a sustained motor improvement until the study was concluded at 44 months. Nonclinical study data did not reveal adverse reactions nor findings with potential impact on patient safety and provided pertinent data on the optimal method of delivery in the clinic. ProSavin was also observed to be well tolerated when co-administered with L-dopa and apomorphine, indicating that it can be used in conjunction with these commonly used Parkinson's disease medications.

In summary, these experiments were determined to demonstrate the long-term safety of therapeutic doses of ProSavin as well as significant efficacy to improve measures of movement and reduce dyskinesias in animal models. These results supported the initiation of clinical trials for ProSavin.

Phase 1/2 Clinical Trial of ProSavin

ProSavin was evaluated for safety and efficacy in a Phase 1/2 study in patients with advanced Parkinson's disease by Oxford BioMedica. In this study, ProSavin was observed to be well-tolerated with sustained improvements on motor function as measured by the Unified Parkinson's Disease Rating Scale ("UPDRS") Part III (motor) score in the state "OFF" levodopa medication, which we refer to as UPDRS Part III "OFF." The Phase 1/2 clinical trial was conducted at sites in the United Kingdom and France on a total of 15 patients with advanced Parkinson's disease. Three dose levels of ProSavin were assessed in four patient cohorts: dose level one (1.9×10^7 transducing units, or "TU"; cohort 1); dose level two (4.0×10^7 TU; cohorts 2a and 2b); and dose level three (1.0×10^8 TU; cohort 3). Cohorts 2b and 3 underwent a modified delivery method to increase the rate of delivery of the viral vector. The primary endpoints were the number and severity of adverse events as well as the UPDRS Part III "OFF" scores at 6 months after gene therapy administration. No serious adverse events related to ProSavin or the surgical procedure were reported. Reported adverse events ("AEs") were generally mild and related to either Parkinson's disease progression or L-dopa-induced dyskinesias that were ameliorated with reduction of L-dopa administration. The most common AEs in the first 12 months were dyskinesia (n=11 subjects), "on-off" motor fluctuations (n=9), headache (n=4), and akinesia (n=3). Across all patients, mean UPDRS Part III "OFF" scores were significantly improved at six months (33% reduction, p-value=0.0001) and 12 months (31% reduction, p-value=0.0001). Sustained improvement was seen through six years of follow-up and the long-term follow-up study is still ongoing (10 years exposure in the earliest subject). Clinical data from this study were published in *The Lancet* in 2014 and long-term follow-up data from this study were published in *Human Gene Therapy Clinical Development* in 2018.

Second-Generation Product Candidate: AXO-Lenti-PD

AXO-Lenti-PD is a re-engineered gene therapy product candidate that was selected following multifactorial experimentation to modify the payload configuration of ProSavin to further improve dopamine production. The modifications included a different ordering of the genes, the fusion of TH and CH1 with a flexible linker, and the removal of a genetic control element between TH and AADC. We believe these changes lead to more balanced stoichiometry of gene expression and colocalization of enzymatic activity. The targeted net result is improved dopamine production in transduced cells.

Nonclinical studies for AXO-Lenti-PD

In vitro experiments with AXO-Lenti-PD demonstrated up to 10-fold increases in dopamine + L-dopa production over ProSavin. In vivo experiments in non-human primate models showed increased AADC activity in the brain with AXO-Lenti-PD compared to ProSavin as measured by PET scans. Functionally, in non-human primate models at approximately 1/5th of the dose, AXO-Lenti-PD demonstrated a similar level of improvement in spontaneous locomotor activity compared to ProSavin. We believe these data provide evidence that AXO-Lenti-PD has greater potency compared to ProSavin in terms of dopamine production, enzymatic activity and functional improvement in animal models of Parkinson's disease.

Clinical Study of AXO-Lenti-PD

In October 2018, we initiated a clinical study of AXO-Lenti-PD in patients with Parkinson's disease with initial clinical data expected to be available in the first half of 2019. The planned study design consists of two parts:

Part A is a non-randomized dose-escalation of multiple potential dose levels.

Part B is a double-blind design with patients randomized either to an active group receiving the optimal dose as determined in Part A, or a control group receiving an imitation "sham" surgical procedure.

The study will evaluate the safety and tolerability of AXO-Lenti-PD as well as assess efficacy using clinical measures of motor function, such as UPDRS Part III, patient diaries and biomarkers.

Manufacturing for AXO-Lenti-PD

We intend to use Oxford Biomedica as our contract manufacturer for current good manufacturing practices ("cGMP") supply of AXO-Lenti-PD pursuant to a clinical supply agreement that will be negotiated by the parties. Oxford Biomedica has already produced the initial cGMP clinical trial material for initiation of the clinical study using an adherent cell process. Oxford Biomedica is currently developing a cell suspension process to support commercial scale cGMP manufacturing of AXO-Lenti-PD, which we intend to use in Part B of the clinical study.

AXO-AAV Programs

Silence-and-Replace Technology

The Silence-and-Replace technology platform is designed to produce a long-term restoration of normal gene function and is achieved by combining RNA interference ("RNAi") (silence) with gene therapy (replace) in a single administration of a single adeno-associated viral ("AAV") vector construct. This approach may be applicable to various genetic diseases, particularly autosomal dominant genetic disorders caused by nucleotide repeat expansion. Multiple neurological and muscular diseases are associated with erroneous expression of a mutated gene. RNAi has shown potential to silence the expression of disease-associated genes. Commonly-used RNAi approaches, in which small interfering RNA ("siRNA") is introduced directly into the cell, achieve only transient gene silencing and are limited by the requirement for repeated administration and variable concentrations of siRNA over time. To provide lasting gene silencing, the Silence-and-Replace technology employs ddRNAi, in which viral vectors deliver a DNA construct that produces short hairpin RNAs ("shRNAs"), which are processed by the cell into siRNAs, which then silence the mutated genes.

In an autosomal dominant genetic disorder, particularly one caused by nucleotide repeat expansion, silencing of the mutant gene can also lead to silencing of the wild type gene, which may be required for normal function. The Silence-and-Replace strategy is designed to address this potential issue by delivering a functional copy of the gene that is re-engineered to be resistant to knockdown. The gene that encodes the functional protein may be contained within the same viral vector as the ddRNAi construct.

AXO-AAV-OPMD Program

Overview

The AXO-AAV-OPMD Program is an investigational gene therapy being developed as a one-time treatment for OPMD, which we licensed from Benitec Biopharma Limited ("Benitec") in July 2018. The Program utilizes an AAV vector to deliver a Silence-and-Replace construct to silence the mutant poly-A binding protein N1 ("PABPN1") gene that causes OPMD and replace it with a functional copy of the PABPN1 gene. This Silence-and-Replace approach aims to knock down the expression of both the wild-type and mutant PABPN1 gene through ddRNAi, while at the same time expressing a re-engineered copy of the PABPN1, which is resistant to silencing and codes for the functional PABPN1 protein. The gene therapy will be delivered in a single administration directly into target muscle tissue to provide long-term correction of muscle pathology and restoration of function.

Oculopharyngeal Muscular Dystrophy Overview

OPMD is a muscular disease that is inherited through a primarily autosomal dominant pattern. OPMD is estimated to affect approximately 15,000 people in North America and Europe. The disease generally presents in patients between the ages of 40 and 70 years old and is characterized primarily by progressive difficulty swallowing, eyelid drooping, and weakness of the proximal extremities. Swallowing difficulties can have life-threatening consequences, including malnutrition and aspiration pneumonia. As the disease progresses, the swallowing difficulties become more severe and other muscles may become involved. There are no products approved for the treatment of OPMD and therefore, treatment options available to patients are limited. OPMD is caused by mutations in the gene coding for PABPN1, a ubiquitously expressed protein that regulates the processing of messenger RNAs. The normal PABPN1 protein contains ten copies of the amino acid alanine, which forms a polyalanine tract. In OPMD, the mutated PABPN1 gene has an expansion of alanine-encoding trinucleotide repeats, resulting in an abnormally long polyalanine tract. The protein that forms from the mutated gene is prone to aggregating into insoluble nuclear inclusion bodies which leads to muscle cell pathology and disease progression.

Nonclinical studies for AXO-AAV-OPMD

Data from mouse models of OPMD showed gene therapy from the AXO-AAV-OPMD Program provided up to 86% inhibition of PABPN1 gene expression, while restoring functional PABPN1 transgene expression up to 63% of normal levels. The A17 mouse model is a well-validated in vivo model that is designed to exhibit many of the key pathological features of OPMD patients. The levels of gene silencing and expression achieved in this model coincided with decreased muscle pathology and a restoration of muscle force and muscle weight to near wild-type levels.

Planned Clinical Study for AXO-AAV-OPMD Program

We expect to initiate a clinical study for the investigational AXO-AAV-OPMD Program in the second half of 2019. The FDA and European Commission have granted Orphan Drug Designation to the AXO-AAV-OPMD Program for the treatment of OPMD.

AXO-AAV-OPMD Program Manufacturing

We plan to use a contract manufacturer for cGMP manufacturing of AXO-AAV-OPMD. We are currently working with a third-party cGMP manufacturer and have completed an engineering run of AXO-AAV-OPMD at the 250L scale using a baculovirus-based suspension production system in anticipation of cGMP manufacturing for clinical trials.

Additional Collaboration Programs

Under our license and collaboration agreement with Benitec (the "Benitec Agreement"), we will pursue five additional investigational gene therapy research plans as part of collaboration programs focused on genetic neurological or neuromuscular disorders utilizing Benitec's technologies. We plan to initiate a research plan to develop gene therapy products targeting the C9orf72 gene, which is associated with ALS and frontotemporal dementia ("FTD"). In addition, we plan to initiate four other research plans focused on undisclosed genetic neurological disorders.

ALS and FTD are neurological disorders that have been linked to hexanucleotide repeats in the C9orf72 gene. Thirty to forty percent of familial ALS cases are associated with C9orf72 gene mutations and these patients have a progressive muscle weakness resulting from the death of motor neurons in the spinal cord and brain. Patients with FTD associated with C9orf72 gene mutations have a progressive brain disorder that affects personality, behavior, language and movement. While the exact role of C9orf72 gene mutation is unknown, both expression of the mutated C9orf72 gene and lack of functional C9orf72 gene are believed to be implicated. We believe Silence-and-Replace gene therapy is a promising approach for the restoration of normal C9orf72 gene function and has the potential to deliver lasting benefits for ALS and FTD patients.

Small Molecule Program

Nelotanserin

Overview

In October 2015, we acquired from our majority shareholder, Roivant Sciences Ltd. ("RSL"), the global rights to nelotanserin, a selective inverse agonist of the 5-HT_{2A} receptor. To date, we have been investigating and developing nelotanserin to address visual hallucinations and RBD in patients with LBD. In January 2018, we reported results for a pilot Phase 2 Visual Hallucination study of nelotanserin in patients with LBD. Nelotanserin was generally well tolerated but did not show any statistical trends of improvement on prespecified analyses of various scales to assess visual hallucinations. However, we did observe positive trends on UPDRS Part III in the primary efficacy population (3.12 point improvement, p=0.075 unadjusted) which will also be assessed in the ongoing RBD study. We expect topline data, anticipated to be available in December 2018, from our currently ongoing Phase 2 study of nelotanserin to address visual hallucinations and RBD in patients with LBD.

Nelotanserin for REM Sleep Behavior Disorder in Lewy Body Dementia

Medical Need

RBD is a common clinical feature of LBD, and is a condition where patients lose normal sleep paralysis resulting in the physical acting out of 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 severe 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.

Clinical Development

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. Due to challenges with recruitment for this study, we elected to close enrollment prior to reaching our enrollment target. Because of this smaller than planned enrollment, the study may not qualify as pivotal. We expect to receive top-line results for this study in December 2018. Patients completing the double-blind portion of this study were eligible to enroll in an open label extension study of nelotanserin.

Our Key Agreements

Oxford BioMedica License Agreement

On June 5, 2018, we, through our wholly owned subsidiary, Axovant Sciences GmbH ("ASG"), entered into the Oxford BioMedica Agreement, pursuant to which we received a worldwide, exclusive, royalty-bearing, sub-licensable license under certain patents and other intellectual property controlled by Oxford BioMedica to develop and commercialize AXO-Lenti-PD and related gene therapy products for all diseases and conditions. In June 2018, as partial consideration for the license, we made an upfront payment to Oxford BioMedica of \$30.0 million, \$5.0 million of which will be applied as a credit against the process development work and clinical supply that Oxford BioMedica will provide to us. Under the terms of the Oxford BioMedica Agreement, we could be obligated to make payments to Oxford BioMedica totaling up to \$55.0 million upon the achievement of specified development milestones and \$757.5 million upon the achievement of specified regulatory and sales milestones. We will also be obligated to pay Oxford BioMedica a tiered royalty from 7% to 10%, based on yearly aggregate net sales of the Gene Therapy Products, subject to specified reductions upon the occurrence of certain events as set forth in the Oxford BioMedica Agreement. These royalties are required to be paid, on a product-by-product and country-by-country basis, until the latest to occur of the expiration of the last to expire valid claim of a licensed patent covering such product in such country, the expiration of regulatory exclusivity for such product in such country, or 10 years after the first commercial sale of such product in such country.

We are solely responsible, at our expense, for all activities related to the development and commercialization of the Gene Therapy Products. Pursuant to the Oxford BioMedica Agreement, we are required to use commercially reasonable efforts to develop, obtain regulatory approval of, and commercialize a Gene Therapy Product in the United States and at least one major market country in Europe. In addition, we are required to meet certain diligence milestones and to include at least one U.S.-based clinical trial site in a pivotal study of a Gene Therapy Product. If we fail to meet any of these specified development milestones, we may cure such failure by paying Oxford BioMedica certain fees, which range from \$0.5 million to \$1.0 million. Pursuant to the Oxford BioMedica Agreement, Oxford Biomedica will be our cGMP manufacturer for AXO-Lenti-PD, subject to a separate clinical and commercial supply agreement to be negotiated between the parties.

Benitec Biopharma License and Collaboration Agreement

On July 8, 2018, we, through our wholly owned subsidiary, ASG, entered into the Benitec Agreement, pursuant to which we received a worldwide, exclusive, royalty-bearing, sub-licensable license under certain patents and other intellectual property controlled by Benitec to develop and commercialize investigational gene therapy AXO-AAV-OPMD and related gene therapy products (collectively, the "AXO-AAV-OPMD Program") for all diseases and conditions.

Under the Benitec Agreement, we will also collaborate with Benitec on five additional research plans ("Collaboration Programs") for other genetic neurological or neuromuscular disorders using Benitec technologies. We will receive a worldwide, exclusive, royalty-bearing, sub-licensable license under certain patents and other intellectual property controlled by Benitec to develop and commercialize products arising from each Collaboration Program.

Under the terms of the Benitec Agreement, we made an upfront payment of \$10.0 million. In addition, we will be obligated to make payments to Benitec totaling up to (i) for the AXO-AAV-OPMD Program, \$67.5 million upon the achievement of specified development and regulatory milestones and \$120.0 million upon the achievement of specified sales milestones, and (ii) for each Collaboration Program, \$33.5 million upon the achievement of specified development and regulatory milestones and \$60.0 million upon the achievement of specified sales milestones. Benitec will receive 30% of net profits of our world-wide sales of products from the AXO-AAV-OPMD Program, subject to an agreed minimum amount for such payments. This profit-sharing payment will be made for so long as we or our affiliates or sublicensees commercialize such products. We will also pay Benitec a tiered royalty based on yearly aggregate net sales of products arising from each Collaboration Program, subject to specified reductions upon the occurrence of certain events as set forth in the Benitec Agreement. These royalties are required to be paid, on a product-by-product and country-by-country basis, until the latest to occur of the expiration of the last to expire valid claim of a licensed patent covering such product in such country, the expiration of regulatory exclusivity for such product in such country, or ten years after the first commercial sale of such product in such country.

Under the Benitec Agreement, Benitec will perform certain research activities for each Collaboration Program and development and manufacturing activities for the AXO-AAV-OPMD Program, and we will reimburse Benitec for its costs incurred, in accordance with an agreed-upon research and development plan and budget. We are solely responsible, at our expense, for all other activities related to the research, development and commercialization of products from the Collaboration Programs and the AXO-AAV-OPMD Program.

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 (the "Arena Development Agreement") with Arena Pharmaceuticals GmbH ("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 Arena Development Agreement, as amended on October 18, 2017. In January 2018, we were notified by Arena that it has assigned all of its rights and obligations under the Arena Development Agreement to an affiliate, 125 Royalty Inc. 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. ("ASI") entered into a services agreement with Roivant Sciences, Inc. ("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 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 ("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 RSI and RSG for their services at a predetermined 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.

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Under the services agreement in effect as of December 31, 2016, we incurred expenses of \$0.9 million and \$1.6 million for the three months ended September 30, 2018 and 2017, respectively, and \$4.3 million and \$4.4 million for the six months ended September 30, 2018 and 2017, 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 and our wholly owned subsidiaries, Axovant Holdings Limited ("AHL"), ASG and ASI entered into a loan and security agreement, as amended on May 24, 2017 and September 22, 2017 (the "Loan Agreement") with Hercules Capital, Inc. ("Hercules") under which we, AHL and ASG (collectively, the "Borrowers") borrowed an aggregate of \$55.0 million (the "Term Loan"). ASI 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. Subsequently, we added our subsidiary Axovant Sciences America, Inc. ("ASA") as a Borrower in July 2017 and our subsidiaries ATH and ATI as Borrowers in April 2018. 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 Loan Agreement until September 1, 2018, followed by monthly installments of principal and interest through March 1, 2021. 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.

The Loan Agreement includes customary affirmative and restrictive covenants and representations and warranties, including a minimum cash covenant, 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.

In connection with the entry into the Loan Agreement, we issued a warrant to Hercules which was exercisable for an aggregate of 274,086 of our common shares at an exercise price of \$12.04 per share. In August 2017, Hercules exercised the warrant on a cashless basis and received a net issuance of 129,827 of our common shares.

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, and preparing for and advancing our product candidates, intepirdine, nelotanserin, RVT-103, RVT-104, AXO-Lenti-PD and AXO-AAV-OPMD, into clinical development. Our research and development expenses include program-specific costs, as well as unallocated internal costs.

Program-specific costs include:

- direct third-party costs, which include expenses incurred under agreements with contract research organizations and contract manufacturing organizations, the cost of consultants who assist with the development of our product

candidates on a program-specific basis, investigator grants, sponsored research, manufacturing costs in connection with producing materials for use in conducting nonclinical and clinical studies, and any other third-party expenses directly attributable to the development of our product candidates; and

upfront payments for the purchase of in-process research and development, which include costs incurred under the Oxford BioMedica Agreement, the Benitec Agreement and the Arena Development Agreement.

Unallocated internal costs include:

- share-based compensation expense for research and development personnel, including expense related to RSL common share awards and RSL options issued by RSL to RSI and RSG employees;
- personnel-related expenses, which include employee-related expenses, such as salaries, benefits and travel expenses, for research and development personnel;
- costs allocated to us under our services agreements with RSI and RSG; and
- other expenses, which includes the cost of consultants who assist with our research and development but are not allocated to a specific program.

Research and development activities will continue to be central to our business model. We expect to continue to incur research and development expense as we continue our development program for nelotanserin in LBD. However, due to the termination of the MINDSET, HEADWAY and Gait and Balance trials of intepirdine, we expect our overall research and development expense to decrease significantly until such time as we undertake additional development programs, including in relation to the AXO-Lenti-PD program, the AXO-AAV-OPMD program, the Collaboration Programs with Benitec and additional product candidates we may in-license or acquire as we pursue our updated business plan. We also expect our share-based compensation and other employee-related expenses for our research and development personnel to increase as a result of the transfer of certain activities from RSI and RSG in July 2018, offset by a reduction in costs allocated to us under our services agreements with RSI and RSG.

Product candidates in later stages of clinical development, such as 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 in advance 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-related expenses, such as salaries, benefits and travel expenses, for general and administrative personnel.

We anticipate that our general and administrative expenses will decrease, primarily as the result of a reduction in share-based compensation and other employee-related expenses for our general and administrative personnel due to the recent reduction in headcount, partially offset by the decision to build out internal administration and finance functions at Axovant and reduce our utilization of RSI services.

Results of Operations for the Three and Six-Months Ended September 30, 2018 and 2017

The following table summarizes our results of operations for the three and six-months ended September 30, 2018 and 2017 (in thousands):

	Three Months Ended September 30,			Six Months Ended September 30,		
	2018	2017	Change	2018	2017	Change
Operating expenses:						
Research and development expenses (includes total share-based compensation expense (benefit) of \$(1,128) and \$5,916 for the three months ended September 30, 2018 and 2017 and \$1,389 and \$12,172 for the six months ended September 30, 2018 and 2017, respectively)	\$21,502	\$38,555	\$(17,053)	\$58,920	\$82,267	\$(23,347)
General and administrative expenses						