Axovant Sciences Ltd.
Form 10-Q
February 07, 2019

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

FORM 10-Q

(Mark One)

ý QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the quarterly period ended December 31, 2018
or
o 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	Not Applicable
(Address of principal executive offices)	(Zip Code)

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 ý No o
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 ý No o
Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer", "accelerated filer", "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange

Act. (Check one):

Large accelerated filer	o	Accelerated filer	x
Non-accelerated filer	o	Smaller reporting company	x
		Emerging growth company	x

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

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

The number of shares outstanding of the Registrant's common shares, $0.00001 par value per share, on February 5, 2019, was 155,534,021.

AXOVANT SCIENCES LTD.
QUARTERLY REPORT ON FORM 10-Q
FOR THE QUARTER ENDED DECEMBER 31, 2018

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PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

AXOVANT SCIENCES LTD.
Condensed Consolidated Balance Sheets
(in thousands, except share and per share data)

	December 31, 2018 (unaudited)	March 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 84,939	$154,337
Prepaid expenses and other current assets	4,883	2,174
Income tax receivable	1,580	1,751
Total current assets	91,402	158,262
Other non-current assets	3,449	—
Property and equipment, net	1,365	2,524
Total assets	$ 96,216	$160,786
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	$ 1,018	$3,949
Due to RSL, RSI and RSG	693	1,011
Accrued expenses	22,911	31,862
Current portion of long-term debt	20,583	9,753
Total current liabilities	45,205	46,575
Long-term debt	28,251	42,925
Total liabilities	73,456	89,500
Commitments and contingencies (Note 11)		
Shareholders' equity:		
Common shares, par value $0.00001 per share, 1,000,000,000 shares authorized, 155,527,771 and 107,788,074 issued and outstanding at December 31, 2018 and March 31, 2018, respectively	2	1
Additional paid-in capital	699,064	628,110
Accumulated deficit	(676,970)	(556,951)
Accumulated other comprehensive income	664	126
Total shareholders' equity	22,760	71,286
Total liabilities and shareholders' equity	$ 96,216	$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 December 31,		Nine Months Ended December 31,	
	2018	2017	2018	2017
Operating expenses:				
Research and development expenses[1] (includes total share-based compensation expense of $1,910 and $2,453 for the three months ended December 31, 2018 and 2017 and $3,299 and $14,625 for the nine months ended December 31, 2018 and 2017, respectively)	$21,483	$ 37,346	$80,403	$ 119,613
General and administrative expenses[2] (includes total share-based compensation expense of $2,648 and $8,186 for the three months ended December 31, 2018 and 2017 and $9,575 and $26,954 for the nine months ended December 31, 2018 and 2017, respectively)	10,933	18,032	33,309	69,662
Total operating expenses	32,416	55,378	113,712	189,275
Other expenses:				
Interest expense	1,906	1,950	5,808	5,702
Other expense (income)	(78)	550	275	324
Loss before income tax expense	(34,244)	(57,878)	(119,795)	(195,301)
Income tax expense	52	24	224	953
Net loss	$(34,296)	$ (57,902)	$(120,019)	$ (196,254)
Net loss per common share — basic and diluted	$(0.27)	$ (0.54)	$(1.01)	$ (1.83)
Weighted average common shares outstanding — basic and diluted	128,771,900	107,719,476	119,183,117	107,241,043

[1] Includes total costs allocated from RSL, RSI and RSG of $0 and $409 for the three months ended December 31, 2018 and 2017, respectively, and $(450) and $5,667 for the nine months ended December 31, 2018 and 2017, respectively.

[2] Includes total costs allocated from RSL, RSI and RSG of $698 and $1,440 for the three months ended December 31, 2018 and 2017, respectively, and $2,772 and $4,936 for the nine months ended December 31, 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 December 31,		Nine Months Ended December 31,	
	2018	2017	2018	2017
Net loss	$(34,296)	$(57,902)	$(120,019)	$(196,254)
Other comprehensive income:				
Foreign currency translation adjustment	92	664	538	414
Total other comprehensive income	92	664	538	414
Comprehensive loss	$(34,204)	$(57,238)	$(119,481)	$(195,840)

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 Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Income	Total Shareholders' Equity
	Shares	Amount				
Balance at March 31, 2018	107,788,074	$ 1	$628,110	$ (556,951)	$ 126	$ 71,286
Issuance of shares upon exercise of stock options	268,352	—	293	—	—	293
Issuance of shares in connection with Private Placement with RSL	14,285,714	—	25,000	—	—	25,000
Shares sold in public offering, net of underwriting discounts and commissions and offering expenses of $1.6 million	33,160,923	1	31,633	—	—	31,634
Shares sold under share sales agreement	24,708	—	61	—	—	61
Share-based compensation expense	—	—	15,534	—	—	15,534
Capital contribution — share-based compensation expense	—	—	(2,660)	—	—	(2,660)
Non-cash capital contribution received by ASG from RSI	—	—	1,093	—	—	1,093
Foreign currency translation adjustment	—	—	—	—	538	538
Net loss	—	—	—	(120,019)	—	(120,019)
Balance at December 31, 2018	155,527,771	$ 2	$699,064	$ (676,970)	$ 664	$ 22,760

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)

	Nine Months Ended December 31,	
	2018	2017
Cash flows from operating activities:		
Net loss	$(120,019)	$(196,254)
Adjustments to reconcile net loss to net cash used in operating activities:		
Disposal of fixed assets	10	—
Foreign currency translation adjustment	538	414
Share-based compensation	12,874	41,579
Depreciation and non-cash amortization	2,123	2,113
Deferred tax assets	—	2,709
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(2,709)	4,362
Other non-current assets	(3,449)	—
Accounts payable	(2,931)	(7,519)
Due to RSL, RSI and RSG	812	(1,559)
Accrued expenses	(8,951)	139
Income tax receivable	171	(2,132)
Net cash used in operating activities	(121,531)	(156,148)
Cash flows from investing activities:		
Purchases of property and equipment	(74)	(4,246)
Net cash used in investing activities	(74)	(4,246)
Cash flows from financing activities:		
Payment of long-term debt	(4,780)	—
Cash proceeds from stock option exercises	293	1,557
Cash proceeds from issuance of common shares, net of costs paid	56,694	134,515
Net cash provided by financing activities	52,207	136,072
Net change in cash and cash equivalents	(69,398)	(24,322)
Cash and cash equivalents—beginning of period	154,337	212,573
Cash and cash equivalents—end of period	$84,939	$188,251
Non-cash financing activities:		
Non-cash capital contribution received by ASG from RSI	$1,093	$—
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. ("ASL"), together with its wholly owned subsidiaries (the "Company"), is a clinical-stage company focused on gene therapy for neurological and neuromuscular diseases. The Company is developing a pipeline of innovative product candidates for the treatment of these debilitating diseases, including GM1 gangliosidosis, GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease), Parkinson's disease, oculopharyngeal muscular dystrophy ("OPMD"), amyotrophic lateral sclerosis ("ALS"), frontotemporal dementia and other neurological and neuromuscular indications. The Company believes gene therapy may offer transformative patient outcomes in areas of high unmet medical need, with the potential for a more capital efficient path to commercialization.

ASL is an exempted limited company incorporated under the laws of Bermuda. It was originally formed under the name Roivant Neurosciences Ltd. in October 2014 and changed its name to ASL in March 2015. ASL has seven wholly owned subsidiaries: Axovant Holdings Limited ("AHL"), a direct wholly owned subsidiary of ASL, 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; Axovant Treasury Holdings, Inc. ("ATH"), a direct wholly owned subsidiary of ASL and Axovant Treasury, Inc. ("ATI"), a direct wholly owned subsidiary of ATH, were each incorporated in Delaware in March 2018; and Axovant Sciences Europe Limited, a direct wholly owned subsidiary of AHL, was incorporated in Ireland in December 2018. As of December 31, 2018, ASG held all of the Company's intellectual property rights and is the principal operating company for conducting the Company’s business.

Since 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.

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 nine-months ended December 31, 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 as amended by Accounting Standards Updates ("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.

The accompanying unaudited condensed consolidated financial statements and notes have been prepared on the basis that the Company will continue as a going concern, and do not include any adjustments to reflect the possible future effects on the recoverability and classification of assets or the amounts and classification of liabilities that may result from the possible inability of the Company to continue as a going concern (See Note 2(B)).

There have been no significant changes in the Company’s accounting policies from those disclosed in its Annual Report, except as follows:

Cash and Cash Equivalents

The Company considers all highly liquid investments purchased with original maturities of 90 days or less at acquisition to be cash equivalents. Cash and cash equivalents include cash held in banks and amounts held in money market funds.

(B) Liquidity:

As of December 31, 2018, the Company’s cash and cash equivalents totaled $84.9 million. For the fiscal year ended March 31, 2018 and the three and nine-months ended December 31, 2018, the Company incurred net losses of $221.6 million, $34.3 million and $120.0 million, respectively. As of December 31, 2018, the Company had aggregate net interest-bearing indebtedness of $48.8 million, of which $20.6 million was due within one year. The Company also had $24.6 million of other non-interest-bearing current liabilities due within one year. The Company’s Loan Agreement (as defined in Note 5) with Hercules Capital, Inc. (“Hercules”) requires that the Company maintain a minimum cash balance, which was previously $35.0 million, but has been reduced to $30.0 million following the achievement of certain clinical milestones as set forth in the Loan Agreement. The Company anticipates that its

current cash and cash equivalent balances will not be sufficient to maintain compliance with the minimum liquidity financial covenant under its Loan Agreement beyond the one-year period following the date that these unaudited condensed consolidated financial statements were issued if the Loan Agreement is not amended or an additional financing is not completed. Failure to meet this minimum covenant would be considered an event of default under the Loan Agreement and could result in the acceleration of the Company's existing indebtedness. These factors raise substantial doubt about the Company's ability to continue as a going concern for the one-year period following the date that these unaudited condensed consolidated financial statements were issued.

The Company is currently in the clinical stage of operations, has not yet achieved profitability, and anticipates that it will continue to incur net losses for the foreseeable future. The Company's principal sources of cash have included proceeds from the issuance of common stock, proceeds from the exercise of stock options and warrants to purchase common stock, and proceeds from the incurrence of debt. The Company's principal uses of cash have included cash used in operations including funding clinical trials, payments relating to purchases of property and equipment, payments related to acquisition and licensing of product candidates, and payment of interest on borrowings. The Company expects that the principal uses of cash in the future will be for continuing operations, further clinical trials, payments of milestones related to its current licenses, acquisition or licensing of additional product candidates, funding of research and development, the hiring of personnel, payment of interest and principal on borrowings and general working capital requirements. Although the Company has successfully obtained financing in the past, and management believes that it will continue to do so in the future, ASC Subtopic 205-40, "Financial Statement Presentation - Going Concern," does not permit future financing activities to be included in the Company's assessment of its liquidity.

The Company will need to raise additional capital resources, which may include proceeds from offerings of the Company's equity securities or debt, cash received from the exercise of outstanding common stock options or warrants, or transactions involving product development, technology licensing or collaboration arrangements, or other sources to complete its currently planned development programs. Adequate additional funding may not be available to the Company on acceptable terms, or at all.

(C) 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, Roivant Sciences Ltd. ("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.

(D) 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 16.8 million common shares were not included in the calculation of diluted weighted-average common shares outstanding for each of the three and nine-months ended December 31, 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 5.3 million and 5.6 million common shares were not included in the calculation of diluted weighted-average common shares outstanding for the three and nine-months ended December 31, 2017, respectively, because they were anti-dilutive given the net loss of the Company.

(E) 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 and cash equivalents, accounts payable and long-term debt. Cash consists of non-interest-bearing deposits denominated in the U.S. dollar and Swiss franc, while cash equivalents consists of interest-bearing money market fund deposits denominated in the U.S. dollar, which are invested in debt securities issued or guaranteed by the U.S. government and repurchase agreements fully collateralized by U.S. Treasury and U.S. government securities. Cash and accounts payable are stated at their respective historical carrying amounts, which approximate fair value due to their short-term nature. The Company measures the fair value of money market funds included in cash and cash equivalents based on quoted prices in active markets for identical securities, which was $30.0 million as of December 31, 2018. The carrying value of the Company’s debt 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 as of December 31, 2018.

(F) Recent Accounting Pronouncements:

The FASB issued ASU No. 2016-02, "Leases (Topic 842)" in February 2016, ASU No. 2018-10, "Codification Improvements to Topic 842, Leases" and ASU No. 2018-11, "Leases (Topic 842): Targeted Improvements" in July 2018, and ASU No. 2018-20, "Narrow-Scope Improvements for Lessors" in December 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 expects to adopt the provisions of the Lease Standards for the fiscal year beginning April 1, 2019. 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 nine-months ended December 31, 2018.

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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"). On December 22, 2017, 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") was enacted in the United States, which introduced a comprehensive set of tax reforms. 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 to provide guidance for companies that would allow for a measurement period of up to one year after the enactment date of the Tax Cuts and Jobs Act, and was effective immediately. The Tax Cuts and Jobs Act did not have a material impact on the Company’s consolidated financial statements since its 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. As a result of finalizing the Company’s fiscal 2018 operating results, the issuance of new interpretative guidance, and other analyses performed, the Company finalized its accounting related to the impacts of the Tax Cuts and Jobs Act and recorded immaterial measurement period adjustments in the period ended December 31, 2018.

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 was applied as a credit against the process development work and clinical supply that Oxford BioMedica is obligated to provide to the Company over the term of the Oxford BioMedica Agreement. 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 $30.0 million upfront nonrefundable payment to Oxford BioMedica under the Oxford BioMedica Agreement was recorded as research and development expense in the Company's unaudited condensed consolidated statements of operations during the nine months ended December 31, 2018. As the remaining $5.0 million of the upfront payment under the licensing agreement represents a nonrefundable payment for process development work and clinical supply that Oxford BioMedica is obligated to provide over the term of the Oxford BioMedica Agreement, the Company fully capitalized this portion of the payment upon execution, with $1.4 million remaining capitalized within prepaid expenses and other current assets and $2.8 million remaining capitalized within other non-current assets in its unaudited condensed consolidated balance sheet as of December 31, 2018, which is recorded to research and development expense as the process development work and clinical supply are provided by Oxford BioMedica. Additionally, the Company incurred $1.9 million and $3.2 million of AXO-LENTI-PD program-specific costs in its unaudited condensed consolidated statements of operations during the three and nine-months ended December 31, 2018, respectively. During the three and nine-months ended December 31, 2018, the Company paid a total of $0.8 million and $30.9 million, respectively, to Oxford BioMedica, including the upfront nonrefundable payment during the nine months ended December 31, 2018.

Benitec Biopharma License and Collaboration Agreement

On July 8, 2018, the Company, through its wholly owned subsidiary 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 nine-months ended December 31, 2018. Additionally, the Company incurred $1.9 million and $3.6 million of AXO-AAV-OPMD program-specific costs in its unaudited condensed consolidated statements of operations during the three and nine-months ended December 31, 2018, respectively. During the three and nine-months ended December 31, 2018, the Company paid a total of $1.4 million and $11.4 million, respectively, to Benitec, including the upfront nonrefundable payment. Further, the Company could 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.

The University of Massachusetts Medical School Exclusive License Agreement

On December 7, 2018, the Company, through its wholly owned subsidiary, ASG, entered into an exclusive license agreement (the "UMMS Agreement"), with the University of Massachusetts Medical School ("UMMS") pursuant to which the Company received a worldwide, royalty-bearing, sub-licensable license under certain patent applications and any patents issuing therefrom, biological materials and know-how controlled by UMMS to develop and commercialize gene therapy product candidates, including AXO-AAV-GM1 and AXO-AAV-GM2, for the treatment of GM1 gangliosidosis and GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease), respectively. This license is exclusive with respect to patents and biological materials and non-exclusive with respect to know-how and is subject to UMMS' retained rights for academic research, teaching and non-commercial patient care purposes, as well as to certain pre-existing rights of the U.S. government.

Under the UMMS Agreement, the Company is solely responsible, at its expense, for the research, development and commercialization of the licensed product candidates. The Company will reimburse UMMS for payments made by UMMS for the manufacture of clinical trial materials for the Company, up to a specified amount. The Company is obligated to use diligent efforts to develop and commercialize the licensed product candidates and is required to achieve certain development and commercial milestones in accordance with the timeline set forth in the agreement.

The Company has evaluated the UMMS 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 upfront payment of $10.0 million made from the Company to UMMS was recorded as research and development expense in the Company's unaudited condensed consolidated statements of operations during the three and nine-months ended December 31, 2018. In addition, the Company could be obligated to make payments to UMMS totaling up to $24.5 million upon the achievement of specified development and regulatory milestones and $39.8 million upon the achievement of specified commercial milestones. The Company is also obligated to pay UMMS tiered mid-single digit royalties based on yearly net sales of the licensed products, subject to a specified annual minimum amount. Additionally, the Company will pay UMMS a percent of any revenues it receives from any third-party sublicenses to licensed products at rates ranging in the mid-single digits to mid-teens.

The UMMS Agreement will expire upon the expiration of the Company's obligations to make royalty payments to UMMS, which continues until the later of the expiration of licensed patents and any applicable orphan designation exclusivity and 10 years after the first commercial sale of the licensed products. Upon such expiration, the licenses granted to the Company by UMMS will automatically convert to perpetual, irrevocable, worldwide royalty-free licenses. The Company has the right to terminate the UMMS Agreement at any time upon 90 days' advance written notice to UMMS. Either party may terminate the UMMS Agreement for the other party's uncured material breach upon 60 days' advance written notice, including in the event that UMMS reasonably determines the Company has not fulfilled its diligence obligations.

Note 4—Accrued Expenses

As of December 31, 2018, and March 31, 2018, the Company’s accrued expenses consisted of the following (in thousands):

	December 31, 2018	March 31, 2018
Research and development expenses	$ 16,449	$21,855
Salaries, bonuses, and other compensation expenses	3,562	7,718
Legal expenses	1,491	779
Other expenses	1,409	1,510
Total accrued expenses	$ 22,911	$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, 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 $30.0 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 (inthousands):

	December 31, 2018	March 31, 2018
Principal amount	$ 50,220	$ 55,000
Less: unamortized discount and debt issuance costs	(1,386)	(2,322)
Loan payable less unamortized discount and debt issuance costs	48,834	52,678
Less: current portion of long-term debt	(20,583)	(9,753)
Long-term loan payable, net of current maturities	$ 28,251	$ 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 $0.7 million and $1.4 million for the three months ended December 31, 2018 and 2017, respectively, and $5.0 million and $5.8 million for the nine months ended December 31, 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. Shankar Ramaswamy, MD, the Chief Business Officer of ASI, and a former employee of RSI, is the brother of Vivek Ramaswamy. Geetha Ramaswamy, MD was 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 and Shankar Ramaswamy, MD (see Note 8(B)(3)).

Salary expenses for Shankar Ramaswamy, MD were $75,000 and $66,950 for the three months ended December 31, 2018 and 2017, respectively, and $225,000 and $200,850 for the nine months ended December 31, 2018 and 2017, respectively. Salary expenses for Geetha Ramaswamy, MD were $133,900 for the nine months ended December 31, 2017.

(C) RSL Financings:

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).

On December 18, 2018, the Company issued and sold 33,160,923 common shares in a follow-on public offering, including 3,160,923 common shares sold pursuant to the exercise of the underwriters' option to purchase additional shares, at an offering price of $1.00 per common share for gross proceeds of $33.2 million, including 10,000,000 shares issued and sold to RSL. The aggregate net proceeds to the Company were approximately $31.6 million, after deducting underwriting discounts and commissions and offering expenses incurred (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 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 the nine months ended December 31, 2018, RSL incurred $0.3 million and RSI incurred $1.1 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 December 31, 2018, approximately $74.9 million of the Company's common shares remained available for sale under the sales agreement.

On December 18, 2018, the Company issued and sold 33,160,923 common shares in a follow-on public offering, including 3,160,923 common shares sold pursuant to the exercise of the underwriters' option to purchase additional shares, at an offering price of $1.00 per common share for gross proceeds of $33.2 million, including 10,000,000 shares issued and sold to RSL (see Note 6(C)). The aggregate net proceeds to the Company were approximately $31.6 million, after deducting underwriting discounts and commissions and offering expenses incurred.

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 December 31, 2018, a total of 7.0 million common shares were available for future grant under the 2015 Plan, and options to purchase approximately 16.8 million common shares were outstanding under the 2015 Plan, with a weighted average exercise price of $4.24 per share.

(A) Stock Options Granted to Employees and Directors:

During the nine months ended December 31, 2018 and 2017, the Company granted options to its employees and directors under the 2015 Plan to purchase a total of 3.8 million and 10.9 million common shares, respectively. The stock options granted during the nine months ended December 31, 2018 include approximately 0.9 million common shares with market-based performance conditions to employees with a weighted average exercise price of $2.56 per share, a contractual term of 10 years, and a corresponding estimated grant date fair value of $1.5 million. As of December 31, 2018, stock options with market-based performance conditions to purchase 1.8 million common shares were outstanding with a weighted-average exercise price of $1.99 per share. The market-based performance options vest based on exceeding certain closing prices of the Company's common shares. As of December 31, 2018, stock options with market-based performance conditions to purchase approximately 0.3 million common shares with a

weighted-average exercise price of $1.46 per share were outstanding and vested, which occurred during the nine months ended December 31, 2018.

The Company recorded total share-based compensation expense related to stock options issued to Company employees and directors of $4.5 million and $9.9 million, respectively, for the three months ended December 31, 2018 and 2017, and $14.3 million and $34.8 million for the nine months ended December 31, 2018 and 2017. At December 31, 2018, total unrecognized compensation expense related to non-vested options was $22.0 million, which is expected to be recognized over the remaining weighted-average service period of 2.36 years.

(B) Share-Based Compensation for Related Parties:

(1) Stock Options Granted to Non-Employees:

During the nine months ended December 31, 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 $(0.3) million and $0.1 million of share-based compensation expense (benefit) for the three months ended December 31, 2018 and 2017, respectively, and $0.1 million and $1.5 million for the nine months ended December 31, 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 $0.3 million as of December 31, 2018, which is expected to be recognized over the remaining weighted-average service period of 2.23 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 $27 thousand and $0.4 million for the three months ended December 31, 2018 and 2017, respectively, and $(2.7) million and $4.8 million for the nine months ended December 31, 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 $0.8 million and $0.8 million for the three months ended December 31, 2018 and 2017, respectively, and $2.5 million and $3.2 million for the nine months ended December 31, 2018 and 2017, respectively, in connection with options vesting for Geetha Ramaswamy, MD and Shankar Ramaswamy, MD.

Shankar Ramaswamy, MD, while previously employed by RSI, was also granted RSL common shares. The Company recorded share-based compensation expense of $0 and $0.2 million for the three months ended December 31, 2018 and 2017, respectively, and $0.1 million and $0.4 million for the nine months ended December 31, 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 December 31, 2018, all compensation expense related to these RSL common share awards had been recognized.

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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 nine months ended December 31, 2018, the Company made cash expenditures of approximately $2.2 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 December 31, 2018
Employee severance and other personnel benefits	$ 2,460	$ —	$ (2,164)	$ —	$ 296

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 (0.2)% and 0.0% for the three months ended December 31, 2018 and 2017, respectively, and (0.2)% and (0.5)% for the nine months ended December 31, 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 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 corporate income tax rate from the previous top marginal rate of 35% to a flat rate of 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 allowed 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 became 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 the Company's consolidated financial statements since its 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. As a result of finalizing the Company's fiscal 2018 operating results, the issuance of new interpretative guidance, and other analyses performed, the Company finalized its accounting related to the impacts of the Tax Cuts and Jobs Act and recorded immaterial measurement period adjustments in the period ended December 31, 2018.

Note 11—Commitments and Contingencies

As of December 31, 2018, the Company had entered into commitments under an exclusive license agreement with UMMS, a license agreement with Oxford BioMedica, a license and collaboration agreement with Benitec, a development, marketing and supply agreement with Arena Pharmaceuticals GmbH, a loan agreement with Hercules, an amended services agreement with RSI and a separate service agreement with RSG (see Note 6(A)). In addition, the Company had 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 nine months ended December 31, 2018, there were no material changes to the Company's specified contractual obligations set forth in the contractual obligations table included in the Annual Report, other than to the lease 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 in August 2018. For the three and nine-months ended December 31, 2018, the Company incurred $0.4 million and $1.3 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 December 31, 2018 (in thousands):

	Total	2019	2020	2021
Rent obligations, net of prepayments	$3,129	$448	$1,791	$890

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

The following discussion and analysis of our financial condition, results of operations and cash flows should be read in conjunction with (1) the unaudited interim condensed consolidated financial statements and the related notes thereto included elsewhere in this Quarterly Report on Form 10-Q, and (2) the audited consolidated financial statements and notes thereto and management's discussion and analysis of financial condition and results of operations for the fiscal year ended March 31, 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 potential commercialization of AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD and AXO-AAV-OPMD;
- our relationships under our license agreements with the University of Massachusetts Medical School ("UMMS"), Oxford BioMedica (UK) Ltd. ("Oxford BioMedica"), and Benitec Biopharma Limited ("Benitec");
- the success of our interactions with the U.S. Food and Drug Administration ("FDA") and 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 or biologics 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.

We have based these forward-looking statements largely on our current expectations and projections about future events, including the responses we expect from 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 company focused on gene therapy for neurological and neuromuscular diseases. We are developing a pipeline of innovative product candidates for the treatment of these debilitating diseases, including GM1 gangliosidosis, GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease), Parkinson's disease, oculopharyngeal muscular dystrophy ("OPMD"), amyotrophic lateral sclerosis ("ALS"), frontotemporal dementia ("FTD") and other neurological and neuromuscular indications.

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 December 31, 2018, we had $84.9 million of cash and cash equivalents. 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 our majority shareholder, Roivant Sciences Ltd. ("RSL"), and in December 2018, we received $31.6 million of aggregate net proceeds, including $10.0 million from RSL, from the issuance and sale of our common shares in a public offering, after deducting underwriting discounts and commissions and offering expenses incurred. We recorded net losses of $34.3 million and $57.9 million for the three months ended December 31, 2018 and 2017, respectively, $120.0 million and $196.3 million for the nine months ended December 31, 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 ("ASG"), our wholly owned subsidiary, holds global commercial rights:

Gene Therapy Program	Clinical Indication	Development Stage
AXO-AAV-GM1	GM1 gangliosidosis	Clinical-ready
AXO-AAV-GM2	GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease)	Clinical
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

AXO-AAV Programs

AXO-AAV-GM1 Program

AXO-AAV-GM1 is an investigational gene therapy currently being developed as a potential one-time disease modifying treatment for GM1 gangliosidosis. The program utilizes an adeno-associated virus ("AAV") vector to deliver a functional copy of the galactosidase beta 1 ("GLB1") gene with the goals of restoring -galactosidase (" -gal") enzyme activity in the central nervous system ("CNS") and reducing GM1 ganglioside accumulation, to ultimately

improve neurological function and extend survival. The therapy is administered intravenously and utilizes the AAV9 capsid, which has been shown to cross the blood-brain barrier. Intravenous administration has the potential to broadly transduce the CNS and peripheral tissues, as well as treat peripheral manifestations of the disease. We licensed exclusive worldwide rights for the development and commercialization of AXO-AAV-GM1 from UMMS in December 2018.

Preclinical studies in GM1 murine and feline models have supported AXO-AAV-GM1's ability to improve -gal enzyme activity, reduce GM1 ganglioside accumulation, improve neuromuscular function, and extend survival. Magnetic resonance imaging of GM1 feline models treated with other GM1 gene therapy demonstrated substantially normal brain architecture through at least two years of age, as compared with untreated GM1 feline models.
AXO-AAV-GM1 will be evaluated in an investigator-initiated clinical program, with the first patient expected to be dosed in the first half of 2019. We expect initial data from this clinical program in the second half of 2019 and expect continued enrollment of patients in this clinical program throughout 2019.
AXO-AAV-GM2 Program
AXO-AAV-GM2 is an investigational gene therapy currently being developed as a potential one-time disease modifying treatment for GM2 gangliosidosis, including Tay-Sachs disease and Sandhoff disease. The AXO-AAV-GM2 program utilizes AAV vectors to deliver functional copies of both the hexosaminidase subunit alpha ("HEXA") gene and the hexosaminidase subunit beta ("HEXB") gene, with the goal of restoring normal beta-hexosaminidase A ("Hex A") enzyme function in the CNS. AXO-AAV-GM2 is administered directly to the brain and utilizes the neurotropic AAVrh.8 capsid. The HEXA and HEXB genes will be delivered in a 1:1 ratio using separate AAvrh.8 vectors. As part of the AXO AAV-GM2 program, we are also exploring a next-generation gene therapy that would utilize a bicistronic vector to deliver both the HEXA and HEXB genes in a single vector using the AAV9 capsid for systemic intravenous administration. We licensed exclusive worldwide rights for the development and commercialization of AXO-AAV-GM2 from UMMS in December 2018.
Administration of AXO-AAV-GM2 in the Sandhoff mouse model showed increases in Hex A enzyme, reductions of GM2 ganglioside in the brain, and improvements in motor coordination. Extension of survival was also observed in the Sandhoff mouse model, with increases in survival in a dose-dependent manner.
AXO-AAV-GM2 is currently being evaluated with the first patient having been dosed in November 2018 under an investigator-initiated protocol approved by the FDA and overseen by UMMS. We expect to obtain initial data from this patient in the first quarter of 2019 and expect to file a subsequent Investigational New Drug application ("IND") to allow for patients to be enrolled in a multi-subject clinical trial in 2019.
GM1 Gangliosidosis, Tay-Sachs and Sandhoff Diseases
GM1 gangliosidosis is a rare, inherited neurodegenerative lysosomal storage disorder characterized by the accumulation of GM1 ganglioside. This accumulation occurs due to a defect in the GLB1 gene. The GLB1 gene codes for the -gal enzyme which catalyzes the hydrolysis of GM1 gangliosides. Impaired -gal activity results in the toxic accumulation of GM1 gangliosides, causing the progressive destruction of nerve cells in the brain and spinal cord and early death. GM1 gangliosidosis is uniformly fatal, and there are no disease-modifying treatment options. The estimated incidence for GM1 gangliosidosis is approximately one in 100,000 live births worldwide.
Tay-Sachs and Sandhoff diseases are a set of rare, inherited neurodegenerative lysosomal storage disorders characterized by buildup of GM2 ganglioside in lysosomes. Defects in the HEXA gene (leading to Tay-Sachs disease) and HEXB gene (leading to Sandhoff disease) cause deficiencies in Hex A enzyme activity. Hex A enzyme deficiency leads to progressive accumulation of GM2 gangliosides in the CNS with ensuing neurodegeneration. Both Tay-Sachs disease and Sandhoff disease are characterized by progressive nervous system dysfunction, resulting in marked cognitive and physical impairment. Tay-Sachs and Sandhoff diseases result in approximately 50% mortality by three and a half years of age and 75% mortality by five years of age. Currently there are no disease-modifying treatment options for Tay-Sachs disease or Sandhoff disease and management is limited to symptomatic treatment. The estimated incidence for Tay-Sachs and Sandhoff diseases is approximately one in 180,000 live births.
We estimate that there are between approximately 600 and 800 patients with GM1 gangliosidosis, Tay-Sachs and Sandhoff diseases in the United States and European Union combined. These diseases, in the severe form, reduce life expectancy to two to four years. The estimated incidence for the combination of GM1 gangliosidosis, Tay-Sachs and Sandhoff diseases is approximately one in 65,000 live births worldwide.
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 as a potential one-time treatment of Parkinson's disease. We licensed the worldwide development and commercialization rights to AXO-LENTI-PD and its first-generation product candidate ProSavin® from 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 ("TH"), the enzyme that converts tyrosine to levodopa ("L-dopa"), Cyclohydrolase 1 ("CH1"), the rate-limiting enzyme for synthesis of Tetrahydrobiopterin ("BH4"), a critical cofactor for production of L-dopa, and Aromatic L-Amino Acid Decarboxylase ("AADC"), the enzyme that converts L-dopa to dopamine. AXO-LENTI-PD is delivered by a one-time magnetic resonance imaging-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 the ability to synthesize dopamine 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 for over six years, 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 responsible for motor control. 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 available treatments for Parkinson's disease are 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 with fluctuating 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 used 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.

First-Generation Product Candidate: ProSavin (OXB-101)

ProSavin, the first-generation 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 less optimized payload configuration. AXO-LENTI-PD was the result of multifactorial experimentation to optimize 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

In nonclinical studies in non-human primate models of Parkinson's disease, ProSavin was shown to be well-tolerated, restored striatal dopamine production to approximately 50% of normal levels and corrected 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. Also, in non-human primate models, treatment with ProSavin plus oral levodopa significantly reduced dyskinesias (p<0.05) compared to an empty vector plus oral levodopa, with effects sustained out to eight weeks. 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 ("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, or 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) compared to baseline. In a long term follow up safety study being performed by Oxford BioMedica for the patients from the phase 1/2 study, ProSavin has been observed to show a favorable long-term safety profile and demonstrated effects on motor function for over six years. 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 optimize the payload configuration of ProSavin to improve endogenous 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 showed 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 $1/5^{th}$ 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 may have

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

The Phase 2 clinical trial of AXO-LENTI-PD, referred to as the SUNRISE-PD study, was initiated in the U.K. in the fourth quarter of 2018. We expect to announce data from the first two patients in March 2019. The SUNRISE-PD study is currently enrolling patients in the United Kingdom, and we plan to file an IND with the FDA in mid-2019 to support enrollment of patients in the United States.

The design of the SUNRISE-PD study consists of two parts, including a single arm dose-escalation portion studying multiple potential dose levels and a sham-controlled portion with patients randomized either to an active group receiving the optimal dose as determined in the first portion, or a control group receiving an imitation "sham" surgical procedure.

The SUNRISE-PD study is evaluating the safety and tolerability of AXO-LENTI-PD as well as assessing efficacy using clinical measures of motor function, patient diaries and biomarkers. The primary endpoint of the double-blind, randomized, sham-controlled portion of the SUNRISE-PD study will be assessed at 12 months using data from Hauser patient diaries, and a key secondary endpoint will include UPDRS Part III "OFF" scores at 12 months.

Silence-and-Replace Technology Platform

The Silence-and-Replace technology platform is designed to produce a long-term restoration of normal gene function by combining RNA interference ("RNAi") (silence) with gene transfer (replace) in a single administration of a single 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 DNA-directed RNA interference ("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 to overcome challenges with administration, transduction and simultaneous expression that may be more likely when the silence and replace genes are contained in separate vectors.

AXO-AAV-OPMD Program

Overview

The AXO-AAV-OPMD program is an investigational gene therapy being developed as a one-time potentially disease-modifying treatment for OPMD, which we licensed from 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 gene, 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 involved in swallowing function to provide long-term correction of muscle pathology and restoration of function.

Oculopharyngeal Muscular Dystrophy Overview

OPMD is a neuromuscular 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 (dysphagia), eyelid drooping (ptosis), and weakness of the proximal extremities. Swallowing difficulties can have life-threating 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

The FDA and European Commission have granted orphan designation to the AXO-AAV-OPMD program for the treatment of OPMD. The AXO-AAV-OPMD clinical program is expected to begin in the second half of 2019, and the final design will be informed by discussions with the FDA and other regulators.

Additional Silence-and-Replace Collaboration Programs

Under our license and collaboration agreement with Benitec (the "Benitec Agreement"), we are able to 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 currently plan to initiate a research plan to develop gene therapy products targeting the C9orf72 gene, which is associated with ALS and 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.

Nelotanserin

In October 2015, we acquired from our majority shareholder, RSL, the global rights to nelotanserin, a selective inverse agonist of the 5-HT_{2A} receptor. We have been investigating and developing nelotanserin to address visual hallucinations and REM sleep behavior disorder ("RBD") in patients with Lewy body dementia ("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. We announced topline data from our Phase 2 clinical study of nelotanserin for the treatment of RBD in patients with LBD in December 2018. These data showed that the primary efficacy endpoint of reduction in frequency of RBD events as measured by sleep laboratory video assessment was not met. Signals of efficacy were observed on secondary measures, including trends in prespecified secondary analyses of study diaries and certain sleep parameters on polysomnography. These findings were generally consistent with previous clinical studies of nelotanserin in patients with insomnia. Nelotanserin was generally well-tolerated in the study.

We do not plan to conduct further clinical studies of nelotanserin.

Our Key Agreements

Oxford BioMedica License Agreement

On June 5, 2018, we, through our wholly owned subsidiary, 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 was applied as a credit against the process development work and clinical supply that Oxford BioMedica is obligated to provide to us over the term of the Oxford BioMedica Agreement. 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.

The University of Massachusetts Medical School Exclusive License Agreement

In December 2018, we, through our wholly owned subsidiary, ASG, entered into an exclusive license agreement (the "UMMS Agreement") with UMMS pursuant to which we received a worldwide, royalty-bearing, sub-licensable license under certain patent applications and any patents issuing therefrom, biological materials and know-how controlled by UMMS to develop and commercialize gene therapy product candidates, including AXO-AAV-GM1 and AXO-AAV-GM2, for the treatment of GM1 gangliosidosis and GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease). This license is exclusive with respect to patents and biological materials and non-exclusive with respect to know-how and is subject to UMMS' retained rights for academic research, teaching and non-commercial patient care purposes, as well as to certain pre-existing rights of the U.S. government.

Under the UMMS Agreement, we are solely responsible, at our expense, for the research, development and commercialization of the licensed product candidates. We will reimburse UMMS for payments made by UMMS for the manufacture of clinical trial materials for us, up to a specified amount. We are obligated to use diligent efforts to develop and commercialize the licensed product candidates and are required to achieve certain development and commercial milestones in accordance with the timeline set forth in the agreement.

Under the terms of the UMMS Agreement, we made an upfront payment of $10.0 million. In addition, we could be obligated to make payments to UMMS totaling up to $24.5 million upon the achievement of specified development and regulatory milestones and $39.8 million upon the achievement of specified commercial milestones. We are also obligated to pay UMMS tiered mid-single digit royalties based on yearly net sales of the licensed products, subject to a specified annual minimum amount. Additionally, we will pay UMMS a percent of any revenues we receive from any third-party sublicenses to licensed products at rates ranging in the mid-single digits to mid-teens.

The UMMS Agreement will expire upon the expiration of our obligations to make royalty payments to UMMS, which continues until the later of the expiration of licensed patents and any applicable orphan designation exclusivity and 10 years after the first commercial sale of the licensed products. Upon such expiration, the licenses granted to us by UMMS will automatically convert to perpetual, irrevocable, worldwide royalty-free licenses. We have the right to terminate the UMMS Agreement at any time upon 90 days' advance written notice to UMMS. Either party may

terminate the UMMS Agreement for the other party's uncured material breach upon 60 days' advance written notice, including in the event that UMMS reasonably determines we have not fulfilled our diligence obligations.

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 could 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.

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.

Under the services agreement in effect as of December 31, 2016, we incurred expenses of $0.7 million and $1.4 million for the three months ended December 31, 2018 and 2017, respectively, and $5.0 million and $5.8 million for the nine months ended December 31, 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.

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 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 ("CROs") 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 UMMS Agreement, the Benitec Agreement, and our development, marketing and supply agreement with Arena Pharmaceuticals, GmbH for nelotanserin.

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 our research and development expense to increase as we advance the AXO-AAV-GM1 program, the AXO-AAV-GM2 program, 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. 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 dose 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 headcount reductions over the past year, as we have changed our focus from small molecules to gene therapies.

Results of Operations for the Three and Nine-Months Ended December 31, 2018 and 2017

The following table summarizes our results of operations for the three and nine-months ended December 31, 2018 and 2017 (in thousands):

	Three Months Ended December 31,			Nine Months Ended December 31,		
	2018	2017	Change	2018	2017	Change
Operating expenses:						
Research and development expenses (includes total share-based compensation expense of $1,910 and $2,453 for the three months ended December 31, 2018 and 2017 and $3,299 and $14,625 for the nine months ended December 31, 2018 and 2017, respectively)	$21,483	$37,346	$(15,863)	$80,403	$119,613	$(39,210)
General and administrative expenses (includes total share-based compensation expense of $2,648 and $8,186 for the three months ended December 31, 2018 and 2017 and $9,575 and $26,954 for the nine months ended December 31, 2018 and 2017, respectively)	10,933	18,032	(7,099)	33,309	69,662	(36,353)
Total operating expenses	$32,416	$55,378	$(22,962)	$113,712	$189,275	$(75,563)
Interest expense	$1,906	$1,950	$(44)	$5,808	$5,702	$106
Income tax expense	$52	$24	$28	$224	$953	$(729)

Research and Development Expenses

Our research and development expenses during the three and nine-months ended December 31, 2018 and 2017 consisted of the following (in thousands):

	Three Months Ended December 31,			Nine Months Ended December 31,		
	2018	2017	Change	2018	2017	Change
Program-specific costs:						
AXO-LENTI-PD	$1,881	$—	$1,881	$28,211	$—	$28,211
AXO-AAV-GM1 and AXO-AAV-GM2	10,024	—	10,024	10,024	—	10,024
AXO-AAV-OPMD	1,858	—	1,858	13,605	—	13,605
Intepirdine	(207)	23,064	(23,271)	2,054	70,794	(68,740)
Nelotanserin	1,334	5,842	(4,508)	8,894	12,635	(3,741)
RVT-103	—	79	(79)	2	691	(689)
RVT-104	—	582	(582)	(73)	1,216	(1,289)
Unallocated internal costs:						
Share-based compensation	1,910	2,453	(543)	3,299	14,625	(11,326)
Personnel-related	3,095	4,035	(940)	7,512	12,631	(5,119)
Services agreements	—	186	(186)	2,352	1,573	779
Other	1,588	1,105	483	4,523	5,448	(925)
Total research and development expenses	$21,483	$37,346	$(15,863)	$80,403	$119,613	$(39,210)

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Research and development expenses were $21.5 million for the three months ended December 31, 2018, and consisted primarily of $10.0 million related to the license fee paid to UMMS in December 2018 for AXO-AAV-GM1 and AXO-AAV-GM2, employee salaries and benefits of $3.1 million, $1.9 million related to AXO-AAV-OPMD, $1.9 million related to AXO-LENTI-PD, share-based compensation expense of $1.9 million and $1.3 million related to nelotanserin clinical studies and related manufacturing.

Research and development expenses were $37.3 million for the three months ended December 31, 2017 and consisted primarily of program-specific costs of $29.6 million, including $23.1 million related to intepirdine clinical studies and related wind-down activities and $5.8 million related to nelotanserin, personnel-related expenses of $4.0 million and share-based compensation expense of $2.5 million. The share-based compensation expense included $0.2 million related to the RSL common share awards and RSL options issued by RSL to RSI employees, net of forfeitures.

Research and development expenses decreased by $15.9 million, to $21.5 million, in the three months ended December 31, 2018, compared to the three months ended December 31, 2017, as intepirdine costs decreased by $23.3 million due to the discontinuation of our intepirdine program, nelotanserin costs decreased by $4.5 million due to the wind-down of our nelotanserin clinical studies, personnel-related expenses decreased by $0.9 million primarily due to decreased headcount related to our previously announced reduction in workforce and share-based compensation expense decreased by $0.5 million, net of forfeitures, primarily due to decreased headcount related to our previously announced reduction in workforce, as well as an increase in forfeitures related to RSL options issued to RSI employees, offset by increases of $10.0 million related to the license fee paid to UMMS in December 2018 for AXO-AAV-GM1 and AXO-AAV-GM2, $1.9 million related to AXO-LENTI-PD and $1.9 million related to AXO-AAV-OPMD.

Research and development expenses were $80.4 million for the nine months ended December 31, 2018, and consisted primarily of $28.2 million related to AXO-LENTI-PD, $13.6 million related to AXO-AAV-OPMD, $10.0 million related to to the license fee paid to UMMS in December 2018 for AXO-AAV-GM1 and AXO-AAV-GM2, $8.9 million related to the nelotanserin clinical study, employee salaries and benefits of $7.5 million and share-based compensation expense of $3.3 million. The share-based compensation expense was net of a benefit of $(2.8) million related to the RSL common share awards and RSL options issued by RSL to RSI employees, net of forfeitures.

Research and development expenses were $119.6 million for the nine months ended December 31, 2017 and consisted primarily of program-specific costs of $85.3 million, including $70.8 million related to intepirdine and $12.6 million related to nelotanserin, share-based compensation expense of $14.6 million, and employee salaries and benefits of $12.6 million. The share-based compensation expense included $4.1 million related to the RSL common share awards and RSL options issued by RSL to RSI employees, net of forfeitures.

Research and development expenses decreased by $39.2 million in the nine months ended December 31, 2018, compared to the nine months ended December 31, 2017, as intepirdine costs decreased by $68.7 million due to the discontinuation of our intepirdine program, share-based compensation expenses decreased by $11.3 million, net of forfeitures, personnel-related expenses decreased by $5.1 million primarily due to reduced headcount and nelotanserin costs decreased by $3.7 million due to the wind-down of our nelotanserin clinical studies, offset by increases of $28.2 million related to AXO-LENTI-PD, which includes the $25.0 million license fee paid to Oxford BioMedica in June 2018, $13.6 million related to AXO-AAV-OPMD, which includes the $10.0 million license fee paid to Benitec in July 2018, and $10.0 million related to the license fee paid to UMMS in December 2018 for AXO-AAV-GM1 and AXO-AAV-GM2.

General and Administrative Expenses

General and administrative expenses were $10.9 million for the three months ended December 31, 2018 and consisted primarily of employee salaries and related benefits of $3.1 million, legal and professional fees of $2.7 million, share-based compensation expense of $2.6 million and $0.7 million of direct and indirect costs allocated to us under the services agreements with RSI and RSG.

General and administrative expenses were $18.0 million for the three months ended December 31, 2017 and consisted primarily of share-based compensation expense of $8.2 million, employee salaries and related benefits of $4.2 million, $1.2 million of direct and indirect costs allocated to us under the services agreement with RSI and RSG, and legal and professional fees of $1.0 million. The share-based compensation expense for the three months ended December 31,

2017 included $0.2 million for RSL common share awards and RSL options issued to RSI employees.
General and administrative expenses decreased by $7.1 million, to $10.9 million, in the three months ended December 31, 2018, compared to the three months ended December 31, 2017, primarily due to decreases in share-based compensation expense of $5.6 million and employee salaries and related benefits of $1.1 million due to decreased headcount, offset by an increase in professional fees of $1.7 million. The remaining decrease of $2.1 million was primarily due to decreases in direct and indirect costs allocated to us under the services agreements with RSI and RSG, depreciation expenses and marketing expenses.

General and administrative expenses were $33.3 million for the nine months ended December 31, 2018 and consisted primarily of share-based compensation expense of $9.6 million, legal and professional fees of $7.2 million, employee salaries and related benefits of $7.2 million and $2.6 million of direct and indirect costs allocated to us under the services agreements with RSI and RSG. The remaining expense of $6.7 million was related primarily to facilities, insurance, depreciation and consulting expenses. The share-based compensation expense for the nine months ended December 31, 2018 included share-based compensation expense of $0.1 million for RSL common share awards and RSL options issued to RSI employees.

General and administrative expenses were $69.7 million for the nine months ended December 31, 2017 and consisted primarily of share-based compensation of $27.0 million, employee salaries and related benefits of $15.2 million, marketing expenses of $8.9 million, legal and professional fees of $5.0 million, and $4.2 million of direct and indirect costs allocated to us under the services agreements with RSI and RSG. The share-based compensation expense for the nine months ended December 31, 2017 included $0.7 million for RSL common share awards and RSL options issued to RSI employees.

General and administrative expenses decreased by $36.4 million, to $33.3 million, in the nine months ended December 31, 2018, compared to the nine months ended December 31, 2017, primarily due to decreases in share-based compensation of $17.4 million, employee salaries and related benefits of $8.0 million due to reduced headcount and marketing expenses of $8.0 million due to the discontinuation of our inteperdine program. The remaining decrease of $3.0 million was primarily due to decreases in direct and indirect costs allocated to us under the services agreements with RSI and RSG, and expenses related to meetings and conferences and travel and entertainment.

Interest Expense

Interest expense was $1.9 million and $5.8 million, respectively for the three and nine-months ended December 31, 2018 and consisted of interest paid and the amortization of debt discount related to the Loan Agreement with Hercules Capital, Inc. (“Hercules”). See “Liquidity and Capital Resources-Loan and Security Agreement with Hercules Capital, Inc.”

Interest expense for the three and nine-months ended December 31, 2017, was $2.0 million and $5.7 million, respectively, and consisted of interest paid and the amortization of debt discount related to the Loan Agreement with Hercules.

Income Tax Expense

Income tax expense was $0.1 million and $0.2 million for the three and nine-months ended December 31, 2018, respectively. Income tax expense was $24 thousand and $1.0 million for the three and nine-months ended December 31, 2017, respectively. The higher income tax expense for the nine months ended December 31, 2017 was due to a valuation allowance recorded to offset our net deferred tax assets, which did not recur during the three and nine-months ended December 31, 2018.

Liquidity and Capital Resources

Overview

In April 2017, we raised net proceeds of approximately $134.5 million, after deducting underwriting discounts and commissions and offering expenses paid by us, from the sale of 7,753,505 common shares in a follow-on public offering.

On June 22, 2018, we entered into a sales agreement with Cowen and Company, LLC ("Cowen") to sell our 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 our agent. Cowen is entitled to compensation for its services in an amount up to 3% of the gross proceeds of any of our common shares sold under the sales agreement. As of December 31, 2018, approximately $74.9 million of our common shares remained available for sale under the sales agreement.

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

On December 18, 2018, we issued and sold 33,160,923 common shares in a follow-on public offering, including 3,160,923 common shares sold pursuant to the exercise of the underwriters' option to purchase additional shares, at an offering price of $1.00 per common share for gross proceeds of $33.2 million, including 10,000,000 shares issued and sold to RSL. The aggregate net proceeds to us were approximately $31.6 million, after deducting underwriting discounts and commissions and offering expenses incurred.

For the nine months ended December 31, 2018, we used $121.5 million of cash in our operating activities. We have incurred and expect to continue to incur significant and increasing operating losses at least for the next several years. We do not expect to generate revenue unless and until after we successfully complete development and obtain regulatory approval for one of our products in development. Our cash utilization may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our planned clinical trials and our expenditures on other research and development activities and activities related to potential commercialization. We anticipate that we will continue to incur significant expenses as we:

- continue development of AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD, and AXO-AAV-OPMD;
- initiate our research program for five targets with Benitec, including our research program on C9orf72;
- seek to identify, acquire, develop and commercialize additional product candidates;
- integrate acquired technologies into a comprehensive regulatory and product development strategy;
- achieve milestones under our agreements with third parties that will require us to make substantial payments to those parties;
- maintain, expand and protect our intellectual property portfolio;
- hire and retain scientific, clinical, regulatory, manufacturing, quality control, commercial and administrative personnel;
- add operational, financial and management information systems and personnel, including personnel to support our drug development efforts;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- scale up external manufacturing capabilities to commercialize our product candidates;
- establish a sales, marketing and distribution infrastructure for product candidates for which we may obtain regulatory approval; and
- operate as a public company.

As of December 31, 2018, we had cash and cash equivalents totaling $84.9 million. For the fiscal year ended March 31, 2018 and the three and nine-months ended December 31, 2018, we incurred net losses of $221.6 million, $34.3 million and $120.0 million, respectively. At December 31, 2018, we had aggregate net interest-bearing indebtedness of $48.8 million, of which $20.6 million was due within one year. We also had $24.6 million of other non-interest-bearing current liabilities due within one year. The Loan Agreement with Hercules requires that we maintain a minimum cash balance, which was previously $35.0 million, but has been reduced to $30.0 million following the achievement of certain clinical milestones as set forth in the Loan Agreement. We anticipate that our current cash and cash equivalent balances will not be sufficient to maintain compliance with the minimum liquidity financial covenant under the Loan Agreement beyond the one-year period following the date that the accompanying unaudited condensed consolidated financial statements were issued if the Loan Agreement is not amended or additional financing is not completed. Failure to meet this minimum covenant would be considered an event of default under the Loan Agreement and could result in the acceleration of our existing indebtedness.

We are currently in the clinical stage of operations, have not yet achieved profitability, and anticipate that we will continue to incur net losses for the foreseeable future. Our existing funds will not be sufficient to enable us to complete all necessary development and to commercially launch any of our products. Accordingly, we will be required to obtain further funding through public or private equity offerings, debt financing, collaboration and licensing arrangements or other sources. Adequate additional funding may not be available to us on acceptable terms, or at all. If we are unable to raise capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of one or more of our product candidates or potentially discontinue operations. Due to these uncertainties, there is substantial doubt about our ability to continue as a going concern.

Loan and Security Agreement with Hercules Capital, Inc.

On February 2, 2017, we and our wholly owned subsidiaries, Axovant Holdings Ltd. ("AHL"), ASG and ASI, entered into the Loan Agreement with Hercules. Pursuant to the Loan Agreement, we, AHL and ASG, as the Borrowers, borrowed an aggregate of $55.0 million. ASI issued a guaranty of the Borrowers' obligations under the Loan

Agreement, and at the closing, we paid Hercules a facility charge of $550,000. Subsequently, we added our subsidiary Axovant Sciences America, Inc. as a Borrower in July 2017 and our subsidiaries Axovant Treasury Holdings, Inc. and Axovant Treasury, Inc. 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 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 through March 1, 2021. The Borrowers' obligations under the Loan Agreement are secured by a first position lien on substantially all of their and ASI's respective assets, other than intellectual property. If we prepay the loan prior to March 1, 2021, we will be obligated to pay Hercules a prepayment charge, based on a percentage of the then-outstanding principal balance, equal to 2.0% if the prepayment occurs after 18 months but prior to 36 months following February 2, 2017, and 1.0% if the prepayment occurs thereafter.

The Loan Agreement includes customary affirmative and restrictive covenants and representations and warranties, including a minimum cash balance of the lesser of at least i) the outstanding amount of debt under the underlying loan agreement plus certain aged accounts payable (as further defined in the Loan Agreement), or ii) $35.0 million, provided that this amount may be lowered to $30.0 million upon the achievement of certain clinical milestones as set forth in the Loan Agreement, a covenant against the occurrence of a "change in control," financial reporting obligations, and certain limitations on indebtedness, liens (including a negative pledge on intellectual property and other assets), investments, distributions (including dividends), collateral, transfers, mergers or acquisitions, taxes, corporate changes, and deposit accounts. The Loan Agreement also includes customary events of default, including payment defaults, breaches of covenants following any applicable cure period, the occurrence of certain events that could reasonably be expected to have a "material adverse effect" as set forth in the Loan Agreement, cross acceleration to the debt and certain events relating to bankruptcy or insolvency. Upon the occurrence of an event of default, a default interest rate of an additional 5.0% may be applied to the outstanding principal balance, and Hercules may declare all outstanding obligations immediately due and payable and take such other actions as set forth in the Loan Agreement. In addition, for so long as the Term Loan remains outstanding, we are required to use commercially reasonable efforts to afford Hercules the opportunity to participate in future underwritten equity offerings of our common shares up to a total of $3.0 million.

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.

Cash Flows

The following table sets forth a summary of our cash flows for the nine months ended December 31, 2018 and 2017 (in thousands):

	Nine Months Ended December 31,	
	2018	2017
Net cash used in operating activities	$(121,531)	$(156,148)
Net cash used in investing activities	(74)	(4,246)
Net cash provided by financing activities	52,207	136,072

Operating Activities

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

For the nine months ended December 31, 2018, net cash used in operating activities was $121.5 million and was primarily attributable to a net loss of $120.0 million, which includes costs incurred for research and development activities, including CRO fees, manufacturing, regulatory and other clinical trial costs and our general and administrative expenses, a decrease of $9.0 million in accrued expenses, an increase of $3.4 million in other non-current assets, a decrease of $2.9 million in accounts payable and an increase of $2.7 million in prepaid assets and other current assets, which were partially offset by $12.9 million of non-cash share-based compensation expense, and $2.1 million of depreciation and amortization and an increase of $0.8 million in amounts due to RSL, RSI and RSG, which includes $1.1 million from a non-cash capital contribution. For the nine months ended December 31, 2017, net cash used in operating activities was $156.1 million and was primarily attributable to a net loss of $196.3 million, which includes costs incurred for research and development activities, including CRO fees, manufacturing, regulatory and other clinical trial costs, and our general and administrative expenses, and a decrease of $7.5 million in accounts payable, partially offset by $41.6 million of non-cash share-based compensation expense.

Investing Activities

For the nine months ended December 31, 2018, net cash used in investing activities was $74 thousand for purchases of computers and software. For the nine months ended December 31, 2017, net cash used in investing activities was $4.2 million, consisting of purchases of leasehold improvements, furniture and equipment.

Financing Activities

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For the nine months ended December 31, 2018, net cash provided by financing activities was approximately $52.2 million and consisted of $56.7 million of net proceeds from the issuance and sale of our common shares in a public offering, a private placement to RSL, and our share sales agreement with Cowen, as well as proceeds of $0.3 million from the exercise of stock options, partially offset by $4.8 million of payments made on long-term debt. For the nine months ended December 31, 2017, net cash provided by financing activities was $136.1 million, which consisted of net proceeds of $134.5 million received from the sale of our common shares in a follow-on public offering and proceeds of $1.6 million from the exercise of stock options.

Contractual Obligations

Our contractual obligations did not materially change during the nine months ended December 31, 2018, as compared to those disclosed in our Annual Report on Form 10-K for the year ended March 31, 2018, except that, in June 2018, we entered into the Oxford BioMedica Agreement; in July 2018, we entered into the Benitec Agreement; in August 2018, we extended our lease 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; and in December 2018, we entered into the UMMS Agreement. The following table provides information regarding remaining contractual rent obligations due within each respective year ending March 31, as of December 31, 2018 (in thousands):

	Total	2019	2020	2021
Rent obligations, net of prepayments	$3,129	$448	$1,791	$890

See “Our Key Agreements” above for additional information regarding the the UMMS Agreement, the Oxford BioMedica Agreement, and the Benitec Agreement, and our commitments thereunder.

Off-Balance Sheet Arrangements

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

Critical Accounting Policies and Significant Judgments and Estimates

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

We define our critical accounting policies as those under U.S. GAAP that require us to make subjective estimates and judgments about matters that are uncertain and are likely to have a material impact on our financial condition and results of operations, as well as the specific manner in which we apply those principles.

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

Our significant accounting policies are more fully described in Note 2 to our unaudited condensed consolidated financial statements in this Quarterly Report on Form 10-Q and Note 2 to our consolidated financial statements in our Annual Report. There have been no material changes to our critical accounting policies and significant judgments and estimates as compared to the critical accounting policies and significant judgments and estimates described in our Annual Report, except as follows:

Cash and Cash Equivalents

We consider all highly liquid investments purchased with original maturities of 90 days or less at acquisition to be cash equivalents. Cash and cash equivalents include cash held in banks and amounts held in money market funds.
Recent Accounting Pronouncements

The Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2016-02, "Leases (Topic 842)" in February 2016, ASU No. 2018-10, "Codification Improvements to Topic 842, Leases" and ASU No. 2018-11, "Leases (Topic 842): Targeted Improvements" in July 2018, and ASU No. 2018-20, "Narrow-Scope Improvements for Lessors" in December 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. We expect to adopt the provisions of the Lease Standards for the fiscal year beginning April 1, 2019. We have implemented a process to identify our outstanding lease portfolio and are currently evaluating our outstanding leases to determine the impact the Lease Standards will have on our 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. We adopted the provisions of ASU No. 2017-01 on April 1, 2018, on a prospective basis. The impact on our consolidated financial statements and disclosures will depend on the facts and circumstances of any specific future transactions. Refer to Note 3, “License and Collaboration Agreements,” in the accompanying notes to the unaudited condensed consolidated financial statements for further information regarding the impact of the adoption of ASU No. 2017-01 on the license agreements executed during the three and nine-months ended December 31, 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"). On December 22, 2017, 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") was enacted in the United States, which introduced a comprehensive set of tax reforms. 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. We expect to adopt the provisions of ASU No. 2018-02 for the fiscal year beginning April 1, 2019. As we have not yet completed our final review of the impact of ASU No. 2018-02 but expect to by March 31, 2019, we have not determined whether the adoption of this guidance will have a material impact on our 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 to provide guidance for companies that would allow for a measurement period of up to one year after the enactment date of the Tax Cuts and Jobs Act, and was effective immediately. The Tax Cuts and Jobs Act did not have a material impact on our consolidated financial statements since our deferred temporary differences are fully offset by a valuation allowance and we do not have any offshore earnings from which to record the mandatory transition tax. As a result of finalizing our fiscal 2018 operating results, the issuance of new interpretative guidance, and other analyses performed, we finalized our accounting related to the impacts of the Tax Cuts and Jobs Act and recorded immaterial measurement period adjustments in the period ended December 31, 2018.

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 Accounting Standards Codification 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. We expect to adopt the provisions of ASU No. 2018-07 for the fiscal year beginning April 1, 2019. As we have not yet completed our final review of the impact of ASU No. 2018-07 but expect to by March 31, 2019, we have not determined whether the adoption of this guidance will have a material impact on our 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. We 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 our consolidated financial statements or disclosures because we do not currently have any Level 3 fair value measurements on a recurring or nonrecurring basis, and also have not had transfers between Level 1 and Level 2 of the fair value hierarchy.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Market risk is the potential loss arising from adverse changes in market rates and market prices such as interest rates, foreign currency exchange rates, and changes in the market value of equity instruments. As of December 31, 2018, we had cash and cash equivalents of $84.9 million, with cash consisting of non-interest-bearing deposits denominated in the U.S. dollar and Swiss franc, and cash equivalents consisting of interest-bearing money market fund deposits denominated in the U.S. dollar, which are invested in debt securities issued or guaranteed by the U.S. government and repurchase agreements fully collateralized by U.S. Treasury and U.S. government securities. We have policies requiring us to invest in high-quality issuers, limit our exposure to any individual issuer, and ensure adequate liquidity. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our cash equivalent investments are in the form of money market funds and marketable securities and are invested in U.S. Treasury obligations. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio. We also have debt that bears interest at a prime-based variable rate. A 10% change in this interest rate would have an impact of approximately $0.5 million on our annual interest expense. We do not believe we are currently exposed to any material market risk.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Under the supervision of our principal executive officer and principal financial officer, we evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2018, the end of the period covered by this Quarterly Report on Form 10-Q. The term "disclosure controls and procedures" (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act of 1934, as amended (the "Exchange Act"), means controls and other procedures of a company that are designed to provide reasonable assurance that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms.

Disclosure controls and procedures include, without limitation, controls and procedures designed to provide reasonable assurance that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow for timely decisions regarding required disclosure. Based on this evaluation, our principal executive officer and principal financial officer concluded that our disclosure controls and

procedures were effective as of December 31, 2018, at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

There was no change in our internal control over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the period covered by this Quarterly Report on Form 10-Q that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Inherent Limitations on Effectiveness of Controls

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

PART II: OTHER INFORMATION

Item 1. Legal Proceedings

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

Item 1A. Risk Factors

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

Risks Related to Our Business, Financial Position and Capital Requirements

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

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

In addition, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. The failure of our Phase 3 MINDSET trial, Phase 2b HEADWAY trial and Phase 2 Gait and Balance trial for intepirdine, as well as our Phase 2 clinical study of nelotanserin, has required us to reevaluate our business and led to dramatic shifts in our strategy and business plan. Our new strategy and business plan have not yet been proven and we may never be successful in developing or commercializing any of our gene therapy product candidates, including our newly licensed product candidates AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD and AXO-AAV-OPMD, which remain in early stages of clinical or preclinical development. If successful in developing and obtaining marketing approval of one of our product candidates, we would need to transition from a company with a product development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

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

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

- successfully commence and complete clinical trials and obtain regulatory approval for the marketing of our product candidates, including AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD and AXO-AAV-OPMD;
- initiate and continue relationships with third-party manufacturers and have clinical development and commercial quantities of our product candidates manufactured at acceptable cost and quality levels;
- set an acceptable price for our product candidates and obtain coverage and adequate reimbursement from third-party payers;
- establish effective sales, marketing and distribution systems for our product candidates;
- add operational, financial and management information systems and personnel, including personnel to support our clinical, manufacturing and planned future commercialization efforts and operations as a public company;
- attract and retain an experienced management and advisory team;

achieve broad market acceptance of our products in the medical community and with third-party payers and consumers;

- launch commercial sales of our products, whether alone or in collaboration with others; and
- maintain, expand and protect our intellectual property portfolio.

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

We are in the process of implementing a business plan that may continue to evolve as we integrate our newly licensed product candidates AXO-LENTI-PD, AXO-AAV-GM1, AXOAAV-GM2, and AXO-AAV-OPMD. Our business plan may lead to the initiation of one or more development programs, the discontinuation of one or more development programs, or the execution of one or more transactions that you do not agree with or that you do not perceive as favorable to your investment.

In early 2018, we began a process to review our strategic alternatives, including identifying potential business development opportunities, following the discontinuation of further development of intepirdine in January 2018. Also beginning in early 2018, we undertook a reassessment of our development plans for nelotanserin in various indications and RVT-104, which included an internal portfolio review of RVT-104 in the context of any newly acquired clinical assets. In October 2018, we discontinued our development plans for RVT-104, and in December 2018, we announced that we do not plan to conduct further clinical studies of nelotanserin.

In June 2018, we announced that we received from Oxford BioMedica a worldwide exclusive license to develop and commercialize AXO-LENTI-PD and its predecessor product candidate ProSavin and related gene therapy products (the Oxford BioMedica Agreement). In July 2018, we announced that we received from Benitec a worldwide exclusive license to develop and commercialize investigational gene therapy AXO-AAV-OPMD and related gene therapy products (the Benitec Agreement). In December 2018, we announced that we had received from UMMS a worldwide exclusive license to develop and commercialize gene therapy product candidates AXO-AAV-GM1 and AXO-AAV-GM2 (the UMMS Agreement). We initially plan to pursue a strategy to leverage our clinical experience and expertise to pursue the clinical development and regulatory approval of AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD and AXO-AAV-OPMD while continuing to engage in business development efforts to identify, evaluate and potentially obtain rights to and develop additional therapeutic and gene therapy product candidates that would complement our strategic goals and leverage our competitive advantages.

This business plan requires us to be successful in a number of challenging, uncertain and risky activities, including pursuing development of AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD and AXO-AAV-OPMD in indications for which we have limited or no human clinical data, building internal or outsourced gene therapy capabilities, converting early stage gene therapy research efforts into clinical development opportunities, identifying additional promising new assets for development that are available for acquisition or in-license and that fit our strategic focus and, if so identified, negotiating and executing an acquisition or in-license agreement for one or more of those programs on favorable terms, and designing and executing a nonclinical and/or clinical development program for our newly acquired product candidates. We may not be successful at one or more of the activities required for us to execute this business plan. In addition, we are also continuing to consider other strategic alternatives, such as mergers, acquisitions, divestitures, joint ventures and collaborations. We cannot be sure when or if any type of transaction will result. Even if we pursue a transaction, such transaction may not be consistent with our shareholders' expectations or may not ultimately be favorable for our shareholders, either in the shorter or longer term.

Our growth prospects and the future value of our company are primarily dependent on the progress of our ongoing and planned clinical development programs for AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD and AXO-AAV-OPMD as well as the outcome of our ongoing business development efforts and pipeline expansion activities, together with the amount of our remaining available cash. The development of AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD and AXO-AAV-OPMD and the outcome of our ongoing business development efforts and pipeline expansion activities are highly uncertain.

We have only very limited data from small, uncontrolled clinical trials, performed by or on behalf of Oxford BioMedica, regarding the safety and tolerability of ProSavin, as the predecessor product candidate to AXO-LENTI-PD, in patients with advanced Parkinson's disease, as well as nonclinical in vitro experiments with AXO-LENTI-PD. Prior ProSavin trials were not powered to demonstrate the efficacy of the therapy with statistical significance. Given the information above, these trials could be underpowered to demonstrate a potential clinical benefit for AXO-LENTI-PD in these indications. In addition, we have no prior clinical data regarding the safety, tolerability and efficacy of AXO-AAV-GM1, AXO-AAV-GM2, AXO-AAV-OPMD or any additional gene therapy product under the Benitec Agreement.

We expect to continue to reassess and make changes to our existing development programs and pipeline expansion strategy. Our plans for our AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD and AXO-AAV-OPMD development programs may be affected by the results of competitors' clinical trials of product candidates addressing GM1 gangliosidosis, GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease), Parkinson's disease, and OPMD, respectively. Our plans for additional gene therapy products under the Benitec Agreement may also be affected by the results of competitors' clinical trials of product candidates addressing our target indications. Our plans for our business development efforts and pipeline expansion activities may also be affected by the results of competitors' ongoing research and development efforts. We may modify, expand or terminate some or all of our development programs, clinical trials or collaborative research programs at any time as a result of new competitive information or as the result of changes to our product pipeline or business development strategy.

We expect that our remaining cash balances will continue to decline as we pursue these development programs, our collaborative research programs and our business development activities until we receive additional funding, if any. As a result, the value of our shareholders' investment may decline.

We expect to incur significant losses for the foreseeable future and may never achieve or maintain profitability.

Investment in pharmaceutical and biological product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that a product candidate will fail to gain regulatory approval or become commercially viable. We have never generated any revenues, and we cannot estimate with precision the extent of our future losses. We do not currently have any products that are available for commercial sale and we may never generate revenue from selling products or achieve profitability. We expect to continue to incur substantial and increasing losses through the projected commercialization of our product candidates. Our product candidates have not been approved for marketing in the United States or any other jurisdiction, and we may never receive any such approvals. Since January 2018, we have discontinued further development of all of our small molecule product candidates, including intepirdine, nelotanserin and RVT-104. While we have recently in-licensed our new product candidates AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD and AXO-AAV-OPMD, these product candidates remain in early stages of clinical or preclinical development. As a result, we are uncertain when or if we will achieve profitability and, if so, whether we will be able to sustain it. Our ability to produce revenue and achieve profitability is dependent on our ability to complete the development of our product candidates, obtain necessary regulatory approvals, and have our product candidates manufactured and successfully marketed and commercialized. We cannot assure you that we will be profitable even if we successfully commercialize our product candidates. If we do successfully obtain regulatory approval to market our product candidates, our revenues will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval, the number of competitors in such markets, the accepted price for our product candidates and whether we own the commercial rights for that territory. If the indication approved by regulatory authorities is narrower than we expect, or the treatment population is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of our product candidates, even if approved. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Failure to become and remain profitable may adversely affect the market price of our common shares and our ability to raise capital and continue operations.

We expect our research and development expenses to be significant as we develop: AXO-AAV-GM1 for the potential treatment of GM1 gangliosidosis; AXO-AAV-GM2 for the potential treatment of GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease); AXO-LENTI-PD for the potential treatment of Parkinson's disease; AXO-AAV-OPMD for the potential treatment of OPMD; and our additional gene therapy products under the Benitec Agreement. In addition, if we obtain regulatory approval for any of our product candidates, we expect to incur increased sales and marketing expenses. As a result, we expect to continue to incur significant and increasing operating losses and negative cash flows for the foreseeable future. These losses have had and will continue to have an adverse effect on our financial position and working capital.

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

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

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

In addition, the process of identifying and acquiring product candidates is highly competitive, and our ability to compete successfully is impacted by the fact that many of the companies with which we compete for these candidates have significantly greater experience, development and commercialization capabilities, name recognition and financial and human resources than we do. Further, our business development efforts are led by our senior executive officers and other management team members and would be significantly impaired if we were to lose the services of any of these executives. The time and resources spent on business development activities may also distract management's attention from our other development and business activities. Even if we are successful in identifying and acquiring additional product candidates, we may choose to focus our efforts and resources on a potential product candidate that ultimately proves to be unsuccessful. If we are unable to identify and acquire suitable product candidates for clinical development, this would adversely impact our business strategy and our financial position and share price.

We are heavily dependent on the success of our gene therapy product candidates, which are still in early stages of clinical or preclinical development. If we are unable to successfully develop and commercialize any of our product candidates, our business will be harmed.

We currently have no products that are approved for commercial sale and may never be able to develop marketable products. We expect that a substantial portion of our efforts and expenditures over the next few years will be devoted to the development of AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD and AXO-AAV-OPMD, all of which are in the early stages of clinical development. Accordingly, our business currently depends heavily on the successful development, regulatory approval and commercialization of these product candidates. We cannot be certain that any of our product candidates will receive regulatory approval or be successfully commercialized even if we receive regulatory approval. The research, testing, manufacturing, labeling, approval, sale, marketing and distribution of our product candidates are and will remain subject to extensive regulation by the FDA, the EMA, the PMDA and other comparable regulatory authorities that each have differing regulations. We are not permitted to market our product candidates in the United States or in any foreign countries until they receive the requisite approvals from the FDA or comparable regulatory authorities in other countries. We have not submitted marketing applications to the FDA or foreign regulatory authorities and do not expect to be in a position to do so for the foreseeable future. Obtaining marketing approval is a lengthy, expensive and inherently uncertain process, and regulatory authorities may delay, limit or deny approval of our product candidates for many reasons, including:

- we may not be able to demonstrate that a product candidate is safe and effective as a treatment for our targeted indications to the satisfaction of the applicable regulatory authorities;
- our biologics license applications ("BLA"), new drug application ("NDA") or other key regulatory filings may be delayed or rejected due to issues, including those related to the FDA's Pharmaceutical Quality/CMC guidance, timing of results from supporting studies, database lock, and data conversion, cleaning, and transfer;
- the regulatory authorities may require additional nonclinical studies or registrational studies of the product candidate in Parkinson's disease or other indications, which would increase our costs and prolong our development;
- the results of our clinical trials may not meet the level of statistical or clinical significance required for marketing approval;
- the regulatory authorities may disagree with the number, design, size, conduct or implementation of our clinical trials;
- the CROs that we retain to conduct clinical trials may take actions outside of our control, or otherwise commit errors or breaches of protocols, that adversely impact our clinical trials;
- the regulatory authorities may not find the data from nonclinical studies and clinical trials sufficient to demonstrate that the clinical and other benefits of the product candidate outweigh its safety risks;
- the regulatory authorities may disagree with our interpretation of data from our nonclinical studies and clinical trials or may require that we conduct additional studies;
- the regulatory authorities may not accept data generated at our clinical trial sites;
- the regulatory authorities may require, as a condition of approval, limitations on approved labeling or distribution and use restrictions;
- the FDA may require development of a risk evaluation and mitigation strategy ("REMS") as a condition of approval;
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- the regulatory authorities may identify deficiencies in the manufacturing processes or facilities of our third-party manufacturers; or
- the regulatory authorities may change their approval policies or adopt new regulations.

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

In February 2017, we and our subsidiaries entered into a loan and security agreement, as amended in May and September 2017 (the "Loan Agreement"), with Hercules. The Loan Agreement is secured by substantially all of our property and that of our subsidiaries that are parties to the Loan Agreement, other than intellectual property.

The Loan Agreement subjects us and our subsidiaries to various affirmative and restrictive covenants, including a minimum cash balance of the lesser of at least (i) the outstanding amount of debt under the Loan Agreement plus certain aged accounts payable (as further defined in the Loan Agreement), or (ii) $35.0 million (which has been reduced to $30.0 million following the achievement of certain clinical milestones as set forth in the Loan Agreement), a covenant against the occurrence of a "change in control," financial reporting obligations, and certain limitations on the incurrence of indebtedness, liens (including a negative pledge on intellectual property and other assets), investments, distributions (including dividends), collateral, transfers, mergers or acquisitions, taxes, corporate changes, and deposit accounts. Compliance with these covenants may limit our flexibility in operating our business and our ability to take actions that might be advantageous to us and our shareholders. For example, if we fail to meet our minimum cash covenant and we are unable to raise additional funds or obtain a waiver or other amendment to the Loan Agreement, we may be required to delay, limit, reduce or terminate certain of our clinical development efforts. Additionally, we may be required to repay the entire amount of outstanding indebtedness under the Loan Agreement in cash if we fail to stay in compliance with our covenants or suffer some other event of default under the Loan Agreement. Under the Loan Agreement, an event of default will occur if, among other things: we fail to make payments under the Loan Agreement; we breach any of our covenants under the Loan Agreement, subject to specified cure periods with respect to certain breaches; there occurs an event that has a material adverse effect on (i) our business, operations, properties, assets or financial condition, (ii) our ability to perform or satisfy our obligations under the Loan Agreement as they become due or Hercules's ability to enforce its rights or remedies with respect to our obligations under the Loan Agreement, or (iii) the collateral or liens securing our obligations under the Loan Agreement; we or our assets become subject to certain legal proceedings, such as bankruptcy proceedings; we are unable to pay our debts as they become due; or we default on contracts with third parties which would permit Hercules to accelerate the maturity of such indebtedness or that could have a material adverse effect on us. We may not have enough available cash or be able to raise additional funds through equity or debt financings to repay such indebtedness at the time any such event of default occurs. In that case, we may be required to delay, limit, reduce or terminate our clinical development efforts or grant to others rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. Hercules could also exercise its rights as collateral agent to take possession and dispose of the collateral securing the loan for its benefit, which collateral includes all of our property other than our intellectual property. Our business, financial condition and results of operations could be substantially harmed as a result of any of these events.

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

We expect to spend substantial amounts to complete the development of, seek regulatory approvals for and commercialize our product candidates. Because the length of time and activities associated with successful development of our product candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and any approved marketing and commercialization activities. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

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

- the initiation, progress, timing and results of our commercialization of our product candidates, if approved for commercial sale.

As of December 31, 2018, we had cash and cash equivalents totaling $84.9 million. For the fiscal year ended March 31, 2018 and the three and nine-months ended December 31, 2018, we incurred net losses of $221.6 million, $34.3 million and $120.0 million, respectively. At December 31, 2018, we had aggregate net interest-bearing indebtedness of $48.8 million, of which $20.6 million was due within one year. We also had $24.6 million of other non-interest-bearing current liabilities due within one year. The Loan Agreement with Hercules requires that we maintain a minimum cash balance, which was previously $35.0 million, but has been reduced to $30.0 million following the achievement of certain clinical milestones as set forth in the Loan Agreement. We anticipate that our current cash and cash equivalent balances will not be sufficient to maintain compliance with the minimum liquidity financial covenant under the Loan Agreement beyond the one-year period following the date that the accompanying unaudited condensed consolidated financial statements were issued if the Loan Agreement is not amended or additional financing is not completed. Failure to meet this minimum covenant would be considered an event of default under the Loan Agreement and could result in the acceleration of our existing indebtedness.

We are currently in the clinical stage of operations, have not yet achieved profitability, and anticipate that we will continue to incur net losses for the foreseeable future. Our existing funds will not be sufficient to enable us to complete all necessary development and to commercially launch any of our products. Accordingly, we will be required to obtain further funding through public or private equity offerings, debt financing, collaboration and licensing arrangements or other sources. Adequate additional funding may not be available to us on acceptable terms, or at all. If we are unable to raise capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of one or more of our product candidates or potentially discontinue operations. Due to these uncertainties, there is substantial doubt about our ability to continue as a going concern.

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

In June 2018, we entered into a license agreement with Oxford BioMedica for AXO-LENTI-PD; in July 2018, we entered into a license agreement with Benitec for AXO-AAV-OPMD and five research collaboration programs; and in December 2018, we entered into a license agreement with UMMS for AXO-AAV-GM1 and AXO-AAV-GM2. Under these agreements, we are subject to significant obligations, including payment obligations upon achievement of specified milestones and payments based on product sales, as well as other material obligations. For example, under our agreement with Oxford BioMedica, we could be obligated to make payments 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. In addition, we will also be obligated to pay Oxford BioMedica a tiered royalty percentage ranging from 7% to 10% based on yearly aggregate net sales of the gene therapy products licensed under the agreement. Under the UMMS Agreement, we could be obligated to make payments to UMMS totaling up to $24.5 million upon the achievement of specified development and regulatory milestones and $39.8 million upon the achievement of specified commercial milestones. We are also obligated to pay UMMS tiered mid-single digit royalties based on yearly net sales of AXO-AAV-GM1 and AXO-AAV-GM2, subject to a specified annual minimum amount. Under the Benitec Agreement, we could 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 under the Benitec Agreement, $33.5 million upon the achievement of specified development and regulatory milestones and $60.0 million upon the achievement of specified sales milestones. In addition, Benitec will receive 30% of net profits of worldwide sales of approved and commercialized products arising from the AXO-AAV-OPMD Program, subject to an agreed minimum amount for such payments, and 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. If these payments become due under the terms of the agreements, we may not have sufficient funds available to meet our obligations, in which case our development efforts would be substantially harmed. Further, failure to make these payments or to meet our other material obligations may result in our counterparties pursuing various remedies under those agreements that could adversely affect our operations.

Raising additional funds by issuing securities may cause dilution to existing shareholders and raising funds through lending and licensing arrangements may restrict our operations or require us to relinquish proprietary rights.

We expect that significant additional capital will be needed in the future to continue our planned operations. Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, strategic alliances and license and development agreements in connection with any collaborations. We do not have any committed external source of funds. To the extent that we raise additional capital by issuing equity securities, including pursuant to our "shelf" registration statement filed with the SEC, our existing shareholders' ownership may experience substantial dilution, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common shareholder. Additional debt financing or preferred equity financing, if available, may involve agreements that include covenants further limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

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

We currently have a limited number of employees and we currently rely in part on Roivant Sciences, Inc. and Roivant Sciences GmbH to provide various services for us.

We currently rely in part on services provided by RSI" and RSG, wholly owned subsidiaries of RSL, pursuant to the Services Agreements we have with these entities. Personnel and support staff who provide services to us under these Services Agreements are not required to treat management and administration of our business as their primary responsibility or act exclusively for us, and we do not expect them to do so. Under the Services Agreements, RSI and RSG have the discretion to determine who, among their employees, will perform services for us. RSI and RSG have limited resources. If either RSI or RSG fails to perform its obligations in accordance with the terms of the Services Agreements or to effectively manage services provided to us, the operations of our business may be adversely affected.

In addition, the level of support we receive from RSI and RSG has decreased and we expect that it will continue to decrease in the near term. As a result, we will be required to replace many of these services with our own internally developed teams or engage external professional service providers. We primarily intend to develop these capabilities internally and may incur increased costs as we hire and train additional personnel. If we are unable to develop these capabilities or we fail to do so in a timely and effective manner, the operations of our business would be adversely affected.

We may not be able to manage our business effectively if we are unable to attract and retain key personnel. In addition, if we are unable to effectively transition and integrate our new executive officers and solidify and implement our updated business strategy, our business and financial performance could be adversely affected.

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

Several members of our senior management team are relatively new to Axovant. Our financial performance will depend in significant part on our senior management team and key employees, including new members of management with expertise in the gene therapy development field. In addition, recent corporate restructurings may have impacted employee morale and led, and may continue to lead, to higher rates of voluntary attrition compared to prior years. We are highly dependent on the skills and leadership of our management team. Our senior management and key employees may terminate their position with us at any time.

If we lose one or more members of our senior management team or key employees, our ability to successfully implement our business strategy could be seriously harmed. Replacing these individuals may be difficult, cause disruption, and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain regulatory approval of and commercialize products successfully. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate additional key personnel. We do not maintain "key person" insurance for any of our executives or other employees.

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

We expect to hire additional employees for our managerial, clinical, scientific and engineering, operational, sales and marketing teams. We may have operational difficulties in connection with identifying, hiring and integrating new personnel. Future growth would impose significant additional responsibilities on our management, including the need to identify, recruit, maintain, motivate and integrate additional employees, consultants and contractors. Also, our management may need to divert a disproportionate amount of its attention away from our day-to-day activities, including development of product candidates, and devote a substantial amount of time to managing these growth activities. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and grow revenues could be reduced, and we may not be able to implement our business strategy. We may not be able to effectively manage the expansion of our operations across our entities, which may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our future financial performance and our ability to commercialize our product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

Many of the other biopharmaceutical companies that we compete against for qualified personnel and consultants have greater financial and other resources, different risk profiles and a longer history in the industry than we do. They also may provide more diverse opportunities and better chances for career advancement. Some of these characteristics may be more appealing to high-quality candidates and consultants than what we have to offer. If we are unable to continue to attract and retain high-quality personnel and consultants, the rate and success at which we can discover and develop product candidates and our business will be limited.

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

Our employees and contractors, including principal investigators, consultants, commercial collaborators, manufacturers, service providers and other vendors, or those of our affiliates, may engage in fraudulent or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or other unauthorized activities that violate the laws and regulations, including those of the FDA and other similar regulatory bodies that require the reporting of true, complete and accurate information; manufacturing standards; federal, state and foreign healthcare fraud and abuse laws and data privacy; or laws that require the true, complete and accurate reporting of financial information or data. In particular, sales, marketing and other business arrangements in the healthcare industry are subject to extensive laws intended to prevent fraud, kickbacks, self-dealing, bribery, corruption, antitrust violations, and other abusive practices. These laws may restrict or prohibit a wide range of business activities, including research, manufacturing, distribution, pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Activities subject to these laws also involve the improper use or misrepresentation of information obtained in the course of clinical trials, the creation of fraudulent data in nonclinical studies or clinical trials or illegal misappropriation of drug product, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter employee or third-party misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. Additionally, we are subject to the risk that a person or government agency could allege such fraud or other misconduct, even if none occurred. If our employees, independent contractors, principal investigators, consultants, commercial collaborators, service providers or other vendors, or those of our affiliates, are alleged or found to be in violation of any such regulatory standards or requirements, or become subject to a corporate integrity agreement or similar agreement and curtailment of our operations, it could have a significant impact on our business and financial results, including the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, suspension or delay in clinical trials, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, FDA debarment, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and oversight, any of which could adversely affect our ability to operate our business and our results of

operations.

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

Our business strategy incorporates international expansion, including establishing and maintaining operations and certain key functions in various jurisdictions around the world and establishing and maintaining relationships with distributors and manufacturers globally. Doing business internationally involves a number of risks, including:

- multiple, conflicting and changing laws and regulations such as tax laws, export and import restrictions, employment laws, anti-bribery and anti-corruption laws, regulatory requirements and other governmental approvals, permits and licenses;

- failure by us or our distributors to obtain appropriate licenses or regulatory approvals for the sale or use of our product candidates, if approved, in various countries;
- difficulties in managing foreign operations;
- complexities associated with managing multiple payer-reimbursement regimes or self-pay systems;
- financial risks, such as longer payment cycles, difficulty enforcing contracts and collecting accounts receivable and exposure to foreign currency exchange rate fluctuations;
- reduced protection for intellectual property rights;
- reduced protection of contractual rights in the event of bankruptcy or insolvency of the other contracting party;
- natural disasters, political and economic instability, including wars, terrorism and political unrest, outbreak of disease, boycotts, curtailment of trade and other business restrictions;
- failure to comply with foreign laws, regulations, standards and regulatory guidance governing the collection, use, disclosure, retention, security and transfer of personal data, including the European Union General Data Privacy Regulation ("GDPR"); and
- failure to comply with the United Kingdom Bribery Act 2010 ("U.K. Bribery Act") and similar anti-bribery and anti-corruption laws in other jurisdictions, and the Foreign Corrupt Practices Act, including its books and records provisions and its anti-bribery provisions, including by failing to maintain accurate information and control over sales and distributors' activities.

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

Legal, political and economic uncertainty surrounding the planned exit of the United Kingdom from the European Union are a source of instability and uncertainty.

The United Kingdom held a referendum on June 23, 2016 to determine whether the United Kingdom should leave the European Union, or remain as a member state, the outcome of which was in favor of leaving the European Union, which is commonly referred to as Brexit. Under Article 50 of the 2009 Lisbon Treaty, the United Kingdom will cease to be a member state when a withdrawal agreement is entered into (such agreement will also require parliamentary approval) or, failing that, two years following the notification of an intention to leave under Article 50, unless the European Council (together with the United Kingdom) unanimously decides to extend this period. On March 29, 2017, the United Kingdom formally notified the European Council of its intention to leave the European Union. It is unclear how long it will take to negotiate a withdrawal agreement, but it appears likely that Brexit will continue to involve a process of lengthy negotiations between the United Kingdom and EU Member States to determine the future terms of the United Kingdom's relationship with the European Union.

Lack of clarity about future U.K. laws and regulations as the United Kingdom determines which EU rules and regulations to replace or replicate in the event of a withdrawal, including financial laws and regulations, tax and free trade agreements, intellectual property rights, supply chain logistics, environmental, health and safety laws and regulations, immigration laws and employment laws, could decrease foreign direct investment in the United Kingdom, increase costs, depress economic activity and restrict access to capital. In addition, if the United Kingdom and the European Union are unable to negotiate acceptable withdrawal terms or if other EU Member States pursue withdrawal, barrier-free access between the United Kingdom and other EU Member States or among the European Economic Area overall could be diminished or eliminated. The long-term effects of Brexit will depend on any agreements (or lack thereof) between the United Kingdom and the European Union and, in particular, any arrangements for the United Kingdom to retain access to European Union markets either during a transitional period or more permanently.

Such a withdrawal from the European Union is unprecedented, and it is unclear how the United Kingdom's access to the European single market for goods, capital, services and labor within the European Union, or the European single market, and the wider commercial, legal and regulatory environment, will impact our U.K. operations. We may also face new regulatory costs and challenges that could have an adverse effect on our operations and development programs. Even prior to any change to the United Kingdom's relationship with the European Union, the announcement of Brexit has created economic uncertainty surrounding the terms of Brexit, and its consequences could negatively

impact our financial condition, results of operations and cash flows.

Our business and operations would suffer in the event of system failures, security breaches or cyber-attacks.

Our computer systems, as well as those of various third parties on which we rely, including those of RSL and its affiliates and our CROs and other contractors, consultants, and law and accounting firms, may sustain damage from computer viruses, unauthorized access, data breaches, phishing attacks, cybercriminals, natural disasters, terrorism, war and telecommunication and electrical failures. We rely on our third-party providers to implement effective security measures and identify and correct for any such failures, deficiencies or breaches. The risk of a security breach or disruption, particularly through cyber-attacks or cyber intrusion, including by computer hackers, foreign governments, and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. We have experienced phishing attacks in the past, which have not had a material impact on our operations; however, we may in the future experience material system failures or security breaches that could cause interruptions in our operations or result in a material disruption of our drug development programs. For example, the loss of nonclinical or clinical trial data from completed, ongoing or planned trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of or damage to our data or applications, or inappropriate disclosure of personal, confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed.

The failure to successfully implement a new enterprise resource planning ("ERP") system, or maintain our current system, could adversely impact our business and results of operations.

As RSL decreases and decentralizes the services it provides to its affiliated companies, we expect to adapt the current ERP system or implement a new ERP system to upgrade certain existing business, operational, and financial processes, upon which we rely. Until such time, we will be reliant on financial systems supported by RSI and RSG. ERP implementations are complex and time-consuming projects that require transformations of business and financial processes in order to reap the benefits of the ERP system; any such transformation involves risk inherent in the conversion to a new computer system, including loss of information and potential disruption to normal operations. Additionally, if the ERP system is not effectively implemented as planned, or the system does not operate as intended, the effectiveness of our internal controls over financial reporting could be adversely affected or our ability to assess those controls adequately could be delayed. Significant delays in documenting, reviewing and testing our internal control could cause us to fail to comply with our SEC reporting obligations related to our management's assessment of our internal control over financial reporting. In addition, if we experience interruptions in service or operational difficulties and are unable to effectively manage our business during or following the implementation of the ERP system, our business and results of operations could be harmed.

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

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

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

The product liability insurance we currently carry, and any additional product liability insurance coverage we acquire in the future, may not be sufficient to reimburse us for any expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive, and in the future, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. A successful product liability claim or series of claims brought against us could cause our share price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business, including preventing or limiting the commercialization of any product candidates we develop.

If we fail to comply with applicable U.S. and foreign privacy and data protection laws and regulations, we may be subject to liabilities that adversely affect our business, operations and financial performance.

Our information security systems are subject to laws and regulations requiring that we take measures to protect the privacy and security of certain information we gather and use in our business. For example, the federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), and its implementing regulations impose, among other requirements, certain regulatory and contractual requirements regarding the privacy and security of personal health information. In addition to HIPAA, numerous other federal and state laws, including, without limitation, state security breach notification laws, state health information privacy laws and federal and state consumer protection laws, govern the collection, use, and storage of personal information.

We may also be subject to or affected by foreign laws and regulation, including regulatory guidance, governing the collection, use, disclosure, security, transfer and storage of personal data, such as information that we collect about patients and healthcare providers in connection with clinical trials and our other operations in the U.S. and abroad. For example, the EU has adopted the GDPR, which introduces strict requirements for processing personal data. The GDPR is likely to increase the compliance burden on us, including by mandating potentially burdensome documentation requirements and granting certain rights to individuals to control how we collect, use, disclose, retain and leverage information about them. The processing of sensitive personal data, such as physical health conditions, may impose heightened compliance burdens under the GDPR and is a topic of active interest among foreign regulators. In addition, the GDPR provides for breach reporting requirements, more robust regulatory enforcement and fines of up to 20 million euros or up to 4% of annual global revenue. While the GDPR affords some flexibility in determining how to comply with the various requirements, significant effort and expense has been, and will continue to be, invested to ensure continuing compliance. Moreover, the requirements under the GDPR may change periodically or may be modified by European Union national law and could have an effect on our business operations if compliance becomes substantially more costly than under current requirements.

The global legislative and regulatory landscape for privacy and data protection continues to evolve, and implementation standards and enforcement practices are likely to remain uncertain for the foreseeable future. This evolution may create uncertainty in our business, result in liability or impose additional costs on us. The cost of compliance with these laws, regulations and standards is high and is likely to increase in the future.

It is possible that each of these privacy laws may be interpreted and applied in a manner that is inconsistent with our practices. Any failure or perceived failure by us to comply with federal, state, or foreign laws or self-regulatory standards could result in negative publicity, diversion of management time and effort and proceedings against us by governmental entities or others. In many jurisdictions, enforcement actions and consequences for noncompliance are rising. As we continue to expand into other foreign countries and jurisdictions, we may be subject to additional laws and regulations that may affect how we conduct business.

Risks Related to Clinical Development, Regulatory Approval and Commercialization

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

Our product candidates are still in development and will require extensive clinical testing before we are prepared to submit an application for marketing approval to regulatory authorities. We cannot predict with any certainty if or when we might submit any such application for regulatory approval for our product candidates or whether any such application will be approved by the applicable regulatory authority in our target markets. Human clinical trials are very expensive and difficult to design and implement, in part because they are subject to rigorous regulatory requirements. For instance, regulatory authorities may not agree with our proposed endpoints for any clinical trials of our product candidates, which may delay the commencement of our clinical trials. The clinical trial process is also time-consuming. We estimate that clinical trials of our product candidates will take at least several years to complete. Furthermore, failure can occur at any stage of the trials, and we could encounter problems that cause us to abandon or repeat clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through nonclinical studies and initial clinical trials, and the results of smaller nonclinical or early clinical trials therefore may not be predictive of the results of large scale or later-stage clinical programs. For example, in January 2018, we announced that intepirdine did not meet its primary efficacy endpoints in the Phase 2B HEADWAY and pilot Phase 2 Gait and Balance studies. In light of the data from these studies, as well as data from the September 2017 MINDSET readout, we discontinued our intepirdine program. Further, in December 2018, we announced that nelotanserin did not meet its primary efficacy endpoint in its Phase 2 clinical study, and we discontinued further clinical development of nelotanserin. Likewise, there can be no assurance that the results of studies conducted by collaborators or other third parties will be viewed favorably or are indicative of our own future study results. A number of companies in the biopharmaceutical industry, and especially in the neurology field, have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials, and in the regulatory approval process. For example, in August 2017, Acorda Therapeutics received a refusal to file letter from the FDA regarding its NDA for INBRIJA, an investigational treatment for symptoms of OFF episodes in patients with Parkinson’s disease taking a carbidopa/levodopa regimen.

Our gene therapy product candidates, AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD and AXO-AAV-OPMD, are in early stages of development. The outcome of nonclinical testing and early clinical trials may not be predictive of the success of later stage clinical trials, interim results of a clinical trial do not necessarily predict final results and results from one completed clinical trial may not be replicated in a subsequent clinical trial with a similar study design. The Phase 1/2 clinical trial of ProSavin conducted by Oxford BioMedica was conducted with a small patient population and was not blinded or placebo-controlled, making it difficult to predict whether the favorable results that we observed in such trials will be repeated in larger and more advanced clinical trials.

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

- failure to obtain regulatory approval to commence a trial;
- unforeseen safety issues;
- determination of dosing issues;
- lack of effectiveness during clinical trials;
- inability to reach agreement on acceptable terms with prospective CROs and clinical trial sites;
- slower than expected rates of patient recruitment or failure to recruit suitable patients to participate in a trial;
- changes in or modifications to clinical trial design;
- failure to manufacture or obtain supply of sufficient quantities of a product candidate or placebo or failure to obtain sufficient quantities of concomitant medication for use in clinical trials;
- inability or unwillingness of medical investigators to follow our clinical and other applicable protocols;
- inability to monitor patients adequately during or after treatment;
- failure to establish sufficient number of clinical trial sites; or
- clinical sites or others deviating from trial protocol, inappropriately unblinding results, or dropping out of a trial.

Further, by way of example, we, a regulatory agency or an institutional review board ("IRB") at a clinical trial site may suspend our clinical trials at any time if it appears that we or our collaborators are failing to conduct a trial in accordance with regulatory requirements, including the FDA's current Good Clinical Practice ("cGCP") regulations, that we are exposing participants to unacceptable health risks, or if the FDA finds deficiencies in our IND submissions or the conduct of these trials. Therefore, we cannot predict with any certainty the schedule for commencement and completion of clinical trials. If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our product candidates could be harmed, and our ability to generate revenues may be delayed. In addition, any delays in our clinical trials could increase our costs, cause a drop in our share price, slow down the approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, financial condition and results of operations.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the applicable regulatory agency, which may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The applicable regulatory agency may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the applicable regulatory agency and may ultimately lead to the denial of marketing approval of one or more of our product candidates.

In addition, we acquired worldwide rights to our product candidates and were not involved in their development prior to such acquisitions. Any difficulties we experience in transitioning and integrating such product candidates into our operations may result in delays in clinical trials as well as problems in our development efforts and regulatory filings, particularly if we do not receive all of the necessary drug products, information, reports and data from third parties in a timely manner. More particularly, we have had no involvement with or control over the nonclinical and clinical development of our product candidates prior to acquiring the rights to them. We are dependent on our predecessors including Oxford BioMedica, UMMS and Benitec, having conducted such research and development in accordance with the applicable protocols, legal, regulatory and scientific standards, having accurately reported the results of all clinical trials and other research conducted prior to our acquisition of the product candidates, having correctly collected and interpreted the data from these trials and other research and having supplied us with complete information, data sets and reports required to adequately demonstrate the results reported through the date of our acquisition of these assets. In addition, we have only very limited data regarding the safety, tolerability and efficacy of AXO-AAV-GM1 for the potential treatment of GM1 gangliosidosis, AXO-AAV-GM2 for the potential treatment of GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease), AXO-LENTI-PD for the potential treatment of Parkinson's disease, and AXO-AAV-OPMD for the potential treatment of OPMD, and we have not previously conducted development activities for a biological product candidate. Problems related to our predecessors, including UMMS, Oxford BioMedica and Benitec, and our limited available data for AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD and AXO-AAV-OPMD could result in increased costs and delays in the development of our product candidates, which could adversely affect our ability to generate future revenues.

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

We may encounter delays in enrolling, or be unable to enroll, a sufficient number of patients to complete any of our clinical trials, and even once enrolled we may be unable to retain a sufficient number of patients to complete any of our trials. Patient enrollment and retention in clinical trials depends on many factors, including the size of the patient population, the nature of the trial protocol, the effectiveness of our patient recruitment efforts, the existing body of safety and efficacy data with respect to the study drug, the perceived risks and benefits of gene therapy approaches for the treatment of neurological diseases, the number and nature of competing existing treatments for our target indications, the number and nature of ongoing trials for other product candidates in development for our target indications, perceived risk of the delivery procedure, patients with pre-existing conditions that preclude their participation in any trial, the proximity of patients to clinical sites and the eligibility criteria for the study. Furthermore, the negative results we have reported in clinical trials to date and any other negative results we may report in clinical trials of any of our product candidates in the future may make it difficult or impossible to recruit and retain patients in other clinical trials of those product candidates. Similarly, negative results reported by our competitors about their product candidates may negatively affect patient recruitment in our clinical trials. Delays or failures in planned patient enrollment or retention may result in increased costs, program delays or both, which could have a harmful effect on our ability to develop our product candidates or could render further development impossible. In addition, we expect to rely on CROs and clinical trial sites to ensure proper and timely conduct of our future clinical trials and, while we intend to enter into agreements governing their services, we will be limited in our ability to control their actual performance.

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

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

We consider our most direct competitor with respect to AXO-LENTI-PD to be Voyager Therapeutics, which is developing VY-AADC, a gene therapy product candidate for the treatment of advanced Parkinson's disease. VY-AADC delivers the AADC gene, one of the three genes contained in AXO-LENTI-PD, via an adeno-associated virus (an "AAV virus-based vector"). Voyager began a Phase 2 study in June 2018. Agilis Biotherapeutics, which was acquired by PTC Therapeutics, is developing AGIL-AADC, another AAV virus-based vector gene therapy that delivers the AADC gene, for the treatment of AADC deficiency, a rare disorder that involves loss of AADC gene function. In addition, DBS (Deep Brain Stimulation) is approved for treating Parkinson's disease and is marketed by multiple device manufacturers, including Medtronic, Abbott and Boston Scientific. DBS treatment involves permanent placement of hardware in the brain via stereotactic neurosurgery and may require follow-up adjustments or even invasive device replacements. Another surgical approach is Abbvie's Duopa which is delivered via a port implanted in the abdominal wall. Further efforts are also underway to develop and commercialize new improved formulations of L-dopa, including Acorda's Inbrija, for which an NDA was approved by the FDA in December 2018, and Mitsubishi Tanabe's ND0612. Adjunct therapies are also being developed or have recently been approved to supplement L-dopa therapy, including Sunovion's sublingual apomorphine and Adamas Pharmaceuticals' GoCovri. Several companies are also trying to develop other disease modifying therapies that could prevent the progression of Parkinson's disease. Examples of these early stage efforts include Denali Therapeutics' LRRK2 inhibitors and anti-alpha synuclein antibodies from Prothena/Roche and Biogen, as well as Prevail Therapeutics' pipeline of AAV-based therapeutics targeting lysosomal dysfunction.

We consider our most direct competitor with respect to AXO-AAV-OPMD to be Enlivex Therapeutics Ltd., formerly BioBlast Pharma, which is developing trehalose, an investigational drug product thought to stabilize mutant proteins and increase autophagy, for the treatment of OPMD and other indications. In addition, there are surgical approaches to address the symptoms of OPMD, such as cricopharyngeal myotomy.

We are unaware of any directly competing commercialized product or clinical-stage product candidate with respect to either AXO-AAV-GM1 or AXO-AAV-GM2 other than LYS-GM101, a gene therapy product candidate for the treatment of GM1 gangliosidosis being developed by Lysogene S.A.

Many of our existing or potential competitors have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, as well as in obtaining regulatory approvals of those product candidates in the United States and in foreign countries. Our current and potential future competitors also have significantly more experience commercializing drugs, particularly gene therapy and other biological products, that have been approved for marketing. Mergers and acquisitions in the pharmaceutical and biotechnology industries could result in even more resources being concentrated among a small number of our competitors.

We will face competition from other drugs or from other non-drug products currently approved or that will be approved in the future in the neurology field, including for the treatment of Parkinson's disease. Therefore, our ability to compete successfully will depend largely on our ability to:

- develop and commercialize drugs that are superior to other products in the market;
- demonstrate through our clinical trials that our product candidates are differentiated from existing and future therapies;
- attract qualified scientific, product development and commercial personnel;
- obtain patent or other proprietary protection for our medicines;
- obtain required regulatory approvals;
- obtain coverage and adequate reimbursement from, and negotiate competitive pricing with, third-party payers; and
- successfully collaborate with pharmaceutical companies in the discovery, development and commercialization of new medicines.

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

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

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

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

We expect to rely on third-party CROs and consultants to assist us in filing and supporting the applications necessary to gain marketing approvals. Securing marketing approval requires the submission of extensive nonclinical and clinical data and supporting information for our product candidates to regulatory authorities for each therapeutic indication to establish safety and efficacy of the product candidate for that indication. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. Delays or errors in the submission of applications for marketing approval or issues, including those related to gathering the appropriate data and the inspection process, may ultimately delay or affect our ability to obtain regulatory approval, commercialize our product candidates and generate product revenues.

Our product candidates may cause adverse effects or have other properties that could delay or prevent their regulatory approval or limit the scope of any approved label or market acceptance.

Adverse events caused by our product candidates or that of adjuncts could cause us, other reviewing entities, clinical trial sites or regulatory authorities to interrupt, delay or halt clinical trials and could result in the denial of regulatory approval. If an unacceptable frequency or severity of adverse events are reported in our clinical trials for our product candidates or any future product candidates, our ability to obtain regulatory approval for such product candidates may be negatively impacted. The laws and regulations governing controlled substances could limit commercialization of our product candidates, and failure to comply with those laws and regulations could also result in adverse regulatory, legal, and operational consequences.

In particular, there have been several significant adverse side effects in gene therapy treatments in the past, including reported cases of leukemia in trials using earlier generation viral vectors. Gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction early after administration which could substantially limit the effectiveness of the treatment or represent safety risks for patients. Another traditional safety concern for gene therapies using viral vectors has been the possibility of insertional mutagenesis by the vectors, leading to malignant transformation of transduced cells. Additionally, in previous clinical trials involving AAV vectors for gene therapy, some subjects experienced the development of a positive ELISPOT test associated with T-cell responses, which is of unclear clinical translatability.

There is also the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material. Possible adverse side effects that may occur with treatment with gene therapy products include an immunologic reaction early after administration that could substantially limit the effectiveness of the treatment or represent safety risks for patients. Many times, side effects are only detectable after investigational products are tested in larger scale, pivotal clinical trials or, in some cases, after they are made available to patients on a commercial scale after approval. In addition to side effects that may be caused by AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD, AXO-AAV-OPMD and our other product candidates, the administration process or related procedures also can cause adverse side effects. For example, integration of AAV DNA into the host cell's genome has been reported to occur. Further, our AAV delivery systems for AXO-AAV-GM1, AXO-AAV-GM2, and AXO-AAV-OPMD have not been validated in human clinical trials previously, and if such delivery systems do not meet the safety criteria or cannot provide the desired efficacy results, then we may be forced to suspend or terminate our development of AXO-AAV-GM1, AXO-AAV-GM2, or AXO-AAV-OPMD.

If additional clinical experience indicates that AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD, AXO-AAV-OPMD or any other product candidate has side effects or causes serious or life-threatening side effects, the development of the product candidate may fail or be delayed, or, if the product candidate has received regulatory approval, such approval may be revoked or limited.

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

- regulatory authorities may withdraw their approval of the product or require a REMS to impose restrictions on its distribution or other risk management measures;

- regulatory authorities may require the addition of labeling statements, such as warnings or contraindications;
- we may be required to change the way the product is administered or to conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients;
- we could elect to discontinue the sale of our product; and
- our reputation may suffer.

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

Our AAV-based gene therapy product candidates AXO-AAV-GM1, AXO-AAV-GM2, and AXO-AAV-OPMD and our lentiviral-based gene therapy product candidate AXO-LENTI-PD are based on new gene transfer technology, which makes it difficult to predict the time and cost of product candidate development and of subsequently obtaining regulatory approval.

We expect to concentrate our research and development efforts in gene therapy on AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD, and AXO-AAV-OPMD. The use of gene therapy in the treatment of GM1 gangliosidosis, GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease), Parkinson's disease, and OPMD is new. There can be no assurance that we will not experience problems or delays in developing new product candidates and that such problems or delays will not cause unanticipated costs, or that any such development problems can be solved. We may also experience delays in developing a sustainable, reproducible and scalable manufacturing process or transferring that process for our gene therapy product candidates from their current manufacturers, which may prevent us from completing our clinical studies or commercializing our products on a timely or profitable basis, if at all.

In addition, the clinical trial requirements of the FDA and other foreign regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of such product candidates. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or more extensively studied product candidates. To date, only a limited number of gene therapies have received marketing authorization from the FDA or foreign regulatory authorities. Until August 2017, the FDA had never approved a gene therapy product. Since that time, it has only approved a small number of product candidates, including Kymriah by Novartis International AG, for pediatric and young adult patients with a form of acute lymphoblastic leukemia, Yescarta by Kite Pharma, Inc., for adult patients with certain forms of non-Hodgkin lymphoma, and Luxturna by Spark Therapeutics, Inc., for patients with an inherited form of vision loss. It is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in either the United States, or other major markets or how long it will take to commercialize our product candidates, if any are approved. Approvals by foreign regulatory authorities may not be indicative of what the FDA may require for approval, and vice versa. Regulatory requirements governing gene therapy products have changed frequently and may continue to change in the future. The FDA has established the Office of Tissues and Advanced Therapies within its Center for Biologics Evaluation and Research ("CBER") to consolidate the review of gene therapy and related products, and has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise the CBER in its review. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the National Institutes of Health ("NIH"), also are potentially subject to review by the NIH Office of Science Policy's Recombinant DNA Advisory Committee (the "RAC") in limited circumstances. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC public review process, if undertaken, can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and authorized its initiation. Conversely, the FDA can put an IND on clinical hold even if the RAC has provided a favorable review or an exemption from in-depth, public review. If we were to engage an NIH-funded institution, to conduct a clinical trial, that institution's institutional biosafety committee as well as its IRB would need to review the proposed clinical trial to assess the safety of the trial and may determine that RAC review is needed. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of any of our product candidates. Similarly, foreign regulatory authorities may issue new guidelines concerning the development and marketing authorization for gene therapy medicinal products and require that we comply with these

new guidelines.

The FDA, NIH and the EMA have each expressed interest in further regulating biotechnology, including gene therapy and genetic testing. For example, the EMA advocates a risk-based approach to the development of a gene therapy product. Agencies at both the federal and state level in the United States, as well as the U.S. Congressional committees and other governments or governing agencies, have also expressed interest in further regulating the biotechnology industry. Such action may delay or prevent commercialization of some or all of our product candidates.

These regulatory review committees and advisory groups and any new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of certain of our product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue, and our business, financial condition, results of operations and prospects would be materially and adversely affected.

The FDA recently announced a series of draft guidance regarding potential accelerated approval endpoints for certain gene therapy products and other clinical and manufacturing issues related to gene therapy products. We cannot be certain of the ultimate impact such guidance will have on our gene therapy candidates or the duration or expense of any applicable regulatory review processes identified therein.

Even if we obtain FDA approval for our product candidates in the United States, we may never obtain approval for or commercialize them in any other jurisdiction, which would limit our ability to realize their full market potential.

In order to market any products in any particular jurisdiction, we must establish and comply with numerous and varying regulatory requirements on a country-by-country basis regarding safety and efficacy. Approval by the FDA in the United States does not ensure approval by regulatory authorities in other countries or jurisdictions. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country. Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and costs for us and require additional nonclinical studies or clinical trials which could be costly and time consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. We do not have any product candidates approved for sale in any jurisdiction, including in international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of any product we develop will be unrealized.

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

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

Regulatory authorities may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product. For example, the holder of an approved NDA or BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the NDA or BLA. The FDA typically advises that patients treated with gene therapy undergo follow-up observations for potential

adverse events for a 15-year period. The holder of an approved NDA or BLA also must submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. These authorities closely regulate the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling. We will be subject to stringent restrictions on manufacturers' communications regarding off-label use and if we do not market our products for their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the DCA or PHSA in the United States, and other comparable regulations in foreign jurisdictions, relating to the promotion of prescription drugs may lead to enforcement actions and investigations alleging violations of U.S. federal and state health care fraud and abuse laws, as well as state consumer protection laws and comparable laws in foreign jurisdictions.

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

- restrictions on manufacturing such products;
- restrictions on the labeling or marketing of such products;
- restrictions on product marketing, distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters;
- withdrawal of the products from the market;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of such products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

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

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

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

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

We expect sales of our product candidates, if approved, to generate substantially all of our product revenues for the foreseeable future. The failure of any of our product candidates, if approved, to find market acceptance would harm our business and could require us to seek additional financing.

Our gene therapy approach for our gene therapy product candidates, AXO-AAV-GM1, AXO-AAV-GM2, and AXO-AAV-OPMD, utilizing AAV vectors, and AXO-LENTI-PD, utilizing a lentiviral vector, are derived from plasmids that encode viral proteins, which may be perceived as unsafe or may result in unforeseen adverse events. Negative public opinion and increased regulatory scrutiny of gene therapy and genetic research may damage public perception of our product candidates or adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.

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Gene therapy remains a novel technology, with only a limited number of gene therapy products approved to date. Public perception may be influenced by claims that gene therapy is unsafe, unethical or immoral, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon the comfort level of physicians to prescribe our product candidates, including AXO-AAV-GM1 (if approved), AXO-AAV-GM2 (if approved), AXO-LENTI-PD (if approved), and AXO-AAV-OPMD (if approved), in lieu of, or in addition to, existing or standard treatments they are already familiar with and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD, or AXO-AAV-OPMD. Earlier gene therapy trials led to several well-publicized adverse events, including cases of leukemia and death seen in such trials using earlier generation vectors. For example, a public backlash developed against gene therapy following the death of a patient in 1999 during a gene therapy trial of research subjects with ornithine transcarbamylase ("OTC") deficiency, a rare disorder in which the liver lacks a functional copy of the OTC gene. The death of the trial subject was due to complications of adenovirus vector administration. In addition, there is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material. Adverse events in our clinical studies, even if not ultimately attributable to our product candidates (such as the many adverse events that typically arise from the conditioning process), or adverse events in other lentiviral gene therapy trials, and the resulting publicity, could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our potential product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

We may not be able to benefit from orphan designation for AXO-AAV-OPMD.

The FDA and European Commission granted AXO-AAV-OPMD orphan designation for the treatment of OPMD in 2018 and 2017, respectively. The designation of AXO-AAV-OPMD as an orphan does not guarantee that any regulatory agency will accelerate regulatory review of, or ultimately approve, that product candidate, nor does it limit the ability of any regulatory agency to grant orphan designation to product candidates of other companies that treat the same indications as our product candidate prior to our product candidate receiving exclusive marketing approval.

We may lose orphan designation and/or exclusivity if the FDA or European Commission determines that the request for designation was materially defective or if we cannot assure sufficient quantity of the applicable biological product to meet the needs of patients with OPMD.

Even if we obtain orphan designation exclusivity for one or more of our product candidates, the exclusivity may not effectively protect our product candidate(s) from competition. For example, regulatory authorities still may approve different therapeutic products for the same condition, or approve the same therapeutic product for the same condition in certain circumstances, such as if clinical superiority of the subsequent product over the approved orphan product is demonstrated. In the United States, there remains uncertainty regarding this developing regulatory area, and we cannot predict whether any orphan designation exclusivity granted for one or more of our product candidates will be maintained. If we cannot obtain or maintain orphan designation exclusivity, the commercialization of our product candidates and our financial condition and results of operations could be adversely impacted.

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

We do not have a full infrastructure for the sales, marketing or distribution of our product candidates should they be approved, and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. In order to market any product that may be approved, we must build our sales, distribution, marketing,

managerial and other non-technical capabilities or make arrangements with third parties to perform these services and obtain requisite licenses. To achieve commercial success for any product for which we have obtained marketing approval, we will need a sales and marketing organization.

We plan to commercialize our product candidates in the United States, the European Union, Japan and other major markets. If our product candidates are approved for marketing, we may build a focused sales, distribution and marketing infrastructure to market them. There are significant expenses and risks involved with establishing our own sales, marketing and distribution capabilities, including our ability to hire, retain and appropriately incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of our internal sales, marketing and distribution capabilities, and any failure to obtain and maintain the requisite licenses, could delay any product launch, which would adversely impact the commercialization of our product candidates.

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

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians or attain adequate numbers of physicians to prescribe any drugs; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

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

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

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

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

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

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

- the federal Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward,

or in return for, either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the federal Anti-Kickback Statute or specific intent to violate it to have committed a violation; in addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act;

- the federal false claims laws and civil monetary penalties laws, including the civil False Claims Act, impose criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent, knowingly making, using or causing to be made or used, a false record or statement material to a false or fraudulent claim, or knowingly making, or causing to be made, a false statement to avoid, decrease or conceal an obligation to pay money to the federal government; in addition, the government may assert that a claim including items and services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act;
- HIPAA imposes criminal and civil liability for, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or making false or fraudulent statements relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it to have committed a violation;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information on health plans, health care clearing houses, and most health care providers, known as covered entities, and their business associates, defined as independent contractors or agents of covered entities that create, receive or obtain protected health information in connection with providing a service for or on behalf of a covered entity;
- a number of federal, state and foreign laws, regulations, guidance and standards that impose requirements regarding the protection of health or other personal data that are applicable to or affect our operations;
- the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to the government information related to payments or other "transfers of value" made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, and requires applicable manufacturers and group purchasing organizations to report annually to the government ownership and investment interests held by the physicians described above and their immediate family members; and
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply to our business practices, including but not limited to, research, distribution, sales, and marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payers, including private insurers, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; and state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures, as well as state and local laws that require the registration of pharmaceutical sales representatives; and state and foreign laws governing the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our current and future business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations, agency guidance or case law involving applicable healthcare laws. If our operations are found to be in violation of any of these or any other health regulatory laws that may apply to us, we may be subject to significant penalties, including the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgement, individual imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement, and curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results

of operations. Even the mere issuance of a subpoena or the fact of an investigation alone, regardless of the merit, may result in negative publicity, a drop in our share price, and other harm to our business, financial condition and results of operations.

Defending against any such actions can be costly, time-consuming and may require significant financial and personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired.

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

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

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

- an annual, nondeductible fee payable by any entity that manufactures or imports specified branded prescription drugs and biologic agents;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer point-of-sale discounts of 50% prior to January 1, 2019, and 70% thereafter, off negotiated prices of applicable brand drugs to eligible beneficiaries under their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D;
- extension of manufacturers' Medicaid rebate liability to individuals enrolled in Medicaid managed care organizations;
- expansion of eligibility criteria for Medicaid programs in certain states;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

We cannot predict the full impact of the Affordable Care Act on pharmaceutical companies, as many of the reforms require the promulgation of detailed regulations implementing the statutory provisions, some of which has not yet fully occurred. For example, in January 2016, the Centers for Medicare and Medicaid Services ("CMS") issued a final rule regarding the Medicaid Drug Rebate Program, effective April 1, 2016, that, among other things, revises the manner in which the "average manufacturer price" is to be calculated by manufacturers participating in the program and implements certain amendments to the Medicaid rebate statute created under the Affordable Care Act. Further, there have been judicial and Congressional challenges to certain aspects of the Affordable Care Act, and we expect there will be additional challenges and amendments to the Affordable Care Act in the future. Since January 2017, the President of the United States has signed two Executive Orders and other directives designed to delay the implementation of certain provisions of the Affordable Care Act or otherwise circumvent some of the requirements for health insurance mandated by the Affordable Care Act. The Tax Cuts and Jobs Act of 2017 includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the Affordable Care Act on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." Additionally, on January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain fees mandated by the Affordable Care Act, including the so-called “Cadillac” tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. Further, the Bipartisan Budget Act of 2018, among other things, amends the Affordable Care Act, effective January 1, 2019, to increase from 50 percent to 70 percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the “donut hole.” In addition, citing legal guidance from the U.S. Department of Justice, the U.S. Department of Health and Human Services has concluded that cost-sharing reduction ("CSR") payments to insurance companies required under the Affordable Care Act have not received necessary appropriations from Congress and announced that it will discontinue these payments immediately until such appropriations are made. The loss of the CSR payments is expected to increase premiums on certain policies issued by qualified health plans under the Affordable Care Act. While Congress is considering legislation to appropriate funds for CSR payments, the future of that legislation is uncertain. Moreover, in July 2018, the Centers for Medicare and Medicaid Services published a final rule permitting further collections and payments to and from certain Affordable Care Act qualified health plans and health insurance issuers under the Affordable Care Act risk adjustment program in response to the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. Congress will likely consider other legislation to replace elements of the Affordable Care Act. The Affordable Care Act is likely to continue the downward pressure on pharmaceutical pricing and may also increase our regulatory burdens and operating costs. We continue to evaluate the effect that the Affordable Care Act and its possible repeal and replacement has on our business.

Other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. For example, in August 2011, President Obama signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee did not achieve a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, triggering the legislation’s automatic reduction to several government programs. This included further reductions to Medicare payments to providers of 2% per fiscal year, which went into effect in April 2013 and, due to subsequent legislative amendments to the statute, will stay in effect through 2027 unless additional Congressional action is taken. Additionally, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers and increased the statute of limitations period in which the government may recover overpayments to providers from three to five years. Additionally, on May 30, 2018, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017 was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational new drug products that have completed a Phase I clinical trial and are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a pharmaceutical

company to make its product candidates available to eligible patients as a result of the Right to Try Act.

Further, there have been several recent U.S. Congressional inquiries and proposed federal and state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the out-of-pocket cost of prescription drugs and reform government program reimbursement methodologies for drugs. Such scrutiny has resulted in several recent Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to pharmaceutical product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. At the federal level, the current administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. Additionally, on May 11, 2018, the President of the United States laid out his administration's "Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs" to reduce the cost of prescription drugs while preserving innovation and cures. The Department of Health and Human Services has already started the process of soliciting feedback on some of these measures and, at the same time, is immediately implementing others under its existing authority. Although some of these and other proposals will require authorization through additional legislation to become effective, Congress and the U.S. presidential administration have each indicated that they will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures have become increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Moreover, the Drug Supply Chain Security Act, which was enacted in 2012 as part of the FDA Safety and Innovation Act, imposes new obligations on manufacturers of pharmaceutical products related to product tracking and tracing. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We are not sure whether additional legislative changes will be enacted, or whether the current regulations, guidance or interpretations will be changed, or what the impact of such changes on our business, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

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

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

Market acceptance and sales of any approved product candidates that we develop will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from third-party payers, including government health administration authorities and private health insurers. In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payers. Third-party payers decide which drugs or therapies they will pay for and establish reimbursement levels. One payer's determination to provide coverage for a product does not assure that other payers will also provide coverage, and adequate reimbursement, for the product. Additionally, a third-party payer's decision to provide coverage for a drug or therapy does not imply that an adequate reimbursement rate will be approved. Each plan determines whether or not it will provide coverage for a drug or therapy, what amount it will pay the manufacturer for the drug or therapy, on what tier of its formulary the drug or therapy will be placed, and whether to require step therapy. The position of a drug on a formulary generally determines the co-payment that a patient will need to make to obtain the drug and can strongly influence the adoption of a drug by patients and physicians. Patients who are prescribed treatments for their conditions and providers prescribing such services generally rely on third-party payers to reimburse all or part of the associated healthcare costs. Patients are unlikely to use our products unless coverage is provided, and reimbursement is adequate

to cover a significant portion of the cost of our products.

The process for determining whether a third-party payer will provide coverage for a product may be separate from the process for setting the price of a product or for establishing the reimbursement rate that such a payer will pay for the product. Even if we do obtain adequate levels of reimbursement, third-party payers, such as government or private healthcare insurers, carefully review and increasingly question the coverage of, and challenge the prices charged for, products. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Increasingly, third-party payers are requiring that pharmaceutical companies provide them with predetermined discounts from list prices and are challenging the prices charged for products. We may also be required to conduct expensive pharmacoeconomic studies to justify the coverage and the amount of reimbursement for particular medications. We cannot be sure that coverage and reimbursement will be available for any product that we commercialize and, if reimbursement is available, what the level of reimbursement will be. Inadequate coverage or reimbursement may impact the demand for, or the price of, any product for which we obtain marketing approval. If coverage and adequate reimbursement are not available, or are available only to limited levels, we may not be able to successfully commercialize any product candidates that we develop.

Risks Related to Our Dependence on Third Parties

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

We are building teams with drug formulation and manufacturing expertise but do not own or operate, nor do we expect to own or operate, facilities for product manufacturing, storage and distribution, or testing. In addition to the technical challenges of drug product formulation and scale-up and environmental compliance aspects of chemical and biologics manufacturing, our vendors of manufacturing services will need to comply with U.S. and foreign regulatory authority licensure and cGMP quality requirements. These obligations are enforced by periodic inspection and audit by regulatory authorities, and any adverse findings or violations discovered on such inspections could distract our vendors and be costly and time consuming to remediate, potentially impacting their supply of clinical and future commercial products to us.

Under the Oxford BioMedica Agreement, Oxford BioMedica will manufacture and supply the AXO-LENTI-PD in accordance with separate clinical and commercial supply agreements, which will be negotiated between us and Oxford BioMedica. The Oxford BioMedica Agreement contains certain key provisions of the clinical and commercial supply agreements, including pricing structure and our ability to transfer the technology to another manufacturer at any time following the completion of formal process characterization, process validation or BLA submission. Further, the process for manufacturing gene therapy products such as AXO-LENTI-PD is more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a gene therapy product such as ours generally cannot be fully characterized. Although we may establish our own manufacturing facility or use that of a third-party contract manufacturer to support a commercial launch of AXO-LENTI-PD, if approved, the timeframe for us to obtain approval for such facility or qualify such third-party contract manufacturer and ensure that all processes, methods and equipment are compliant with cGMP requirements is uncertain. In addition, our ability to receive damages from our CROs in connection with such failures is generally contractually limited. As a result, we will heavily depend on Oxford BioMedica and its key personnel to manufacture sufficient quantities of AXO-LENTI-PD drug product for future clinical trials as well as in commercial quantities if such product candidate receives regulatory approval.

Under the Benitec Agreement, Benitec will be responsible for certain 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 development plan and budget. Benitec and a third-party cGMP manufacturer are responsible for completing the cGMP manufacturing processes necessary to initiate clinical trials of AXO-AAV-OPMD. If Benitec or the third-party cGMP manufacturer fails to complete these processes in a timely manner, our clinical development of AXO-AAV-OPMD may be delayed.

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

- failure to satisfy their contractual duties or obligations;
- inability to meet our product specifications and quality requirements consistently;
- delay or inability to procure or expand sufficient manufacturing capacity;
- manufacturing and/or product quality issues related to manufacturing development and scale-up;
- costs and validation of new equipment and facilities required for scale-up;
- failure to comply with applicable laws, regulations and standards, including cGMP and similar foreign standards;
- deficient or improper record-keeping;
- contractual restrictions on our ability to engage additional or alternative manufacturers;
- inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us;
- reliance on a limited number of sources, and in some cases, single sources for product components, such that if we are unable to secure a sufficient supply of these product components, we will be unable to manufacture and sell our product candidates or any future product candidate in a timely fashion, in sufficient quantities or under acceptable terms;

- lack of access or licenses to proprietary manufacturing methods used by third-party manufacturers to make our product candidates;
- lack of qualified backup suppliers for those components that are currently purchased from a sole or single source supplier;
- operations of our third-party manufacturers or suppliers could be disrupted by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier or regulatory sanctions related to the manufacture of our or other company's products;
- carrier disruptions or increased costs that are beyond our control; and
- failure to deliver our products under specified storage conditions and in a timely manner.

Our product candidates AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD, and AXO-AAV-OPMD are manufactured using technically complex processes requiring specialized facilities, highly specific raw materials and other production constraints. The complexity of these processes, as well as strict government standards for the manufacture and storage of our product candidates, subjects us to manufacturing risks for this product candidate. If supply from a manufacturing facility is interrupted, there could be a significant disruption in supply of our product candidates. If we are unable to engage other manufacturers or suppliers, we may not be able to enter into arrangements with on favorable terms or at all. Use of new third-party manufacturers could increase the risk of delays in production or insufficient supplies of our product candidates as we transfer our manufacturing technology to these manufacturers and as they gain experience manufacturing our product candidates.

Any of these events affecting our product candidates or those of adjuncts could lead to clinical trial delays, cost overruns, delay or failure to obtain regulatory approval or impact our ability to successfully commercialize our products, as well as potential product liability litigation, product recalls or product withdrawals. Some of these events could be the basis for FDA action, including injunction, recall, seizure, or total or partial suspension of production.

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

We intend to rely on CROs and nonclinical and clinical trial sites to ensure the proper and timely conduct of our nonclinical studies and our clinical trials, and we expect to have limited influence over their actual performance. In addition, pursuant to the UMMS Agreement, the Oxford BioMedica Agreement, and the Benitec Agreement, we may rely on UMMS employees for certain services in connection with the transition of AXO-AAV-GM1 and AXO-AAV-GM2, Oxford BioMedica employees for certain services in connection with the transition of AXO-LENTI-PD, and Benitec employees for certain services in connection with the transition of AXO-AAV-OPMD. We will not have complete control over those employees or their execution of services provided to us under the UMMS Agreement, the Oxford BioMedica Agreement, or the Benitec Agreement.

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

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

result, our financial results and the commercial prospects for any product candidate that we develop could be harmed, our costs could increase, and our ability to generate revenues could be delayed.

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

Certain intellectual property of Oxford BioMedica relating to AXO-LENTI-PD and other gene therapy products that we have licensed from Oxford BioMedica are subject to a lien under Oxford BioMedica's debt agreements. The foreclosure on such intellectual property or exercise of other remedies available to the lenders under such debt agreements could materially adversely affect our rights under the Oxford BioMedica Agreement and our future prospects.

Certain intellectual property and other intangible assets of Oxford BioMedica, excluding the gene therapy product-specific intellectual property licensed under the Oxford BioMedica Agreement, are encumbered by an existing loan agreement between Oxford BioMedica and certain of its lenders. There can be no assurance that Oxford BioMedica will remain in compliance with its obligations under the loan agreement. In the event of foreclosure or exercise of other remedies by the lenders under such agreement on the assets (including such intellectual property) pledged to such lenders, our ability to use and develop AXO-LENTI-PD and other gene therapy product candidates under the license may be materially adversely affected, and we may be required to negotiate with third-party lenders with whom we do not have a prior relationship.

We may seek to enter into collaborations in the future with other third parties. If we are unable to enter into such collaborations, or if these collaborations are not successful, our business could be adversely affected.

We will seek to enter into additional collaborations in the future, including sales, marketing, distribution, development, licensing, and/or broader collaboration agreements. Our likely collaborators include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies, biotechnology companies, and medical device manufacturers. However, we may not be able to enter into additional collaborations on favorable terms or at all. Our ability to generate revenues from our collaborations will depend on our and our collaborators' abilities to successfully perform the functions assigned to each of us in these arrangements. In addition, our collaborators have the ability to abandon research or development projects and terminate applicable agreements. Moreover, an unsuccessful outcome in any clinical trial for which our collaborator is responsible could be harmful to the public perception and prospects of our existing product candidate pipeline.

Our relationship with any future collaborations may pose several risks, including the following:

- collaborators have significant discretion in determining the amount and timing of the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- the nonclinical studies and clinical trials conducted as part of these collaborations may not be successful;
- collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on nonclinical study or clinical trial results, changes in the collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay nonclinical studies and clinical trials, provide insufficient funding for nonclinical studies and clinical trials, stop a nonclinical study or clinical trial or abandon a product candidate, repeat or conduct new nonclinical studies or clinical trials or require a new formulation of a product candidate for nonclinical studies or clinical trials;
- we may not have access to, or may be restricted from disclosing, certain information regarding product candidates being developed or commercialized under a collaboration and, consequently, may have limited ability to inform our stockholders about the status of such product candidates;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- product candidates developed in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- a collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of any such product candidate;
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disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development of any product candidates, may cause delays or termination of the research, development or commercialization of such product candidates, may lead to additional responsibilities for us with respect to such product candidates or may result in litigation or arbitration, any of which would be time consuming and expensive;

- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- disputes may arise with respect to the ownership or inventorship of intellectual property developed pursuant to our collaborations;

- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- the terms of our collaboration agreement may restrict us from entering into certain relationships with other third parties, thereby limiting our options; and
- collaborations may be terminated for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

We will face significant competition in seeking appropriate collaborators, and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration agreement with any future collaborators will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of several factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. We may also be restricted under future license agreements from entering into agreements on certain terms with potential collaborators.

Risks Related to Our Intellectual Property

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

We rely, and will continue to rely, upon a combination of patents, trademarks, trade secret protection and confidentiality agreements with employees, consultants, collaborators, advisors and other third parties to protect the intellectual property related to our current and future drug development programs and product candidates. Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries for our current product candidates and any future product candidates. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our current and future product development programs and product candidates. The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. The patent applications we have in-licensed cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such application(s). The licensed patent applications may fail to result in issued patents with claims that cover AXO-AAV-GM1, AXO-AAV-GM2, AXO-AAV-OPMD or other gene therapy product candidates in the United States or in foreign countries. As a result, our in-licensed patent portfolio alone may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our current product candidates or any future product candidates in the United States or in other foreign countries. We may also inadvertently make statements to regulatory agencies during the regulatory approval process that may be inconsistent with positions that have been taken during prosecution of our patents which may result in such patents being narrowed, invalidated, or held unenforceable. The patents and patent applications that we own or in-license may fail to result in issued patents with claims that cover our current product candidates or any future product candidates in the United States or in other foreign countries.

The patent rights that we own or have licensed relating to our product candidates may be limited in ways that may affect our ability to exclude third parties from competing against us if we obtain regulatory approval to market these product candidates. For our current product candidates or future product candidates for which we do not hold or do not obtain composition of matter patents, competitors who obtain the requisite regulatory approval can offer products with the same composition as our products so long as the competitors do not infringe any method patents that we may hold. Method patents only protect the product when used or sold for the specified method. However, this type of patent protection does not limit a competitor from making and marketing a product that is identical to our product that is labeled for an indication that is outside of the patented method, or for which there is a substantial use in commerce outside the patented method.

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

Our owned or in-licensed pending applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. If the patent applications we hold or have in-licensed with respect to our development programs and product candidates fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our current or future product candidates, it could dissuade companies from collaborating with us to develop product candidates and threaten our

ability to commercialize future products. Any such outcome could have an adverse effect on our business.

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

Patent reform legislation could increase uncertainties and costs surrounding the prosecution of our owned and in-licensed patent applications and the enforcement or defense of our owned or in-licensed issued patents. On September 16, 2011, the Leahy-Smith America Invents Act (the "Leahy-Smith Act") was signed into law. The Leahy-Smith Act made a number of significant changes to United States patent laws. These include provisions that affect the way patent applications are prosecuted and challenged at the U.S. Patent and Trademark Office ("USPTO") and may also affect patent litigation. The USPTO has developed and continues to develop new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act, subsequent rulemaking, and judicial interpretation of the Leahy-Smith Act and regulations with have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement and/or defense of our issued patents, all of which could have an adverse effect on our business and financial condition.

Moreover, we may be subject to a third-party pre-issuance submission of prior art to the USPTO, or become involved in opposition, derivation, reexamination, inter partes review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights.

Even if our patent applications that we own or license issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products.

The inventorship and/or ownership rights for patents we own or in-license may be challenged by third parties. Such challenges could result in loss of exclusive rights to such patents, which could limit our ability to stop others from using or commercializing similar or identical technology and products or require us to obtain a license from such third parties on commercially reasonable terms to secure exclusive rights, or our business could be harmed. If any such challenges to inventorship and/or ownership were asserted, there is no assurance that a court would find in our favor or that, if we choose to seek a license, such license would be available to us on acceptable terms or at all.

Moreover, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after the first non-provisional filing date. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Without patent protection for our current or future product candidates, we may be open to competition from biosimilar or generic versions of such products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

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

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

Depending upon the timing, duration and specifics of FDA marketing approval of our product candidates, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent term extension of up to five years beyond the normal expiration of the patent as compensation for patent term lost during development and the FDA regulatory review process, which is limited to the approved indication (or any additional indications approved during the period of extension). However, the total patent term including the period of extension cannot exceed 14 years from the product's approval date. Furthermore, this extension is limited to only one patent that covers the approved product, the approved use of the product, or a method of manufacturing the product. However, the applicable authorities, including the FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. We may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request.

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

The validity, scope and enforceability of any patents that cover our biologic product candidates can be challenged by third parties.

For biologics, such as AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD and AXO-AAV-OPMD, the Biologics Price Competition and Innovation Act ("BPCIA") provides a mechanism for one or more third parties to seek FDA approval to manufacture or sell biosimilar or interchangeable versions of brand name biological products. Due to the large size and complexity of biological products, as compared to small molecules, a biosimilar must be "highly similar" to the reference product with "no clinically meaningful differences between the biological product and the reference product in terms of the safety, purity, and potency of the product." The BPCIA does not require reference product sponsors to list patents in an Orange Book and does not include an automatic 30-month stay of FDA approval upon the timely filing of a lawsuit. The BPCIA, however, does provide, among other things, for a formal pre-litigation process which includes the exchange of information between a biosimilar applicant and a reference product sponsor that can include the identification of relevant patents and each parties' basis for infringement and invalidity. After the exchange of this information (if the exchange is elected), we must then initiate an infringement lawsuit within 30 days for the patents identified in the exchange. If the biosimilar applicant successfully challenges the asserted patent claims it could result in the invalidation of, or render unenforceable, some or all of the relevant patent claims or result in a finding of non-infringement.

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

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

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

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

A third-party may hold intellectual property, including patent rights and trade secrets that are important or necessary to the development of our current or future product candidates. It may be necessary for us to use the patented or proprietary technology of one or more third parties to manufacture or commercialize our product candidates, in which case we would be required to obtain a license from these third parties on commercially reasonable terms, or our business could be harmed, possibly materially. If any such patents were to be asserted against us, there is no assurance that a court would find in our favor or that, if we choose or are required to seek a license, a license to any of these patents would be available to us on acceptable terms or at all.

It may be necessary to use a patented or proprietary AAV-related technology of one or more third parties to manufacture and commercialize AXO-AAV-GM1, AXO-AAV-GM2, or AXO-AAV-OPMD. If we are unable to obtain licenses from such third parties when needed or on commercially reasonable terms, our ability to commercialize AXO-AAV-GM1 (if approved), AXO-AAV-GM2 (if approved), or AXO-AAV-OPMD (if approved), would likely be delayed or impaired.

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

Our commercial success depends in part on our avoiding infringement and other violations of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, derivation and administrative law proceedings, inter partes review, and post-grant review before the USPTO, as well as oppositions and similar proceedings in foreign jurisdictions. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we and our collaborators are developing product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, and as we gain greater visibility and market exposure as a public company, the risk increases that our product candidates or other business activities may be subject to claims of infringement of the patent and other proprietary rights of third parties. Third parties may assert that we are infringing their patents or employing their proprietary technology without authorization.

There may be third-party patents or patent applications with claims to compositions, materials, formulations, methods of manufacture or methods for treatment related to our current or future product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our current or future product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our product candidates, any molecules formed during the manufacturing process, any final product itself or the intended method of treatment of the product, including combination therapy, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents

expire.

A license may not be available on commercially reasonable terms or at all. In addition, we may be subject to claims that we are infringing other intellectual property rights, such as trademarks or copyrights, or misappropriating the trade secrets of others, and to the extent that our employees, consultants or contractors use intellectual property or proprietary information owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful infringement or other intellectual property claim against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our affected products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates, and we have done so from time to time. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize one or more of our product candidates, which could harm our business significantly. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

We cannot provide any assurances that third-party patents do not exist which might be enforced against our product candidates, resulting in either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties or other forms of compensation to third parties.

We are aware of a third-party patent application directed to methods for producing a recombinant lentiviral vector that could adversely affect the potential commercialization of AXO-LENTI-PD. While we do not believe that any such claims that would cover the methods of making AXO-LENTI-PD are patentable, we may be incorrect in this belief.

We may not identify relevant third-party patents or may incorrectly interpret the relevance, scope or expiration of a third-party patent, which might adversely affect our ability to develop and market our products.

We cannot guarantee that any of our or our licensors' patent searches or analyses, including the identification of relevant patents, the scope of patent claims or the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States and abroad that is or may be relevant to or necessary for the commercialization of our product candidates in any jurisdiction. Patent applications in the United States and elsewhere are not published until approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. In addition, U.S. patent applications filed before November 29, 2000 and certain U.S. patent applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Therefore, patent applications covering our products could have been filed by others without our knowledge. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our product candidates or the use of our products.

The scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history. Our interpretation of the relevance or the scope of a patent or a pending application may be incorrect, which may negatively impact our ability to market our products. We may incorrectly determine that our products are not covered by a third-party patent or may incorrectly predict whether a third party's pending application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States or abroad that we consider relevant may be incorrect, and our failure to identify and correctly interpret relevant patents may negatively impact our ability to develop and market our products.

If we fail to identify and correctly interpret relevant patents, we may be subject to infringement claims. We cannot guarantee that we will be able to successfully settle or otherwise resolve such infringement claims. If we fail in any such dispute, in addition to being forced to pay damages, we may be temporarily or permanently prohibited from commercializing any of our products that are held to be infringing. We might, if possible, also be forced to redesign products or services so that we no longer infringe the third-party intellectual property rights. Any of these events, even if we were ultimately to prevail, could require us to divert substantial financial and management resources that we would otherwise be able to devote to our business.

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

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We have licensed rights to intellectual property from third parties in order to commercialize our product candidates, and, in the case of AXO-LENTI-PD, intend to enter into commercial supply and manufacturing agreements with Oxford BioMedica. In particular, our product candidate AXO-LENTI-PD is dependent on the Oxford BioMedica Agreement. Pursuant to such agreement, we received from Oxford BioMedica 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. We also received from Oxford BioMedica an exclusive option to obtain a worldwide license to other patents and know-how controlled by Oxford BioMedica related to certain technology processes. Under the terms of the Oxford BioMedica Agreement, we and Oxford BioMedica have each agreed to customary non-compete restrictions limiting our respective abilities to develop certain directly-competing gene therapy products. We are solely responsible, at our expense, for all activities related to the development and commercialization of the gene therapy products under the license. We must provide Oxford BioMedica with regular forecasts and updates with respect to our development and commercialization activities. 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. We are required to meet certain diligence milestones relating to clinical site selection, obtaining regulatory advice for a gene therapy product, and inclusion of at least one U.S. based clinical site in a pivotal study of a gene therapy product.

In December 2018, we entered into the UMMS Agreement with UMMS. In particular, our product candidates AXO-AAV-GM1 and AXO-AAV-GM2 are dependent on the UMMS Agreement. Pursuant to such agreement, we received from UMMS a worldwide, royalty-bearing, sub-licensable license under certain patent applications and any patents issuing therefrom, biological materials and know-how controlled by UMMS to develop and commercialize gene therapy product candidates, including AXO-AAV-GM1 and AXO-AAV-GM2, for the potential treatments of GM1 gangliosidosis and GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease), respectively. This license is exclusive with respect to patents and biological materials and non-exclusive with respect to know-how and is subject to UMMS' retained rights for academic research, teaching and non-commercial patient care purposes, as well as to certain pre-existing rights of the U.S. government. Under the UMMS Agreement, we are solely responsible, at our expense, for the research, development and commercialization of the licensed product candidates. We will reimburse UMMS for payments made by UMMS for the manufacture of clinical trial materials for us, up to a specified amount. We are obligated to use diligent efforts to develop and commercialize the licensed product candidates and are required to achieve certain development and commercial milestones in accordance with the timeline set forth in the agreement.

In July 2018, we entered into the Benitec Agreement with Benitec. In particular, our product candidate AXO-AAV-OPMD is dependent on the Benitec Agreement. Pursuant to such agreement, we received from Benitec a worldwide, exclusive, royalty-bearing, sub-licensable license under certain patents and other intellectual property controlled by Benitec to develop and commercialize AXO-AAV-OPMD and related gene therapy products. Under the Benitec Agreement, Benitec also agreed to collaborate on five additional research plans for other genetic neurological 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. We are required to use commercially reasonable efforts to develop and to seek regulatory approval for at least one collaboration product candidate from each collaboration program in the United States and major market countries in Europe. In addition, we are required to use commercially reasonable efforts to develop and to seek regulatory approval for at least one AXO-AAV-OPMD product candidate for OPMD in each of the United States, Canada, France, and Israel. We will be solely responsible for the cost for the development, manufacture, and commercialization of AXO-AAV-OPMD product candidates and collaboration product candidates, with contract manufacturing performed by a third-party cGMP manufacturer.

Disputes may arise between us and any of these counterparties regarding intellectual property rights that are subject to such agreements, including, but not limited to:

- the scope of rights granted under the agreement and other interpretation-related issues;
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whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the agreement;

- our right to sublicense patent and other rights to third parties;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations;
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners;
- our right to transfer or assign our license; and
- the effects of termination.

These or other disputes over intellectual property that we have licensed (or will license or acquire in the future) may prevent or impair our ability to maintain our current arrangements on acceptable terms or may impair the value of the arrangement to us. Any such dispute could have an adverse effect on our business.

If we materially breach or fail to perform any provision under these license and collaboration agreements, including failure to make payments to a licensor or collaborator when due for royalties and failure to use commercially reasonable efforts to develop and commercialize our product candidates, such as AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD, or AXO-AAV-OPMD, such licensors and collaborators have the right to terminate our agreement, and upon the effective date of such termination, our right to practice the licensed patent rights and other intellectual property would end. Any uncured, material breach under the agreements could result in our loss of rights to practice the patent rights and other intellectual property licensed to us under the agreements and to liability for potential damages.

Our intellectual property agreements with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology.

Certain provisions in our intellectual property agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could affect the scope of our rights to the relevant intellectual property or technology or affect financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

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

Competitors may infringe or otherwise violate our patents, the patents of our licensors or our other intellectual property rights. To counter infringement or unauthorized use, we may be required to file legal claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid or is unenforceable or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing. The initiation of a claim against a third-party may also cause the third-party to bring counter claims against us such as claims asserting that our patents are invalid or unenforceable.

In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, non-enablement, or lack of statutory subject matter. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant material information from the USPTO, or made a materially misleading statement, during prosecution. Third parties may also raise similar validity claims before the USPTO in post-grant proceedings such as ex parte reexaminations, inter partes review, or post-grant review, or oppositions or similar proceedings outside the United States, in parallel with litigation or even outside the context of litigation. The outcome following legal assertions of invalidity and unenforceability is unpredictable. We cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. For the patents and patent applications that we have licensed, we may have limited or no right to participate in the defense of any licensed patents against challenge by a third-party. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of any future patent protection on our current or future product candidates. Such a loss of patent protection could harm our business.

We may not be able to detect or prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States. Any litigation or other proceedings to enforce our intellectual property rights may fail, and even if successful, may result in substantial costs and distract our management and other employees.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could

also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have an adverse effect on the price of our common shares.

Because of the expense and uncertainty of litigation, we may not be in a position to enforce our intellectual property rights against third parties.

Because of the expense and uncertainty of litigation, we may conclude that even if a third-party is infringing our issued patent, any patents that may be issued as a result of our pending or future patent applications or other intellectual property rights, the risk-adjusted cost of bringing and enforcing such a claim or action may be too high or not in the best interest of our company or our shareholders. In such cases, we may decide that the more prudent course of action is to simply monitor the situation or initiate or seek some other non-litigious action or solution.

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

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

Similarly, changes in patent law and regulations in other countries or jurisdictions or changes in the governmental bodies that enforce them or changes in how the relevant governmental authority enforces patent laws or regulations may weaken our ability to obtain new patents or to enforce patents that we have licensed or that we may obtain in the future.

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

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

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

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

Many countries, including European Union countries, India, Japan and China, have compulsory licensing laws under which a patent owner may be compelled under specified circumstances to grant licenses to third parties. In those countries, we may have limited remedies if patents are infringed or if we are compelled to grant a license to a third-party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

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Because we expect to rely on third parties to manufacture our product candidates, and we expect to continue to collaborate with third parties on the development of our current and future product candidates, we must, at times, share trade secrets with them. We also conduct joint research and development programs that may require us to share trade secrets under the terms of our collaboration or similar agreements. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Any disclosure, either intentional or unintentional, by our employees, the employees of third parties with whom we share our facilities or third-party consultants and vendors that we engage to perform research, clinical trials or manufacturing activities, or misappropriation by third parties (such as through a cybersecurity breach) of our trade secrets or proprietary information could enable competitors to duplicate or surpass our technological achievements, thus eroding our competitive position in our market. Further, adequate remedies may not exist in the event of unauthorized use or disclosure. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor’s discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have an adverse effect on our business and results of operations.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets, although our agreements may contain certain limited publication rights. Policing unauthorized use of our or our licensors' intellectual property is difficult, expensive and time-consuming, and we may be unable to determine the extent of any unauthorized use. Moreover, enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any of our third-party collaborators. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

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

We employ individuals who were previously employed at other biotechnology or pharmaceutical companies. Although we seek to protect our ownership of intellectual property rights by ensuring that our agreements with our employees, collaborators, and other third parties with whom we do business include provisions requiring such parties to assign rights in inventions to us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed confidential information of our employees’ former employers or other third parties. We may also be subject to claims that former employers or other third parties have an ownership interest in our patents. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and if we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Even if we are successful, litigation could result in substantial cost and be a distraction to our management and other employees. Moreover, any such litigation or the threat thereof may adversely affect our reputation, our ability to form strategic alliances or sublicense our rights to collaborators, engage with scientific advisors or hire employees or consultants, each of which would have an adverse effect on our business, results of operations and financial condition.

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

against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

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

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

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

In addition to seeking patents for our current and future product candidates, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect our trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets.

If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

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

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

If we attempt to enforce our trademarks and assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

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

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

advantage. The following examples are illustrative:

- others may be able to make products that are the same as or similar to our product candidates, but that are not covered by the claims of the patents or other intellectual property rights that we own that we have exclusively licensed and have the right to enforce;
- we, our licensor or any collaborators might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own;
- we or our licensor might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;

- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own may not provide us with any competitive advantages, or may be held invalid or unenforceable as a result of legal challenges;
- our competitors might conduct research and development activities in the United States and other countries that provide a safe harbor from patent infringement claims for certain research and development activities, as well as in countries where we do not have patent rights, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets; and
- we may not develop additional proprietary technologies that are patentable.

Risks Related to Our Common Shares

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

Although our common shares are listed on the Nasdaq Global Select Market ("Nasdaq"), we cannot assure you that an active trading market for our common shares will continue to develop or be sustained. In addition, as a result of RSL owning 63.8% of our common shares as of December 31, 2018, trading in our common shares may be less liquid than the shares of companies with broader public ownership. If an active market for our common shares is not sustained, you may not be able to sell your shares quickly or at the market price. An inactive market may also impair our ability to raise capital to continue to fund operations by selling common shares and may impair our ability to acquire other companies or technologies by using our common shares as consideration.

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

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

- any additional delays in the commencement, enrollment and ultimate completion of our clinical trials;
- results of clinical trials of our product candidates or those of our competitors, such as our announcement of the failure of our Phase 2B HEADWAY clinical trial of intepirdine in patients with DLB and the pilot Phase 2 Gait and Balance clinical trial of intepirdine in patients with dementia and gait impairment to meet their respective primary endpoints, 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, and the December 2018 announcement that our Phase 2 clinical study of nelotanserin for the treatment of RBD in patients with LBD did not meet its primary endpoint;
- any delay in filing applications for marketing approval of AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD, or AXO-AAV-OPMD, and any adverse development or perceived adverse development with respect to applicable regulatory authorities' review of those applications;
- failure to successfully develop and commercialize AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD, or AXO-AAV-OPMD or any other of our current or future product candidates;
- failure to maintain our relationship with UMMS, Oxford BioMedica, or Benitec or comply with the terms of the UMMS Agreement, the Oxford BioMedica Agreement, or the Benitec Agreement;
- inability to obtain additional funding;
- inability to obtain, protect or maintain necessary intellectual property;
- regulatory or legal developments in the United States and other countries applicable to our product candidates, including gene therapies;
- adverse regulatory decisions or statements;
- changes in the structure of healthcare payment systems;
- inability to obtain adequate product supply for our current product candidates or any future product candidate, or the inability to do so at acceptable prices;
- introduction of new products, services or technologies by our competitors;
- failure to meet or exceed financial projections we provide to the public;
- failure to meet or exceed the estimates and projections of the investment community;
- changes in the market valuations of similar companies;
- market conditions in the pharmaceutical and biotechnology sectors, and the issuance of new or changed securities analysts' reports or recommendations;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- significant lawsuits, including patent or shareholder litigation, and disputes or other developments relating to our proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- additions or departures of key scientific or management personnel;
- short sales of our common shares;
- sales of our common shares by us or our shareholders in the future;

- negative coverage in the media or analyst reports, whether accurate or not;
- issuance of subpoenas or investigative demands, or the public fact of an investigation by a government agency, whether meritorious or not;
- trading volume of our common shares;
- general economic, industry and market conditions; and
- the other factors described in this "Risk Factors" section.

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

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

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

We are a "controlled company" within the meaning of the applicable rules of the Nasdaq and, as a result, qualify for exemptions from certain corporate governance requirements. If we rely on these exemptions, you will not have the same protections afforded to shareholders of companies that are subject to such requirements.

RSL controls a majority of the voting power of our outstanding common shares. As a result, we are a "controlled company" within the meaning of the Nasdaq corporate governance requirements. Under these rules, a company of which more than 50% of the voting power for the election of directors is held by an individual, group or another company is a "controlled company" and may elect not to comply with certain corporate governance requirements, including the requirements:

- that a majority of its board of directors consists of independent directors;
- for an annual performance evaluation of the nominating and corporate governance and compensation committees;
- to require director nominees to be selected, or recommended for the board of directors' selection, either by independent directors constituting a majority of the Board's independent directors in a vote in which only independent directors participate or a nominations committee comprised solely of independent directors; and
- to have a compensation committee that is composed entirely of independent directors with a written charter addressing the committee's purpose and responsibility.

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

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

Based on common shares outstanding as of December 31, 2018, RSL beneficially owns approximately 63.8% of the voting power of our outstanding common shares and has the ability to substantially influence us through this ownership position. For example, RSL may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. RSL's interests may not always coincide with our corporate interests or the interests of other shareholders, and they may act in a manner with which you may not agree or that may not be in the best interests of our other shareholders. So long as RSL continues to own a significant amount of our equity, RSL will continue to be able to strongly influence or effectively control our decisions.

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

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

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

If securities or industry analysts cease to publish research or reports about our business, or publish negative reports about our business, our share price and trading volume could decline.

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

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

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

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

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

All of the shares sold in our initial public offering ("IPO") and our follow-on offerings described below, as well as shares issued upon the exercise of options granted to persons other than our officers and directors, are freely transferable without restrictions or further registration under the Securities Act. As of December 31, 2018, 99,285,714 of our outstanding common shares, representing a majority of our common shares, were held by RSL. If RSL or any of our executive officers or directors were to sell our common shares, or if the market perceived that RSL or any of our executive officers or directors intend to sell our common shares, it could negatively affect our share price. Prior to RSL’s corporate reorganization and recapitalization in December 2015, any decision by RSL to sell or otherwise dispose of our shares required the unanimous agreement of all of the directors of RSL, including Vivek Ramaswamy, our former director and former principal executive officer. Subsequent to RSL’s corporate reorganization and recapitalization in December 2015, any such decision no longer requires a unanimous vote of RSL’s directors, meaning that all or a portion of the shares of our common stock held by RSL may be sold without Vivek Ramaswamy’s consent. However, any such sales must still be made in compliance with the Securities Act and the rules and regulations thereunder, which could limit the number of our shares that RSL could sell in any 90-day period.

We have filed registration statements on Form S-8 under the Securities Act to register the common shares that may be issued under our equity incentive plans from time to time. Shares registered under these registration statements are available for sale in the public market subject to vesting arrangements and exercise of options, as well as Rule 144 in

the case of our affiliates. We also filed a "shelf" registration statement on Form S-3 under the Securities Act in December 2016, allowing us, from time to time, to offer up to $750 million of any combination of registered common shares, preferred shares, debt securities and warrants. In April 2017 and December 2018, we offered and sold approximately $143.7 million and $33.2 million, respectively, of our common shares, gross of underwriting discounts and commissions and offering expenses, pursuant to this registration statement.

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

As a public company, we have incurred and will continue to incur significant legal, accounting and other expenses. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of Nasdaq and other applicable securities rules and regulations impose various requirements on public companies. Our management and other personnel devote a substantial amount of time to compliance with these requirements. Moreover, changing rules and regulations may increase our legal and financial compliance costs and make some activities more time-consuming and more costly. If, notwithstanding our efforts to comply with new or changing laws, regulations and standards, we fail to comply, regulatory authorities may initiate legal proceedings against us, and our business may be harmed.

Further, failure to comply with these laws, regulations and standards may make it more difficult and more expensive for us to obtain directors' and officers' liability insurance, which could make it more difficult for us to attract and retain qualified members of our Board of Directors or members of senior management.

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

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and to protect from fraudulent, illegal or unauthorized transactions. Effective disclosure controls and procedures enable us to make timely and accurate disclosure of financial and non-financial information that we are required to disclose. If we cannot provide effective controls and reliable financial reports and other disclosures, our business and operating results could be harmed. We have in the past discovered, and may in the future discover, areas of our internal controls over financial reporting or disclosure controls and procedures that, even if effective, could be improved. For example, with respect to disclosure controls and procedures, in January 2018, we issued a press release disclosing clinical trial results that included an erroneous statistical value. We issued a correction the next day, and we are taking steps to further enhance controls over our clinical data disclosure process. We are required, pursuant to Section 404 of the Sarbanes-Oxley Act, to furnish a report by management on the effectiveness of our internal control over financial reporting as of the end of each fiscal year. Our independent registered public accounting firm will not be required to attest to the effectiveness of our internal control over financial reporting until our first annual report required to be filed with the SEC following the later of the date we are deemed to be a "large accelerated filer," as defined in the Exchange Act, or the date we are no longer an "emerging growth company," as defined in the JOBS Act.

If material weaknesses or control deficiencies occur or our disclosure controls and procedures are ineffective in the future, we may be unable to report our financial results or make other disclosures accurately on a timely basis, which could cause our reported financial results or other disclosures to be materially misstated and result in the loss of investor confidence and cause the market price of our common shares to decline.

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

We are an "emerging growth company," as defined in the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including exemption from compliance with the auditor attestation requirements of Section 404, reduced disclosure obligations regarding executive compensation and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved. We will remain an emerging growth company until the earliest of (1) March 31, 2021, (2) the last day of the fiscal year in which we have total annual gross revenue of at least $1.07 billion, (3) the date on which we are deemed to be a "large accelerated filer," which means the market value of our common shares that are held by non-affiliates exceeds $700.0 million as of the prior September 30, the end of our second fiscal quarter, and (4) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

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

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

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

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

We are a Bermuda exempted company. As a result, the rights of our shareholders are governed by Bermuda law and our memorandum of association and bye-laws. The rights of shareholders under Bermuda law may differ from the rights of shareholders of companies incorporated in another jurisdiction. It may be difficult for investors to enforce in the United States judgments obtained in U.S. courts against us based on the civil liability provisions of the U.S. securities laws. It is doubtful whether courts in Bermuda will enforce judgments obtained in other jurisdictions, including the United States, against us or our directors or officers under the securities laws of those jurisdictions or entertain actions in Bermuda against us or our directors or officers under the securities laws of other jurisdictions.

Bermuda law differs from the laws in effect in the United States and may afford less protection to our shareholders.

We are incorporated under the laws of Bermuda. As a result, our corporate affairs are governed by the Bermuda Companies Act 1981, as amended (the "Companies Act"), which differs in some material respects from laws typically applicable to U.S. corporations and shareholders, including the provisions relating to interested directors, amalgamations, mergers and acquisitions, takeovers, shareholder lawsuits and indemnification of directors. Generally, the duties of directors and officers of a Bermuda company are owed to the company only. Shareholders of Bermuda companies typically do not have rights to take action against directors or officers of the company and may only do so in limited circumstances. Shareholder class actions are not available under Bermuda law. The circumstances in which shareholder derivative actions may be available under Bermuda law are substantially more proscribed and less clear than they would be to shareholders of U.S. corporations. The Bermuda courts, however, would ordinarily be expected to permit a shareholder to commence an action in the name of a company to remedy a wrong to the company where the act complained of is alleged to be beyond the corporate power of the company or illegal, or would result in the violation of the company’s memorandum of association or bye-laws. Furthermore, consideration would be given by a Bermuda court to acts that are alleged to constitute a fraud against the minority shareholders or, for instance, where an act requires the approval of a greater percentage of the company’s shareholders than those who actually approved it. When the affairs of a company are being conducted in a manner that is oppressive or prejudicial to the interests of some shareholders, one or more shareholders may apply to the Supreme Court of Bermuda, which may make such order as it sees fit, including an order regulating the conduct of the company’s affairs in the future or ordering the purchase of the shares of any shareholders by other shareholders or by the company. Additionally, under our bye-laws and as permitted by Bermuda law, each shareholder has waived any claim or right of action against our directors or officers for any action taken by directors or officers in the performance of their duties, except for actions involving fraud or dishonesty. In addition, the rights of our shareholders and the fiduciary responsibilities of our directors under Bermuda law are not as clearly established as under statutes or judicial precedent in existence in jurisdictions in the United States, particularly the State of Delaware. Therefore, our shareholders may have more difficulty protecting their interests than would shareholders of a corporation incorporated in a jurisdiction within the United States.

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

Common shares may be offered or sold in Bermuda only in compliance with the provisions of the Companies Act and the Bermuda Investment Business Act 2003, which regulates the sale of securities in Bermuda. In addition, the Bermuda Monetary Authority must approve all issues and transfers of shares of a Bermuda exempted company. However, the Bermuda Monetary Authority has, pursuant to its statement of June 1, 2005, given its general permission under the Exchange Control Act 1972 and related regulations for the issue and free transfer of our common shares to and among persons who are non-residents of Bermuda for exchange control purposes as long as the shares are listed on an appointed stock exchange, which includes Nasdaq. Specific permission of the Bermuda Monetary Authority has also been obtained dated June 8, 2015 to the issue and transfer of our shares, options, warrants, depositary receipts, rights, loan notes, debt instruments and our other securities to persons resident and non-resident of Bermuda for exchange control purposes while our shares are listed on an appointed stock exchange. The general permission and the specific permission would cease to apply if we were to cease to be listed on

Nasdaq or any other appointed stock exchange.

Our bye-laws enable our board of directors to issue preference shares, which may discourage a change of control.

Our bye-laws contain provisions that enable our board of directors to determine the powers, preferences, and rights of our preference shares and to issue the preference shares without shareholder approval.

This could discourage, delay or prevent a transaction involving a change in control of our company and may prevent our shareholders from receiving the benefit from any premium to the market price of our common shares offered by a bidder in a takeover context. Even in the absence of a takeover attempt, the existence of this provision may adversely affect the prevailing market price of our common shares if it is viewed as discouraging takeover attempts in the future.

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

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

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

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

We are incorporated under the laws of Bermuda, where we are not subject to any income or withholding taxes. We are centrally managed and controlled in the United Kingdom, and under current U.K. tax law, a company which is centrally managed and controlled in the United Kingdom is regarded as resident in the United Kingdom for taxation purposes. Accordingly, we expect to be subject to U.K. taxation on our income and gains, except where an exemption applies. We may be treated as a dual resident company for U.K. tax purposes. As a result, our right to claim certain reliefs from U.K. tax may be restricted, and changes in law or practice in the United Kingdom could result in the imposition of further restrictions on our right to claim U.K. tax reliefs. We may also become subject to income, withholding or other taxes in certain jurisdictions by reason of our activities and operations, and it is also possible that taxing authorities in any such jurisdictions could assert that we are subject to greater taxation than we currently anticipate. Any such additional tax liability could adversely affect our results of operations. For example, ASGis our principal operating company for conducting our business and is the entity that holds our intellectual property rights, including AXO-AAV-GM1, AXO-AAV-GM2, AXO-LENTI-PD and AXO-AAV-OPMD. The establishment of this Swiss entity as our principal operating company and the transfer of our intellectual property rights to this entity may result in a higher overall effective tax rate.

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

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

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

Significant judgment is required in evaluating our tax positions and determining our provision for income taxes. During the ordinary course of business, there are many transactions and calculations for which the ultimate tax determination is uncertain. For example, our effective tax rates could be adversely affected by changes in foreign currency exchange rates or by changes in the relevant tax, accounting, and other laws, regulations, principles, and interpretations. As we intend to operate in numerous countries and taxing jurisdictions, the application of tax laws can be subject to diverging and sometimes conflicting interpretations by tax authorities of these jurisdictions. It is not uncommon for taxing authorities in different countries to have conflicting views, for instance, with respect to, among other things, the manner in which the arm's length standard is applied for transfer pricing purposes, or with respect to the valuation of intellectual property. In addition, tax laws are dynamic and subject to change as new laws are passed and new interpretations of the law are issued or applied. For example, on December 22, 2017, 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") was enacted in the United States, which introduced a comprehensive set of tax reforms. Certain impacts of this legislation have been taken into account, including the reduction of the U.S. federal statutory corporate income tax rate from the previous top marginal rate of 35 percent to a flat rate of 21 percent. The Tax Cuts and Jobs Act did not have a material impact on our consolidated financial statements since our deferred temporary differences are fully offset by a valuation allowance and we do not have any offshore earnings from which to record the mandatory transition tax. Also, in September 2018, the Swiss Parliament approved a new tax bill known as Tax Proposal 17, which will enter into force in January 2020 absent a referendum that halts its effectiveness. Tax Proposal 17 would implement a set of changes to Swiss federal and cantonal tax laws, such as the amendment of the capital tax to provide a uniform rate of 0.1%, a new patent box regime, and a reduction in the statutory profit tax rate in Canton Basel-Stadt that will result in a combined Swiss federal and cantonal tax rate of 13.04%. We continue to assess the impact of such changes in tax laws on our business and may determine that changes to our structure, practice or tax positions are necessary in light of Tax Proposal 17. Tax Proposal 17, in conjunction with the tax laws of other jurisdictions in which we operate, however, may require consideration of changes to our structure and the manner in which we conduct our business. Such changes may nevertheless be ineffective in avoiding an increase in our consolidated tax liability, which could adversely affect our financial condition, results of operations and cash flows.

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

Our tax position could be adversely impacted by changes in tax rates, tax laws, tax practice, tax treaties or tax regulations or changes in the interpretation thereof by the tax authorities in Europe (including the United Kingdom and Switzerland), the United States, Bermuda, and other jurisdictions as well as being affected by certain changes currently proposed by the Organisation for Economic Co-operation and Development and their action plan on Base Erosion and Profit Shifting. Such changes may become more likely as a result of recent economic trends in the jurisdictions in which we operate, particularly if such trends continue. If such a situation was to arise, it could adversely impact our tax position and our effective tax rate. Failure to manage the risks associated with such changes, or misinterpretation of the laws providing such changes, could result in costly audits, interest, penalties, and reputational damage, which could adversely affect our business, results of our operations, and our financial condition. Our actual effective tax rate may vary from our expectation and that variance may be material. A number of factors may increase our future effective tax rates, including: (1) the jurisdictions in which profits are determined to be earned and taxed; (2) the resolution of issues arising from any future tax audits with various tax authorities; (3) changes in the valuation of our deferred tax assets and liabilities; (4) increases in expenses not deductible for tax purposes, including transaction costs and impairments of goodwill in connection with acquisitions; (5) changes in the taxation of share-based compensation; (6) changes in tax laws or the interpretation of such tax laws, and changes in generally accepted accounting principles; and (7) challenges to the transfer pricing policies related to our structure.

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

Generally, if, for any taxable year, at least 75% of our gross income is passive income, or at least 50% of the value of our assets is attributable to assets that produce passive income or are held for the production of passive income, including cash, we would be characterized as a PFIC for U.S. federal income tax purposes. For purposes of these tests,

passive income includes dividends, interest, and gains from the sale or exchange of investment property and rents and royalties other than rents and royalties which are received from unrelated parties in connection with the active conduct of a trade or business. Additionally, a look-through rule generally applies with respect to 25% or more owned subsidiaries. If we are characterized as a PFIC, U.S. holders of our common shares may suffer adverse tax consequences, including having gains realized on the sale of our common shares treated as ordinary income rather than capital gain, the loss of the preferential tax rate applicable to dividends received on our common shares by individuals who are U.S. holders, and having interest charges apply to distributions by us and the proceeds of sales of our common shares.

Our status as a PFIC will depend on the nature and composition of our income and the nature, composition and value of our assets from time to time. The 50% passive asset test described above is generally based on the fair market value of each asset, with the value of goodwill and going concern value determined in large part by reference to the market value of our common shares, which may be volatile. Our status may also depend, in part, on how quickly we utilize the cash proceeds from our IPO and subsequent financings in our business. With respect to the taxable year that ended on March 31, 2018, we believe that we were not a PFIC, however, with respect to foreseeable future taxable years, because the PFIC tests are based upon the value of our assets, including any goodwill and going concern value, and the nature and composition of our income and assets, which cannot be known at this time, we cannot predict whether we will or will not be classified as a PFIC. Because the determination of whether we are a PFIC for any taxable year is a fact-intensive determination made annually after the end of each taxable year, and because certain aspects of the PFIC rules are uncertain, we cannot provide any assurances regarding our PFIC status for the current or future taxable years.

In our current taxable year ending March 31, 2019, we have implemented structures and arrangements intended to mitigate the possibility that we will be classified as a PFIC. There can be no assurance that the IRS will not successfully challenge these structures and arrangements, which may result in an adverse impact on the determination of whether we are classified as a PFIC.

U.S. holders that own 10 percent or more of the vote or value of our common shares may suffer adverse tax consequences because we and/or any of our non-U.S. subsidiaries are expected to be characterized as a controlled foreign corporation ("CFC"), under Section 957(a) of the U.S. Internal Revenue Code of 1986, as amended ("the Code").

A non-U.S. corporation is considered a CFC if more than 50 percent of (1) the total combined voting power of all classes of stock of such corporation entitled to vote, or (2) the total value of the stock of such corporation, is owned, or is considered as owned by applying certain constructive ownership rules, by United States shareholders (U.S. persons who own stock representing 10% or more of the vote or, for taxable years of non-U.S. corporations beginning after December 31, 2017 and for taxable years of shareholders with or within which such taxable years of non-U.S. corporations end, 10% or more of the value) on any day during the taxable year of such non-U.S. corporation. Certain United States shareholders of a CFC generally are required to include currently in gross income such U.S. shareholders' share of the CFC's "Subpart F income", a portion of the CFC's earnings to the extent the CFC holds certain U.S. property, and a portion of the CFC's "global intangible low-taxed income" (as defined under Section 951A of the Code). Such United States shareholders are subject to current U.S. federal income tax with respect to such items, even if the CFC has not made an actual distribution to such shareholders. "Subpart F income" includes, among other things, certain passive income (such as income from dividends, interests, royalties, rents and annuities or gain from the sale of property that produces such types of income) and certain sales and services income arising in connection with transactions between the CFC and a person related to the CFC. "Global intangible low-taxed income" may include most of the remainder of a CFC's income over a deemed return on its tangible assets.

As a result of certain changes in the U.S. tax law introduced by the Tax Cuts and Jobs Act, we believe that we and our non-U.S. subsidiaries are classified as CFCs in the current taxable year. For U.S. holders who hold 10% or more of the vote or value of our common shares, this may result in adverse U.S. federal income tax consequences, such as current U.S. taxation of Subpart F income and of any such shareholder's share of our accumulated non-U.S. earnings and profits (regardless of whether we make any distributions), taxation of amounts treated as global intangible low-taxed income under Section 951A of the Code with respect to such shareholder, and being subject to certain reporting requirements with the U.S. Internal Revenue Service. Any such U.S. holder who is an individual generally would not be allowed certain tax deductions or foreign tax credits that would be allowed to a U.S. corporation. If you are a U.S. holder who holds 10% or more of the vote or value of our common shares, you should consult your own tax advisors regarding the U.S. tax consequences of acquiring, owning, or disposing our common shares and the impact of the Tax Cuts and Jobs Act, especially the changes to the rules relating to CFCs.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds
None.

Item 3. Defaults Upon Senior Securities.
None.

Item 4. Mine Safety Disclosures.
Not applicable.

Item 5. Other Information.
None.

Item 6. Exhibits.

Exhibit Number	Description of Document	Incorporated by Reference			
		Schedule/Form	File No.	Exhibit	Filing Date
3.1	Certificate of Incorporation.	S-1	333-204073	3.1	05/11/2015
3.2	Memorandum of Association.	S-1	333-204073	3.2	05/11/2015
3.3	Amended and Restated Bye-laws.	8-K	001-37418	3.1	12/21/2017
10.1*+	Exclusive License Agreement, dated as of December 7, 2018, by and between the Registrant and the University of Massachusetts.				
31.1*	Certification of Principal Executive Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.				
31.2*	Certification of Principal Financial Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.				
32.1*#	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.				
32.2*#	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.				
101.INS*	XBRL Instance Document				
101.SCH*	XBRL Taxonomy Extension Schema				
101.CAL*	XBRL Taxonomy Extension Calculation Linkbase				
101.DEF*	XBRL Taxonomy Extension Definition Linkbase				
101.LAB*	XBRL Taxonomy Extension Label Linkbase				
101.PRE*	XBRL Taxonomy Extension Presentation Linkbase				

* Filed herewith.
+ Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment and have been separately filed with the Securities and Exchange Commission.
# These certifications are being furnished solely to accompany this Quarterly Report on Form 10-Q pursuant to 18 U.S.C. Section 1350, and are not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and are not to be incorporated by reference into any filing of the Registrant, whether made before or after the date hereof, regardless of any general incorporation language in such filing.

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SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

AXOVANT SCIENCES LTD.

By: /s/ Gregory Weinhoff
Gregory Weinhoff
(Duly Authorized Officer and Principal Financial Officer)

Date: February 7, 2019