

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

**FORM 10-Q**

(Mark One)

- QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**  
For the quarterly period ended **December 31, 2019**  
or  
 **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**  
For the transition period from \_\_\_\_\_ to \_\_\_\_\_  
Commission file number **001-37418**

**Axovant Gene Therapies Ltd.**

(Exact name of registrant as specified in its charter)

**Bermuda**

(State or other jurisdiction of  
incorporation or organization)

**98-1333697**

(I.R.S. Employer  
Identification No.)

**Suite 1, 3rd Floor  
11-12 St. James's Square  
London SW1Y 4LB, United Kingdom**  
(Address of principal executive offices)

**Not Applicable**  
(Zip Code)

Registrant's telephone number, including area code: **+44 203 997 8931**

Securities registered pursuant to Section 12(b) of the Act:

Title of each Class	Trading Symbol	Name of each exchange on which registered
Common Shares, par value \$0.00001 per share	AXGT	The Nasdaq Stock Market LLC

Securities registered pursuant to Section 12(g) of the Act: None

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer", "accelerated filer", "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer	<input type="checkbox"/>	Accelerated filer	<input checked="" type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input checked="" type="checkbox"/>
		Emerging growth company	<input checked="" type="checkbox"/>

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

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

The number of shares outstanding of the Registrant's common shares, \$0.00001 par value per share, on February 6, 2020, was 22,791,669.

**AXOVANT GENE THERAPIES LTD.**  
**QUARTERLY REPORT ON FORM 10-Q**  
**FOR THE QUARTER ENDED DECEMBER 31, 2019**

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**PART I. FINANCIAL INFORMATION**

**Item 1. Financial Statements (Unaudited)**

**AXOVANT GENE THERAPIES LTD.**  
**Condensed Consolidated Balance Sheets**  
*(Unaudited, in thousands, except share and per share data)*

	December 31, 2019	March 31, 2019
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 27,812	\$ 106,999
Prepaid expenses and other current assets	3,621	5,859
Income tax receivable	1,988	1,726
Total current assets	33,421	114,584
Long-term investment	5,871	5,871
Other non-current assets	46	973
Operating lease right-of-use assets	1,946	—
Property and equipment, net	980	1,278
Total assets	\$ 42,264	\$ 122,706
<b>Liabilities and Shareholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 347	\$ 1,698
Accrued expenses	20,334	20,619
Current portion of operating lease liabilities	1,156	—
Current portion of long-term debt	8,851	21,182
Total current liabilities	30,688	43,499
Operating lease liabilities, net of current portion	77	—
Long-term debt	6,452	22,994
Total liabilities	37,217	66,493
<b>Commitments and contingencies (Note 11)</b>		
Shareholders' equity:		
Common shares, par value \$0.00001 per share, 1,000,000,000 shares authorized, 22,791,669 and 22,779,891 issued and outstanding at December 31, 2019 and March 31, 2019, respectively	—	—
Additional paid-in capital	746,845	741,318
Accumulated deficit	(741,996)	(686,016)
Accumulated other comprehensive income	198	911
Total shareholders' equity	5,047	56,213
Total liabilities and shareholders' equity	\$ 42,264	\$ 122,706

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

**AXOVANT GENE THERAPIES 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,	
	2019	2018	2019	2018
<b>Operating expenses:</b>				
<b>Research and development expenses<sup>(1)</sup></b>				
(includes share-based compensation expense of \$876 and \$1,910 for the three months ended December 31, 2019 and 2018 and \$2,006 and \$3,299 for the nine months ended December 31, 2019 and 2018, respectively)	\$ 8,267	\$ 21,483	\$ 36,190	\$ 80,403
<b>General and administrative expenses<sup>(2)</sup></b>				
(includes share-based compensation expense of \$1,436 and \$2,648 for the three months ended December 31, 2019 and 2018 and \$3,332 and \$9,575 for the nine months ended December 31, 2019 and 2018, respectively)	5,409	10,933	16,928	33,309
Total operating expenses	13,676	32,416	53,118	113,712
<b>Other (income) expenses:</b>				
Interest expense	1,066	1,906	3,937	5,808
Other (income) expense	(694)	(78)	(1,231)	275
Loss before income tax expense	(14,048)	(34,244)	(55,824)	(119,795)
Income tax expense (benefit)	(9)	52	156	224
Net loss	\$ (14,039)	\$ (34,296)	\$ (55,980)	\$ (120,019)
Net loss per common share — basic and diluted	\$ (0.62)	\$ (2.13)	\$ (2.46)	\$ (8.06)
Weighted-average common shares outstanding — basic and diluted	22,791,669	16,096,488	22,785,006	14,897,890

<sup>(1)</sup> Includes total costs (benefit) allocated from certain wholly owned subsidiaries of our parent company, Roivant Sciences Ltd., of \$0 and \$(450) for the nine months ended December 31, 2019 and 2018, respectively. No such costs (benefit) were allocated during the three months ended December 31, 2019 and 2018.

<sup>(2)</sup> Includes total costs allocated from certain wholly owned subsidiaries of our parent company, Roivant Sciences Ltd., of \$27 and \$698 for the three months ended December 31, 2019 and 2018, respectively, and \$103 and \$2,772 for the nine months ended December 31, 2019 and 2018, respectively.

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

**AXOVANT GENE THERAPIES LTD.**  
**Condensed Consolidated Statements of Comprehensive Loss**  
*(Unaudited, in thousands)*

	Three Months Ended December 31,		Nine Months Ended December 31,	
	2019	2018	2019	2018
Net loss	\$ (14,039)	\$ (34,296)	\$ (55,980)	\$ (120,019)
Other comprehensive (loss) income:				
Foreign currency translation adjustment	(293)	92	(713)	538
Total other comprehensive (loss) income	(293)	92	(713)	538
Comprehensive loss	<u>\$ (14,332)</u>	<u>\$ (34,204)</u>	<u>\$ (56,693)</u>	<u>\$ (119,481)</u>

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

**AXOVANT GENE THERAPIES LTD.**  
**Condensed Consolidated Statements 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	13,473,512	\$ —	\$ 628,111	\$ (556,951)	\$ 126	\$ 71,286
Issuance of shares upon exercise of stock options	875	—	10	—	—	10
Share-based compensation expense	—	—	5,369	—	—	5,369
Capital contribution — share-based compensation expense	—	—	490	—	—	490
Foreign currency translation adjustment	—	—	—	—	559	559
Net loss	—	—	—	(51,888)	—	(51,888)
Balance at June 30, 2018	<u>13,474,387</u>	<u>\$ —</u>	<u>\$ 633,980</u>	<u>\$ (608,839)</u>	<u>\$ 685</u>	<u>\$ 25,826</u>
Issuance of shares upon exercise of stock options	11,090	—	108	—	—	108
Shares issued for private placement offering	1,785,714	—	25,000	—	—	25,000
Shares sold under share sales agreement	743	—	14	—	—	14
Share-based compensation expense	—	—	5,635	—	—	5,635
Capital contribution — share-based compensation expense	—	—	(3,178)	—	—	(3,178)
Non-cash capital contribution received from Roivant Sciences, Inc.	—	—	422	—	—	422
Foreign currency translation adjustment	—	—	—	—	(113)	(113)
Net loss	—	—	—	(33,835)	—	(33,835)
Balance at September 30, 2018	<u>15,271,934</u>	<u>\$ —</u>	<u>\$ 661,981</u>	<u>\$ (642,674)</u>	<u>\$ 572</u>	<u>\$ 19,879</u>
Issuance of shares upon exercise of stock options	21,572	—	175	—	—	175
Shares sold in public offering, net of underwriting discounts and commissions and offering expenses of \$1.6 million	4,145,115	—	31,634	—	—	31,634
Shares sold under share sales agreement	2,344	—	47	—	—	47
Share-based compensation expense	—	—	4,530	—	—	4,530
Capital contribution — share-based compensation expense	—	—	28	—	—	28
Non-cash capital contribution received from RSI	—	—	671	—	—	671
Foreign currency translation adjustment	—	—	—	—	92	92
Net loss	—	—	—	(34,296)	—	(34,296)
Balance at December 31, 2018	<u>19,440,965</u>	<u>\$ —</u>	<u>\$ 699,066</u>	<u>\$ (676,970)</u>	<u>\$ 664</u>	<u>\$ 22,760</u>

**AXOVANT GENE THERAPIES LTD.**  
**Condensed Consolidated Statements of Shareholders' Equity**  
*(Unaudited, in thousands, except share data)*

	Common Shares		Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Income (Loss)	Total Shareholders' Equity
	Shares	Amount				
Balance at March 31, 2019	22,779,891	\$ —	\$ 741,318	\$ (686,016)	\$ 911	\$ 56,213
Issuance of shares upon exercise of stock options	781	—	5	—	—	5
Share-based compensation expense	—	—	2,135	—	—	2,135
Non-cash capital contribution received from Roivant Sciences, Inc.	—	—	28	—	—	28
Foreign currency translation adjustment	—	—	—	—	(972)	(972)
Net loss	—	—	—	(28,057)	—	(28,057)
Balance at June 30, 2019	<u>22,780,672</u>	<u>\$ —</u>	<u>\$ 743,486</u>	<u>\$ (714,073)</u>	<u>\$ (61)</u>	<u>\$ 29,352</u>
Issuance of shares upon exercise of stock options	10,997	—	81	—	—	81
Share-based compensation expense	—	—	891	—	—	891
Non-cash capital contribution received from Roivant Sciences, Inc.	—	—	48	—	—	48
Foreign currency translation adjustment	—	—	—	—	552	552
Net loss	—	—	—	(13,884)	—	(13,884)
Balance at September 30, 2019	<u>22,791,669</u>	<u>\$ —</u>	<u>\$ 744,506</u>	<u>\$ (727,957)</u>	<u>\$ 491</u>	<u>\$ 17,040</u>
Share-based compensation expense	—	—	2,312	—	—	2,312
Non-cash capital contribution received from Roivant Sciences, Inc.	—	—	27	—	—	27
Foreign currency translation adjustment	—	—	—	—	(293)	(293)
Net loss	—	—	—	(14,039)	—	(14,039)
Balance at December 31, 2019	<u>22,791,669</u>	<u>\$ —</u>	<u>\$ 746,845</u>	<u>\$ (741,996)</u>	<u>\$ 198</u>	<u>\$ 5,047</u>

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

**AXOVANT GENE THERAPIES LTD.**  
**Condensed Consolidated Statements of Cash Flows**  
*(Unaudited, in thousands)*

	Nine Months Ended December 31,	
	2019	2018
<b>Cash flows from operating activities:</b>		
Net loss	\$ (55,980)	\$ (120,019)
Adjustments to reconcile net loss to net cash used in operating activities:		
Loss on disposal of fixed assets	17	10
Unrealized foreign currency translation adjustment	(713)	538
Amortization of operating lease right-of-use assets	1,203	—
Share-based compensation expense	5,338	12,874
Depreciation and non-cash amortization	1,175	2,123
Change in operating lease liabilities	(1,318)	—
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	2,376	(2,709)
Income tax receivable	(262)	171
Other non-current assets	341	(3,449)
Accounts payable	(1,351)	(2,931)
Due to/from Roivant Sciences Ltd. and certain of its wholly owned subsidiaries	(138)	(281)
Accrued expenses	(251)	(8,951)
Net cash used in operating activities	(49,563)	(122,624)
<b>Cash flows from investing activities:</b>		
Purchases of property and equipment	(250)	(74)
Net cash used in investing activities	(250)	(74)
<b>Cash flows from financing activities:</b>		
Payments on long-term debt	(29,563)	(4,780)
Capital contribution received from Roivant Sciences, Inc.	103	1,093
Cash proceeds from stock option exercises	86	293
Cash proceeds from issuance of common shares, net of costs	—	56,694
Net cash (used in) provided by financing activities	(29,374)	53,300
<b>Net change in cash and cash equivalents</b>	<b>(79,187)</b>	<b>(69,398)</b>
Cash and cash equivalents—beginning of period	106,999	154,337
Cash and cash equivalents—end of period	\$ 27,812	\$ 84,939
<b>Non-cash operating activities:</b>		
Operating lease right-of-use assets recognized upon and since the adoption of ASC 842, <i>Leases</i> , on April 1, 2019	\$ 3,103	\$ —
Operating lease liabilities recognized upon and since the adoption of ASC 842, <i>Leases</i> , on April 1, 2019	2,517	—
Amounts reclassified from other non-current assets to operating lease right-of-use assets upon the adoption of ASC 842, <i>Leases</i> , on April 1, 2019	586	—

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



**AXOVANT GENE THERAPIES LTD.**  
**Notes to Condensed Consolidated Financial Statements (Unaudited)**

**Note 1—Description of Business**

Axovant Gene Therapies Ltd. ("AGT"), together with its wholly owned subsidiaries (the "Company"), is a clinical-stage company focused on gene therapy for neurological diseases. The Company is developing a pipeline of innovative product candidates for the treatment of these debilitating diseases, including Parkinson's disease, GM1 gangliosidosis and GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease). The Company is dedicated to realizing the potential of gene therapies to offer transformative patient outcomes in areas of high unmet medical need.

AGT is an exempted limited company incorporated under the laws of Bermuda, which was originally formed under the name Roivant Neurosciences Ltd. in October 2014 and changed its name to Axovant Sciences Ltd. in March 2015 and to Axovant Gene Therapies Ltd. in March 2019. AGT has seven wholly owned subsidiaries:

- Axovant Holdings Limited ("AHL");
- Axovant Sciences, Inc. ("ASI");
- Axovant Sciences GmbH ("ASG");
- Axovant Sciences America, Inc. ("ASA");
- Axovant Treasury Holdings, Inc. ("ATH");
- Axovant Treasury, Inc. ("ATI");  
and
- Axovant Sciences Europe Limited.

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.

These unaudited condensed consolidated financial statements and accompanying notes 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 unaudited condensed consolidated financial statements and accompanying notes 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, 2019 (the "Annual Report"), filed with the Securities and Exchange Commission ("SEC") on June 11, 2019. 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 periods presented have been included. Operating results for the three and nine-months ended December 31, 2019 are not necessarily indicative of the results that may be expected for the year ending March 31, 2020, 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"). These unaudited condensed consolidated financial statements and accompanying notes 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. Certain prior period balances have been reclassified to conform to the current period presentation.

These unaudited condensed consolidated financial statements and accompanying 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)).

A 1-for-8 reverse share split of the Company's outstanding common stock was effected on May 8, 2019 as approved by the Company's Board of Directors and a majority of its shareholders, which reduced the number of common shares issued and outstanding from approximately 182.2 million to 22.8 million as of May 8, 2019. As such, all references to share and per share amounts in these unaudited condensed consolidated financial statements and accompanying notes have been retroactively restated to reflect the 1-for-8 reverse share split, except for the authorized number of shares of the Company's common stock and the par value per share, which were not affected.

There have been no significant changes in the Company's accounting policies from those disclosed in its Annual Report, except for the Company's adoption of Topic 842, "*Leases (Topic 842)*" ("*Topic 842*") (see Note 2(F) and Note 5).

**(B) Going Concern and Management's Plans:**

The Company assesses and determines its ability to continue as a going concern in accordance with the provisions of ASC Topic 205-40, "*Presentation of Financial Statements—Going Concern*" ("*ASC Topic 205-40*"), which requires the Company to evaluate whether there are conditions or events that raise substantial doubt about its ability to continue as a going concern within one year after the date that its annual and interim consolidated financial statements are issued. Certain additional financial statement disclosures are required if such conditions or events are identified. If and when an entity's liquidation becomes imminent, financial statements should be prepared under the liquidation basis of accounting. Determining the extent, if any, to which conditions or events raise substantial doubt about the Company's ability to continue as a going concern, or the extent to which mitigating plans sufficiently alleviate any such substantial doubt, as well as whether or not liquidation is imminent, requires significant judgment by management.

The Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date the consolidated financial statements are issued.

As of December 31, 2019, the Company's cash and cash equivalents totaled \$27.8 million and its accumulated deficit was \$742.0 million. For the nine months ended December 31, 2019 and the fiscal year ended March 31, 2019, the Company incurred net losses of \$56.0 million and \$129.1 million, respectively. As of December 31, 2019, the Company had aggregate net interest-bearing indebtedness of \$15.3 million, of which \$8.9 million was due within one year. The Company expects to continue to incur significant operating and net losses, as well as negative cash flows from operations, for the foreseeable future as it continues to develop its gene therapy product candidates and prepares for potential future regulatory approvals and commercialization of its products, if approved. The Company has not generated any revenue to date and does not expect to generate product revenue unless and until it successfully completes development and obtains regulatory approval for at least one of its product candidates, and its current cash and cash equivalents balance will not be sufficient to complete all necessary development activities and commercially launch its products. The Company anticipates that its current cash and cash equivalents balance will not be sufficient to sustain operations beyond three months following the date that these unaudited condensed consolidated financial statements and notes were issued, which raises substantial doubt about the Company's ability to continue as a going concern.

To continue as a going concern, the Company will need, among other things, to raise additional capital in the near term. The Company continually assesses multiple options to obtain additional funding to support its operations, including proceeds from offerings of the Company's equity securities or debt, cash received from the exercise of outstanding common stock options, or transactions involving product development, technology licensing or collaboration arrangements, or other sources of capital to complete its currently planned development programs. Management can provide no assurances that it can raise a sufficient amount of financing for the Company on favorable terms, if at all. 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 does not permit future financing activities that are not probable of being implemented and probable of alleviating the conditions that raise substantial doubt to be included in the Company's assessment of its liquidity.

Due to these uncertainties, there is substantial doubt about the Company's ability to continue as going concern. These unaudited condensed consolidated financial statements and accompanying 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.

**(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 certain assets, including which costs are charged to research and development and general and administrative expense, as well as assumptions used to estimate its ability to continue as a going concern and estimate the fair value of its stock option awards. 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. In periods in which the Company reports a net loss, all common share equivalents are deemed anti-dilutive such that basic net loss per common share and diluted net loss per common share are equivalent. Potentially dilutive common shares have been excluded from the diluted net loss per common share computations in all periods presented because such securities have an anti-dilutive effect on net loss per common share due to the Company's net loss. There are no reconciling items used to calculate the weighted-average number of total common shares outstanding for basic and diluted net loss per common share data. Stock options and Restricted Stock Units ("RSUs") to purchase approximately 2.9 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, 2019 because they were anti-dilutive given the net loss of the Company. Stock options to purchase approximately 2.1 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, 2018 because they were anti-dilutive given the net loss of the Company.

**(E) Financial Instruments and Fair Value Measurement:**

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, a long-term investment, 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 carrying value of the Company's money market fund included in cash and cash equivalents of \$10.6 million at December 31, 2019 approximated fair value, which is based on quoted prices in active markets for identical securities.

At December 31, 2019, the Company held a long-term investment in nonredeemable convertible preferred stock, which is accounted for in accordance with the provisions of ASC 321, "Investments - Equity Securities" whereby the Company elected to use the measurement alternative therein (see Note 4).

The following table summarizes the fair value of the Company's money market fund included in cash equivalents based on the inputs used at December 31, 2019 in determining such values (in thousands):

	Fair Value	Price Quotations (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Money market fund	\$ 10,555	\$ 10,555	\$ —	\$ —

The carrying value of the Company's debt of \$15.3 million at December 31, 2019 approximated fair value, which was based on current interest rates for similar types of borrowings and is in Level 2 of the fair value hierarchy.

**(F) Recent Accounting Pronouncements:**

In February 2016, the FASB issued Topic 842, which requires lessees to recognize on their consolidated balance sheets a liability to make lease payments and a right-of-use asset representing their right to use the underlying asset for the lease term for both finance and operating leases with lease terms greater than twelve months. Topic 842 is effective for annual periods beginning after December 15, 2018, and interim periods within fiscal years beginning after December 15, 2018. Early adoption is permitted. A modified retrospective transition approach is required, applying the new standard to all leases existing at the date of initial application. Topic 842 allows entities to choose to use either (i) the effective date or (ii) the beginning of the earliest comparative period presented in the financial statements as the date of initial application. Topic 842 provides a number of optional practical expedients in transition. The Company adopted Topic 842 on April 1, 2019 using the optional modified retrospective transition method. Comparative periods were not restated. For leases that commenced prior to April 1, 2019, the Company elected the following package of practical expedients when assessing the transition impact: (1) not to reassess whether any expired or existing contracts are or contain leases; (2) not to reassess the lease classification for any expired or existing leases; and (3) not to reassess initial direct costs for any existing leases. The Company also elected to: (1) use the total lease term in its initial incremental borrowing rate calculation; (2) combine its lease and non-lease components and account for them as a single lease component; and (3) not apply the use of hindsight in determining the lease term when considering lessee options to extend or terminate the lease and to purchase the underlying asset. Upon adoption, the Company recorded corresponding aggregate operating lease right-of-use assets and operating lease liabilities of \$3.0 million and \$2.4 million, respectively, including \$0.6 million of prepaid rent previously classified within other non-current assets in the Company's consolidated balance sheet that was reclassified to operating lease right-of-use assets. The adoption of the new standard did not materially impact the Company's consolidated results of operations and cash flows and did not have an impact on the Company's beginning accumulated deficit balance. For additional information regarding the Company's leases, see Note 5 for further information regarding the impact of the Company's adoption of Topic 842.

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 adopted the provisions of ASU No. 2018-02 for the fiscal year beginning April 1, 2019, which did not have a material impact on its consolidated financial statements and related disclosures.

In June 2018, the FASB issued ASU No. 2018-07, *Compensation - Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting* ("ASU No. 2018-07"). ASU No. 2018-07 requires equity-classified share-based payment awards issued to nonemployees to be measured on the grant date, rather than remeasuring the awards through the performance completion date as previously required. Additionally, for nonemployee awards with performance conditions, compensation cost associated with the award is to be recognized when achievement of the performance condition is probable, rather than upon achievement of the performance condition. Further, the requirement to reassess the liability or equity classification for nonemployee awards upon vesting is eliminated, except for awards in the form of convertible instruments. ASU No. 2018-07 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 adopted the provisions of ASU No. 2018-07 for the fiscal year beginning April 1, 2019, which did not have a material impact on its consolidated financial statements and related disclosures.

In December 2019, the FASB issued ASU No. 2019-12, *Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes* ("ASU No. 2019-12"). ASU No. 2019-12 simplifies the accounting for income taxes, eliminates certain exceptions within *Income Taxes (Topic 740)*, and clarifies certain aspects of the current guidance to promote consistency among reporting entities, and is effective for fiscal years, and for interim periods within those fiscal years, beginning after December 15, 2020, with early adoption permitted. Most amendments within ASU No. 2019-12 are required to be applied on a prospective basis, while certain amendments must be applied on a retrospective or modified retrospective basis. The Company is currently in the process of evaluating the effects of ASU No. 2019-12 on its consolidated financial statements, including potential early adoption.

Other recent accounting pronouncements issued by the FASB (including its Emerging Issues Task Force), the American Institute of Certified Public Accountants, and the SEC did not, or are not believed by management to, have a material impact on the Company's present or future consolidated financial position, results of operations or cash flows.

### **Note 3—License and Collaboration Agreements**

#### **(A) Oxford BioMedica License Agreement**

On June 5, 2018, the Company, through its wholly owned subsidiary, ASG, entered into an exclusive license agreement with Oxford BioMedica (UK) Ltd. ("Oxford"), pursuant to which the Company received a worldwide, exclusive, royalty-bearing, sub-licensable license under certain patents and other intellectual property controlled by Oxford to develop and commercialize AXO-Lenti-PD and related gene therapy products for all diseases and conditions (the "Oxford Agreement"). In June 2018, as consideration for the license, the Company made an upfront nonrefundable payment to Oxford of \$30.0 million, \$5.0 million of which was applied as a credit against the process development work and clinical supply that Oxford is obligated to provide to the Company over the term of the Oxford Agreement. Under the terms of the Oxford Agreement, the Company could be obligated to make payments to Oxford 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 April 2019, certain development milestones were achieved resulting in a \$13.0 million net payment due to Oxford. The Company will also be obligated to pay Oxford 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 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 Agreement. Pursuant to the Oxford 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 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 Agreement. If the Company fails to meet any of these specified development milestones, it may cure such failure by paying Oxford certain fees, which range from \$0.5 million to \$1.0 million.

The Company evaluated the Oxford Agreement and 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 under the Oxford Agreement was recorded as research and development expense in the Company's condensed consolidated statements of operations during the three months ended June 30, 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 is obligated to provide over the term of the Oxford Agreement, the Company fully capitalized this portion of the payment upon execution, with \$1.8 million remaining capitalized within prepaid expenses and other current assets in its unaudited condensed consolidated balance sheet as of December 31, 2019, which will be recorded to research and development expense as the process development work and clinical supply are provided by Oxford. The Company has the right to terminate the Oxford Agreement at any time upon two months' advance written notice prior to the first commercial sale of a product, or for a specified period of advance written notice after the first commercial sale of a product. Either party may terminate the Oxford Agreement for the other party's uncured material breach or with respect to a failure to make a required payment.

Additionally, excluding the upfront nonrefundable payment and development milestones achieved, the Company incurred \$2.4 million and \$6.5 million of AXO-Lenti-PD program-specific costs within research and development expenses in its unaudited condensed consolidated statements of operations during the three and nine-months ended December 31, 2019, respectively, and \$1.9 million and \$3.2 million during the three and nine-months ended December 31, 2018, respectively. The Company paid Oxford a total of \$1.7 million and \$8.2 million during the three and nine-months ended December 31, 2019, respectively, and \$0.8 million and \$30.9 million during the three and nine-months ended December 31, 2018, respectively, including the upfront nonrefundable payment and payments made on development milestones achieved.

#### **(B) Benitec Biopharma License and Collaboration Agreement**

In July 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 which the Company made an upfront payment of \$10.0 million and 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. The Company terminated the Benitec Agreement in its entirety, effective September 3, 2019.

The Company evaluated the Benitec Agreement and 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 was recorded as research and development expense in the Company's unaudited condensed consolidated statement of operations during the three months ended September 30, 2018. The Company also incurred \$(0.3) million and \$1.8 million of AXO-AAV-OPMD program-specific costs within research and development expenses in its unaudited condensed consolidated statement of operations during the three and nine-months ended December 31, 2019, respectively, and \$1.9 million and \$3.6 million during the three and nine-months ended December 31, 2018, respectively, excluding the upfront nonrefundable payment. The Company paid Benitec a total of \$0.4 million and \$2.8 million during the three and nine-months ended December 31, 2019, respectively, and \$1.4 million and \$11.4 million during the three and nine-months ended December 31, 2018, respectively, including the upfront nonrefundable payment.

#### **(C) The University of Massachusetts Medical School Exclusive License Agreement**

In December 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 gene therapy 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 gene therapy product candidates and is required to achieve certain development and commercial milestones in accordance with the timeline set forth in the agreement.

The Company evaluated the UMMS Agreement and 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 statement of operations during the three 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. In February 2019, certain development and regulatory milestones were achieved resulting in a \$1.0 million payment due to UMMS, and in October 2019, further development and regulatory milestones were achieved resulting in an additional \$1.0 million payment due to UMMS. 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 percentage 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.

During the three and nine-months ended December 31, 2019, the Company incurred \$1.8 million and \$4.5 million, respectively, of program-specific costs excluding development and regulatory milestones achieved related to AXO-AAV-GM1 and AXO-AAV-GM2 within research and development expenses in its unaudited condensed consolidated statement of operations. Including the upfront payment and regulatory and development milestones achieved, the Company paid a total of \$1.1 million and \$2.9 million to UMMS during the three and nine-months ended December 31, 2019, respectively, and a total of \$10.0 million during the three and nine-months ended December 31, 2018.

#### **Note 4—Long-term Investment**

On February 13, 2019, ASG entered into a share subscription agreement (the "Subscription Agreement") to purchase up to approximately 8.1 million shares of nonredeemable convertible preferred stock of Arvelle Therapeutics B.V. ("Arvelle") in exchange for €0.00001 per share paid in cash, as well as certain goods and services provided by ASG to Arvelle. The first closing under the Subscription Agreement occurred on February 25, 2019 with ASG purchasing approximately 5.9 million nonredeemable convertible preferred shares of Arvelle, which was initially recorded at a fair value of \$5.9 million and was capitalized as a long-term investment in the Company's consolidated balance sheet and recorded to other non-operating income in the Company's consolidated statement of operations. ASG also received the right to purchase up to approximately 2.2 million additional nonredeemable convertible preferred shares of Arvelle at a price of €0.00001 per share upon a potential future second closing under the Subscription Agreement. The Company accounts for its investment in Arvelle in accordance with the provisions of ASC 321, "Investments - Equity Securities", and elected to use the measurement alternative therein. The investment will be remeasured upon future observable price change(s) in orderly transaction(s) or upon impairment, if any.

On February 13, 2019, ASI and ASG entered into a transition services agreement with two wholly owned subsidiaries of Arvelle, whereby ASI and ASG received reimbursement for any expenses they, or third parties acting on their behalf, incurred on behalf of Arvelle or its subsidiaries (the "Transition Services Agreement"). For any general and administrative and research and development activities performed by its employees, ASI billed the employee compensation expense plus a predetermined mark-up, whereby the Company recorded \$0.2 million to other income in its unaudited condensed consolidated statement of operations during the nine-months ended December 31, 2019 for such costs, inclusive of the mark-up. The Company billed Arvelle for all other charges at cost under the terms of the Transition Services Agreement. The Transition Services Agreement ended in September 2019.

## Note 5—Leases

The Company adopted the provisions of Topic 842 on April 1, 2019 using the effective date as its date of initial application and applied the optional modified retrospective transition method. Comparative periods were not restated. For leases that commenced prior to April 1, 2019, the Company elected the following package of practical expedients when assessing the transition impact: (1) not to reassess whether any expired or existing contracts are or contain leases; (2) not to reassess the lease classification for any expired or existing leases; and (3) not to reassess initial direct costs for any existing leases. The Company also elected to: (1) use the total lease term in its initial incremental borrowing rate calculation; (2) combine its lease and non-lease components and account for them as a single lease component; and (3) not apply the use of hindsight in determining the lease term when considering lessee options to extend or terminate the lease and to purchase the underlying asset. Upon adoption, the Company recorded corresponding aggregate operating lease right-of-use assets and operating lease liabilities of \$3.0 million and \$2.4 million, respectively, including \$0.6 million of prepaid rent previously classified within other non-current assets in the Company's consolidated balance sheet that was reclassified to operating lease right-of-use assets. Operating right-of-use assets and obligations were recognized based on the present value of remaining lease payments over the lease term using an incremental borrowing rate of 12.9%. As the Company's operating leases do not provide an implicit rate, estimated incremental borrowing rates were used based on the information available at the adoption date in determining the present value of lease payments. Operating lease expense is recognized on a straight-line basis over the lease term. Variable lease costs such as common area costs and other operating costs are expensed as incurred. Leases with an initial term of 12 months or less are not recorded within the Company's unaudited condensed balance sheet. In addition, the Company reviews agreements at inception to determine if they include a lease, and when they do, uses its incremental borrowing rate or implicit interest rate to determine the present value of the future lease payments.

The Company leases office facilities in New York, New York, Durham, North Carolina, and Princeton, New Jersey, with corresponding lease terms ending in January 2021, November 2022, and October 2020, respectively, whereby the last payment under the lease agreement for the office facility in New York, New York is due in September 2020. These leases are classified as operating leases in accordance with the provisions of Topic 842. The aggregate weighted-average remaining lease term and aggregate weighted-average discount rate were 1.2 years and 12.9%, respectively, for the Company's contractual rent obligations for operating leases as of December 31, 2019.

In each of the three- and nine-month periods ended December 31, 2019 and 2018, the Company incurred \$0.4 million and \$1.3 million, respectively, in rent expense associated with contractual rent obligations for its operating leases. During the nine months ended December 31, 2019, the Company paid \$1.5 million related to its contractual rent obligations associated with operating lease right-of-use assets. The following table provides a reconciliation of the Company's remaining undiscounted contractual rent obligations due within each respective fiscal year ending March 31 to the operating lease liabilities recognized as of December 31, 2019 (in thousands):

<b>Fiscal Year Ending March 31,</b>	<b>Amount</b>
2020	\$ 306
2021	943
2022	46
2023	31
2024	—
Thereafter	—
Total undiscounted payments	1,326
Less: Present value adjustment	(93)
Present value of future payments	\$ 1,233



**Note 6—Accrued Expenses**

As of December 31, 2019, and March 31, 2019, accrued expenses consisted of the following (in thousands):

	December 31, 2019	March 31, 2019
Research and development expenses	\$ 17,348	\$ 13,416
Salaries, bonuses, and other compensation expenses	1,816	3,899
Legal expenses	363	1,319
Other expenses	807	1,985
Total accrued expenses	<u>\$ 20,334</u>	<u>\$ 20,619</u>

**Note 7—Long-term Debt**

On February 2, 2017, AGT and its subsidiaries, AHL, ASG and ASI, entered into a loan and security agreement (as amended on May 24, 2017, September 22, 2017 and November 27, 2019) (the "Loan Agreement") with Hercules, under which AGT, AHL and ASG (the "Borrowers") borrowed an aggregate of \$55.0 million (the "Term Loan"). Subsequently, ASA was added as a Borrower in July 2017, ATH and ATI were added as Borrowers in April 2018 and RSL was added as a guarantor in November 2019. Pursuant to the Loan Agreement, ASI and RSL have issued guarantees of the Borrowers' obligations under the Loan Agreement, and the Borrowers and ASI have granted Hercules a first position lien on substantially all of their respective assets, excluding intellectual property. The Term Loan initially bore 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%, which was subsequently changed in connection with the November 2019 amendment of the Loan Agreement to a variable per annum rate calculated for any day as the greater of either (i) the prime rate plus 6.80%, and (ii) 11.55%. The Term Loan has a scheduled maturity date of March 1, 2021. The Borrowers were initially 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. Subsequent to the November 2019 amendment of the Loan Agreement, the Borrowers are obligated to make monthly payments of accrued interest from December 1, 2019 until August 1, 2020, followed by monthly installments of principal and interest from September 1, 2020 through March 1, 2021. Prepayment of the Term Loan is subject to penalty. The Loan Agreement with Hercules initially required a covenant that the Borrowers maintain an aggregate minimum cash balance equal to the lesser of \$30.0 million or the outstanding amount due under the Term Loan, which was removed as a result of the November 2019 amendment of the Loan Agreement. The Borrowers prepaid 50%, or approximately \$15.7 million, of outstanding principal due without penalty in connection with the November 2019 amendment of the Loan Agreement, which was accounted for as a modification of debt in accordance with applicable accounting guidance.

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 have the Borrowers been in default under the provisions of the Loan Agreement. In addition, for so long as the Term Loan remains outstanding, the Borrowers shall be required to use their 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 34,260 of the Company's common shares at an exercise price of \$96.32 per share (the "Warrant"). In August 2017, Hercules exercised the Warrant on a cashless basis and received a net issuance of 16,228 of the Company's common shares. The Warrant was accounted for 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 of approximately \$2.3 million was estimated using the Black-Scholes model. In addition, at the closing of the Term Loan, the Company paid transaction costs of \$1.5 million. These amounts were recorded as a discount on the debt and are 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 were as follows (in thousands):

	December 31, 2019	March 31, 2019
Principal amount	\$ 15,733	\$ 45,295
Less: unamortized discount and debt issuance costs	(430)	(1,119)
Loan payable less unamortized discount and debt issuance costs	15,303	44,176
Less: current portion of long-term debt	(8,851)	(21,182)
Long-term loan payable, net of current maturities	<u>\$ 6,452</u>	<u>\$ 22,994</u>

Annual maturities of debt outstanding as of December 31, 2019 are as follows (in thousands):

	Fiscal year ending March 31,
2020	\$ —
2021	15,733
Thereafter	—
Total	<u>\$ 15,733</u>

## Note 8—Related Party Transactions

### (A) Services Agreements:

In October 2014, AGT and ASI entered into a services agreement with Roivant Sciences, Inc. ("RSI"), a wholly owned subsidiary of RSL, under which RSI agreed to provide certain administrative and research and development services to AGT and ASI (the "RSI Services Agreement"). AGT and ASI amended and restated the RSI Services Agreement with RSI on October 13, 2015, effective for the fiscal year commencing April 1, 2015. Under the RSI Services Agreement, as amended and restated, AGT and ASI pay or reimburse RSI for any expenses it, or third parties acting on its behalf, incurs for AGT and ASI. 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 AGT and ASI matters. All other costs are billed back at cost.

In February 2017, AGT and ASI amended and restated the RSI Services Agreement, effective as of December 13, 2016, to add ASG as a services recipient. In addition, in February 2017, ASG entered into a separate services agreement with Roivant Sciences GmbH ("RSG"), a wholly owned subsidiary of RSL, effective as of December 13, 2016 (the "RSG Services Agreement"), 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. In June 2019, both the RSI Services Agreement and the RSG Services Agreement were amended, whereby RSI and RSG, respectively, as service providers, have agreed to indemnify AGT, ASI and ASG and each of their officers, employees and directors against all losses arising out of, due to or in connection with the provision of services (or the failure to provide services) under the applicable services agreement, subject to certain limitations set forth in the applicable services agreement. In addition, AGT, ASI and ASG have agreed to indemnify RSI and RSG, respectively, and their respective affiliates and officers, employees and directors against all losses arising out of, due to or in connection with the receipt of services under the applicable services agreement, subject to certain limitations set forth in the applicable services agreement. Such indemnification obligations will not exceed the payments made by AGT, ASI and ASG under the applicable services agreement for the specific service that allegedly caused or was related to the losses during the period in which such alleged losses were incurred. The term of each of the services agreements will continue until terminated upon 90 days' written notice by any party with respect to the services such party provides or receives thereunder.

Under the RSI Services Agreement, expenses of \$27 thousand and \$103 thousand were incurred during the three and nine-months ended December 31, 2019, respectively, inclusive of the mark-up. Under the RSI Services Agreement and the RSG Services Agreement, expenses of \$0.7 million and \$5.0 million were incurred during the three and nine-months ended December 31, 2018, respectively, inclusive of the mark-up.

**(B) Information Sharing and Cooperation Agreement:**

In March 2015, the Company entered into an information sharing and cooperation agreement (the "Cooperation Agreement") with RSL. The Cooperation Agreement, among other things, grants the Company a right of first review on any potential dementia-related product or investment opportunity that RSL may consider pursuing and obligates the Company to deliver periodic financial statements and other financial information to RSL and comply with other specified financial reporting requirements.

On June 5, 2018, in connection with a share purchase placement agreement with RSL (the "Private Placement"), the Company entered into an amended and restated information sharing and cooperation agreement (the "Restated Cooperation Agreement") with RSL, which became effective as of July 9, 2018, the closing date of the Private Placement. The Restated Cooperation Agreement, among other things: (1) obligates the Company to deliver to RSL periodic financial statements and other information upon reasonable request and to comply with other specified financial reporting requirements; (2) requires the Company to supply certain material information to RSL to assist it in preparing any future SEC filings; and (3) requires the Company to implement and observe certain policies and procedures related to applicable laws and regulations. The Company agreed to indemnify RSL and its affiliates and their respective officers, employees and directors against all losses arising out of, due to or in connection with RSL's status as a shareholder under the Restated Cooperation Agreement and the operations of or services provided by RSL or its affiliates or their respective officers, employees or directors to the Company or any of its subsidiaries, subject to certain limitations set forth in the Restated Cooperation Agreement.

Subject to specified exceptions, the Restated Cooperation Agreement will terminate at such time as RSL is no longer required (a) under U.S. GAAP to consolidate the Company's results of operations and financial position, (b) under U.S. GAAP to account for its investment in the Company under the equity method of accounting, or (c) otherwise to include separate financial statements of the Company in its filings with the SEC pursuant to any SEC rule. In addition, the Cooperation Agreement may be terminated upon mutual written consent of the parties or upon written notice from RSL to the Company in the event of the Company's bankruptcy, liquidation, dissolution or winding-up.

**(C) RSL Financings:**

In July 2018, the Company received \$25.0 million of net proceeds from RSL in exchange for the issuance and sale of 1,785,714 of the Company's common shares to RSL at a purchase price of \$14.00 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 9(B)).

In December 2018, the Company issued and sold 4,145,115 common shares in a follow-on public offering, including 395,115 common shares sold pursuant to the exercise of the underwriters' option to purchase additional shares, at an offering price of \$8.00 per common share for gross proceeds of \$33.2 million, including 1,250,000 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 9(B)).

In March 2019, the Company issued and sold 3,333,333 common shares at an offering price of \$12.00 per common share for gross proceeds of \$40.0 million, including 833,333 shares issued and sold to RSL. The aggregate net proceeds to the Company were approximately \$37.9 million, after deducting underwriting discounts and commissions and offering expenses incurred (see Note 9(B)).

**Note 9—Shareholders' Equity**

**(A) Overview:**

The Company's Memorandum of Association, filed on October 31, 2014 in Bermuda, authorized the issuance of one class of shares. The total number of shares authorized was 1,000,000,000 with a par value per share of \$0.00001 at December 31, 2019. A 1-for-8 reverse share split of the Company's outstanding common stock was effected on May 8, 2019 as approved by the Company's Board of Directors and a majority of its shareholders, which reduced the number of common shares issued and outstanding from approximately 182.2 million to 22.8 million as of May 8, 2019. As such, all references to share and per share amounts in these unaudited condensed consolidated financial statements and accompanying notes have been retroactively restated to reflect the 1-for-8 reverse share split, except for the authorized number of shares of the Company's common stock and the par value per share, which were not affected.

**(B) Transactions:**

During the nine months ended December 31, 2019, expenses of \$103 thousand were incurred by RSI on behalf of the Company that were treated as capital contributions (see Note 8(A)).

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 1,785,714 of its common shares at a purchase price of \$14.00 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 8(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, 2019, 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 4,145,115 common shares in a follow-on public offering, including 395,115 common shares sold pursuant to the exercise of the underwriters' option to purchase additional shares, at an offering price of \$8.00 per common share for gross proceeds of \$33.2 million, including 1,250,000 shares issued and sold to RSL (see Note 8(C)). The aggregate net proceeds to the Company were approximately \$31.6 million, after deducting underwriting discounts and commissions and offering expenses incurred.

In March 2019, the Company issued and sold 3,333,333 common shares, including 833,333 shares issued and sold to RSL, at an offering price of \$12.00 per common share for gross proceeds of \$40.0 million. The aggregate net proceeds to the Company were approximately \$37.9 million, after deducting underwriting discounts and commissions and offering expenses incurred.

**Note 10—Share-Based Compensation**

In March 2015, the Company adopted its 2015 Equity Incentive Plan, which was amended and restated in June 2017 by its Board of Directors and became effective upon shareholder approval in August 2017 (the "2015 Plan"). A 1-for-8 reverse share split of the Company's outstanding common stock was effected on May 8, 2019 as approved by the Company's Board of Directors and a majority of its shareholders. As such, all references to share and per share amounts in these unaudited condensed consolidated financial statements and accompanying notes have been retroactively restated to reflect the 1-for-8 reverse share split, except for the authorized number of shares of the Company's common stock and the par value per share, which were not affected. In April 2019, the number of common shares authorized for issuance under the 2015 Plan increased automatically to approximately 4.0 million common shares in accordance with the terms of the 2015 Plan. Under the 2015 Plan at December 31, 2019, a total of 1.0 million common shares were available for future grant, RSUs underlying approximately 0.3 million common shares were outstanding, and options to purchase approximately 2.5 million common shares were outstanding with a weighted-average exercise price of \$16.53 per share.

**(A) Equity Awards:**

During the nine months ended December 31, 2019 and 2018, the Company granted options to purchase a total of 1.5 million and 0.6 million of its common shares, respectively, with weighted-average exercise prices of \$8.12 and \$16.54, respectively, and estimated grant date fair values of \$7.6 million and \$6.2 million, respectively, under the 2015 Plan. The stock options granted to employees during the nine months ended December 31, 2019 and 2018 include options with market-based performance conditions to purchase 0.4 million and 0.1 million common shares, respectively, with weighted-average exercise prices of \$8.07 and \$20.49 per share, respectively, and corresponding estimated grant date fair values of \$1.2 million and \$1.5 million, respectively, which were estimated using Monte Carlo Simulation methods under the income approach. As of December 31, 2019, options with market-based performance conditions to purchase 0.4 million common shares at a weighted-average exercise price of \$10.77 per share were outstanding. The market-based performance options vest based on the trading price for the Company's common shares exceeding certain closing price thresholds. As of December 31, 2019, stock options with market-based performance conditions to purchase approximately 27 thousand common shares with a weighted-average exercise price of \$11.68 per share were outstanding and vested.

During the nine months ended December 31, 2019, the Company granted RSUs underlying a total of 0.4 million common shares with an aggregate grant date fair value of \$2.7 million to its employees under the 2015 Plan, with one-half of the RSUs granted vesting on January 31, 2020 and the remainder vesting on July 31, 2020.

The Company recorded total share-based compensation expense of \$2.2 million and \$4.2 million for the three months ended December 31, 2019 and 2018, respectively, and \$5.3 million and \$14.4 million for the nine months ended December 31, 2019 and 2018, respectively, related to options and RSUs granted to its employees, directors and consultants. The share-based compensation expense was recorded as research and development and general and administrative expenses in the Company's unaudited condensed consolidated statements of operations. At December 31, 2019, total unrecognized compensation expense related to non-vested outstanding equity awards related to options and RSUs granted to its employees, directors and consultants was \$11.1 million, which is expected to be recognized over the remaining weighted-average service period of 2.34 years.

**(B) Share-Based Compensation for Related Parties:**

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

The Company has incurred 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 has been allocated to the Company by RSL based upon the relative percentage of time utilized by RSL, RSG and RSI employees on Company matters. The Company recorded no share-based compensation expense for the three and nine-months ended December 31, 2019 and share-based compensation expense (benefit) of \$27 thousand and \$(2.7) million for the three and nine-months ended December 31, 2018, respectively, in relation to the RSL common share awards and options issued by RSL to RSL, RSG and RSI employees.

**(2) RSL Common Share Awards and Options:**

Certain employees of the Company have been granted RSL common share awards and options. The Company recorded share-based compensation expense of \$0.1 million and \$0.3 million for the three months ended December 31, 2019 and 2018, respectively, and \$16 thousand and \$1.1 million for the nine months ended December 31, 2019 and 2018, respectively, related to these awards.

**Note 11—Commitments and Contingencies**

As of December 31, 2019, the Company had entered into commitments under the Oxford Agreement (see Note 3), the UMMS Agreement (see Note 3), the Loan Agreement (see Note 7), the services agreements with RSI and RSG (see Note 8(A)), and agreements to rent office space. In addition, the Company has entered into services agreements with third parties for pharmaceutical manufacturing and research activities. See Note 5 for information regarding rent expense incurred, and remaining contractual rent obligations due, in connection with the Company's outstanding contractual rent obligations.

In June 2019, the Company entered into a manufacturing services agreement with a third-party for the manufacture of cGMP grade viral vector, which can be terminated without cause by the Company with six months written notice. Under the terms of this agreement, the Company is committed to pay a minimum of approximately \$1.5 million for manufacturing services through December 2020, which remains outstanding at December 31, 2019.

The Company has the right to terminate the Oxford Agreement at any time upon two months' advance written notice prior to the first commercial sale of a product, or for a specified period of advance written notice after the first commercial sale of a product. Either party may terminate the Oxford Agreement for the other party's uncured material breach or with respect to a failure to make a required payment.

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.

## 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 interim unaudited 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, 2019, included in our Annual Report on Form 10-K, filed with the Securities and Exchange Commission (the "SEC") on June 11, 2019. Unless the context requires otherwise, references in this report to "Axovant", the "Company", "we," "us," and "our" refer to Axovant Gene Therapies 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 throughout 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 our product candidates;
- our relationships under our license agreements;
- 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, as well as subsequent portions or cohorts of our ongoing clinical trials;
- the receipt of approvals or endorsements by data monitoring or other committees necessary for commencement or continuation of 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 executive officers or other 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 therapies that are or may become available; and
- our stated objective of building the world's leading gene therapy company for the treatment of neurological 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.

In addition, statements that "we believe" and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this report, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain, and investors are cautioned not to unduly rely upon these statements as predictions of future events.

### Overview

We are a clinical-stage company focused on developing innovative gene therapies for neurological diseases. We currently have three clinical stage programs: (i) AXO-Lenti-PD for the treatment of Parkinson's disease, which is comprised of the first generation ProSavin® in which 15 patients were previously dosed in a Phase 1/2 study, and the second generation AXO-Lenti-PD in which we have dosed two patients in Cohort 1 of the dose ranging study and are currently dosing Cohort 2 that will enroll four patients; (ii) the AXO-AAV-GM1 program for the treatment of GM1 gangliosidosis in which two patients have been dosed and we expect to continue to dose patients in the study this year; and (iii) the AXO-AAV-GM2 program for the treatment of GM2 gangliosidosis (including Tay-Sachs and Sandhoff diseases) in which two expanded access patients have been dosed and we expect to dose additional patients this year.

We are dedicated to realizing the potential of gene therapies to offer transformative patient outcomes in areas of high unmet medical need. We have assembled a portfolio of gene therapies in partnership with leading scientific institutions and have built a team with extensive experience in the gene therapy space. We will continue to build integrated internal development capabilities from product development through commercialization, with a focus on accelerating the pace of product development in the clinic. As part of our ongoing business strategy we continue to explore potential opportunities to acquire or license new product candidates as well as opportunities for partnership or collaboration on our existing products in development. Our vision is to build the world's leading gene therapy company for the treatment of neurological diseases by progressing our current programs and identifying, developing and commercializing other novel gene therapy treatments for neurological diseases.

### Our Product Pipeline

The following table summarizes the status of our gene therapy development programs. Our wholly owned subsidiary, Axovant Sciences GmbH ("ASG"), holds global commercial rights to each of the following programs:

<b>Gene Therapy Program</b>	<b>Clinical Indication</b>	<b>Clinical Development Stage</b>
<i>AXO-Lenti-PD</i>	Parkinson's disease	Phase 2
<i>AXO-AAV-GM1</i>	GM1 gangliosidosis	Phase 1/2
<i>AXO-AAV-GM2</i>	GM2 gangliosidosis (including Tay-Sachs and Sandhoff diseases)	Phase 1/2

## AXO-Lenti-PD Program

### Overview

AXO-Lenti-PD 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 ProSavin from Oxford BioMedica (UK) Ltd. ("Oxford"), under an exclusive license agreement entered into in June 2018 (the "Oxford Agreement"). Currently, we have six years of data on 15 patients dosed in a Phase 1/2 clinical trial of ProSavin and 12-month data on two patients dosed in Cohort 1 of the Phase 2 clinical trial of the SUNRISE-PD study. We expect initial six-month data from the first two patients dosed in Cohort 2 of the SUNRISE-PD study in the first quarter of calendar year 2020.

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 stereotactic guided 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 previously conducted a Phase 1/2 clinical study with ProSavin. 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 construct relative to ProSavin that has been demonstrated to increase dopamine production in nonclinical studies.

### Parkinson's Disease

Parkinson's disease is a chronic neurodegenerative disorder that primarily results in progressive and debilitating motor symptoms. It is estimated that up to 1,000,000 people in the United States and 7,000,000 to 10,000,000 people worldwide suffer from Parkinson's disease. It typically develops between the ages of 55 and 65 years and affects approximately 1% of people 60 years of age. 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 treatment of Parkinson's disease is currently limited to symptomatic treatments, as no therapies have proven effective in altering the course of the disease or addressing the underlying pathophysiological processes. The mainstay of treatment typically involves the daily administration of oral L-dopa, the precursor to dopamine. While L-dopa is effective in controlling motor symptoms early in the disease, progressive loss of dopaminergic neurons and chronic L-dopa therapy are believed to contribute to the "*wearing off*" of L-dopa's efficacy in the more advanced stages of the disease. Patients become increasingly less responsive to oral L-dopa therapy and require higher doses to manage their symptoms. More advanced Parkinson's disease patients often begin to experience "*on-off*" motor fluctuations, characterized by unpredictable "OFF periods" of reduced mobility and increased rigidity and tremor. In addition, abnormal and involuntary movements known as dyskinesias may occur at higher L-dopa blood levels. Approximately 10% of patients per year develop "*on-off*" motor fluctuations after starting L-dopa therapy.

As Parkinson's disease progresses, other therapies can be 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 periods. 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.



### ***Earlier-Generation Product Candidate: ProSavin (OXB-101)***

ProSavin, the earlier-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 improved motor function 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. 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 ("U.K.") and France on a total of 15 patients with advanced Parkinson's disease. Three dose levels of ProSavin were assessed in four patient cohorts: Low Dose:  $1.9 \times 10^7$  transducing units ("TU") in Cohort 1 (n=3); Medium Dose:  $4.0 \times 10^7$  TU in Cohorts 2a (n=3) and 2b (n=3); High Dose:  $1.0 \times 10^8$  TU in Cohort 3 (n=6). 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 six months after gene therapy administration. No serious adverse events related to ProSavin or the surgical procedure were reported. Reported adverse events 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 adverse events 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 for the patients from the Phase 1/2 study, ProSavin has been observed to show a favorable long-term safety profile and demonstrated positive 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.

### ***Next-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 increase 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. Both ProSavin and AXO-Lenti-PD utilize the same exact fourth generation lentiviral vector. We believe these changes lead to more balanced stoichiometry of gene expression and colocalization of enzymatic activity. The targeted net result is increased 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 positron emission tomography ("PET") scans. Functionally, in non-human primate models at approximately 1/5th of the dose, AXO-Lenti-PD demonstrated a similar level of improvement in spontaneous locomotor activity compared to ProSavin. A recent placebo-controlled study in a non-human primate model of Parkinson's disease published in *Molecular Therapy: Methods and Clinical Development* compared two doses of AXO-Lenti-PD against control-group animals receiving a placebo. The study demonstrated statistically significant differences in Parkinson's disease clinical response scores at six months in this diseased-animal model ( $p < 0.0002$  for AXO-Lenti-PD compared to control), dose-dependent increases in PET signaling using a 6-[(18)F]fluoro-m-tyrosine radiotracer ( $p < 0.001$  for AXO-Lenti-PD compared to control), and dose-dependent increases in gene expression for AADC, TH, and CH1 in transduced striatal tissue. 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.

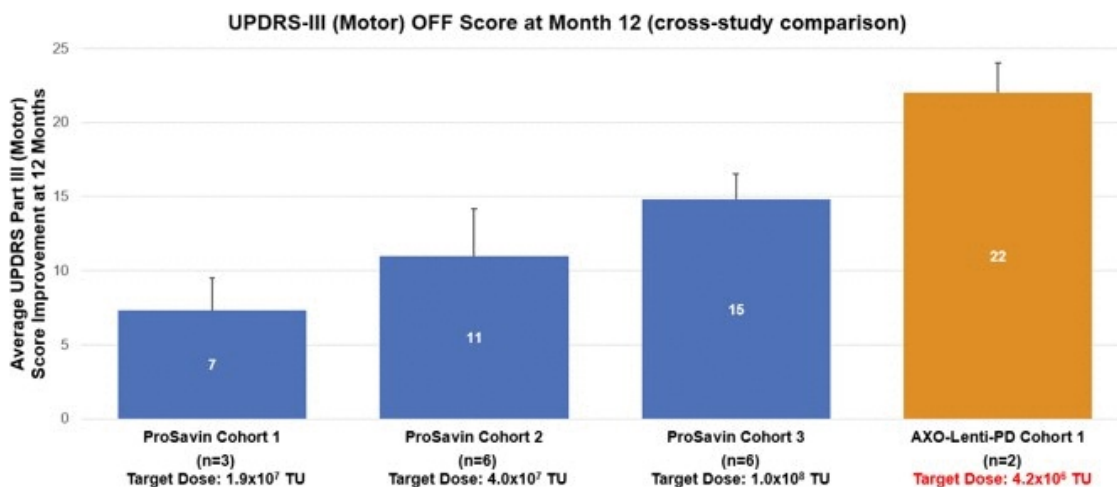
### ***SUNRISE-PD Phase 2 Clinical Trial of AXO-Lenti-PD***

In the fourth quarter of calendar year 2018, we initiated the Phase 2 clinical trial of the SUNRISE-PD study in the U.K. The SUNRISE-PD study is currently enrolling patients in the U.K., and we plan to file an investigational new drug application ("IND") with the FDA to support enrollment of patients in the United States. Additionally, we have filed a Clinical Trial Application to support local enrollment in France.

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. Assuming successful completion of the dose-escalation portion of the study and successful development of a suspension-based manufacturing process, we expect to initiate the randomized, sham-controlled part of the SUNRISE-PD Phase 2 study by the end of the calendar year 2020.

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. We expect the primary endpoint of the double-blind, randomized, sham-controlled portion of the SUNRISE-PD study will be assessed at six months and may include data from Hauser patient diaries and the UPDRS Part III "OFF" scores and other measures being assessed in the study.

In January 2020, we reported 12-month data from Cohort 1 in the open-label, dose-escalation portion of the SUNRISE-PD Phase 2 study. AXO-Lenti-PD was observed to be generally well tolerated, with no serious adverse events attributable to the gene therapy. At month 12, the patients experienced an average improvement from baseline in UPDRS III (motor) score, in the state "OFF" levodopa therapy, of 22 points, representing an average improvement of 37% from baseline. Individual patient improvements from baseline at 12 months of 20 points and 24 points were observed (from 58 to 38 and from 60 to 36, respectively). Previously, at six months post-dosing, these patients demonstrated an average 17-point change from baseline, or 29% improvement, on the same scale. Improvement in the UPDRS Part III "OFF" score in Cohort 1 exhibited evidence of dose response when compared to the low (n="3"), medium (n="6"), and high (n="6") dose cohorts of ProSavin that were previously evaluated in a separate Phase 1/2 study at 12 months, as follows:



These data are based on a cross-trial comparison and not a head-to-head clinical trial. As a result, these data may not be directly comparable.

In addition, the patients experienced an average improvement of approximately 13 points from baseline on the UPDRS Part II (activities of daily living) "OFF" score at 12 months post-dosing, representing an average improvement of 44% from baseline, and an average improvement of 3 points from baseline on the UPDRS Part IV (complications of therapy) "OFF" score at six months post-dosing. The 12-month timepoint is considered an important timeframe for assessment of therapeutic response, differentiation from sham/placebo effect, and durability of gene therapy in Parkinson's disease.

Only one of two patients in Cohort 1 was able to record a Hauser diary. Improvements were observed across various diary measures from baseline to 12 months for the single patient. The Parkinson's Disease Questionnaire-39 score index, a well-validated quality of life measure in Parkinson's disease, demonstrated an average 15-point change from baseline for the patients in Cohort 1, or 30% improvement from baseline to 12 months.

We dosed the first patient in Cohort 2 of the SUNRISE-PD clinical study in April 2019 and expect to continue enrollment of patients during calendar year 2020 with initial six-month data from the first two patients dosed in Cohort 2 expected in the first quarter of calendar year 2020. The dose being tested in Cohort 2 is  $1.4 \times 10^7$  TU, which is three times higher than the dose used in Cohort 1.

## AXO-AAV-GM1 and AXO-AAV-GM2 Programs

### Overview

We are developing AXO-AAV-GM1 and AXO-AAV-GM2 as potential one-time disease-modifying treatments for GM1 and GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease), respectively.

### **GM1 Gangliosidosis and GM2 Gangliosidosis (Including 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 galactosidase beta 1 ("*GLB1*") gene. The *GLB1* gene codes for the  $\beta$ -galactosidase (" $\beta$ -gal") enzyme which catalyzes the hydrolysis of GM1 gangliosides. Impaired  $\beta$ -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. In 2019, we collaborated with the National Institutes of Health ("NIH") to publish on comprehensive retrospective study characterizing the natural history of Type 1 GM1 gangliosidosis in *Molecular Genetics and Metabolism* (Lang et.al., 2019). This paper describes a rapidly progressive clinical course of Type 1 GM1 gangliosidosis, in which almost all patients experience significant multi-organ system dysfunction and neurodevelopmental regression between six and 18 months of age.

GM2 gangliosidosis, also known as Tay-Sachs or Sandhoff diseases, is a rare, inherited neurodegenerative lysosomal storage disorders characterized by buildup of GM2 ganglioside in lysosomes. Defects in the hexosaminidase subunit alpha ("*HEXA*") gene (leading to Tay-Sachs disease) and hexosaminidase subunit beta ("*HEXB*") gene (leading to Sandhoff disease) cause deficiencies in beta-hexosaminidase A ("Hex A") enzyme activity. Hex A enzyme deficiency leads to progressive accumulation of GM2 gangliosides in the central nervous system ("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 either 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 worldwide.

The estimated incidence for the combination of GM1 gangliosidosis, Tay-Sachs and Sandhoff diseases is approximately one in 65,000 live births worldwide. We estimate that there are between approximately 600 and 1,000 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.

### **AXO-AAV-GM1**

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 *GLB1* gene with the goals of restoring  $\beta$ -gal enzyme activity in the 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 the University of Massachusetts Medical School ("UMMS") in December 2018. In November 2019, we announced that the FDA had granted orphan drug designation for AXO-AAV-GM1.

Preclinical studies in GM1 murine and feline models have supported AXO-AAV-GM1's ability to improve  $\beta$ -gal enzyme activity, reduce GM1 ganglioside accumulation, improve neuromuscular function, and extend survival. Magnetic resonance imaging ("MRI") 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 is currently being evaluated with the first patient dosed in May 2019 and a second patient dosed in September 2019 under an IND overseen by the NIH. We presented an update from the first child dosed with AXO-AAV-GM1 in the fourth quarter of calendar year 2019, who was observed to have clinically significant improvements from baseline gene transfer to six month follow-up based on neurological exam, the Vineland-3 scale, Clinical Global Impression assessments, and nutritional status. The Vineland-3 scale is an individually administered measure of adaptive behavior that is widely used to assess individuals with intellectual, developmental, and other disabilities. In addition, AXO-AAV-GM1 was observed to be generally well tolerated with no reports of serious adverse events related to the investigational gene therapy or intravenous administration of the vector. We expect to complete enrollment in Part A of the registrational study of late infantile and juvenile onset (type II) GM1 patients by mid-calendar year 2020 with initial data available during the fourth quarter of calendar year 2020. We also plan to expand the clinical development plan to include infantile GM1 subjects (Type I), the population most severely affected by the disease.

#### ***AXO-AAV-GM2***

AXO-AAV-GM2 is an investigational gene therapy that we are developing 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 dual vectors to deliver functional copies of both the *HEXA* gene and the *HEXB* gene, with the goal of restoring normal Hex A enzyme function in the CNS. AXO-AAV-GM2 is administered directly to the brain and CNS 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. Patients with infantile Tay-Sachs disease, late-infantile or juvenile disease, and adult disease have been shown to have less than 0.1%, approximately 0.5%, and between 2% and 4% of normal Hex A activity, respectively. In addition, patients with infantile Tay-Sachs disease, late-infantile or juvenile disease, and adult disease have a median survival time from birth of three to four, 10 to 15, and over 18 years, respectively. Hex A activity of between 5% and 10% or more of normal is believed to be compatible with a disease-free life. We believe that the restoration of Hex A activity to 0.5% of normal activity could represent a clinically meaningful effect. AXO-AAV-GM2 is currently being evaluated in two patients under an expanded access IND overseen by UMMS. Initial observations suggested that AXO-AAV-GM2 was generally well-tolerated and associated with improvement in bioactivity outcomes. In addition, data suggested attainment of normal neurodevelopmental milestones and improvement in myelination on brain MRI.

We submitted an IND to the FDA in late calendar year 2019 to support the initiation of our registrational clinical trial in patients with GM2 gangliosidosis. Following the review of the IND, the FDA did not raise concerns related to animal toxicology or safety from the investigator-sponsored study. Rather, due to chemistry, manufacturing and controls and device-related questions posed by the FDA, the FDA placed the IND on clinical hold. In January 2020, we provided responses to the questions posed by the FDA. Pending clearance by FDA, we expect to initiate the AXO-AAV-GM2 trial in calendar year 2020.

#### ***Patient #1***

AXO-AAV-GM2 is currently being evaluated with the first patient having been dosed in November 2018 under the expanded access IND overseen by UMMS. In March 2019 and October 2019, the three-month and six-month data were reported from the first patient, a 30-month-old with advanced infantile Tay-Sachs disease, who received a total dose of  $1.0 \times 10^{14}$  vg of AXO-AAV-GM2 administered into the cisterna magna (75% of vector) and lumbar spinal canal (25% of vector) only. Due to the patient's advanced disease, a co-delivered intrathalamic injection of AXO-AAV-GM2 was not administered.

Hex A activity was determined using the 4MUGS assay, the standard assay for assessing activity of the enzyme. At baseline, the patient's enzyme activity in the cerebrospinal fluid ("CSF") was 0.37% of normal. At three months following administration, there was an apparent increase in enzyme activity in the CSF to 1.22% of normal, which was maintained at the six-month timepoint at 1.06% of normal, representing an approximately 3-fold increase in CSF enzyme activity at both three and six-months post-injection. Serum Hex A enzyme activity also increased from baseline (0.26% of normal) at all time points measured (0.42% of normal at three months and 0.28% at six months) following administration of AXO-AAV-GM2. Additionally, the CSF GM2 levels were noted to decrease by about 25% relative to baseline at three months, and then increase by approximately 25% compared to baseline at six months post infusion. The clinical relevance of these changes is unknown.

AXO-AAV-GM2 was observed to be generally well-tolerated with no reports of serious adverse events ("SAE" or "SAEs") related to the investigational gene therapy as of either the three-month or six-month visits. No clinically relevant laboratory abnormalities were observed following administration. Within the safety parameters that were summarized by the investigator was a high CSF pressure (42 cm H<sub>2</sub>O) that was noted to be 21 cm H<sub>2</sub>O after the infusion (normal 18 cm H<sub>2</sub>O). Baseline aspartate transaminase elevation was noted to be well described in patients with Tay-Sachs disease. There was a slight transient rise in liver function tests that were considered related to the oral medications (Sirolimus or Keppra). The SAE of possible aspiration pneumonia was considered unrelated to the investigational therapy by the investigator. From an immunology standpoint, the patient was noted to have achieved adequate immunosuppression with no humoral or T-cell response to the vector. The patient's clinical condition was stable from baseline to month six without clinical deterioration observed on neurological exam. In addition, there was no deterioration in the MRI of the brain from baseline to the MRI at month three post infusion.

#### *Patient #2*

In June 2019, a six month old child with early symptomatic infantile Tay-Sachs disease received AXO-AAV-GM2 prior to the onset of severe symptoms, delivered into the thalamus bilaterally as well as into the cisterna magna and lumbar intrathecal space, the planned routes of administration for patients in the registrational program. The surgical procedure was well tolerated with no neurological defects noted. There were baseline elevated transaminases noted with a transient increase. This child is clinically stable at three months after dosing with no loss or gain of milestones. Importantly, no seizure activity and no exaggerated startle responses were observed. By contrast, the patient's untreated, two older siblings with Tay-Sachs disease exhibited rapid disease progression, clinical regression and seizure onset at 12 to 18 months of age. In addition, a brain MRI taken three months after administration (at 10 months of age) demonstrated no damage to the thalamus and normal new myelin deposition. By contrast, commonly reported MRI findings in infantile Tay-Sachs disease at this age include demyelination and cerebellar atrophy. The CHOP INTEND score, a 16-item scale of motor function that has been validated in infants with neuromuscular disorders, was 58 out of 64 at baseline, increasing to a total score of 60 at month three following gene transfer and ranging between 58-61 from week two to month three.

At baseline, the CSF Hex A enzyme activity was 1.57% of normal and subsequently increased to 1.8% of normal enzyme activity at three months following administration. Serum Hex A enzyme activity also increased from baseline (0.26% of normal) at all time points measured (0.50% of normal at three months) following administration of AXO-AAV-GM2. A sustained level  $\geq 0.5\%$  of normal enzyme activity may correlate with a clinically meaningful effect.

AXO-AAV-GM2 was observed to be generally well-tolerated with no reports of SAEs related to the investigational gene therapy. One SAE was a febrile urinary tract infection due to *Klebsiella* and was considered unrelated to the investigational therapy by the investigator. No clinically relevant laboratory abnormalities were observed following administration, with a transient positive immune response related to the Sirolimus. The patient's clinical condition was stable from baseline to month three without gain or loss of milestones and a stable neurological exam with lower extremity weakness that is unchanged from baseline. In addition, the MRI showed no injury to the thalamus and an increase of myelin that is seen with normal brain development.

## **Our Key Agreements**

### ***Oxford BioMedica License Agreement***

In June 2018, we, through our wholly owned subsidiary, ASG, entered into the Oxford Agreement, pursuant to which we received a worldwide, exclusive, royalty-bearing, sub-licensable license under certain patents and other intellectual property controlled by Oxford 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 of \$30.0 million, \$5.0 million of which was applied as a credit against the process development work and clinical supply that Oxford is obligated to provide to us over the term of the Oxford Agreement. Under the terms of the Oxford Agreement, we could be obligated to make payments to Oxford 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 April 2019, certain development milestones were achieved resulting in a \$13.0 million net payment due to Oxford. We will also be obligated to pay Oxford 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 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 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 certain fees, which range from \$0.5 million to \$1.0 million. Pursuant to the Oxford Agreement, Oxford is expected to be our cGMP manufacturer for AXO-Lenti-PD, subject to a separate clinical and commercial supply agreement to be negotiated between the parties. We have the right to terminate the Oxford Agreement at any time upon two months' advance written notice prior to the first commercial sale of a product, or for a specified period of advance written notice after the first commercial sale of a product. Either party may terminate the Oxford Agreement for the other party's uncured material breach or with respect to a failure to make a required payment.

#### ***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. In February 2019, certain development and regulatory milestones were achieved resulting in a \$1.0 million payment to UMMS, and in October 2019, further development and regulatory milestones were achieved resulting in an additional \$1.0 million payment due to UMMS. 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.

#### **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 gene therapy 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, and acquiring, 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 and milestone payments, which include costs incurred under our agreements with Oxford and UMMS, as well as costs incurred for our discontinued AXO-AAV-OPMD, intepirdine and nelotanserin programs.

Unallocated internal costs include:

- share-based compensation expense for research and development personnel, including expense related to common share awards and option awards issued by our parent company, Roivant Sciences Ltd. ("RSL"), to its employees and employees of its wholly owned subsidiaries, Roivant Sciences, Inc. ("RSI") and Roivant Sciences GmbH ("RSG"), as well as to certain of our 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 our current gene therapy product candidate programs and additional product candidates we may in-license or acquire as we pursue our updated business plan. For the three and nine-months ended December 31, 2019, the majority of our research and development expenses were associated with our gene therapy product candidates. For the three months ended December 31, 2018, the majority of our research and development expenses were associated with our gene therapy product candidates, including an upfront nonrefundable payment to UMMS under the UMMS Agreement. For the nine months ended December 31, 2018, the majority of our research and development expenses were associated with our gene therapy product candidates, including upfront nonrefundable payments to Oxford under the Oxford Agreement, to UMMS under the UMMS Agreement, and to Benitec Biopharma Limited ("Benitec") under a license and collaboration agreement to develop and commercialize investigational gene therapy AXO-AAV-OPMD and related gene therapy products, which was terminated effective September 3, 2019.

Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials.

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

- the number of trials required for approval;
- the per patient trial costs;
- the number of patients who participate in the trials;
- the number of sites included in the trials;
- the countries in which the trials are conducted;
- the length of time required to enroll eligible patients;
- the 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 gene therapy 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 gene therapy 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, and employee-related expenses, such as salaries, benefits and travel expenses for general and administrative personnel. General and administrative expenses also include fees for services received by ASG under the services agreements with RSI and RSG.

We anticipate that our general and administrative expenses will continue to decrease during the fiscal years ending March 31, 2020 and 2021, although at a lower rate than in the periods presented in this Quarterly Report on Form 10-Q, primarily as the result of further reductions expected in overhead-related expenses.

#### **Results of Operations for the Three and Nine-Months Ended December 31, 2019 and 2018**

The following table summarizes our results of operations for the three and nine-months ended December 31, 2019 and 2018 (in thousands):

	Three Months Ended December 31,			Nine Months Ended December 31,		
	2019	2018	Change	2019	2018	Change
Operating expenses:						
Research and development expenses						
(includes share-based compensation expense of \$876 and \$1,910 for the three months ended December 31, 2019 and 2018 and \$2,006 and \$3,299 for the nine months ended December 31, 2019 and 2018, respectively)	\$ 8,267	\$ 21,483	\$ (13,216)	\$ 36,190	\$ 80,403	\$ (44,213)
General and administrative expenses						
(includes share-based compensation expense of \$1,436 and \$2,648 for the three months ended December 31, 2019 and 2018 and \$3,332 and \$9,575 for the nine months ended December 31, 2019 and 2018, respectively)	5,409	10,933	(5,524)	16,928	33,309	(16,381)
Total operating expenses	13,676	32,416	(18,740)	53,118	113,712	(60,594)
Interest expense	1,066	1,906	(840)	3,937	5,808	(1,871)
Other (income) expense	(694)	(78)	(616)	(1,231)	275	(1,506)
Loss before income tax expense	(14,048)	(34,244)	20,196	(55,824)	(119,795)	63,971
Income tax expense (benefit)	(9)	52	(61)	156	224	(68)
Net loss	\$ (14,039)	\$ (34,296)	\$ 20,257	\$ (55,980)	\$ (120,019)	\$ 64,039

### Research and Development Expenses

Our research and development expenses during the three and nine-months ended December 31, 2019 and 2018 consisted of the following (in thousands):

	Three Months Ended December 31,			Nine Months Ended December 31,		
	2019	2018	Change	2019	2018	Change
<i>Program-specific costs:</i>						
AXO-Lenti-PD	\$ 2,417	\$ 1,881	\$ 536	\$ 19,540	\$ 28,211	\$ (8,671)
AXO-AAV-OPMD	(321)	1,858	(2,179)	1,822	13,605	(11,783)
AXO-AAV-GM1 and AXO-AAV-GM2	2,831	10,024	(7,193)	5,533	10,024	(4,491)
Intepirdine	17	(207)	224	(940)	2,054	(2,994)
Nelotanserin	39	1,334	(1,295)	251	8,894	(8,643)
<i>Unallocated internal costs:</i>						
Share-based compensation expense	876	1,910	(1,034)	2,006	3,299	(1,293)
Personnel-related	1,610	3,095	(1,485)	5,294	7,512	(2,218)
Services agreements	—	—	—	—	2,352	(2,352)
Other	798	1,588	(790)	2,684	4,452	(1,768)
<b>Total research and development expenses</b>	<b>\$ 8,267</b>	<b>\$ 21,483</b>	<b>\$ (13,216)</b>	<b>\$ 36,190</b>	<b>\$ 80,403</b>	<b>\$ (44,213)</b>

Research and development expenses were \$8.3 million for the three months ended December 31, 2019 compared to \$21.5 million for the three months ended December 31, 2018. Research and development expenses decreased by \$4.2 million in the current year period, after excluding the upfront license fee of \$10.0 million paid to UMMS in the prior year period and \$1.0 million due to UMMS for development and regulatory milestones achieved in the current year period. While program-specific costs increased during the period for the programs currently under development, they were offset by decreases related to the discontinuation of our legacy AXO-AAV-OPMD and nelotanserin programs, as well as decreases in share-based compensation and personnel-related costs primarily associated with a decrease in headcount.

Research and development expenses were \$36.2 million for the nine months ended December 31, 2019 compared to \$80.4 million for the nine months ended December 31, 2018. Research and development expenses decreased by \$13.2 million in the current year period, after excluding a net amount of \$13.0 million due to Oxford for a development milestone achieved and \$1.0 million due to UMMS for development and regulatory milestones achieved in the current year period, as well as the upfront license fees of \$25.0 million paid to Oxford, \$10.0 million paid to Benitec and \$10.0 million paid to UMMS in the prior year period. While program-specific costs increased during the period for the programs currently under development, they were offset by decreases related to the discontinuation of our legacy intepirdine, nelotanserin and AXO-AAV-OPMD programs, as well as decreases in share-based compensation and personnel-related costs primarily associated with a decrease in headcount, in addition to costs allocated under our services agreements with RSI and RSG as a result of the decentralization of the services provided to us.

### General and Administrative Expenses

General and administrative expenses were \$5.4 million for the three months ended December 31, 2019 compared to \$10.9 million for the three months ended December 31, 2018, with the decrease of \$5.5 million primarily related to reductions in (i) personnel costs of \$1.7 million and share-based compensation expense of \$1.2 million attributable to reduced headcount, (ii) legal, accounting and other administrative professional fees of \$1.6 million, and (iii) costs allocated under our services agreements with RSI and RSG of \$0.6 million as a result of the decentralization of the services provided to us. Going forward, the costs allocated to us under our services agreements with RSI and RSG are expected to continue to be insignificant.

General and administrative expenses were \$16.9 million for the nine months ended December 31, 2019 compared to \$33.3 million for the nine months ended December 31, 2018, with the decrease of \$16.4 million primarily related to reductions in (i) share-based compensation expense of \$6.2 million and personnel costs of \$1.5 million attributable to reduced headcount, (ii) legal, accounting and other administrative professional fees of \$4.7 million, and (iii) costs allocated under our services agreements with RSI and RSG of \$2.5 million as a result of the decentralization of the services provided to us.

### ***Interest Expense***

Interest expense was \$1.1 million and \$3.9 million for the three and nine-months ended December 31, 2019, respectively, and \$1.9 million and \$5.8 million for the three and nine-months ended December 31, 2018, respectively, which consisted of interest paid and the amortization of debt discount related to our loan and security agreement (the "Loan Agreement") with Hercules Capital, Inc. ("Hercules"), whereby we prepaid approximately \$15.7 million of outstanding principal due without penalty in connection with the November 2019 amendment of the Loan Agreement. See "—Liquidity and Capital Resources—Loan and Security Agreement with Hercules Capital, Inc."

### **Liquidity and Capital Resources**

#### ***Sources of Liquidity***

Since our initial public offering in June 2015, our operations have been financed primarily through sales of common shares and borrowings under our credit facilities. As of December 31, 2019, we had total cash and cash equivalents of \$27.8 million.

#### ***Capital Requirements***

We are currently in the clinical stage of operations and have not yet achieved profitability. We expect to continue to incur significant operating and net losses, as well as negative cash flows from operations, for the foreseeable future as we continue to develop our gene therapy product candidates and prepares for potential future regulatory approvals and commercialization of our products. We have not generated any revenue to date and do not expect to generate product revenue unless and until we successfully complete development and obtain regulatory approval for at least one of our gene therapy product candidates. Our current cash and cash equivalents balance will also not be sufficient to complete all necessary development activities and commercially launch our products.

We expect to spend substantial amounts to complete the development of, seek regulatory approvals for and commercialize our gene therapy product candidates. We will require cash to pay principal and interest on our term loan with Hercules, whereby a \$15.7 million aggregate principal amount remains outstanding as of December 31, 2019 and \$0.3 million in interest payments are scheduled to be made through March 31, 2020 in accordance with the current terms of our Loan Agreement with Hercules. The principal amount outstanding at March 31, 2020 under our term loan with Hercules will be \$15.7 million unless we prepay some or all of the principal amount. In addition, as part of our business development strategy, we generally structure our license agreements and collaboration agreements so that a significant portion of the total license cost is contingent upon the successful achievement of specified development, regulatory or commercial milestones. As a result, we will require cash to make payments upon achievement of these milestones under these agreements. Based on our anticipated timeline for the achievement of development, regulatory and commercial milestones, we expect we will make total milestone payments under our license agreements and collaboration agreements of approximately \$7.0 million from January 1, 2020 through March 31, 2020. To the extent we achieve additional development, regulatory and commercial milestones through March 31, 2020, we may pay additional milestone payments under our license agreements and collaboration agreements.

Because the length of time and activities associated with successful development of our gene therapy 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 gene therapy product candidates;
- the outcome, timing and cost of meeting regulatory requirements established by the FDA, the European Medicines Agency, or Japan's Pharmaceutical and Medical Devices Agency, 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 gene therapy product candidates or any future gene therapy product candidates;
- the effect of competing technological and market developments;
- the cost and timing of completion of clinical-stage and commercial-scale manufacturing activities;

- the cost of establishing sales, marketing and distribution capabilities for our gene therapy 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 gene therapy product candidates, if approved for commercial sale.

As of December 31, 2019, our cash and cash equivalents totaled \$27.8 million and our accumulated deficit was \$742.0 million. For the nine months ended December 31, 2019 and the fiscal year ended March 31, 2019, we incurred net losses of \$56.0 million and \$129.1 million, respectively. As of December 31, 2019, we had aggregate net interest-bearing indebtedness of \$15.3 million, of which \$8.9 million was due within one year. We anticipate that our current cash and cash equivalents balance will not be sufficient to sustain operations beyond three months following the date that the accompanying unaudited condensed consolidated financial statements and notes were issued, which raises substantial doubt about our ability to continue as a going concern.

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

In addition, if we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds 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 prefer to develop and market ourselves.

To continue as a going concern, we will need, among other things, to raise additional capital in the near term. We continually assess multiple options to obtain additional funding to support our operations, including proceeds from offerings of our equity securities or debt, or transactions involving product development, technology licensing or collaboration arrangements, or other sources of capital to complete our currently planned development programs. Sources of a sufficient amount of financing may not be available to us on favorable terms, if at all, and due to these uncertainties, there is substantial doubt about our ability to continue as a going concern.

#### ***At-the-Market Equity Offering Program***

In December 2016, we filed a registration statement with the SEC for the offer and sale from time to time of up to \$750.0 million of any combination of registered common shares, preferred shares, debt securities, and warrants.

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, 2019, approximately \$74.9 million of our common shares remained available for sale under the sales agreement.

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

On February 2, 2017, we and our wholly owned subsidiaries, Axovant Holdings Ltd. ("AHL"), ASG and Axovant Sciences, Inc. ("ASI"), entered into the Loan Agreement with Hercules as agent and lender, which was amended on May 24, 2017, September 22, 2017 and November 27, 2019. Pursuant to the Loan Agreement, we, AHL and ASG (collectively, the "Borrowers"), borrowed an aggregate of \$55.0 million (the "Term Loan"). ASI issued a guarantee 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, our subsidiaries Axovant Treasury Holdings, Inc. and Axovant Treasury, Inc. as Borrowers in April 2018, and RSL as a guarantor in November 2019. Pursuant to the Loan Agreement, ASI and RSL have issued guarantees of the Borrowers' obligations under the Loan Agreement, and the Borrowers and ASI have granted Hercules a first position lien on substantially all of their respective assets, excluding intellectual property. The Term Loan initially bore 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%, which was subsequently changed as a result of the November 2019 amendment of the Loan Agreement to a variable per annum rate calculated for any day as the greater of either (i) the prime rate plus 6.80%, and (ii) 11.55%. The Term Loan has a scheduled maturity date of March 1, 2021. We were initially 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. Subsequent to the November 2019 amendment of the Loan Agreement, we are obligated to make monthly payments of accrued interest from December 1, 2019 until August 1, 2020, followed by monthly installments of principal and interest from September 1, 2020 through March 1, 2021. If we prepay the loan prior to March 1, 2021, we will be obligated to pay Hercules a prepayment charge of 1.0% of the then-outstanding principal balance. The Loan Agreement with Hercules initially required a covenant that we maintain an aggregate minimum cash balance equal to the lesser of \$30.0 million or the outstanding amount due under the Term Loan, which was removed as a result of the November 2019 amendment of the Loan Agreement. We prepaid 50%, or approximately \$15.7 million, of outstanding principal due without penalty in connection with the November 2019 amendment of the Loan Agreement, which was accounted for as a modification of debt in accordance with applicable accounting guidance.

The Loan Agreement currently includes customary affirmative and restrictive covenants and representations and warranties, including 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. 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 4,260 of our common shares at an exercise price of \$96.32 per share. In August 2017, Hercules exercised the warrant on a cashless basis and received a net issuance of 16,228 of our common shares.

### *Cash Flows*

The following table sets forth a summary of our cash flows for the nine months ended December 31, 2019 and 2018 (in thousands):

	Nine Months Ended December 31,	
	2019	2018
Net cash used in operating activities	\$ (49,563)	\$ (122,624)
Net cash used in investing activities	(250)	(74)
Net cash (used in) provided by financing activities	(29,374)	53,300

### *Operating Activities*

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

For the nine months ended December 31, 2019, net cash used in operating activities was \$49.6 million and was primarily attributable to a net loss of \$56.0 million that includes costs incurred for research and development activities, including CRO fees, manufacturing, regulatory and other clinical trial costs, as well as our general and administrative expenses, which was partially offset by \$5.3 million of non-cash share-based compensation expense.

For the nine months ended December 31, 2018, net cash used in operating activities was \$122.6 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, as well as our general and administrative expenses, as well as a net decrease of \$9.0 million in accrued expenses, a net 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.

#### *Investing Activities*

Cash used in investing activities was \$250 thousand and \$74 thousand for the nine months ended December 31, 2019 and 2018, respectively, and related to purchases of software, equipment and computers.

#### *Financing Activities*

For the nine months ended December 31, 2019, net cash used in financing activities was approximately \$29.4 million and consisted primarily of principal payments made on long-term debt. For the nine months ended December 31, 2018, net cash provided by financing activities was approximately \$53.3 million and consisted primarily 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 sales agreement with Cowen, which was partially offset by \$4.8 million of principal payments made on long-term debt.

#### **Contractual Obligations**

Our contractual obligations did not materially change during the nine months ended December 31, 2019 as compared to those disclosed in our Annual Report on Form 10-K for the year ended March 31, 2019, except that in November 2019, we amended our Loan Agreement with Hercules, and June 2019, we entered into a manufacturing services agreement with a third-party for the manufacture of cGMP grade viral vector, which can be terminated without cause by us with six months written notice. Under the terms of this manufacturing services agreement, we are committed to pay a minimum of approximately \$1.5 million for manufacturing services through December 2020, which remains outstanding at December 31, 2019.

The following table provides information with respect to our outstanding debt obligations as of December 31, 2019 (in thousands):

	<b>Total</b>	<b>Under 1 year</b>	<b>1-3 years</b>	<b>3-5 years</b>	<b>Over 5 years</b>
Debt obligations, including interest <sup>(1)</sup>	\$ 17,428	\$ 10,413	\$ 7,015	\$ —	\$ —

<sup>(1)</sup> Amounts estimated using interest rate in effect at December 31, 2019. Debt obligations are described below under "—Debt Obligations."

#### *Debt Obligations*

Debt obligations reflect our obligations to pay interest-only on the outstanding principal amount of \$15.7 million as of December 31, 2019 until August 1, 2020 under the Loan Agreement with Hercules, and to make periodic interest payments and principal repayments from September 1, 2020 through maturity on March 1, 2021. Our debt obligation under the Hercules Loan Agreement 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) 11.55%. The related interest on the aggregate principal amounts outstanding to Hercules included in the above table was estimated using the interest rate in effect as of December 31, 2019. See Note 7, "Long-term Debt," in the accompanying unaudited interim consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q for further discussion of the Loan Agreement with Hercules.

#### **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.

### **Critical Accounting Policies and Significant Judgments and Estimates**

Our management's discussion and analysis of our financial condition and results of operations is based on our unaudited condensed consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles ("GAAP"). The preparation of these unaudited condensed consolidated financial statements and accompanying notes 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 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 stock option awards. 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.

Our significant accounting policies are more fully described in Note 2, "Summary of Significant Accounting Policies," to our unaudited condensed consolidated financial statements in this Quarterly Report on Form 10-Q and in Note 2, "Summary of Significant Accounting Policies," to our audited consolidated financial statements in our Annual Report. Not all of these significant accounting policies, however, require that we make estimates and assumptions that we believe are "critical accounting estimates." We believe that our estimates relating to share-based compensation, research and development accruals, income taxes and our ability to continue as a going concern have the greatest potential impact on our consolidated financial statements and consider these to be our critical accounting policies and estimates and are "critical accounting estimates." 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 for our adoption of Financial Accounting Standards Board Accounting Standards Codification Topic 842, "*Leases (Topic 842)*." See "Note 2(F)—Recent Accounting Pronouncements" in the accompanying notes to the unaudited condensed consolidated financial statements included in "Item 1—Financial Statements" of this Quarterly Report on Form 10-Q for additional information.

### **Recent Accounting Pronouncements**

For a discussion of recent accounting pronouncements, see "Note 2(F)—Recent Accounting Pronouncements" in the accompanying notes to the unaudited condensed consolidated financial statements included in "Item 1—Financial Statements" of this Quarterly Report on Form 10-Q for additional information.

### **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, 2019, we had cash and cash equivalents of \$27.8 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 \$0.2 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, 2019, 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 Securities 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, 2019, at the reasonable assurance level.

##### **Changes in Internal Control over Financial Reporting**

There was no change in our internal control over financial reporting (as defined in Rules 13a-15(d) and 15d-15(d) under 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 Gene Therapies 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. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties of which we are unaware, or that we currently believe are not material, may also become important factors that adversely affect our business. 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. 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 clinical-stage or 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, the failure of our clinical trials for, and the discontinuation of development of, intepirdine and 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 gene therapy product candidates, which remain in early stages of clinical development.

Our ability to generate revenue and become profitable depends upon our ability to successfully complete the development of our product candidates 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 gene therapy product candidates;
- establish effective sales, marketing and distribution systems for our gene therapy product candidates;
- add operational, financial and management information systems and personnel, including personnel to support our clinical, manufacturing and planned future commercialization efforts and operations as a public company;
- initiate and continue relationships with third-party suppliers and manufacturers, including Oxford BioMedica (UK) Ltd. ("Oxford"), Nationwide Children's Hospital, Yposkesi and other third-party cGMP manufacturers, and have clinical and commercial quantities of our gene therapy product candidates manufactured at acceptable cost and quality levels;
- attract and retain an experienced management and advisory team;
- raise additional funds when needed and on terms acceptable to us;
- achieve broad market acceptance of our products in the medical community and with third-party payors and consumers;
- launch commercial sales of our products, whether alone or in collaboration with others;
- compete effectively with other biotechnology and gene therapy companies targeting neurological diseases; and
- obtain, maintain, expand and protect necessary intellectual property rights.

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 United States Food and Drug Administration ("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 gene therapy product candidates. 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 June 2018, we announced that we received from Oxford a worldwide exclusive license to develop and commercialize AXO-Lenti-PD and its predecessor product candidate ProSavin® and related gene therapy products (the "Oxford Agreement"). In July 2018, we announced that we received from Benitec Biopharma Limited ("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 the University of Massachusetts Medical School ("UMMS") a worldwide exclusive license to develop and commercialize gene therapy product candidates AXO-AAV-GM1 and AXO-AAV-GM2 (the "UMMS Agreement"). We are pursuing a strategy to leverage our clinical experience and expertise for the clinical development and regulatory approval of our gene therapy product candidates. As part of our ongoing business strategy, we continue to explore potential opportunities to acquire or license new product candidates and to collaborate on our existing products in development.

We cannot be certain that our newly licensed product candidates will be successfully developed, or that the early clinical trial results of these product candidates will be predictive of future clinical trial results. We may determine at any time that one or more of our in-licensed product candidates is not suitable for continued development due to cost, feasibility of obtaining regulatory approvals or any other reason, and may terminate the related license. For example, in June 2019, we decided to terminate the Benitec Agreement following our decision to no longer pursue development of AXO-AAV-OPMD and related gene therapy product candidates. In addition, we have limited data from small, uncontrolled clinical trials, performed by or on behalf of Oxford, 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.

This business plan requires us to be successful in a number of challenging, uncertain and risky activities, including pursuing development of our newly licensed gene therapy product candidates in indications for which we have limited or no human clinical data, designing and executing a nonclinical and/or clinical development program for our newly licensed product candidates, 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 identifying potential partners to collaborate on our products. 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, partnerships 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 our product candidates 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 our product candidates and the outcome of our ongoing business development efforts and pipeline expansion activities are highly uncertain.

We expect to continue to reassess and make changes to our existing development programs and pipeline expansion strategy. Our plans for our development programs may be affected by the results of competitors' clinical trials of product candidates addressing our current target indications, and 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 to incur significant losses for the foreseeable future and may never achieve or maintain profitability. Our independent registered public accounting firm has expressed substantial doubt about our ability to continue as a going concern.***

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. 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 newly licensed 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 our gene therapy product candidates. 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 operating losses and negative cash flows from operations for the foreseeable future. These losses have had and will continue to have an adverse effect on our financial position and working capital.

Our independent registered public accounting firm has issued a going concern opinion on our consolidated financial statements for the one-year period following the date that our consolidated financial statements for the year ended March 31, 2019 were issued, which have been prepared assuming that we will continue as a going concern. We have not made 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 our continuing as a going concern. We will need to raise additional capital when needed or adjust our operational plans to continue as a going concern. We continually assess multiple options to obtain additional funding to support our operations, including proceeds from offerings of our equity securities or debt, or transactions involving product development, technology licensing or collaboration arrangements, or other sources of capital to complete our currently planned development programs. Sources of a sufficient amount of financing may not be available to us on favorable terms, if at all.

***We may not be successful in our efforts to identify and acquire additional gene therapy product candidates, or to enter into collaborations or strategic alliances for the development and commercialization of any such future 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:

- pre-clinical and early clinical results of any product candidates we acquire may not be predictive of future clinical results;
- 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 could adversely impact our business strategy, our financial position and share price.

We may also decide to collaborate with other pharmaceutical companies for the development and potential commercialization of our product candidates in the United States or other countries or territories of the world. We will face significant competition in seeking appropriate collaborators. We may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for our product candidates because they may be deemed to be at too early of a stage of development for collaborative effort and third parties may not view our product candidates as having the requisite potential to demonstrate safety and efficacy. If and when we collaborate with a third party for development and commercialization of a product candidate, we can expect to relinquish some or all of the control over the future success of that product candidate to the third party. Our ability to reach a definitive agreement for a collaboration 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 a number of factors.

***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 our newly licensed gene therapy product candidates, 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 BLA or other key regulatory filings may be delayed or rejected due to issues, including those related to product quality and manufacturing, timing of results from supporting studies, database lock and data transfer;
- the regulatory authorities may require additional nonclinical studies or clinical 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 contract research organizations ("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;
- 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.

We submitted an Investigational New Drug application ("IND") in late 2019 to support the initiation of a company-sponsored clinical trial of AXO-AAV-GM2 for the treatment of patients with GM2 gangliosidosis. Following its review of the IND, while the FDA had no concerns over animal toxicology or clinical safety in the AXO-AAV-GM2 program, the FDA placed the IND on clinical hold pending review of our responses to CMC and device-related questions. We are working with the FDA to allow for the clinical hold to be lifted. However, there can be no assurance that this clinical hold will be lifted in the near term or at all. If the FDA does not lift the clinical hold in part or in full, our development of AXO-AAV-GM2 may be delayed or discontinued, which would adversely affect our business, financial condition and results of operations.

***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 2017, September 2017 and November 2019 (as amended, the "Loan Agreement"), with Hercules Capital, Inc. ("Hercules"). The Loan Agreement is guaranteed by our parent company, Roivant Sciences Ltd. ("RSL") and 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, currently including 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.

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 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 are currently in the clinical stage of operations and have not yet achieved profitability. We expect to continue to incur significant operating and net losses, as well as negative cash flows from operations, for the foreseeable future as we continue to develop our gene therapy product candidates and prepare for potential future regulatory approvals and commercialization of our products. We have not generated any revenue to date and do not expect to generate product revenue unless and until we successfully complete development and obtain regulatory approval for at least one of our gene therapy product candidates. Our current cash and cash equivalents balance will also not be sufficient to complete all necessary development activities and commercially launch our products.

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 clinical-stage and 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, 2019, our cash and cash equivalents totaled \$27.8 million and our accumulated deficit was \$742.0 million. For the nine months ended December 31, 2019 and the fiscal year ended March 31, 2019, we incurred net losses of \$56.0 million and \$129.1 million, respectively. As of December 31, 2019, we had aggregate net interest-bearing indebtedness of \$15.3 million, of which \$8.9 million was due within one year. We anticipate that our current cash and cash equivalents balance will not be sufficient to sustain operations beyond three months following the date that these unaudited condensed consolidated financial statements and notes were issued, which raises substantial doubt about our ability to continue as a going concern.

To continue as a going concern, we will need, among other things, to raise additional capital in the near term. We continually assess multiple options to obtain additional funding to support our operations, including proceeds from offerings of our equity securities or debt, or transactions involving product development, technology licensing or collaboration arrangements, or other sources of capital to complete our currently planned development programs. Sources of a sufficient amount of financing may not be available to us on favorable terms, if at all, and due to these uncertainties, there is substantial doubt about our ability to continue as going concern.

***Raising additional funds by issuing securities may cause dilution to existing shareholders, raising additional funds through debt financings may involve additional restrictive covenants, 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 U.S. Securities and Exchange Commission (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 may be required to make significant payments to third parties under the agreements pursuant to which we acquired our gene therapy product candidates.***

Under our agreements with Oxford and UMMS, 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. Some of these milestones require us to make payments prior to generating any product sales. We may not have sufficient funds available to meet our obligations at such time as any of these payments become due, in which case our development efforts would be 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 harm our operations.

***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, 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, and the cost of living in the New York City area. If we are not able to attract and retain necessary personnel to accomplish our business objectives, we may experience constraints that will 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 us. 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.

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, our ability to effectively manage any future growth and our business will be limited.



***Our employees, independent contractors, principal investigators, consultants, commercial collaborators, manufacturers, 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, manufacturers, 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.

***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, or may rely on in the future, including our CRO's 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 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, suffer reputational damage, and the further development of our product candidates could be delayed.

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

Our business strategy includes establishing and maintaining operations and certain key third party arrangements in various jurisdictions around the world. 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;

- unexpected changes in tariffs or trade barriers;
- complexities associated with managing multiple payor-reimbursement regimes or self-pay systems;
- financial risks, such as longer payment cycles, difficulty enforcing contracts and collecting accounts receivable and exposure to foreign currency exchange rate fluctuations;
- reduced protection for intellectual property rights;
- reduced protection of contractual rights in the event of bankruptcy or insolvency of the other contracting party;
- natural disasters, political and economic instability, including wars, terrorism and political unrest, outbreak of disease, boycotts, curtailment of trade and other business restrictions;
- 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 current or future international 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 ("U.K.") from the European Union ("EU") are a source of instability and uncertainty.***

On June 23, 2016, the U.K. held a referendum in which a majority of the eligible members of the electorate voted for the U.K. to leave the EU. The U.K.'s withdrawal from the EU is commonly referred to as "Brexit". The U.K. and the EU have agreed to a withdrawal agreement (the "Withdrawal Agreement"), which was approved by the U.K. Parliament on January 24, 2020. Under the Withdrawal Agreement, the U.K. is subject to a transition period until December 31, 2020 (the "Transition Period"), during which EU rules will continue to apply. Negotiations between the U.K. and the EU are expected to continue in relation to the customs and trading relationship between the U.K. and the EU following the expiration of the Transition Period.

The uncertainty concerning the U.K.'s legal, political and economic relationship with the EU after the Transition Period may be a source of instability in the international markets, create significant currency fluctuations, and/or otherwise adversely affect trading agreements or similar cross-border cooperation arrangements (whether economic, tax, fiscal, legal, regulatory or otherwise). These developments, or the perception that any of them could occur, have had, and may continue to have, a significant negative impact on global economic conditions and the stability of global financial markets, and could significantly reduce global market liquidity and limit the ability of key market participants to operate in certain financial markets. In particular, it could also lead to a period of considerable uncertainty in relation to the regulatory process in Europe.

If the U.K. and the EU are unable to negotiate acceptable trading and customs terms or if other EU member states pursue withdrawal, barrier-free access between the U.K. 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 U.K. and the EU and, in particular, any arrangements for the U.K. to retain access to EU markets after the Transition Period. Such a withdrawal from the EU is unprecedented, and it is unclear how the U.K.'s access to the European single market for goods, capital, services and labor within the EU, or 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 as a result of Brexit that could have an adverse effect on our operations and development programs. There may continue to be economic uncertainty surrounding the consequences of Brexit which could negatively impact our financial condition, results of operations and cash flows.

***Brexit may adversely impact our ability to obtain regulatory approvals of our product candidates in the U.K. or the EU and may require us to incur additional expenses to develop and commercialize our product candidates in the U.K. or the EU or receive clinical supply of our product candidates from manufacturing partners in the U.K.***

Brexit has created political and economic uncertainty, particularly in the U.K. and the EU, and this uncertainty may persist for years. A withdrawal could, among other outcomes, disrupt the free movement of goods, services and people between the U.K. and the EU, and result in increased legal and regulatory complexities, as well as potentially higher costs of conducting business in Europe. The U.K.'s vote to exit the EU could also result in similar referendums or votes in other European countries in which we do business. Given the lack of comparable precedent, it is unclear what financial, trade and legal implications the withdrawal of the U.K. from the EU would have and how such withdrawal would affect us.

Since a significant proportion of the regulatory framework in the U.K. applicable to our business and our product candidates is derived from EU directives and regulations, Brexit, following the Transition Period, could materially impact the regulatory regime with respect to the development, approval and commercialization of our product candidates in the U.K. or the EU. For example, as a result of the uncertainty surrounding Brexit, the EMA relocated to Amsterdam from London. Following the Transition Period, the U.K. will no longer be covered by the centralized procedures for obtaining EU-wide marketing authorization from the EMA and, unless a specific agreement is entered into, a separate process for authorization of drug products, including our product candidates, will be required in the U.K., the potential process for which is currently unclear. In the U.K., this transition may result in delays in granting clinical trial authorization or marketing authorization, disruption of importation and export of active substance and other components of new drug formulations, and disruption of the supply chain for clinical trial product and final authorized formulations.

We anticipate that Oxford, which is based in the U.K., will continue to provide clinical and commercial supply for our AXO-Lenti-PD program. Brexit, following the Transition Period, could affect the clearance or timing of the release of our clinical trial materials out of the U.K. Any such delays could result in our clinical study sites outside of the U.K. not having sufficient clinical trial materials and could adversely affect the timing and completion of our clinical trials.

Any new regulations could add time and expense to the conduct of our business, as well as the process by which our products receive regulatory approval in the U.K., the EU and elsewhere. In addition, the announcement of Brexit and the withdrawal of the U.K. from the EU have had and may continue to have a material adverse effect on global economic conditions and the stability of global financial markets, and may significantly reduce global market liquidity and restrict the ability of key market participants to operate in certain financial markets. Any of these effects of Brexit, among others, could adversely affect our business, our results of operations, liquidity and financial condition.

***Use of social media platforms presents new risks.***

We believe that our potential patient population is active on social media. Social media practices in the pharmaceutical and biotechnology industries are evolving, which creates uncertainty and risk of noncompliance with regulations applicable to our business. For example, patients may use social media platforms to comment on the effectiveness of, or adverse experiences with, a product candidate, which could result in reporting obligations. In addition, there is a risk of inappropriate disclosure of sensitive information or negative or inaccurate posts or comments about us or our product candidates on any social networking website. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face restrictive regulatory actions or incur other harm to our business.

***The failure to maintain our current enterprise resource planning system ("ERP") could adversely impact our business and results of operations.***

If our ERP system does not continue to 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 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 monetary 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 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.

***Changes in funding for the FDA and other government agencies could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new products and services from being developed or commercialized in a timely manner, which could negatively impact our business.***

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would harm our business. For example, over the last several years, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could harm our business.

***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.***

We 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. In addition, in June 2018, California enacted the California Consumer Privacy Act ("CCPA") which took effect on January 1, 2020. The CCPA gives California residents expanded rights to access and require deletion of their personal information, opt out of certain personal information sharing, and receive detailed information about how their personal information is used. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that may increase data breach litigation. Although the CCPA includes exemptions for certain clinical trials data, and HIPAA protected health information, the law may increase our compliance costs and potential liability with respect to other personal information we collect about California residents. The CCPA has prompted a number of proposals for new federal and state privacy legislation that, if passed, could increase our potential liability, increase our compliance costs and adversely affect our business.

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. 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. 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 EU national law and could have an effect on our business operations if compliance becomes more costly than under current requirements.

It is possible that each of these privacy laws may be interpreted and applied in a manner that is inconsistent with our practices. Further, Brexit has created uncertainty with regard to data protection regulation in the U.K. In particular, it is unclear whether, post Brexit, the U.K. will enact data protection legislation equivalent to the GDPR and how data transfers to and from the U.K. will be regulated. 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 expensive, time-consuming, difficult to design and implement and involve an uncertain outcome.***

Our gene therapy 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 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 gene therapy 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.

Failure can occur at any stage of our clinical 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.

Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including due to changes in regulatory policy during the period of our product candidate development. Any such failures or delays could negatively impact our business, financial condition, results of operations and prospects.

All of our gene therapy product candidates 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 clinical trials conducted to date for our gene therapy product candidates have involved a small number of patients, 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. In addition, the design of a clinical trial, such as endpoints, inclusion and exclusion criteria, statistical analysis plans, data access protocols and trial sizing, can determine whether its results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. Furthermore, as we are exploring new disease areas without any approved treatments, we may need to qualify new and unproven endpoints as we are continuing the development of our product candidates, which may increase uncertainty.

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

- failure to obtain regulatory approval to commence a trial, such as the recent clinical hold placed by the FDA on our AXO-AAV-GM2 program;
- 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 gene therapy product candidate or placebo or failure to obtain sufficient quantities of concomitant medication for use in clinical trials;
- inability to monitor patients adequately during or after treatment;
- inability or unwillingness of medical investigators to follow our clinical and other applicable protocols;
- 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 gene therapy 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 gene therapy product candidates.

In addition, we acquired worldwide rights to our gene therapy product candidates and were not involved in their development prior to such acquisitions. Any difficulties we experience in transitioning and integrating such gene therapy 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 materials, 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 gene therapy product candidates prior to acquiring the rights to them. We are dependent on our predecessors, including Oxford and UMMS, 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 gene therapy 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 limited data regarding the safety, tolerability and efficacy of our gene therapy product candidates and their potential indications, and we have not previously conducted development activities for a biological product candidate. Problems related to our predecessors, including UMMS and Oxford, and our limited available data for our gene therapy product candidates could result in increased costs and delays in the development of our gene therapy product candidates, which could adversely affect our ability to generate future revenues.

***Interim "top-line" and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.***

From time to time, we may publish interim "top-line" or preliminary data from our clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data over a longer follow-up period become available. Preliminary or "top-line" data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. In addition, our clinical trials have involved small patient populations; the interim results of these clinical trials may be subject to substantial variability and may not be indicative of final results. As a result, interim and preliminary data should be viewed with caution until the final data are available. Adverse differences between preliminary or interim data and final data could significantly harm our business prospects.

***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 candidate, 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 gene therapy product candidates in the future may make it difficult or impossible to recruit and retain patients in other clinical trials of those gene therapy 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 gene therapy 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, there are several large and small pharmaceutical companies focused on delivering therapeutics for the treatment of these diseases. Further, it is likely that additional therapies will become available in the future for the treatment of our target indications.

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

The availability of our competitors' products could limit the demand, and the price we are able to charge, for any gene therapy product candidate we develop. The inability to compete with existing or subsequently introduced products 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 gene therapy 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 therapies 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 gene therapy product candidates, and our ability to generate revenue will be materially impaired.***

The activities associated with the development and commercialization of our gene therapy 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 gene therapy product candidates will prevent us from commercializing them.

We have not received approval from regulatory authorities to market any gene therapy product candidate in any jurisdiction, and we will need to complete pivotal clinical trials successfully for our gene therapy product candidates before we can submit any application for regulatory approval. It is possible that our gene therapy 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 gene therapy product candidates to regulatory authorities for each therapeutic indication to establish safety and efficacy of the gene therapy 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 gene therapy product candidates and generate product revenues.

***Our gene therapy 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 gene therapy 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 gene therapy 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 gene therapy 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 gene therapy 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 and AXO-AAV-GM2 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 or AXO-AAV-GM2.

If additional clinical experience indicates that any of our gene therapy product candidates has side effects or causes serious or life-threatening side effects, the development of the gene therapy product candidate may fail or be delayed, or, if the gene therapy 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 gene therapy product candidate and could increase the costs of commercializing our gene therapy product candidates.

***Our AAV-based gene therapy product candidates and our lentiviral-based gene therapy product candidate 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.***

The use of gene therapy in the treatment of GM1 gangliosidosis, GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease) and Parkinson's disease is new. We may experience problems or delays in developing new gene therapy product candidates and such problems or delays may cause unanticipated costs, and such development problems may not be solvable. 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 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, Luxturna by Spark Therapeutics, Inc. for patients with an inherited form of vision loss, and Zolgensma by Novartis International AG, for children less than 2 years old with spinal muscular atrophy. It is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our gene therapy product candidates in either the United States, or other major markets or how long it will take to commercialize our gene therapy 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. 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. 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 gene therapy 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 gene therapy 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 gene therapy 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 gene therapy 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.

***Even if we obtain FDA approval for our gene therapy 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 product, 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 BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the 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 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. Prescription drugs may be promoted only for the approved indications in accordance with the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label may be subject to significant liability. However, physicians may, in their independent medical judgment, prescribe legally available products for off-label uses. The FDA does not regulate the behavior of physicians in their choice of treatments but the FDA does restrict manufacturer's communications on the subject of off-label use of their products. Violations of the FDCA or PHS 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. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained.

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

Even if our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community, 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 and patients' willingness to pay for our products in the absence of such 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.

***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.***

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, 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 our gene therapy product candidates. 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. 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. Among the risks in any gene therapy product based on viral vectors are the risks of immunogenicity, elevated liver enzymes, and insertional oncogenesis, which is the process whereby the insertion of a functional gene near a gene that is important in cell growth or division results in uncontrolled cell division, which could potentially enhance the risk of malignant transformation. If our vectors demonstrate a similar effect we may decide or be required to halt or delay further clinical development of our AAV-based product candidates. 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 product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

***Increasing demand for compassionate use or expanded access of our unapproved therapies could negatively affect our reputation and harm our business.***

We are developing our product candidates for life-threatening illnesses for which there are currently limited to no available therapeutic options. It is possible for individuals or groups to target companies with disruptive social media campaigns related to a request for access to unapproved drugs for patients with significant unmet medical need. If we experience a similar social media campaign regarding our decision to provide or not provide our product candidates under an expanded access corporate policy, our reputation may be negatively affected and our business may be harmed.

Recent media attention to individual patients' expanded access requests has resulted in the introduction of legislation at the local and national level referred to as "Right to Try" laws, such as the Right to Try Act, which are intended to give patients access to unapproved therapies. New and emerging legislation regarding expanded access to unapproved drugs for life-threatening illnesses could negatively impact our business in the future.

A possible consequence of both activism and legislation in this area is the need for us to initiate an unanticipated expanded access program or to make our product candidates more widely available sooner than anticipated. We are a small company with limited resources and unanticipated trials or access programs could result in diversion of resources from our primary goals.

In addition, some patients who receive access to unapproved drugs through compassionate use or expanded access programs have life-threatening illnesses and have exhausted all other available therapies. The risk for serious adverse events in this patient population is high which could have a negative impact on the safety profile of our product candidates if we were to provide them to these patients in accordance with our expanded access corporate policy, which could cause significant delays or an inability to successfully commercialize our product candidates, which could materially harm our business. If we were to provide patients with our product candidates under an expanded access program, we may in the future need to restructure or pause ongoing compassionate use and/or expanded access programs in order to perform the controlled clinical trials required for regulatory approval and successful commercialization of our product candidates, which could prompt adverse publicity or other disruptions related to current or potential participants in such programs.

***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 an 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 EU, 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;
- the inability to negotiate with payors regarding reimbursement for our products; 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 payors, and customers will be subject to applicable healthcare regulatory laws, which could expose us to penalties.***

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations.

These laws may regulate the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute our products for which we obtain marketing approval. Such laws include:

- the federal Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the federal Anti-Kickback Statute or specific intent to violate it to have committed a violation; in addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act;
- the federal false claims laws including the civil False Claims Act, which can be enforced through civil whistleblower or qui tam actions, and civil monetary penalties laws, which impose criminal and civil penalties 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 certain 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 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 payors, including private insurers, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; and state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers, marketing expenditures or drug pricing, 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, 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 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 licensure framework for follow on biologic products;
- 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.

There remain judicial and Congressional challenges to certain aspects of the Affordable Care Act, as well as efforts by the current administration to repeal or replace certain aspects of the Affordable Care Act. 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, the 2020 federal spending package permanently eliminates, effective January 1, 2020, the Affordable Care Act's "Cadillac" tax on high-cost employer-sponsored health coverage and medical device tax and, effective January 1, 2021, also eliminates the health insurer tax.

Further, the Bipartisan Budget Act of 2018, among other things, amended the Affordable Care Act, effective January 1, 2019, to increase from 50% to 70% 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, the federal government eliminated federal cost-sharing reduction ("CSR") payments to insurance companies. The loss of such federal CSR payments has resulted in increased premiums on certain policies issued by qualified health plans under the Affordable Care Act. Moreover, in December 2018, the Centers for Medicare and Medicaid Services ("CMS") published a new 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. On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas ruled that the individual mandate is a critical and inseparable feature of the Affordable Care Act, and therefore, because it was repealed as part of the Tax Cuts and Jobs Act of 2017, the remaining provisions of the Affordable Care Act are invalid as well. In December 2019, the U.S. Court of Appeals for the Fifth Circuit upheld the District Court ruling that the individual mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the Affordable Care Act are invalid as well. Additionally, on December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court ruling that the individual mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the Affordable Care Act are invalid as well. It is unclear how this decision, future decisions, subsequent appeals, if any, and other efforts to repeal and replace the Affordable Care Act will impact the Affordable Care Act and our business. 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. See "Item 1. Business-Government Regulation."

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 2029 unless additional Congressional action is taken. Additionally, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers and increased the statute of limitations period in which the government may recover overpayments to providers from three to five years.

Further, there have been several recent U.S. Congressional inquiries and proposed federal and state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the out-of-pocket cost of prescription drugs and reform government program reimbursement methodologies for drugs. 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 2020 contains further drug price control measures that could be enacted during the 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 solicited feedback on some of these measures and is implementing others under its existing authority. For example, in May 2019, CMS issued a final rule to allow Medicare Advantage Plans the option of using step therapy for Part B drugs beginning January 1, 2020. This final rule codified CMS's policy change that was effective January 1, 2019. 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.

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, whether the current regulations, guidance or interpretations will be changed, or what the impact of such changes on our business, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

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

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

Market acceptance and sales of any approved product candidates that we develop will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from third-party payors, including government health administration authorities and private health insurers. In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors. Third-party payors decide which drugs or therapies they will pay for and establish reimbursement levels. One payor's determination to provide coverage for a product does not assure that other payors will also provide coverage, and adequate reimbursement, for the product. Additionally, a third-party payor's decision to provide coverage for a drug 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 payors to reimburse all or part of the associated healthcare costs. Patients are unlikely to use our products unless coverage is provided, and reimbursement is adequate to cover a significant portion of the cost of our products.

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

Additionally, there have been a number of legislative and regulatory proposals to change the healthcare system in the United States and in some foreign jurisdictions that could affect our ability to sell any future drugs profitably. There can be no assurance that our product candidates, if approved, will be considered medically reasonable and necessary, that it will be considered cost-effective by third-party payors, that coverage or an adequate level of reimbursement will be available, or that reimbursement policies and practices in the United States and in foreign countries where our products are sold will not adversely affect our ability to sell our product candidate profitably, if approved for sale.

### **Risks Related to Our Dependence on Third Parties**

*Gene therapies are novel, complex and difficult to manufacture. 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 in the foreseeable future, 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 Agreement, Oxford 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. The Oxford 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. The UMMS Agreement provides that UMMS will supply material manufactured at Nationwide Children's Hospital to support the AXO-AAV-GM1 and AXO-AAV-GM2 Phase 1/2 clinical studies. Our strategic partnership with Yposkesi will provide us with preferred access and reserved capacity for cGMP grade viral vector production for our gene therapy programs, with an initial focus on the AAV-based gene therapies.

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 qualified backup suppliers for those components that are currently purchased from a sole or single source supplier;
- lack of access or licenses to proprietary manufacturing methods used by third-party manufacturers to make our product candidates;
- 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.

The process for manufacturing gene therapy products, including our product candidates, is more complex than those required for most chemical pharmaceuticals, require substantial expertise, specialized facilities, highly specific raw materials and significant capital investment and involve other production constraints. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a gene therapy product such as ours generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner or that the dosing will be uniform in our products. Accordingly, we and our manufacturers employ multiple steps to control our manufacturing process to assure that the process works and that our product candidates are made strictly and consistently in compliance with the process. Problems with the manufacturing process, including even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims or insufficient inventory. We may encounter problems achieving adequate quantities and quality of clinical-grade or commercial-grade materials that meet FDA, EMA or other applicable standards or specifications with consistent and acceptable production yields and costs. In addition, the FDA, EMA and other regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, EMA or other regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay product launches or clinical trials, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

Due to the complexity and constraints associated with manufacturing gene therapy products, there is a limited number of suppliers that can adequately and timely provide the raw materials, including vectors, for our product candidates, particularly if we commence larger clinical trials and studies for our product candidates. If supply from a manufacturing facility is interrupted, including due to equipment malfunctions, facility contamination, material shortages or contamination, natural disasters, disruption in utility services, human error or disruptions in the operations of suppliers, 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 them 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. Further, due to intense competition among companies developing gene therapy product candidates, we may encounter difficulties in sourcing adequate supply for our gene therapy products on a timely or cost-efficient basis.

We have no experience manufacturing any of our product candidates. Building our own manufacturing facility, if we decide to do so in the future, would require substantial additional investment, would be time-consuming and may be subject to delays, including those resulting from compliance with regulatory requirements. We also may encounter problems hiring and retaining the experienced specialist scientific, quality control and manufacturing personnel needed to operate our manufacturing process, which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements. Although we may establish our own manufacturing facility or use that of a third-party contract manufacturer to support a commercial launch of our gene therapy product candidates, 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. We must supply all necessary documentation in support of a BLA or other MAA on a timely basis and must adhere to the FDA's and EMA's cGMP requirements before any of our product candidates can obtain marketing approval. To date, to our knowledge, a limited number of cGMP gene therapy manufacturing facilities have received approval from the FDA for the manufacture of an approved gene therapy product and, therefore, the timeframe required for us to obtain such approval is uncertain. We are subject to audits from FDA, EMA and other authorities that may result in observations of non-compliance from cGMP requirements. In addition, our ability to receive damages from our CROs in connection with such failures is generally contractually limited.

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.

***Any contamination in our manufacturing process, shortages of raw materials or failure of any of our key suppliers to deliver necessary components could result in delays in our clinical development or marketing schedules.***

Given the nature of biologics manufacturing, there is a risk of contamination. Any contamination could harm our ability to produce product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage. Some of the raw materials required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates could harm or disrupt the commercial manufacturing or the production of clinical material, which could harm our development timelines and our business, financial condition, results of operations and prospects.

***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 our agreements with Oxford and UMMS, we may rely on their respective employees for certain services in connection with the transition of the respective gene therapy product candidates to us. We do not have complete control over those employees or their execution of services provided to us, and these employees may not perform such services in a timely or satisfactory manner, which could harm our business and development programs.

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. Although we may rely on CROs to conduct our GLP-compliant preclinical studies and GCP-compliant clinical trials, we will remain responsible for ensuring that each of our GLP preclinical studies and GCP clinical trials is conducted in accordance with its investigational plan and protocol and applicable laws and regulations, and our reliance on the CROs does not relieve us of our regulatory responsibilities. 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.

***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;
- 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 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 gene therapy 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 or own cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such application(s). Our in-licensed and/or owned patent applications may fail to result in issued patents with claims that cover our current product candidates or other gene therapy product candidates in the United States or in foreign countries. Our in-licensed and owned 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 in-licensed or owned patents which may result in such patents being narrowed, invalidated, or held unenforceable.

The patent rights that we own or have licensed relating to our product candidates may be limited in ways that may affect our ability to exclude third parties from competing against us if we obtain regulatory approval to market these product candidates. For our 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 other patents, such as method patents, that we may hold or obtain rights to. 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 as patents, 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 will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement 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 or patent applications 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. Certain 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 patents 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 owned or in-licensed U.S. patents or patent applications, once issued, 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-Lenti-PD, AXO-AAV-GM1 and AXO-AAV-GM2, 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 provides, 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 complex in nature, may be 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.***

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 sooner, 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 or AXO-AAV-GM2. 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) or AXO-AAV-GM2 (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, plasmids, vectors, cell lines, etc. formed during the manufacturing process; any final product itself or the intended method of treatment using 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.

Third-party patent applications directed to methods for producing recombinant lentiviral vectors or recombinant AAV vectors could adversely affect the potential commercialization of our current gene therapy product candidates, if patents issue from such applications that include claims that would cover the methods of making our current gene therapy product candidates. While we do not believe that such pending third-party claims that would cover the methods of making our current gene therapy product candidates are likely to be 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 or patents 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 multiple factors including 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, processes, 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.***

We have licensed rights to intellectual property from Oxford and UMMS in order to commercialize our product candidates, and we have or intend to enter into one or more commercial supply and manufacturing agreements for our current product candidates.

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;
- 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 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 government may have certain rights in patent applications and patents issuing therefrom that we in-license or own. 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. Furthermore, if the U.S. Government has rights related to a product candidate under the Bayh-Dole Act, we may be obligated to substantially manufacture in the U.S. such product if it was invented using government funding. Under certain circumstances, we may be able to obtain a waiver to manufacture outside the U.S., however, such waivers are not guaranteed.

***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 EU 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.***

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 or 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 or license;
- 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 or license 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 be sustained. In addition, as a result of RSL owning approximately 58.1% of our common shares as of December 31, 2019, 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, including as a result of the recent clinical hold placed on our AXO-AAV-GM2 program;

- results of clinical trials of our product candidates or those of our competitors;
- any delay in filing applications for marketing approval of our product candidates, and any adverse development or perceived adverse development with respect to applicable regulatory authorities' review of those applications;
- failure to successfully develop and commercialize our product candidates;
- failure to maintain our relationship with Oxford or UMMS or comply with the terms of the Oxford Agreement or the UMMS 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, 2019, RSL beneficially owns approximately 58.1% of the voting power of our outstanding common shares and has the ability to substantially influence us through this ownership position. For example, RSL is 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 RSL may act in a manner with which you may not agree or that may not be in the best interests of our other shareholders. RSL has closed a transaction with Sumitomo Dainippon Pharma Co., Ltd. ("Sumitomo") that includes a grant to Sumitomo of a right of first refusal with respect to our common shares held by RSL, which could result in RSL taking actions that may not coincide with our corporate interests or the interests of other shareholders, and could impact our ability to undertake certain corporate transactions. Further, RSL is a privately held company whose ownership and governance structure is not transparent to our other shareholders. There may be changes to the management or ownership of RSL that could impact RSL's interests in a way that may not coincide with our corporate interests or the interests of other shareholders. So long as RSL continues to own a significant amount of our equity, 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.

In addition, we are party to certain related party agreements with RSL and its wholly owned subsidiaries, Roivant Sciences, Inc. ("RSI") and Roivant Sciences GmbH ("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.***

As of December 31, 2019, 13,244,047 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. Such a decrease in our share price could also in turn impair our ability to raise capital through the sale of additional equity securities.

Further, 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. Sales of these common shares may negatively impact our share price.

In addition, we have filed a "shelf" registration statement on Form S-3 under the Securities Act, 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. We have also entered into a sales agreement with Cowen and Company, LLC to sell 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. To the extent we issue new common shares as a result of needing additional capital, such shares could constitute a material portion of our then outstanding common shares and cause dilution our existing shareholders.

***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.

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 by-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. Additionally, we have sought and obtained specific permission from the Bermuda Monetary Authority for the issue and transfer of our common 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 second amended and restated bye-laws enable our board of directors to issue preference shares, which may discourage a change of control.***

Our second amended and restated 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 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 U.K., and under current U.K. tax law, a company which is centrally managed and controlled in the U.K. is regarded as resident in the U.K. for taxation purposes. Accordingly, we expect to be subject to U.K. taxation on our income and gains and subject to the U.K.'s controlled foreign company rules, 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 U.K. 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.

***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 majority shareholder, are incorporated under the laws of Bermuda. We currently have subsidiaries in the U.K., 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 services agreements between us 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. We continue to assess the impact of such changes in tax laws and interpretations on our business and may determine that changes to our structure, practice, tax positions or the manner in which we conduct our business are necessary in light of such changes and developments in the tax laws of the jurisdictions in which we operate. 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 U.K. 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 that own 10% 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% 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 value of all outstanding stock of such non-U.S. corporation) 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 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.

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 ("IRS"). 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 of our common shares.

***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 certain distributions by us and the proceeds of sales or other dispositions of our common shares that result in a gain to the U.S. holder. In addition, special information reporting may be required.

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 our cash on-hand and cash from future financings in our business. With respect to the taxable year that ended on March 31, 2019, and foreseeable future tax years, we believe that we were not a PFIC and presently do not anticipate that we will be a PFIC based upon the expected value of our assets, including any goodwill, and the expected nature and composition of our income and assets. However, our status as a PFIC is a fact-intensive determination made on an annual basis, and we cannot provide any assurances regarding our PFIC status for the current or future taxable years. Our U.S. counsel expresses no opinion with respect to our PFIC status for our current or future taxable years. We will determine whether we were a PFIC or not for each taxable year and make such determination available to U.S. holders.

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 in the current and future taxable years. In addition, recently proposed U.S. Treasury Regulations, of we are continuing to assess the impact, may also adversely affect the treatment of these structures and arrangements with respect to our PFIC status.

**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.**

As previously announced, on September 6, 2019, our parent company, Roivant Sciences Ltd. (“RSL”), and Sumitomo Dainippon Pharma Co., Ltd. (“Sumitomo”) entered into a non-binding memorandum of understanding (the “MOU”) related to the creation of a strategic alliance between the companies (the “Strategic Alliance”).

As contemplated by the MOU, RSL announced that on October 31, 2019, Sumitomo, RSL and certain of RSL’s subsidiaries entered into a definitive agreement (the “Transaction Agreement”) related to the creation of the Strategic Alliance. Upon the closing of the Strategic Alliance transaction, which occurred on December 27, 2019, and in connection with the transactions contemplated by the Transaction Agreement, RSL and Sumitomo entered into the Right of First Refusal and Notice Agreement (“the ROFR Agreement”), granting Sumitomo, among other things, a right of first refusal with respect to our common shares held by RSL (the “ROFR”).

For more information regarding the terms of the ROFR and a copy of the ROFR Agreement, refer to the Current Report on Form 8-K filed by us with the SEC on September 6, 2019 and the Schedule 13D/A filed by RSL with the SEC on December 31, 2019.

**Item 6. Exhibits.**

Exhibit Number	Description of Document	Incorporated by Reference			
		Schedule/Form	File No.	Exhibit	Filing Date
3.1	<a href="#">Certificate of Incorporation.</a>	S-1	333-204073	3.1	05/11/2015
3.2	<a href="#">Memorandum of Association.</a>	S-1	333-204073	3.2	05/11/2015
3.3	<a href="#">Second Amended and Restated Bye-laws.</a>	8-K	001-37418	3.1	12/21/2017
10.1	<a href="#">Third Amendment to Loan and Security Agreement, dated November 27, 2019, by and among the Registrant, Axovant Holdings Limited, Axovant Sciences GmbH, Axovant Sciences America, Inc., Axovant Treasury, Inc., Axovant Treasury Holdings, Inc., Axovant Sciences, Inc., and Hercules Capital, Inc.</a>	8-K	001-37418	10.1	12/03/2019
10.2+	<a href="#">Employment Agreement, dated November 4, 2019, by and between Pavan Cheruvu and Axovant Sciences, Inc.</a>	8-K	001-37418	10.1	11/8/2019
10.3+	<a href="#">Employment Agreement, dated November 4, 2019, by and between David Nassif and Axovant Sciences, Inc.</a>	8-K	001-37418	10.2	11/8/2019
10.4+	<a href="#">Employment Agreement, dated November 4, 2019, by and between Gavin Corcoran and Axovant Sciences, Inc.</a>	8-K	001-37418	10.3	11/8/2019
31.1*	<a href="#">Certification of Principal Executive Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</a>				
31.2*	<a href="#">Certification of Principal Financial Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</a>				
32.1*#	<a href="#">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.</a>				
32.2*#	<a href="#">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.</a>				
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.

+ Indicates management contract or compensatory plan.

# 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.

**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 GENE THERAPIES LTD.**

Date: February 10, 2020

By: /s/ David Nassif  
Name: David Nassif  
Title: Principal Financial Officer and Principal  
Accounting Officer

## CERTIFICATION

I, Pavan Cheruvu, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Axovant Gene Therapies Ltd.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: February 10, 2020

By: /s/ Pavan Cheruvu

Pavan Cheruvu

*Principal Executive Officer*

## CERTIFICATION

I, David Nassif, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Axovant Gene Therapies Ltd.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: February 10, 2020

By: /s/ David Nassif

David Nassif

*Principal Financial Officer and Principal Accounting Officer*

**CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,  
AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Quarterly Report on Form 10-Q of Axovant Gene Therapies Ltd. (the "Company") for the period ended December 31, 2019 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Pavan Cheruvu, Principal Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

- (1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: February 10, 2020

By: /s/ Pavan Cheruvu

Pavan Cheruvu

*Principal Executive Officer*

A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the SEC or its staff upon request.



**CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,  
AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Quarterly Report on Form 10-Q of Axovant Gene Therapies Ltd. (the "Company") for the period ended December 31, 2019 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, David Nassif, Principal Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

- (1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: February 10, 2020

By: /s/ David Nassif

David Nassif

*Principal Financial Officer and Principal Accounting Officer*

A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the SEC or its staff upon request.