



## Axovant Announces Financial Results and Corporate Updates for Fourth Quarter and Fiscal Year Ended March 31, 2019

June 11, 2019

- Established diversified pipeline of three clinical-stage gene therapy programs for serious neurological disorders including Parkinson's disease, GM1 gangliosidosis, and Tay-Sachs
- SUNRISE-PD Phase 2 trial of AXO-Lenti-PD for Parkinson's disease continues to enroll patients in second dose cohort with data expected in the fourth quarter of 2019
- Reported initial data from Tay-Sachs patient dosed with AXO-AAV-GM2 in March 2019; additional data from patients dosed with AXO-AAV-GM1 and AXO-AAV-GM2 expected in second half of 2019
- Raised \$94.5 million in net equity capital from healthcare-dedicated investors in fiscal year ended March 31, 2019
- Net loss for the quarter ended March 31, 2019 was \$9.0 million, or \$0.45 per share, compared to a net loss of \$25.3 million, or \$1.88 per share, for the prior-year quarter

BASEL, Switzerland, June 11, 2019 (GLOBE NEWSWIRE) -- Axovant Gene Therapies Ltd (NASDAQ: AXGT), a clinical-stage company developing innovative gene therapies, today provided financial results and corporate updates for its fiscal year ended March 31, 2019.

"The past year has been transformational as Axovant has been rebuilt around a singular mission of becoming an industry leader in the clinical development of gene therapies for neurological diseases," said Pavan Cheruvu, Chief Executive Officer of Axovant. "We have assembled a world-class team with decades of experience in gene therapy development and built a diversified pipeline of clinical-stage programs in collaboration with leading gene therapy institutions. Two of these programs, AXO-Lenti-PD and AXO-AAV-GM2, have already generated encouraging data readouts and continue to enroll patients. Additionally, in historic moments for the field of neurological gene therapies, children with GM1 gangliosidosis and Tay-Sachs disease were dosed with gene therapy for the first time at our partner institutions."

"We expect a data-rich second half of the year with key clinical readouts across all three of our gene therapy programs. Over the last year, we have taken steps to direct our focus and resources towards the clinical programs that will drive long-term value for our shareholders. I'm incredibly proud of the new direction that Axovant is taking, and we look forward to continuing to drive innovative science to transform the lives of patients and their families."

### Key Highlights and Development Updates

- Reported six-month data from the first cohort of the dose escalation portion of the SUNRISE-PD study of AXO-Lenti-PD in Parkinson's disease patients. AXO-Lenti-PD was observed to be generally well tolerated at six months and continued to demonstrate benefits in both patients across multiple measures after a single administration. Enrollment in the second dose cohort has begun, with initial data from up to six patients in this cohort expected in the fourth quarter of 2019.
- Reported initial data from the AXO-AAV-GM2 study in March 2019, which suggested that the gene therapy was generally well-tolerated and no serious adverse events in a patient with advanced Tay-Sachs disease. An apparent increase in  $\beta$ -Hexosaminidase A enzyme activity at three months was reported, and the patient's clinical condition was stable from baseline. Axovant expects to enroll patients in a multi-subject clinical trial in the second half of 2019.
- AXO-AAV-GM1 is currently being evaluated for the potential treatment of GM1 gangliosidosis, with the first patient dosed in May 2019 by collaborators at the National Institutes of Health. Axovant expects initial data from this clinical program in the second half of 2019 with continued enrollment of patients throughout 2019.
- Terminated the license and collaboration agreement with Benitec Biopharma Limited following the decision to no longer pursue development of AXO-AAV-OPMD and related gene therapy products.
- Raised \$94.5 million in net equity in fiscal year ended March 31, 2019, including \$37.9 million of net equity financing in March 2019 led by existing shareholders and several new healthcare-dedicated investors.
- Announced the formation of Arvelle Therapeutics and the strategic transition of the legacy small molecule team with Axovant receiving an approximate 5% preferred equity stake in Arvelle.
- Hosted first R&D Day for investors and analysts in March 2019 to highlight development updates, scientific and clinical insights from prominent physicians and researchers, and patient perspectives across Axovant's gene therapy pipeline.
- Effected a reverse share split of common shares on a 1-for-8 basis in May 2019, reducing the shares outstanding from approximately 182.2 million common shares to approximately 22.8 million, and completed name change to Axovant Gene Therapies and ticker change to AXGT.

## Fiscal Fourth Quarter Financial Summary

For the fiscal fourth quarter ended March 31, 2019, research and development expenses were \$7.1 million, of which \$1.5 million was attributable to non-cash, share-based compensation expense. Research and development expenses decreased by \$14.7 million in the fiscal fourth quarter ended March 31, 2019 compared to the quarter ended March 31, 2018, primarily related to a decrease of \$17.3 million associated with the discontinuation of our development programs for intepirdine and nelotanserin, partially offset by an increase of \$4.1 million related to our AXO-LENTI-PD, AXO-AAV-OPMD, AXO-AAV-GM1 and AXO-AAV-GM2 programs. Excluding non-cash share-based compensation, research and development expenses decreased by \$14.1 million in the fiscal fourth quarter ended March 31, 2019, compared to the fiscal fourth quarter ended March 31, 2018.

General and administrative expenses for the fiscal fourth quarter ended March 31, 2019 were \$6.2 million, of which \$2.1 million was non-cash, share-based compensation expense. General and administrative expenses increased by \$3.9 million in the fiscal fourth quarter ended March 31, 2019, compared to the fiscal fourth quarter ended March 31, 2018. Excluding non-cash share-based compensation, general and administrative expenses decreased by \$9.9 million in the fiscal fourth quarter ended March 31, 2019, compared to the fiscal fourth quarter ended March 31, 2018, primarily related to personnel-related expenses, as well as direct and indirect costs allocated to us under the services agreements with RSI and RSG.

Net loss for the fiscal fourth quarter ended March 31, 2019 was \$9.0 million, or \$0.45 per share, compared to a net loss of \$25.3 million, or \$1.88 per share, for the prior-year quarter. Net cash used in operating activities for the fiscal fourth quarter ended March 31, 2019 was \$12.7 million.

## Fiscal Year Financial Summary

For the fiscal year ended March 31, 2019, research and development expenses were \$87.6 million, of which \$4.8 million was attributable to non-cash, share-based compensation expense. Research and development expenses decreased by \$53.9 million in the year ended March 31, 2019 compared to the year ended March 31, 2018. Research and development expenses decreased by approximately \$92.3 million, primarily due to the discontinuation and wind-down of our intepirdine and nelotanserin development programs. However, these decreases in research and development expenses were partially offset by increases in our AXO-LENTI-PD, AXO-AAV-OPMD, AXO-AAV-GM1 and AXO-AAV-GM2 program expenses, primarily due to the incurrence of \$45 million of upfront license fees paid to the licensors of those programs.

General and administrative expenses for the fiscal year ended March 31, 2019 were \$39.5 million, of which \$11.7 million was non-cash, share-based compensation expense. General and administrative expenses decreased \$32.4 million in the year ended March 31, 2019, compared to the year ended March 31, 2018, primarily due to a \$13.1 million decrease in personnel-related expenses resulting from reduced headcount, employee severance and other benefits and an \$8.7 million decrease in marketing expenses due to the discontinuation of our intepirdine program.

Net loss for the year ended March 31, 2019 was \$129.1 million, or \$8.02 per share, compared to a net loss of \$221.6 million or \$16.51 per share, in the prior-year period. Net cash used in operating activities was \$134.2 million for the fiscal year ended March 31, 2019, or \$84.2 million excluding \$50.0 million in upfront payments made to our licensing partners. Excluding milestone payments, we expect our net cash used in operations to be lower during the fiscal year ending March 31, 2020 due to a reduced operating cost structure, the discontinuation of our legacy small molecule programs and the termination of the license and collaboration agreement with Benitec Biopharma Limited.

As of March 31, 2019, we had \$107.0 million of cash and cash equivalents, working capital of \$71.1 million, and long-term debt of \$23.0 million.

## About Axovant

Axovant, part of the Roivant family of companies, is a clinical-stage gene therapy company focused on developing a pipeline of innovative product candidates for debilitating neurological and neuromuscular diseases. Our current pipeline of gene therapy candidates targets GM1 gangliosidosis, GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease), and Parkinson's disease. Axovant is focused on accelerating product candidates into and through clinical trials with a team of experts in gene therapy development and through external partnerships with leading gene therapy organizations. For more information, visit [www.axovant.com](http://www.axovant.com).

## About Roivant

Roivant Sciences aims to improve health by rapidly delivering innovative medicines and technologies to patients. It does this by building Vants – nimble, entrepreneurial biotech and healthcare technology companies with a unique approach to sourcing talent, aligning incentives, and deploying technology to drive greater efficiency in R&D and commercialization. For more information, please visit [www.roivant.com](http://www.roivant.com).

## Forward-Looking Statements

This press release contains forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995 and other federal securities laws. The use of words such as “may,” “might,” “will,” “would,” “should,” “expect,” “believe,” “estimate,” and other similar expressions are intended to identify forward-looking statements. For example, all statements Axovant makes regarding the initiation, timing, progress, and reporting of results of its preclinical programs, clinical trials, and research and development programs; cash to be used in operating activities; its ability to advance its gene therapy product candidates into and successfully initiate, enroll, and complete clinical trials; the potential clinical utility of its product candidates; its ability to continue to develop its gene therapy platforms; its ability to develop and manufacture its products and successfully transition manufacturing processes; its ability to perform under existing collaborations with, among others, Oxford BioMedica, Benitec and UMass Medical School, and to add new programs to its pipeline; its ability to enter into new partnerships or collaborations; its ability to retain and successfully integrate its leadership and personnel; the timing or likelihood of its regulatory filings and approvals, and the timing of its expected name change and ticker symbol change are forward-looking. All forward-looking statements are based on estimates and assumptions by Axovant's management that, although Axovant believes to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that Axovant expected. Such risks and uncertainties include, among others, the initiation and conduct of preclinical studies and clinical trials; the availability of data from clinical trials; the expectations for regulatory submissions and approvals; the continued development of its small molecule and gene therapy product candidates and platforms; Axovant's scientific approach and general development progress; and the availability or commercial potential of Axovant's product candidates. These statements are also subject to a number of material risks and uncertainties that are described in Axovant's most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission on June 11, 2019, as updated by its subsequent filings with the Securities and Exchange Commission. Any forward-looking statement speaks only as of the date on which it was made. Axovant undertakes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise.

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**AXOVANT GENE THERAPIES LTD.****Consolidated Statements of Operations**

(in thousands, except share and per share amounts)

	<b>Three Months Ended March 31,</b>		<b>Years Ended March 31,</b>	
	<b>2019</b>	<b>2018</b>	<b>2019</b>	<b>2018</b>
	<b>(unaudited)</b>	<b>(unaudited)</b>	<b>(unaudited)</b>	
Operating expenses:				
Research and development expenses <sup>(1)</sup>				
(includes total share-based compensation expense of \$1,459 and \$1,972 for the three months ended March 31, 2019 and 2018 and \$4,758 and \$16,597 for the years ended March 31, 2019 and 2018, respectively)	\$ 7,149	\$ 21,799	\$ 87,552	\$ 141,412
General and administrative expenses <sup>(2)</sup>				
(includes total share-based compensation expense of \$2,096 and \$(11,673) for the three months ended March 31, 2019 and 2018 and \$11,671 and \$15,281 for the years ended March 31, 2019 and 2018, respectively)	6,157	2,244	39,466	71,906
Total operating expenses	13,306	24,043	127,018	213,318
Other expenses:				
Interest expense	1,722	1,843	7,530	7,545
Other income	(5,891)	(535)	(5,616)	(211)
Loss before income tax expense (benefit)	(9,137)	(25,351)	(128,932)	(220,652)
Income tax expense (benefit)	(91)	(32)	133	921
Net loss	\$ (9,046)	\$ (25,319)	\$ (129,065)	\$ (221,573)
Net loss per common share — basic and diluted	\$ (0.45)	\$ (1.88)	\$ (8.02)	\$ (16.51)
Weighted average common shares outstanding — basic and diluted	19,962,170	13,473,509	16,100,686	13,421,984

(1) Includes total costs allocated from Roivant Sciences Ltd., Roivant Sciences, Inc. and Roivant Sciences GmbH of \$0 and \$1,367 for the three months ended March 31, 2019 and 2018, respectively, and \$(450) and \$7,034 for the years ended March 31, 2019 and 2018, respectively.

(2) Includes total costs allocated from Roivant Sciences Ltd., Roivant Sciences, Inc. and Roivant Sciences GmbH of \$126 and \$1,947 for the three months ended March 31, 2019 and 2018, respectively, and \$2,898 and \$6,883 for the years ended March 31, 2019 and 2018, respectively.

**AXOVANT GENE THERAPIES LTD.****Consolidated Balance Sheets**

(in thousands, except share and per share amounts)

	<b>March 31,</b>	<b>March 31,</b>
	<b>2019</b>	<b>2018</b>
	<b>(unaudited)</b>	
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 106,999	\$ 154,337
Prepaid expenses and other current assets	5,859	2,174
Income tax receivable	1,726	1,751
Total current assets	114,584	158,262
Long-term investment	5,871	—
Other non-current assets	973	—
Property and equipment, net	1,278	2,524
Total assets	\$ 122,706	\$ 160,786
<b>Liabilities and Shareholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 1,698	\$ 3,949
Due to Roivant Sciences Ltd., Roivant Sciences, Inc. and Roivant Sciences GmbH	—	1,011
Accrued expenses	20,619	31,862

Current portion of long-term debt	21,182	9,753
Total current liabilities	43,499	46,575
Long-term debt	22,994	42,925
Total liabilities	66,493	89,500
Shareholders' equity:		
Common shares, par value \$0.00001 per share, 1,000,000,000 shares authorized, 22,779,891 and 13,473,512 issued and outstanding at March 31, 2019 and March 31, 2018, respectively	—	—
Accumulated other comprehensive income	911	126
Additional paid-in capital	741,318	628,111
Accumulated deficit	(686,016)	(556,951)
Total shareholders' equity	56,213	71,286
Total liabilities and shareholders' equity	\$ 122,706	\$ 160,786



Source: Axovant Sciences Ltd.