Axovant Announces Corporate Updates and Financial Results for Second Fiscal Quarter Ended September 30, 2019

November 8, 2019

- First evidence of clinical stabilization in two children with Tay-Sachs disease that received AXO-AAV-GM2 presented at ESGCT in October 2019
- Continued enrollment of patients in AXO-AAV-GM1 registrational study, with 6-month data from Part A of the registrational study expected in mid-2020
- 3-month data from second cohort of dose-escalation study of AXO-Lenti-PD expected in 1H 2020, and company plans to initiate a randomized, sham-controlled study in Parkinson's disease by the end of 2020
- Net loss for the quarter ended September 30, 2019 was $13.9 million, or $0.61 per share, compared to a net loss of $33.8 million, or $2.24 per share for the prior-year quarter

NEW YORK and BASEL, Switzerland, Nov. 08, 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 second fiscal quarter ended September 30, 2019.

"Presenting early evidence of disease stabilization in children with Tay-Sachs was a significant accomplishment for Axovant and our academic collaborators, and suggests an opportunity for disease modification with gene replacement therapy in monogenic pediatric lysosomal storage disorders such as GM1 and GM2 gangliosidoses," said Pavan Cheruvu M.D., Chief Executive Officer of Axovant. "This year, we achieved our goal of commencing dosing across all three gene therapy programs in our portfolio. We remain committed to delivering transformative gene therapies to patients with devastating neurologic diseases, and look forward to a productive 2020 during which we expect to provide several meaningful updates across our programs, including providing safety and efficacy data from the second dose cohort in Parkinson's disease and Part A data at 6-months from our ongoing registrational study in GM1 gangliosidosis."

Key Highlights and Development Updates

- **AXO-AAV-GM2:** Presented first evidence of clinical disease stabilization in two patients dosed with AXO-AAV-GM2 at the European Society of Gene and Cell Therapy (ESGCT) 27th Annual Congress in October 2019. Initial 3-month follow-up data from a child with early symptomatic Tay-Sachs disease treated at 6-months of age suggests a pattern of stable CHOP-INTEND scores, an improvement from baseline in myelination on brain MRI, and an increase in enzyme activity in CSF. The treatment was generally observed to be well-tolerated in both patients with no serious adverse events related to therapy.
- **AXO-AAV-GM1:** The first two patients in the AXO-AAV-GM1 Phase 1/2 program were dosed intravenously earlier this year, and the therapy has been generally well tolerated to date with no serious adverse events attributed to the therapy. Axovant expects to present an update from the first child dosed with AXO-AAV-GM1 gene therapy in Q4 2019, and data from Part A (n=5), which is expected to focus on safety and tolerability as well as exploratory measures of efficacy after 6 months of follow-up, is expected in mid-2020. Axovant also announced plans to expand the clinical development plan for AXO-AAV-GM1 to include infantile GM1 gangliosidosis subjects (Type I), the most severely affected population with the disease.
- **AXO-Lenti-PD:** Axovant expects to present 3-month data from the second dose cohort (n=4) of the SUNRISE-PD Phase 2 study of AXO-Lenti-PD in the first half of calendar year 2020, allowing for an evaluation of safety, tolerability, and various measures of clinical efficacy in Parkinson's disease. Axovant is actively initiating new clinical sites to expedite patient enrollment by expanding neurosurgical capacity for current and future studies. Based on the outcome of dose-escalation studies, Axovant expects to initiate a randomized, sham-controlled study of AXO-Lenti-PD by the end of calendar year 2020.

Fiscal Second Quarter Financial Summary

Research and development expenses were $6.8 million for the three months ended September 30, 2019 compared to $21.5 million for the three months ended September 30, 2018. Excluding a $10.0 million upfront license fee paid in the prior year period to Benitec Biopharma Limited ("Benitec"), research and development expenses decreased by $4.7 million in the current year period, primarily due to the discontinuation of our small

molecule drug programs.

General and administrative expenses decreased by $5.5 million from $10.6 million for the three months ended September 30, 2018 to $5.1 million in the three months ended September 30, 2019, primarily due to reductions in (i) share-based compensation expense of $3.1 million, attributable to reduced headcount, (ii) legal and accounting fees of $0.8 million, (iii) costs allocated under our services agreements with Roivant Sciences, Inc. ("RSI") and Roivant Sciences GmbH ("RSG") of $0.7 million as a result of the decentralization of the services provided to us, and (iv) a reduction in marketing costs of $0.6 million. Going forward, the costs allocated to us under our services agreements with RSI and RSG are expected to continue to be insignificant.

Net loss for the three months ended September 30, 2019 was $13.9 million, or $0.61 per share, based on a weighted-average of 22.8 million common shares outstanding, compared to a net loss of $33.8 million, or $2.24 per share, based on a weighted-average of 15.1 million common shares outstanding for the three months ended September 30, 2018.

Fiscal First-Half Financial Summary

Research and development expenses were $27.9 million for the six months ended September 30, 2019 compared to $58.9 million for the six months ended September 30, 2018. Excluding a net amount of $13.0 million due to Oxford BioMedica (UK) Ltd. ("Oxford") for a development milestone achieved in the current year period for AXO-Lenti-PD, as well as upfront license fees of $35.0 million paid in the prior year period to Oxford and Benitec, research and development expenses decreased by $9.0 million in the current year period, primarily due to the discontinuation of our small molecule drug programs.

General and administrative expenses decreased by $10.9 million from $22.4 million for the six months ended September 30, 2018 to $11.5 million in the six months ended September 30, 2019, primarily due to reductions in (i) share-based compensation expense of $5.0 million attributable to reduced headcount, (ii) legal and accounting fees of $3.0 million, and (iii) costs allocated under our services agreements with RSI and RSG of $1.9 million as a result of the decentralization of the services provided to us.

Net loss for the six months ended September 30, 2019 was $41.9 million, or $1.84 per share, based on a weighted-average of 22.8 million common shares outstanding, compared to a net loss of $85.7 million, or $6.00 per share, based on a weighted-average of 14.3 million common shares outstanding for the six months ended September 30, 2018. Net cash used in operating activities for the six months ended September 30, 2019 was $36.5 million, or $30.5 million excluding a total of $6.0 million paid to Oxford and the University of Massachusetts Medical School for development milestones achieved. Excluding milestone payments, we expect our net cash used in operating activities to be lower during the fiscal year ending March 31, 2020 than in the prior fiscal year due to a streamlined operating structure, the discontinuation of our small molecule drug programs and the termination of the license and collaboration agreement with Benitec.

As of September 30, 2019, we had $60.3 million of cash and cash equivalents, which exceeded the minimum cash balance currently required by our loan and securities agreement with Hercules Capital, Inc. by $30.3 million, working capital of $19.6 million, total debt of $34.4 million, net of discount, of which $11.7 million is classified as long-term debt.

About Axovant

Axovant Gene Therapies, part of the Roivant family of companies, is a clinical-stage company focused on developing a pipeline of innovative gene therapy product candidates for debilitating neurological diseases. The company's 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.

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.

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 and the University of Massachusetts 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; and the timing or likelihood of its regulatory filings and approvals 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on November 8, 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.
Condensed Consolidated Statements of Operations
(Unaudited, in thousands, except share and per share amounts)

	Three Months Ended September 30,		Six Months Ended September 30,	
	2019	**2018**	**2019**	**2018**
Operating expenses:				
Research and development expenses[1] (includes share-based compensation expense (benefit) of $409 and $(1,128) for the three months ended September 30, 2019 and 2018 and $1,130 and $1,389 for the six months ended September 30, 2019 and 2018, respectively)	$ 6,833	$ 21,502	$ 27,923	$ 58,920
General and administrative expenses[2] (includes share-based compensation expense of $482 and $3,585 for the three months ended September 30, 2019 and 2018 and $1,896 and $6,927 for the six months ended September 30, 2019 and 2018, respectively)	5,051	10,622	11,519	22,376
Total operating expenses	11,884	32,124	39,442	81,296
Other (income) expenses:				
Interest expense	1,313	1,932	2,871	3,902
Other (income) expense	560	(315)	(537)	353
Loss before income tax expense	(13,757)	(33,741)	(41,776)	(85,551)
Income tax expense	127	94	165	172
Net loss	$ (13,884)	$ (33,835)	$ (41,941)	$ (85,723)
Net loss per common share — basic and diluted	$ (0.61)	$ (2.24)	$ (1.84)	$ (6.00)
Weighted-average common shares outstanding — basic and diluted	22,783,182	15,107,932	22,781,657	14,295,301

[1] Includes total costs (benefit) allocated from certain wholly owned subsidiaries of RSL of $0 and $(3,069) for the three months ended September 30, 2019 and 2018, respectively, and $0 and $(450) for the six months ended September 30, 2019 and 2018, respectively.

[2] Includes total costs allocated from certain wholly owned subsidiaries of RSL of $48 and $772 for the three months ended September 30, 2019 and 2018, respectively, and $76 and $2,074 for the six months ended September 30, 2019 and 2018, respectively.

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

	September 30, 2019	**March 31, 2019**
Assets		
Current assets:		
Cash and cash equivalents	$ 60,255	$ 106,999
Prepaid expenses and other current assets	4,503	5,859
Income tax receivable	2,071	1,726
Total current assets	66,829	114,584
Long-term investment	5,871	5,871
Other non-current assets	46	973
Operating lease right-of-use assets	2,237	—
Property and equipment, net	1,081	1,278
Total assets	$ 76,064	$ 122,706

Liabilities and Shareholders' Equity

Current liabilities:		
Accounts payable	$ 1,567	$ 1,698
Accrued expenses	21,427	20,619
Current portion of operating lease liabilities	1,672	—
Current portion of long-term debt	22,613	21,182
Total current liabilities	47,279	43,499
Operating lease liabilities, net of current portion	3	—
Long-term debt	11,742	22,994
Total liabilities	59,024	66,493
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 September 30, 2019 and March 31, 2019, respectively	—	—
Additional paid-in capital	744,506	741,318
Accumulated deficit	(727,957)	(686,016)
Accumulated other comprehensive income	491	911
Total shareholders' equity	17,040	56,213
Total liabilities and shareholders' equity	$ 76,064	$ 122,706

Source: Axovant Sciences Ltd.