Axovant Announces Corporate Updates and Financial Results for First Fiscal Quarter Ended June 30, 2020

August 11, 2020

- ***Data readouts for AXO-Lenti-PD and AXO-AAV-GM1 remain on-track for Q4 2020***
- ***Completed 3-year manufacturing and supply agreement with Oxford BioMedica for AXO-Lenti-PD***
- ***Company had $55.5 million of cash and cash equivalents as of June 30, 2020***

NEW YORK and BASEL, Switzerland, Aug. 11, 2020 (GLOBE NEWSWIRE) -- Axovant Gene Therapies Ltd. (NASDAQ: AXGT), a clinical-stage gene therapy company developing innovative gene therapies for neurodegenerative diseases, today provided financial results for its first fiscal quarter ended June 30, 2020.

“During our first fiscal quarter, we continued to make significant progress across our three clinical-stage gene therapy development programs in Parkinson’s disease, GM1 gangliosidosis and Tay-Sachs/Sandhoff diseases and remain on-track for key data readouts later this year,” said Pavan Cheruvu, M.D., Chief Executive Officer of Axovant. “Notably this quarter, we completed Type II (Juvenile) patient enrollment in the low dose cohort of our dose-escalation study of AXO-AAV-GM1 and have begun preparations to initiate treatment of Type I (infantile) GM1 later this year. Additionally, our gene therapy manufacturing strategy has meaningfully progressed as we extended our supply collaboration with Oxford BioMedica around a new scalable, suspension-based manufacturing process to support our clinical studies and future commercialization of AXO-Lenti-PD. In the coming months, we look forward to presenting program updates across each of our three clinical-stage programs and will work tirelessly to execute our strategy on behalf of patients, families, caregivers and our shareholders.”

Key Highlights and Development Updates

AXO-Lenti-PD gene therapy for Parkinson’s disease

- Completed enrollment of the second dose cohort with the enrollment of four patients and remain on-track to deliver 6-month safety and efficacy data in Q4 2020.
- Entered into a clinical supply and manufacturing agreement with Oxford BioMedica to produce clinical trial material at-scale to support randomized, controlled Phase 2 and Phase 3 clinical studies and eventual commercialization.
- Anticipated completion of the first batch of AXO-Lenti-PD manufactured using a suspension-based process by year-end 2020.
- Enrollment of the first subject in a randomized, sham-controlled Phase 2 study of AXO-Lenti-PD anticipated in 2021.
- Presented an oral presentation at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting in May 2020 summarizing the mechanistic rationale and development status of AXO-Lenti-PD by Gregory Stewart, Ph.D., SVP, Scientific Affairs.

AXO-AAV-GM1 gene therapy for GM1 gangliosidosis

- Enrolled five Type II (juvenile) patients in the low-dose cohort of Stage 1 of the registrational study with 6-month safety and efficacy data expected in Q4 2020.
- Expanded protocol through successful IND amendment to enable dosing of Type I (infantile) patients and to explore a higher dose of 4.5×10^{13} vg/kg in both infantile and juvenile patients.
- Enrollment of both Type I and Type II patients at the low and high-dose cohorts expected to continue throughout the second half of 2020.
- Presented an oral presentation at the ASGCT Annual Meeting in May 2020 demonstrating the superior efficacy of intravenous GM1 gene therapy administration in the feline model from collaborator Amanda Gross from Auburn University (Poster #495).

AXO-AAV-GM2 gene therapy for Tay-Sachs and Sandhoff disease

- Company-sponsored investigational new drug (IND) application clearance is expected in Q4 2020.
- Presented clinical and preclinical data at the ASGCT Annual Meeting in May 2020 highlighting the mechanistic rationale for gene replacement in Tay-Sachs/Sandhoff disease and preliminary evidence of safety and efficacy from a first-in-human gene therapy study in Tay-Sachs disease.
  - Oral presentation on clinical data from two children treated in an expanded access clinical trial for the first-in-human gene therapy trial for Tay-Sachs Disease reported by Terence Flotte, M.D., Provost & Executive Deputy Chancellor, University of Massachusetts Medical School and Principal Investigator (Poster #1299).
  - Poster presentation on the surgical technique for bilateral intrathalamic infusion of rAAVrh8-*HEXA*/*HEXB* gene therapy in

an infant with Tay-Sachs Disease from collaborator Oguz Cataltepe, M.D., Professor, University of Massachusetts Medical School and Director of Pediatric Neurosurgery, UMass Memorial Medical Center (Poster #666).
- Oral presentation on preclinical data from intravenous AAV gene therapy demonstrating improved lifespan and clinical metrics in feline Sandhoff Disease from collaborator Anne Maguire from Auburn University (Poster #493).

Corporate Updates

- Axovant continues its corporate transformation to align corporate structure and governance with current and future business activity. Specific changes intended to occur by March 31, 2021 include:
  - Redomiciliation to Delaware from Bermuda
  - Plans to initiate a corporate name change of Axovant Gene Therapies Ltd.
  - Transition of the Board of Directors to a majority of independent members

First Fiscal Quarter Financial Summary

For the first fiscal quarter ended June 30, 2020, research and development expenses were $5.2 million, a decrease of $15.9 million compared to the prior year quarter. Excluding a development milestone of $13.0 million achieved and due to our partner, Oxford BioMedica (UK) Ltd., in the prior year quarter, research and development expenses decreased by $2.9 million, primarily related to the termination of our legacy AXO-AAV-OPMD program in September 2019.

General and administrative expenses for the first fiscal quarter ended June 30, 2020 were $4.6 million, a decrease of $1.9 million compared to the prior year quarter, primarily due to reductions in (i) personnel costs of $0.8 million and share-based compensation expense of $0.4 million attributable to reduced headcount and (ii) pharmaceutical market research costs of $0.6 million.

Net loss for the first fiscal quarter ended June 30, 2020 was $8.6 million, or $0.20 per share, compared to a net loss of $28.1 million, or $1.23 per share, in the prior year quarter. Net cash used in operating activities was $13.5 million for the first fiscal quarter ended June 30, 2020.

As of June 30, 2020, we had $55.5 million of cash and cash equivalents. The Company holds no short-term or long-term debt on the balance sheet. We expect the cash and cash equivalents to sustain our operations into the second calendar quarter of 2021.

About Axovant

Axovant Gene Therapies is a clinical-stage gene therapy company focused on developing a pipeline of innovative product candidates for debilitating neurodegenerative diseases. Our current pipeline of gene therapy candidates targets GM1 gangliosidosis, GM2 gangliosidosis (also known as 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.

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 "intended", "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 costs associated with its operating activities 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 impact of the Covid-19 pandemic on our operations, the initiation and conduct of preclinical studies and clinical trials; the availability of data from clinical trials; the scaling up of manufacturing, the expectations for regulatory submissions and approvals; the continued development of our 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 August 11, 2020, 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 June 30,	
	2020	**2019**
Operating expenses:		
Research and development expenses (includes $563 and $721 of share-based compensation expense for the three months ended June 30, 2020 and 2019, respectively)	$ 5,194	$ 21,090
General and administrative expenses (includes $1,027 and $1,414 of share-based compensation expense for the three months ended June 30, 2020 and 2019, respectively)	4,640	6,468
Total operating expenses	9,834	27,558
Other (income) expenses:		
Interest expense	796	1,558
Other income	(2,066)	(1,097)
Loss before income tax expense	(8,564)	(28,019)
Income tax expense	30	38
Net loss	$ (8,594)	$ (28,057)
Net loss per common share — basic and diluted	$ (0.20)	$ (1.23)
Weighted-average common shares outstanding — basic and diluted	43,287,222	22,780,114

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

	June 30, 2020	**March 31, 2020**
Assets		
Current assets:		
Cash and cash equivalents	$ 55,482	$ 80,752
Prepaid expenses and other current assets	3,998	2,971
Income tax receivable	1,717	1,707
Total current assets	61,197	85,430
Long-term investment	8,055	5,871
Other non-current assets	46	46
Operating lease right-of-use assets	1,105	1,532
Property and equipment, net	633	801
Total assets	$ 71,036	$ 93,680
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	$ 2,970	$ 4,412
Accrued expenses	8,949	11,319
Current portion of operating lease liabilities	465	889
Current portion of long-term debt	—	15,423
Total current liabilities	12,384	32,043
Operating lease liabilities, net of current portion	71	79
Total liabilities	12,455	32,122
Shareholders' equity:		
Common shares, par value $0.00001 per share, 1,000,000,000 shares authorized, 40,973,380 and 39,526,299 issued and outstanding at June 30, 2020 and March 31, 2020, respectively	—	—
Additional paid-in capital	825,830	820,257
Accumulated deficit	(767,238)	(758,644)
Accumulated other comprehensive loss	(11)	(55)
Total shareholders' equity	58,581	61,558
Total liabilities and shareholders' equity	$ 71,036	$ 93,680

Source: Axovant Sciences Ltd.