# Axovant Announces Corporate Updates and Financial Results for First Fiscal Quarter Ended June 30, 2019

August 9, 2019

- 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 calendar 2019
- Data from patients dosed with AXO-AAV-GM1 and AXO-AAV-GM2 expected in fourth quarter of calendar 2019
- Net loss for the quarter ended June 30, 2019 was $28.1 million, or $1.23 per share, compared to a net loss of $51.9 million, or $3.85 per share, for the prior-year quarter

BASEL, Switzerland, Aug. 09, 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 first quarter ended June 30, 2019.

“I’m proud of the continued progress across Axovant’s pipeline as we prepare to report data from all three of our clinical-stage programs in the fourth quarter of 2019, including results from the second cohort of our AXO-LENTI-PD study and data from additional children dosed with AXO-AAV-GM1 and AXO-AAV-GM2,” said Pavan Cheruvu, M.D., Chief Executive Officer of Axovant. “Our reduced expenses this quarter reflect ongoing efforts to streamline operations and drive long-term value for our shareholders. Additionally, our newly forged partnership with Yposkesi, a leading manufacturing group, provides us with sufficient manufacturing capacity for our AAV-based gene therapy pipeline. Finally, I’m pleased to welcome David Nassif and Senthil Sundaram, seasoned leaders with decades of biopharmaceutical industry experience, to help guide the company through an exciting period ahead.”

**Key Highlights and Development Updates**

- Initiated second dose cohort of the SUNRISE-PD study of AXO-LENTI-PD in April, and we expect initial data from up to six patients in this cohort in the fourth quarter of calendar 2019. In June, we provided a six-month update on the first cohort 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. At month six, the patients experienced an average improvement from baseline in UPDRS III (motor) OFF score of 17 points, representing an average improvement of 29%, and the Parkinson’s Disease Questionnaire-39 (“PDQ-39”) Summary Index score showed a 32-point reduction from baseline, an approximate 65% improvement.
- Preclinical data of AXO-LENTI-PD was published in *Molecular Therapy: Methods and Clinical Development*. The publication titled “Gene Therapy for Parkinson’s Disease: preclinical evaluation of an optimally configured TH:CH1 fusion for maximal Dopamine synthesis” reports safety and efficacy of AXO-LENTI-PD in the MPTP macaque model of Parkinson’s disease.
- Investigators recently dosed a second patient with AXO-AAV-GM2. The patient was a 7-month old with early symptomatic infantile Tay-Sachs disease, and AXO-AAV-GM2 was delivered into the thalamus bilaterally as well as into the cisterna magna and lumbar intrathecal space, the planned route of administration for patients in the program. We expect to enroll patients in a multi-subject clinical trial in the second half of calendar 2019 and into 2020 and expect to provide additional data in fourth quarter of calendar 2019.
- Dosed first patient in the AXO-AAV-GM1 clinical program, with initial data expected in the fourth quarter of calendar 2019. Enrollment of patients will continue throughout calendar 2019 and 2020.
- Entered into a strategic partnership with Yposkesi, a leading Contract Development and Manufacturing Organization (CDMO) spinout from Genethon, for preferred access and reserved capacity for cGMP grade viral vector production. Yposkesi will support our gene therapy programs as they proceed through development and commercialization.
- Appointed Mr. David Nassif as Chief Financial Officer and expanded our Board of Directors with the addition of Mr. Senthil Sundaram.
- Data from the AXO-AAV-GM2 and AXO-LENTI-PD programs were presented at the 22nd Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT) and 5$^{th}$Congress of the European Academy of Neurology (EAN).

**Fiscal First Quarter Financial Summary**

Research and development expenses were $21.1 million for the first fiscal quarter ended June 30, 2019, including a net payment of $13.0 million due to Oxford BioMedica (UK) Ltd. ("Oxford") for a development milestone achieved for our AXO-LENTI-PD program. Research and development expenses were $37.4 million for the first fiscal quarter ended June 30, 2018, including the $25.0 million upfront license fee paid to Oxford. Excluding the net development milestone achieved and upfront license fee paid, research and development expenses decreased by $4.3 million from $12.4 million in the first fiscal quarter ended June 30, 2018 to $8.1 million in the first fiscal quarter ended June 30, 2019, which was primarily due to the discontinuation of our intepirdine and nelotanserin small molecule drug programs.

General and administrative expenses decreased by $5.3 million from $11.8 million for the three months ended June 30, 2018 to $6.5 million in the three months ended June 30, 2019. The decrease was primarily related to reductions in i) professional fees of $2.3 million supporting business development and financing activities in the prior year, ii) share-based compensation expense of $1.9 million attributable to reduced headcount, and iii) costs allocated under the Roivant services agreements of $1.2 million as a result of the decentralization of the services provided to us. Going forward, the costs allocated to us under the services agreements are expected to be insignificant.

Net loss for the fiscal first quarter ended June 30, 2019 was $28.1 million, or $1.23 per share, based on a weighted-average of 22.8 million common shares outstanding, compared to a net loss of $51.9 million, or $3.85 per share, based on a weighted-average of 13.5 million common shares outstanding for the quarter ended June 30, 2018. Net cash used in operating activities for the fiscal first quarter ended June 30, 2019 was $17.7 million, or $16.7 million excluding $1.0 million paid to the University of Massachusetts Medical School for a development milestone achieved. Excluding milestone payments, we expect our net cash used in operations to be lower during the fiscal year ending March 31, 2020 than in the prior year due to a reduced operating cost structure, the discontinuation of our legacy small molecule drug programs and the termination of the license and collaboration agreement with Benitec Biopharma Limited.

As of June 30, 2019, we had $84.2 million of cash and cash equivalents, working capital of $37.7 million, total net debt of $39.4 million and long-term debt of $17.5 million. As of June 30, 2019, we had 22.8 million common shares outstanding.

**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.

**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 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 August 9, 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 June 30, | |
|---|---|---|
| | 2019 | 2018 |
| Operating expenses: | | |
| Research and development expenses[1] (includes $721 and $2,517 of share-based compensation expense for the three months ended June 30, 2019 and 2018, respectively) | $ 21,090 | $ 37,418 |
| General and administrative expenses[2] (includes $1,414 and $3,342 of share-based compensation expense for the three months ended June 30, 2019 and 2018, respectively) | 6,468 | 11,754 |
| Total operating expenses | 27,558 | 49,172 |
| Other expenses: | | |
| Interest expense | 1,558 | 1,970 |
| Other (income) expense | (1,097) | 668 |
| Loss before income tax expense | (28,019) | (51,810) |
| Income tax expense | 38 | 78 |
| Net loss | $ (28,057) | $ (51,888) |
| Net loss per common share — basic and diluted | $ (1.23) | $ (3.85) |
| Weighted-average common shares outstanding — basic and diluted | 22,780,114 | 13,473,740 |

[1] Includes total costs allocated from our parent company, Roivant Sciences Ltd. ("RSL") and certain of its wholly owned subsidiaries of $0 and $2,619 for the three months ended June 30, 2019 and 2018, respectively.

[2] Includes total costs allocated from RSL and certain of its wholly owned subsidiaries of $28 and $1,302 for the three months ended June 30, 2019 and 2018, respectively.

**AXOVANT GENE THERAPIES LTD.**

**Condensed Consolidated Balance Sheets**

*(Unaudited, in thousands, except share and per share amounts)*

| | June 30, 2019 | March 31, 2019 |
|---|---|---|
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 84,176 | $ 106,999 |
| Prepaid expenses and other current assets | 5,657 | 5,859 |
| Income tax receivable | 1,916 | 1,726 |
| Total current assets | 91,749 | 114,584 |
| Long-term investment | 5,871 | 5,871 |
| Other non-current assets | 46 | 973 |
| Operating lease right-of-use assets | 2,664 | — |
| Property and equipment, net | 1,115 | 1,278 |
| Total assets | $ 101,445 | $ 122,706 |
| | | |
| **Liabilities and Shareholders' Equity** | | |
| Current liabilities: | | |
| Accounts payable | $ 2,896 | $ 1,698 |
| Accrued expenses | 27,698 | 20,619 |
| Current portion of operating lease liabilities | 1,586 | — |
| Current portion of long-term debt | 21,855 | 21,182 |
| Total current liabilities | 54,035 | 43,499 |
| Operating lease liabilities, net of current portion | 525 | — |
| Long-term debt | 17,533 | 22,994 |
| Total liabilities | 72,093 | 66,493 |
| | | |
| Shareholders' equity: | | |
| Common shares, par value $0.00001 per share, 1,000,000,000 shares authorized, 22,780,672 and 22,779,891 issued and outstanding at June 30, 2019 and March 31, 2019, respectively | — | — |
| Additional paid-in capital | 743,486 | 741,318 |
| Accumulated deficit | (714,073) | (686,016) |
| Accumulated other comprehensive (loss) income | (61) | 911 |
| Total shareholders' equity | 29,352 | 56,213 |
| Total liabilities and shareholders' equity | $ 101,445 | $ 122,706 |

Source: Axovant Sciences Ltd.