Axovant Announces 6-Month Follow-Up Data From First Cohort of SUNRISE-PD Phase 2 Trial of AXO-Lenti-PD and Pipeline Update

June 6, 2019

- 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
- Totality of data across the program at 6 months supports overall favorable product profile
- Initial three-month data from second dose cohort of SUNRISE-PD Phase 2 study is expected in the fourth quarter of 2019

BASEL, Switzerland, June 06, 2019 (GLOBE NEWSWIRE) -- Axovant Gene Therapies, Ltd. (Nasdaq: AXGT), a clinical-stage company developing innovative gene therapies, today reported six-month follow-up data from the first dose cohort in the open-label, dose-escalation portion of the ongoing SUNRISE-PD Phase 2 trial of AXO-Lenti-PD for the treatment of Parkinson's disease. AXO-Lenti-PD was observed to be generally well tolerated, with no serious adverse events related to the product or the procedure and patients showed continued improvement from baseline across multiple measurements. In addition, the Company is providing a pipeline update.

AXO-Lenti-PD 6-month data

"We continue to be encouraged by the consistency of the data and improvements in quality of life seen at six months in the two low-dose cohort patients, as we enroll additional patients in the second cohort of the SUNRISE-PD study," said Dr. Gavin Corcoran, Axovant's Chief Research and Development Officer. "Our patient-focused goal of improving motor function, reducing dyskinesia, lowering the requirement for oral levodopa, and improving quality of life is made possible by the continuous dopamine replacement strategy of AXO-Lenti-PD gene therapy. These data at six months highlight the potential for a clinically meaningful improvement over the currently available standard of care for those patients with moderate to advanced Parkinson's disease."

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%. Individual patient improvements at 6 months were 14 and 20 points, respectively. The patients experienced an average improvement of approximately 20 points from baseline on the UPDRS Part II (activities of daily living) OFF score, and an average improvement of 3 points from baseline on the UPDRS Part IV (complications of therapy) OFF score.

The average levodopa equivalent daily dose (LEDD) at baseline was 1117mg and at month six was 884mg, a decrease of 233 mg, or 21% from baseline. On the Rush Dyskinesia Rating Scale, at month six, patients experienced a mean 18% improvement in functional disability during activities of daily living while on oral levodopa.

The Parkinson's Disease Questionnaire-39 ("PDQ-39") Summary Index score, a patient and caregiver reported quality of life measure was recorded at baseline, month three and month six. The average score at baseline was 50 points, which at month three had improved to an average score of 31 points (a reduction of 19 points from baseline). At month 6, the average PDQ-39 Summary Index score was further improved to 18 points (a reduction of 32 points from baseline). These scores demonstrate an approximate 37% improvement from baseline at month three and an approximate 65% improvement from baseline at month six. These reductions in total score at both three and six months appear to indicate a substantial clinical benefit in this quality of life measure.

In addition, a patient-recorded diary was collected again at six months. On average, the patients experienced an improvement from baseline of ON time without dyskinesia of 2.7 hours, a reduction of 2.4 hours in ON time with non-troublesome dyskinesias, a reduction of ON time with troublesome dyskinesias of 1.5 hours, and an increase in OFF time of 0.9 hours.

Pipeline Updates

Axovant announced today that it has terminated the license and collaboration agreement with Benitec Biopharma Limited in its entirety. This agreement included AXO-AAV-OPMD, in preclinical development for the treatment of oculopharyngeal muscular dystrophy (OPMD), as well as discovery-stage research collaboration programs including amyotrophic lateral sclerosis (ALS) and frontotemporal dementia.

In May 2019, Axovant announced dosing of the first child with GM1 gangliosidosis with AXO-AAV-GM1, and expects data in the second half of 2019. In April 2019, the first patient in the second cohort of the SUNRISE-PD clinical study was dosed and Axovant expects initial three-month data in the fourth quarter of 2019. In March 2019, Axovant reported three-month data from the first child dosed with AXO-AAV-GM2, and expects to report data from additional patients in the second half of 2019.

Pavan Cheruvu, M.D., Chief Executive Officer of Axovant, commented: "We are committed to our mission of developing clinical-stage gene therapies for the treatment of neurological diseases. Based on the encouraging data seen in the Parkinson's disease and GM2 gangliosidosis programs, the team will focus its efforts on our clinical programs."

About AXO-Lenti-PD

AXO-Lenti-PD is an investigational gene therapy for the treatment of Parkinson's disease that is designed to deliver three genes (tyrosine hydroxylase, cyclohydrolase 1, and aromatic L-amino acid decarboxylase) via a single lentiviral vector to encode a set of critical enzymes required for dopamine synthesis, with the goal of reducing variability and restoring steady levels of dopamine in the brain. The investigational gene therapy aims to provide patient benefit for years following a single administration.

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. 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 and Information

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," "will," "expect," "would," "intend," "future," and other similar expressions are intended to identify forward-looking statements. All forward-looking statements are based on estimates and assumptions by Axovant's management that, although Axovant believes to be reasonable, are inherently uncertain, including the timing of data from AXO-AAV-GM1, AXO-AAV-GM2 and AXO-Lenti-PD clinical programs. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that Axovant expected. In addition, Axovant's business is subject to additional risks and uncertainties, including among others, the initiation and conduct of preclinical studies and clinical trials; the timing and availability of data from clinical trials; the expectations for regulatory submissions and approvals; any potential safety concerns or profile of Axovant's product candidates; and the availability or commercial potential of 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 for the quarterly period ended December 31, 2018, filed with the Securities and Exchange Commission on February 7, 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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