Axovant Announces Clinical Updates from AXO-AAV-GM2 and AXO-Lenti-PD Studies

March 29, 2019

-R&D Day featuring leading physicians and scientists in Parkinson's disease, GM1 gangliosidosis and GM2 gangliosidosis (Tay-Sachs disease) will be webcast today at 8:30a.m. ET from New York-

-Approximately 25% decrease in GM2 ganglioside in CSF observed at end of three-month period in the first child dosed with AXO-AAV-GM2-

-Interim data from additional patients in the AXO-AAV-GM2 program expected in H2 2019-

-First patient expected to be dosed in second cohort of SUNRISE-PD study of AXO-Lenti-PD in Q2 2019-

BASEL, Switzerland, March 29, 2019 (GLOBE NEWSWIRE) -- Axovant Gene Therapies Ltd. (NASDAQ: AXGT), a clinical-stage company developing innovative gene therapies, today announced additional data and plans for the AXO-AAV-GM2 and AXO-Lenti-PD programs, which will be highlighted at the Company's R&D day for investors and analysts.

AXO-AAV-GM2 for Tay-Sachs disease
Axovant recently reported three-month data from an investigator-initiated study administering investigational AXO-AAV-GM2 gene therapy in a patient with advanced infantile Tay-Sachs disease. Additional independent assays and samplings were conducted to further evaluate the biological activity of AXO-AAV-GM2. There was a reduction of approximately 25% in GM2 ganglioside from baseline in the cerebral spinal fluid (CSF). This suggests that the previously announced approximately 3-fold increase from baseline in β-Hexosaminidase A enzyme activity in the CSF is associated with a decrease in GM2 ganglioside, which accumulates in Tay-Sachs disease patients and is believed to cause disease progression.

Dr. Terence Flotte, a principal investigator on the AXO-AAV-GM2 study, will provide a more detailed discussion of these findings at Axovant's R&D day.

Interim data from additional patients is expected in the second half of 2019.

AXO-Lenti-PD for Parkinson's disease

Axovant's previously reported interim results from the first cohort of the SUNRISE-PD Phase 2 study of AXO-Lenti-PD in Parkinson's disease patients demonstrated that the product was generally well-tolerated with no serious adverse events reported at the low dose (4.2×10^6 TU). In addition, consistent improvements over the study period were observed across multiple motor function and dyskinesia scales, including improvements on the UPDRS Part III OFF score of 14-points and 36-points, respectively, in the two patients studied in the low-dose cohort. Axovant plans to proceed to the second cohort of the SUNRISE-PD study, at a higher dose of 1.4×10^7 TU, with the first subject expected to be dosed in the second quarter of 2019.

R&D Day Webcast Information

The R&D Day event will be webcast live on March 29, 2019 at 8:30am ET on the investor relations section of Axovant's website at www.axovant.com. An archived webcast will be available on Axovant's website for 30 days following the event.

About Axovant Gene Therapies

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), Parkinson's disease, oculopharyngeal muscular dystrophy (OPMD), amyotrophic lateral sclerosis (ALS) and frontotemporal dementia. 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," "might," "will," "expect," "plan," "anticipate," "believe," "intend," "future," or "continue" and other similar expressions are intended to identify forward-looking statements. For example, all statements Axovant makes regarding the following are forward looking: the success and timing of its ongoing development of AXO-Lenti-PD, AXO-AAV-GM2 and its other product candidates; the anticipated start dates, durations and completion dates of its ongoing and future clinical trials; the anticipated designs of its future clinical studies; and the success of its interactions with the FDA. In addition, promising interim results or other preliminary analyses do not in any way ensure that later or final results in a clinical trial or in related or similar clinical trials will replicate those interim results. The product candidates discussed are investigational and not approved and there can be no assurance that the clinical programs will be successful in demonstrating safety and/or efficacy, that Axovant will not encounter problems or delays in clinical development, or that any product candidate will ever receive regulatory approval or be successfully commercialized. 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 timing and availability of data from clinical trials; the expectations for regulatory submissions and approvals; the 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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