Axovant Gene Therapies to Present AXO-Lenti-PD Program Update During Virtual Parkinson's Disease R&D Day on October 30, 2020

October 29, 2020

- Patient-level data in cohort 2 of SUNRISE-PD study demonstrates consistent, clinically meaningful outcomes and evidence of dose response
- Delays in manufacturing process expected to postpone the start of enrollment in randomized, controlled study
- Virtual R&D Day webcast on October 30th at 11:30 AM Eastern time

NEW YORK, Oct. 29, 2020 (GLOBE NEWSWIRE) -- Axovant Gene Therapies Ltd. (Nasdaq: AXGT), a clinical-stage company developing innovative gene therapies, today announced program updates to be presented as part of its virtual R&D Day on Friday, October 30, 2020 at 11:30 AM Eastern time, for the Company's AXO-Lenti-PD gene therapy for Parkinson's disease.

Axovant's Parkinson's disease R&D Day will be moderated by Chief R&D Officer, **Gavin Corcoran, M.D.**, and will feature presentations on the current treatment landscape, unmet medical need for people living with Parkinson's disease, and a summary of data from the second cohort of the Phase 2 SUNRISE-PD trial for AXO-Lenti-PD.

AXO-Lenti-PD Program Updates

- Summary of available individual patient-data from the second dose cohort at the 6-month timepoint following one-time dosing with AXO-Lenti-PD gene therapy demonstrate consistent treatment outcomes including:
  - Favorable safety and tolerability profile, with no serious adverse events attributable to gene therapy
  - Improvement in Hauser diary "Good ON time" and "OFF time" changes from baseline for all 4 patients
  - Improvement in UPDRS Part II and Part III "OFF" score in 2 evaluable patients
  - Reduction in Levodopa-equivalent daily dose (LEDD) from baseline
- 23-point improvement from baseline to 12 months in UPDRS Part III "OFF" score observed for the first patient in cohort 2 who has reached the 12-month evaluation, which was performed as a remote assessment.
- Based on new information received from our manufacturing partner, Oxford Biomedica, in mid-October regarding delays in CMC data and third-party fill/finish issues, the development of a suspension-based manufacturing process for AXO-Lenti-PD will take longer than expected. As a result, the Company believes that it is unlikely that its planned randomized, sham-controlled trial of AXO-Lenti-PD will enroll patients by the end of calendar year 2021. Manufacturing of several GMP batches is now underway and planned at Oxford Biomedica with a goal of generating material for use in future clinical trials as soon as possible. The Company expects to provide an update on program timelines in the first quarter of 2021 or as program timelines are clarified.

Dr. Stéphane Palfi, M.D., Ph.D., Professor of Neurosurgery and Head of the Neurosurgery Department at Henri Mondor Medical Center, Paris University, commented, "As an investigator in this program for several years, I am grateful to all the patients and families who have participated in the study. I am increasingly encouraged by the emerging body of evidence generated so far in the SUNRISE-PD study as suggestive of clinically meaningful improvements and dose-dependent responses following one-time administration of AXO-Lenti-PD gene therapy. New therapeutic approaches are urgently needed to address the suffering and disability of patients with Parkinson's disease, and I believe AXO-Lenti-PD has the potential to meaningfully improve their lives."

R&D Day Information

- To register for the R&D webcast, please click here.
- An 8-K with a copy of the R&D Day presentation has been filed with the Securities and Exchange Commission (SEC).
- A live audio webcast of the R&D Day can be accessed through the Events & Presentations section of the company's website at investors.axovant.com. An archived replay of the webcast will be available on the company's website following the event.

About Axovant Gene Therapies

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 target 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 "will," "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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