Axovant Announces Positive Six-Month Follow-Up Data From Second Cohort of SUNRISE-PD Phase 2 Trial of AXO-Lenti-PD Gene Therapy

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• AXO-Lenti-PD was well-tolerated with no treatment-related serious adverse events at 6 months
• 21-point mean improvement in UPDRS Part III "OFF" score, a 40% improvement from baseline, exceeding pre-defined criteria for success
• Greater than 2-hour improvement from baseline in both diary "good ON time" and diary OFF time assessments
• EXPLORE-PD, a randomized, sham-controlled study of AXO-Lenti-PD is expected to begin dosing in 2021

NEW YORK, Oct. 06, 2020 (GLOBE NEWSWIRE) -- Axovant Gene Therapies Ltd. (NASDAQ: AXGT), a clinical-stage gene therapy company developing innovative gene therapies for neurodegenerative diseases, today reported six-month follow-up data from the second cohort of patients receiving a total dose of 1.4×10^7 TU of gene therapy in the open-label, dose-escalation SUNRISE-PD Phase 2 trial of AXO-Lenti-PD for the treatment of Parkinson's disease.

"These data, showing over 20 points of improvement on the UPDRS Part III 'OFF' motor function score, as well as meaningful improvements in quality of life measures, underscores the potentially best-in-class profile of AXO-Lenti-PD gene therapy in Parkinson's disease. The totality of data seen thus far reinforces my belief that AXO-Lenti-PD has the potential to transform the treatment of patients with Parkinson's disease through one-time administration of gene therapy. Building on these encouraging results, we expect to begin dosing in EXPLORE-PD, a randomized, sham-controlled study in 2021, and will additionally evaluate the safety and tolerability of higher volumes of infusion. We look forward to sharing additional data and program updates at our upcoming Parkinson's disease R&D Day on October 30," said Gavin Corcoran, M.D., Chief R&D Officer at Axovant.

Key study results at 6 months follow-up:

- AXO-Lenti-PD was observed to be generally well-tolerated in all 4 patients receiving gene therapy, with no serious adverse events attributable to the vector at six months after a single administration. The 4 subjects had an average age of 57 years and an average duration of Parkinson's disease of 13 years.
- Two evaluable patients in Cohort 2 demonstrated a 21-point mean improvement in the UPDRS Part III "OFF" score, which assesses motor function, representing a 40% improvement from the baseline average score of 52 in these patients.
  ° Improvement in the UPDRS Part III "OFF" score in AXO-Lenti-PD Cohort 2 exhibited evidence of dose response when compared to Cohort 1 (n=2) and the low (n=3), medium (n=6), and high (n=6) dose cohorts of ProSavin that were previously evaluated in a separate Phase 1/2 study.
- Because of COVID-19 and a patient refusal, 2 out of 4 patients in the cohort at our U.K. clinical trial sites were unable to participate in UPDRS assessments and the mandatory washout of background levodopa therapy at the 6-month time point. However, all 4 subjects were able to complete all other efficacy assessments at 6 months, including the patient-recorded Hauser diaries. The Company is working with sites and investigators to ensure safe and ethical data collection at future time points through the pandemic in accordance with regulatory guidance.
- Diary "good ON time," defined as the sum of ON time without dyskinesias and ON time with non-troublesome dyskinesias, improved an average of 2.2 hours (baseline good ON time 10.2 hours) across the four patients.
  ° 100% of patients (4 out of 4) demonstrated improvement from baseline in diary good ON time.
- Diary OFF time, defined as time when medication has worn off and is no longer providing benefit with regards to mobility, improved an average of 2.3 hours (baseline OFF time 5.8 hours) across the four patients.
  ° 100% of patients (4 out of 4) demonstrated improvement from baseline in diary OFF time.
- Two evaluable patients in Cohort 2 demonstrated a 14-point mean improvement in the UPDRS Part II "OFF" score, which assesses activities of daily living, representing a 71% improvement from baseline.

With the completion of this cohort, the Company expects to investigate the safety and tolerability of a higher volume and flow rate to increase putaminal coverage and decrease operating room time (Cohort 3). After successful development of the suspension-based manufacturing process, Axovant expects to dose the first patient in EXPLORE-PD in 2021. Additional program updates will be provided at Axovant's Parkinson's disease R&D Day on October 30, 2020.

Pavan Cheruvu, MD, Chief Executive Officer at Axovant, added, "We are highly encouraged by the growing evidence of dose response and apparent clinical improvement observed in the SUNRISE-PD study, combined with a favorable safety profile. These results exceed our pre-defined base case criteria of 10-15 points of improvement from baseline on the UPDRS Part III 'OFF' score and are above a threshold considered clinically meaningful in the medical literature and as compared to standard of care. Although the COVID-19 pandemic impacted our ability to collect full UPDRS data from the cohort, the safety of our patients and staff is our first priority, and we have begun work on validating new technologies that may enable remote-based clinical assessments at future time points in order to obtain complete data sets. Based on the data seen thus far, we are excited to advance our clinical development program with the first randomized, controlled study to evaluate the continuous dopamine replacement strategy of AXO-Lenti-PD gene therapy in Parkinson's disease in 2021."

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. Axovant expects to dose the first patient in EXPLORE-PD, a randomized, sham controlled study in 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 "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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