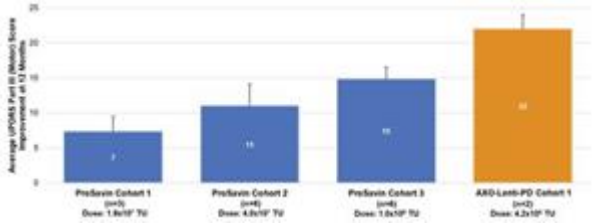
Axovant Announces Positive 12-month Data on AXO-Lenti-PD and Provides Updates Across Gene Therapy Pipeline Programs

January 13, 2020

NEW YORK and BASEL, Switzerland, Jan. 13, 2020 (GLOBE NEWSWIRE) -- Axovant Gene Therapies Ltd. (NASDAQ: AXGT), a clinical-stage company developing innovative gene therapies for neurological diseases, today announced recent progress in its gene therapy programs, including a 12-month update on its Parkinson's program.



Average UPDRS Part III (motor) "OFF" score change from baseline to 12 months (cross-study comparison)

Major 2019 Accomplishments and Upcoming 2020 Milestones

AXO-Lenti-PD gene therapy in Parkinson's disease:

- At 12 months post-dosing, patients in the first dose cohort (4.2 x 10^6 TU) demonstrated an average 22-point change from baseline in motor function as assessed by the UPDRS Part III "OFF" score, which represents a 37% improvement. Individual patient improvements were 24-points and 20-points, respectively. Previously, at 6 months post-dosing, these patients demonstrated an average 17-point change from baseline, or 29% improvement, on the same scale. The 12-month timepoint is considered an important timeframe for assessment of therapeutic response, differentiation from sham/placebo effect, and durability of gene therapy in Parkinson's disease.
  • AXO-Lenti-PD was observed to be well-tolerated with no serious adverse events attributable to the gene therapy.
  • Improvement in the UPDRS Part III "OFF" score in the first cohort exhibited evidence of dose response when compared to 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 at 12 months (see Exhibit 1).
  • The UPDRS Part II "OFF" score, which assesses activities of daily living, showed a 13-point average change from baseline, or a 44% improvement from baseline to 12 months.
  • Only one of two patients in the first cohort was able to record a Hauser diary. Improvements were observed across various diary measures from baseline to 12 months for the single patient.
  • The PDQ-39 score index, a well-validated quality of life measure in Parkinson's disease, demonstrated an average 15-point change from baseline, or 30% improvement from baseline to 12 months.
- Enrollment continues in the second dose cohort of the SUNRISE-PD Phase 2 study of AXO-Lenti-PD. In Q1 2020, Axovant expects to present initial 6-month data from the first two patients in the second cohort (1.4 x 10^7 TU) who were administered a 3-fold higher dose of vector than the first dose cohort.
- Based on the outcome of the dose-escalation part of the study and successful development of a suspension-based manufacturing process, Axovant expects to initiate the randomized, sham-controlled part of the SUNRISE-PD Phase 2 study by the end of calendar year 2020.
- A recent placebo-controlled study in a non-human primate model of Parkinson's disease published in *Molecular Therapy: Methods and Clinical Development* (Badin et.al., 2019) compared two doses of AXO-Lenti-PD against control-group animals receiving a null vector. The study demonstrated statistically significant differences in PD clinical response scores at 6-months in this diseased-animal model ($p<0.0002$ for AXO-Lenti-PD vs. control), dose-dependent increases in PET signaling using a 6-FMT radiotracer ($p<0.001$ for AXO-Lenti-PD vs. control), and dose-dependent increases in gene expression for AADC, TH, and CH1 in transduced striatal tissue.

AXO-AAV-GM1 gene therapy in GM1 gangliosidosis:

- Reported data from the first child dosed with AXO-AAV-GM1 under an expanded access protocol, which suggested tolerability and clinical improvement between baseline and 6-months after vector administration with no reports of serious adverse events attributable to the gene therapy.
- Continued enrollment in Part A of the registrational study of late infantile and juvenile onset (Type II) GM1 patients evaluating safety, tolerability, and exploratory measures of efficacy. The study remains on track to report data from Part A in mid-2020.
- Granted orphan drug designation (ODD) by the U.S. Food and Drug Administration (FDA) in November 2019.
- Axovant co-authored the first comprehensive retrospective study characterizing the natural history of Type 1 GM1 gangliosidosis in *Molecular Genetics and Metabolism* (Lang et.al., 2019) together with collaborators at the National Institutes of Health (NIH). This paper describes a rapidly progressive clinical course of Type 1 GM1 gangliosidosis, in which almost all patients experience significant multi-organ system dysfunction and neurodevelopmental regression between 6 and 18 months of age.

AXO-AAV-GM2 gene therapy in Tay-Sachs and Sandhoff diseases:

- In October 2019, at the *European Society of Gene and Cell Therapy (ESGCT) 27th Annual Congress*, Dr. Terry Flotte presented evidence of clinical disease stabilization in two patients dosed with AXO-AAV-GM2 under an investigator-initiated IND. Initial observations suggested that AXO-AAV-GM2 was generally well-tolerated and associated with improvement in bioactivity outcomes. In addition, data suggested attainment of normal neurodevelopmental milestones and improvement in myelination on brain MRI.
- The Company submitted an IND in late 2019 to support the initiation of Axovant's registrational clinical trial in patients with GM2 gangliosidosis. Following the review of the IND, the FDA did not raise concerns related to animal toxicology or safety from the investigator-sponsored study. Rather, due to CMC and device-related questions posed by the FDA beyond the 30-day review period, the Agency placed the IND on clinical hold. Pending clearance by FDA, Axovant expects to initiate the AXO-AAV-GM2 trial in 2020.

"In 2019, Axovant advanced its vision of creating a new genetic medicines company around an experienced leadership team, innovative gene therapy pipeline, and patient-focused mission. We are unique in having three promising clinical-stage gene therapy programs focused on life-threatening neurodegenerative diseases," said Pavan Cheruvu, M.D., Chief Executive Officer of Axovant. "I am particularly excited to share a new 12-month data update on safety and efficacy from the first dose cohort of our Parkinson's disease program, which highlights the potential for AXO-Lenti-PD gene therapy to produce both durable and clinically meaningful improvements over the currently available standard of care in Parkinson's disease. We believe that a 12-month evaluation is a key regulatory timepoint in the assessment of treatment durability and separation from placebo effect. In the first quarter of 2020, we expect to present 6-month data from two patients in the second cohort treated with a higher dose of AXO-Lenti-PD. Axovant is committed to advancing each of our gene therapy programs with a sense of urgency on behalf of our patient communities. We believe we are well-positioned to deliver on the promise of these programs for patients and families affected by Parkinson's disease, GM1 gangliosidosis, and Tay-Sachs and Sandhoff diseases."

About Axovant Gene Therapies

Axovant Gene Therapies, 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 neurodegenerative 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.

In 2018, Axovant licensed exclusive worldwide rights from the University of Massachusetts Medical School (UMMS) for the development and commercialization of gene therapy programs for GM1 gangliosidosis and GM2 gangliosidosis, including Tay-Sachs and Sandhoff diseases.

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; costs associated with its 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 BioMedica, the University of Massachusetts Medical School, and the National Institutes of Health (NIH); and the timing and likelihood of its regulatory filings and agency interactions to advance its gene therapy programs and clinical studies, are all 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; interactions and submissions with regulatory agencies; 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 November 8, 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.

Media and Investor Contact:

Parag Meswani
Axovant Gene Therapies
(212) 547-2523
investors@axovant.com
media@axovant.com

A photo accompanying this announcement is available at https://www.globenewswire.com/NewsRoom/AttachmentNg/5e9dccfe-81e4-43e4-9d18-2f5e49111e72

Source: Axovant Sciences Ltd.