Axovant Reports Positive Interim Results From First Cohort Of SUNRISE-PD Phase 2 Trial Of AXO-Lenti-PD Gene Therapy In Parkinson's Disease

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- 25-point (42%) mean improvement in the UPDRS Part III (motor) OFF score, with individual patient improvements of 14-points and 36-points respectively
- Benefit seen across all UPDRS OFF subscales at 3 months
- Reductions in ON state dyskinesias seen on the Rush Dyskinesia Rating Scale and diary ON time with dyskinesia
- Generally well-tolerated with no serious adverse events at 3 months
- First patient in second cohort expected to be dosed in Q2 2019
- Axovant to present at the Cowen and Company 39th Annual Health Care Conference on March 11th at 12:00 PM (Eastern Time)

NEW YORK and BASEL, Switzerland, March 11, 2019 (GLOBE NEWSWIRE) -- Axovant (Nasdaq: AXGT), a clinical-stage company developing innovative gene therapies, today reported 3-month 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. The cohort consisted of two patients with advanced Parkinson's disease who received a one-time administration of the lowest dose of AXO-Lenti-PD ($4.2x10^6$ TU). AXO-Lenti-PD was generally well-tolerated, and no serious adverse events were reported.

"Our focus in this first cohort of the SUNRISE-PD study was on the safety and tolerability of AXO-Lenti-PD, as well as the evaluation of efficacy using well-validated, objective measures. These early data support the safety of the lowest dose of AXO-Lenti-PD, similar to what was observed with the earlier generation construct, ProSavin, and also suggest substantially greater biological activity than the highest dose of ProSavin previously tested," said Dr. Gavin Corcoran, Axovant's Executive Vice President of Research and Development. "These findings are highly encouraging, and we look forward to advancing to higher dose cohorts where we will explore the full clinical potential of AXO-Lenti-PD in patients with Parkinson's disease."

The Unified Parkinson's Disease Rating Scale (UPDRS) Part III score is a physician-rated scale assessing motor function, ranging from 0 to 108 with lower scores indicating improvement. The OFF score is assessed after patients are washed out of their oral levodopa therapy, thereby capturing the benefit of therapy without the potentially confounding effect of background medical treatment. Patients in the first cohort experienced an average UPDRS Part III (motor) OFF score improvement of 25 points at 3 months after administration of AXO-Lenti-PD, representing an average improvement of 42% from baseline. Individual patient improvements at 3 months were 14 points and 36 points, respectively.

Improvements were seen across all subparts of the UPDRS scale, with an average UPDRS Total OFF score improvement of 54.5 points at 3 months after receiving AXO-Lenti-PD, representing an average improvement of 55% from baseline. Patients experienced an average improvement of 22 points from baseline on the UPDRS Part II (activities of daily living) OFF score, and an average improvement of 7 points from baseline on the UPDRS Part IV (complications of therapy) OFF score.

Taken together, these results suggest that the lowest dose of AXO-Lenti-PD at 3 months may have greater efficacy compared to the highest dose of ProSavin previously tested. A detailed summary of results on the UPDRS OFF scale for AXO-Lenti-PD from the SUNRISE-PD study and for ProSavin from a prior clinical trial is shown in Table 1 below.

Table 1[†]

Measure	**AXO-Lenti-PD Cohort 1 (Low Dose: $4.2x10^6$ TU) at 3 months (N=2)***	**ProSavin Cohort 3 (High Dose: $1.0x10^8$ TU) at 3 months (N=6)****
UPDRS Total OFF Score. Range from 0 to 199		
Baseline	99.0	79.3
Month 3	44.5	61.3
Improvement from Baseline	*54.5*	*18.0*
UPDRS Part I OFF Score (Mentation, Behavior and Mood). Range from 0 to 16		
Baseline	1.0	2.5
Month 3	0.5	0.8
Improvement from Baseline	*0.5*	*1.7*
UPDRS Part II OFF Score (Activities of Daily Living). Range from 0 to 52		
Baseline	29.5	21.7
Month 3	7.5	19.3
Improvement from Baseline	*22.0*	*2.3*
UPDRS Part III OFF Score (Motor). Range from 0 to 108		
Baseline	59.0	44.8
Month 3	34.0	34.0

Improvement from Baseline	*25.0*	*10.8*
UPDRS Part IV OFF Score (Complications of Therapy). Range from 0 to 23		
Baseline	9.5	10.3
Month 3	2.5	7.2
Improvement from Baseline	*7.0*	*3.2*

† **These data are based on a cross-trial comparison and not a head-to-head clinical trial, and as a result, these data may not be directly comparable**
*** Ongoing SUNRISE-PD Phase 2 trial of AXO-Lenti-PD**
**** Prior Phase 1/2 open-label trial of ProSavin**

Patients in Cohort 1 also experienced a mean 18% improvement in dyskinesia as measured by the Rush Dyskinesia Rating Scale ON score, which assesses functional disability during activities of daily living while patients are on oral levodopa.

Additionally, a patient-recorded diary was collected from patients in Cohort 1. Although variability was present in the patient-recorded diary entries, both patients exhibited improvement in diary ON time with dyskinesia, with an average reduction of 3.5 hours (57%) from baseline. An improvement was also observed in diary ON time with troublesome dyskinesia, with an average reduction of 1.3 hours (85%) from baseline. Results from the patient-recorded diary are summarized in Table 2 below.

Table 2

Diary Measure	**Baseline**	**Month 3**	**Improvement from Baseline**
ON time without dyskinesia	8.0 hours	10.1 hours	2.1 hours
ON time with dyskinesia	6.1 hours	2.6 hours	3.5 hours
ON time without troublesome dyskinesia	12.5 hours	12.4 hours	-0.1 hours
ON time with troublesome dyskinesia	1.5 hours	0.2 hours	1.3 hours
OFF time	1.8 hours	3.4 hours	-1.6 hours

At month 3, the average levodopa equivalent daily dose (LEDD) was decreased by 208 mg for patients in Cohort 1. This represents an average reduction of 19% from baseline.

Based on initial feedback received from members of the Data Monitoring Committee (DMC), Axovant plans to proceed to the planned second dose cohort of AXO-Lenti-PD. The dose to be tested in the second cohort of patients is $1.4x10^7$ TU. The first subject in this second cohort is expected to be dosed in the second quarter of 2019.

“This early data suggests that AXO-Lenti-PD has the potential to significantly improve motor function in patients with advancing Parkinson’s disease. The mechanism of action of AXO-Lenti-PD, which is designed to deliver all three genes necessary for endogenous dopamine biosynthesis, as well as our prior clinical experience with ProSavin, led us to expect that the major benefit would be in improving the OFF state – and the results so far are very encouraging in this regard,” said Dr. Roger Barker, one of the principal investigators on the SUNRISE-PD Phase 2 study and Professor of Clinical Neuroscience and Honorary Consultant in Neurology at the University of Cambridge and Addenbrooke's Hospital. “I am hopeful that this development program will translate into a significant new therapeutic option for patients with Parkinson’s disease.”

Pavan Cheruvu, M.D., chief executive officer of Axovant, will present at the Cowen and Company 39th Annual Health Care Conference on March 11, 2019 at 12:00 PM (Eastern Time). A copy of the Company’s presentation slides and link to a live webcast of the presentation can be found below.

http://investors.axovant.com/investors

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), 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; 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 candidate discussed is 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 the 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 availability of data from clinical trials; the expectations for regulatory submissions and approvals; the availability or commercial potential of product candidates; and receipt of confirmatory concurrence from the DMC regarding the planned second dose cohort of AXO-Lenti-PD. 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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