# Axovant Announces Dosing of First Patient in Clinical Study of AXO-Lenti-PD, a Novel Gene Therapy for Patients With Parkinson's Disease

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**Initial data from the first cohort expected in first half of 2019**

BASEL, Switzerland, Oct. 25, 2018 (GLOBE NEWSWIRE) -- Axovant Sciences (NASDAQ:AXON), a company developing innovative gene therapies for neurologic and neuromuscular diseases, today announced dosing of the first patient in a clinical study of AXO-Lenti-PD (also known as OXB-102), an investigational gene therapy for the treatment of Parkinson's disease. AXO-Lenti-PD is a novel gene therapy that enables the expression of a set of three critical enzymes required for end-to-end dopamine synthesis in the brain.

AXO-Lenti-PD was administered to the first patient in the study at the Clinical Research Facility affiliated with the National Institute for Health Research (NIHR) and University College London Hospitals (UCLH). The patient experienced no complications related to the surgery or to administration of the vector and was discharged home as planned.

"Mid to late-stage Parkinson's disease remains a challenge to treat, with current therapies leading to debilitating adverse events and unpredictable therapeutic effects over time," said Stéphane Palfi, M.D., Ph.D., coordinating investigator on the AXO-Lenti-PD clinical study. "We are pleased to advance AXO-Lenti-PD in the clinic and are eager to see the trial expand upon the long-term safety and efficacy results we observed in the phase I/II clinical trial of ProSavin®."

The ongoing clinical study of AXO-Lenti-PD will evaluate safety and tolerability, as well as collect efficacy data including standard measures of motor function in patients with Parkinson's disease. Initial data from the first cohort of patients in the clinical trial is expected in the first half of 2019. This study will build upon the long-term safety and efficacy results reported in the phase I/II clinical trial of ProSavin, which showed up to six-year durability of reductions in UPDRS Part III "OFF" scores in updated data presented last week at the Annual Congress of the European Society of Gene and Cell Therapy and recently published in *Human Gene Therapy, Clinical Development*[1,2].

"The millions of patients with Parkinson's disease globally have limited treatment options available to them today. We are very excited to bring AXO-Lenti-PD into clinical development and believe it will be an important new therapy for patients with Parkinson's disease who suffer from motor fluctuations on the current standard of care. Building upon the evidence of safety and durable improvements in motor symptoms seen up to six years in the prior clinical study of ProSavin, we feel a sense of urgent responsibility to accelerate the development of AXO-Lenti-PD," said Pavan Cheruvu, M.D., chief executive officer of Axovant. "This marks the first of our gene therapy programs to enter the clinic, and our focus now is on rapid execution of the clinical study. We look forward to sharing initial data in the first half of 2019."

**About AXO-Lenti-PD**

AXO-Lenti-PD, also known as OXB-102, is an investigational gene therapy for Parkinson's disease. The therapy delivers three genes *in vivo* via a lentiviral vector to encode a set of critical enzymes required for dopamine synthesis in the brain and is expected to provide patient benefit for many years following a single administration. AXO-Lenti-PD is a next-generation gene therapy designed to further increase endogenous dopamine production over the first-generation product, Prosavin, by modifying the payload configuration. Preclinical studies directly comparing AXO-Lenti-PD to ProSavin demonstrate increased AADC activity and dopamine productivity of the new vector configuration. Oxford BioMedica has successfully completed a phase I/II study for ProSavin, which met its primary endpoint. The results, which were published in *The Lancet* in 2014, demonstrate favorable safety and tolerability and a statistically significant improvement from baseline of motor function as measured by the UPDRS Part III score at 6 and 12 months ($p=0.0001$)[3]. This improvement has been observed to be sustained in patients for up to six years despite the progressively degenerative nature of Parkinson's disease. Initial data from the ongoing AXO-Lenti-PD clinical program is expected in the first half of 2019.

**About Axovant Sciences**

Axovant is a clinical-stage gene therapy company focused on developing a pipeline of innovative product candidates for debilitating neurological and neuromuscular diseases such as Parkinson's disease, oculopharyngeal muscular dystrophy (OPMD), amyotrophic lateral sclerosis (ALS), frontotemporal dementia, and other indications. For more information, visit www.axovant.com

**Forward-Looking Statements and Information**

*Statements made in this press release contain forward-looking statements, including statements regarding Axovant's plans to advance the development of its investigational gene therapy candidate, AXO-Lenti-PD, and Axovant's expectations about timing of the results for its clinical study for AXO-Lenti-PD in Parkinson's disease, and other elements of Axovant's clinical development and regulatory strategy. Forward-looking statements can be identified by the words "believe," "anticipate," "continue," "estimate," "project," "expect," "plan," "potential," "intend," "will," "would," "could," "should," or the negative or plural of these words or other similar expressions that are predictions or indicate future events, trends or prospects. Forward-looking statements are subject to risks and uncertainties that could cause actual results to differ materially and reported results should not be considered as an indication of future performance. These risks and uncertainties include, but are not limited to: risks associated with the success, cost and timing of our product development activities and clinical trials; the approval and commercialization of Axovant's product candidates, including AXO-Lenti-PD; the ability to obtain issued patents, and identify and in-license or acquire rights to third party patents; and regulatory requirements. These statements are also subject to the risk that clinical trial data are subject to differing interpretations, and regulatory agencies, medical and scientific experts and others may not share Axovant's views of the clinical study data. 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 Axovant's clinical programs, including the AXO-Lenti-PD program, will be successful in demonstrating safety and/or efficacy, that Axovant will not encounter problems or delays in clinical development, or that any of Axovant's product candidates will ever receive regulatory approval or be successfully commercialized. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to Axovant's business in general, see the "Risk Factors" section of Axovant's quarterly report on Form 10-Q filed with the Securities and Exchange Commission on August 7, 2018, and other filings that Axovant makes with the SEC from time to time. These forward-looking statements are based on information available to Axovant as of the date of this press release and speak only as of the date of this press release. Axovant disclaims any obligation to update these forward-looking statements, except as may be required by law.*

References:


1. Palfi S, et al. ProSavin®, a dopamine gene therapy for advanced Parkinson's disease: 6 years phase I/II clinical update. Poster at ESGCT 2018.
2. Palfi S, et al. Long-Term Follow-Up of a Phase I/II Study of ProSavin, a Lentiviral Vector Gene Therapy for Parkinson's Disease. *Hum Gene Ther Clin Dev. 2018 Sep;29(3):148-155*.
3. Palfi S, et al. Long-term safety and tolerability of ProSavin, a lentiviral vector-based gene therapy for Parkinson's disease: a dose escalation, open-label, phase 1/2 trial. *Lancet. 2014 Mar 29;383(9923):1138-46*


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