Axovant Announces Feedback From FDA Meeting Regarding AXO-Lenti-PD for Parkinson's Disease and Progress in Ongoing SUNRISE-PD Phase 2 Clinical Trial

December 6, 2018

- *Meeting with FDA confirmed that studies previously conducted using first generation ProSavin® may be considered part of a single development program with AXO-Lenti-PD*
- *Confirmed with FDA that the proposed current manufacturing process and quality control testing is adequate for the clinical program*
- *Dosed second patient in the SUNRISE-PD phase 2 clinical trial of AXO-Lenti-PD in November 2018, with data expected in March 2019*

BASEL, Switzerland, Dec. 06, 2018 (GLOBE NEWSWIRE) -- Axovant Sciences (NASDAQ: AXON), a clinical-stage company developing innovative gene therapies for neurological conditions, today announced feedback from a face-to-face pre-IND meeting with the U.S. Food and Drug Administration (FDA) regarding AXO-Lenti-PD for patients with Parkinson's disease. Based on the discussion at the meeting, the totality of data collected on the initial vector construct, ProSavin, including over six years of phase 1/2 clinical data and IND-enabling preclinical data, may be supportive of the planned development program for AXO-Lenti-PD.

The phase 2 clinical trial of AXO-Lenti-PD (NCT03720418), now called SUNRISE-PD, was initiated in the U.K. in the fourth quarter of 2018. The SUNRISE-PD study is advancing as planned with dosing of the second patient in November 2018. To date, both patients tolerated the surgical procedure well and were discharged home with no serious adverse events observed. Axovant expects to announce data from the first two patients in March 2019.

During the meeting discussion and subsequent written meeting minutes, Axovant received feedback from the FDA on several key features of the AXO-Lenti-PD development program:

- The target patient population will be adult patients with Parkinson's disease who are refractory to additional medical management due to motor complications
- The ongoing SUNRISE-PD clinical study of AXO-Lenti-PD constitutes an early-phase, exploratory trial that may support a future marketing application if safety and efficacy data are meaningful
- The primary efficacy measure of the randomized, sham-controlled portion of the phase 2 study will be assessed at 12 months using data from Hauser patient diaries
- Additional secondary efficacy data will be collected on the UPDRS Part III "OFF" score, a motor function assessment completed by clinicians after oral levodopa has been washed out
- The proposed current manufacturing process and quality control testing is adequate for the clinical program
- FDA agreed in principle with the proposed approach to demonstrate compatibility between the current manufacturing process and the planned serum-free, suspension manufacturing process that will be used to support scale-up and commercialization.

In addition, Axovant was encouraged to return for an End-of-Phase 2 meeting after completion of the ongoing SUNRISE-PD study, during which the available data generated in the study will be discussed in the context of a pivotal program design.

"We are pleased with the feedback received at our meeting with the FDA, which reaffirmed our strategy to view the ongoing SUNRISE-PD clinical study of AXO-Lenti-PD as a continuation of the previous ProSavin program, and part of a single development program," said Gavin Corcoran, M.D., executive vice president of research and development for Axovant. "We believe we are well-positioned to continue to execute on the clinical trial as designed, manufacture drug product at scale, and activate clinical trial sites in the U.S. during the randomized, sham-controlled portion of our SUNRISE-PD study."

About AXO-Lenti-PD
AXO-Lenti-PD, also known as OXB-102, is an investigational gene therapy for Parkinson's disease. The product delivers three genes *in vivo* via a lentiviral vector to encode the set of enzymes required for dopamine synthesis in the brain and is expected to provide patient benefit for many years following a single administration. A phase 1/2 study for ProSavin, a first-generation version of AXO-Lenti-PD, 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). This improvement has been observed to be sustained in patients for up to six years despite the progressively degenerative nature of Parkinson's disease. Data from the first two patients enrolled in the ongoing AXO-Lenti-PD phase 2 (SUNRISE-PD) study are expected in March 2019.

About Axovant Sciences
Axovant is a clinical-stage gene therapy company focused on developing a pipeline of innovative product candidates for debilitating neurological 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," "may," "can," "might," "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 initiation, conduct, success, cost, and timing of our product development activities and clinical trials; the ability to manufacture Axovant's product candidates and successfully transition manufacturing processes; 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 technology; 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. These statements are also subject to the risk that the FDA may have a different view of or later reach a different determination relating to the AXO-Lenti-PD development program and the feedback provided to us. 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 manufacturing, 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 November 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, whether as a result of new information, future events or otherwise, except as may be required by law.

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