Axovant Reports Results of Exploratory Phase 2 Clinical Study of Nelotanserin in Lewy Body Dementia Patients Experiencing REM Sleep Behavior Disorder

December 10, 2018

- *Primary efficacy endpoint assessed by sleep laboratory video assessment was not met*
- *Axovant is discontinuing clinical development of nelotanserin*

BASEL, Switzerland, Dec. 10, 2018 (GLOBE NEWSWIRE) -- Axovant Sciences (NASDAQ: AXON) a clinical-stage gene therapy company, today reported topline results of the 34-patient, exploratory phase 2 clinical study of nelotanserin for the treatment of REM sleep behavior disorder (RBD) in patients with Lewy body dementia (LBD). The primary efficacy endpoint of reduction in frequency of RBD events as measured by sleep laboratory video assessment was not met.

Nelotanserin was generally well-tolerated in the study. Signals of efficacy were observed on secondary measures, including trends in prespecified analyses of study diaries and certain sleep parameters on polysomnography (PSG). These findings are consistent with nelotanserin's mechanism of action and previous clinical studies of nelotanserin in patients with insomnia.

"While secondary measures of efficacy suggest biologic activity for nelotanserin, Axovant has been focused on developing innovative gene therapies and we will not undertake further clinical studies with our legacy small molecule portfolio, including nelotanserin," said Pavan Cheruvu, M.D., chief executive officer of Axovant. "The completion of this study closes a chapter in the company's history. We are grateful to the patients and clinical investigators who participated in this study, and we look forward to advancing Axovant's gene therapy pipeline through multiple important milestones in 2019."

Axovant has been focused on gene therapies since the in-licensing of AXO-Lenti-PD in June 2018, and has since then strengthened its capabilities in the development, manufacturing and commercialization of gene therapies with the addition of experts to the team and continued expansion of its pipeline. AXO-Lenti-PD is an investigational gene therapy for Parkinson's disease that delivers three genes *in vivo* via a lentiviral vector to encode the set of enzymes required for dopamine synthesis in the brain. The SUNRISE-PD phase 2 clinical study of AXO-Lenti-PD is ongoing, with data expected in March 2019. In addition, Axovant is developing AXO-AAV-OPMD, a gene therapy utilizing novel Silence-and-Replace technology to restore normal muscle function in patients with oculopharyngeal muscular dystrophy (OPMD), and plans to initiate a clinical study for the therapy in the second half of 2019.

About the Nelotanserin REM Sleep Behavior Disorder Study
This multi-center, randomized, double-blind, placebo-controlled phase 2 clinical study evaluated the efficacy of nelotanserin in 34 patients with LBD who were experiencing RBD. The prespecified primary endpoint of the pilot study was to determine efficacy in reducing frequency of RBD behaviors compared to placebo through sleep laboratory video assessment. Secondary efficacy assessments in the study included measures of RBD derived from a study diary and various objective sleep parameters.

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
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 initiation, timing, progress, and reporting of results of its preclinical programs, clinical trials, and research and development programs; its ability to advance its product candidates into and successfully initiate, enroll, and complete clinical trials; and the timing or likelihood of its regulatory filings and approvals, 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 initiation and conduct of preclinical studies and clinical trials; the availability of data from clinical trials; the expectations for regulatory submissions and approvals; the continued development of its 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 for the quarterly period ended September 30, 2018, filed with the Securities and Exchange Commission on November 7, 2018, 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, except as required by law.

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Source: Axovant Sciences Ltd.