Axovant Sciences Announces Start Of Confirmatory Phase 3 MINDSET Study And Special Protocol Assessment (SPA) Agreement With FDA

October 6, 2015

-- MINDSET will seek to confirm results of prior study in which RVT-101 demonstrated statistically significant improvements in cognition and function in mild-to-moderate Alzheimer's disease patients

HAMILTON, Bermuda, Oct. 6, 2015 /PRNewswire/ -- Axovant Sciences Ltd. (NYSE: AXON), a leading clinical-stage biopharmaceutical company focused on the treatment of dementia, today announced the first patients screened in MINDSET, a confirmatory global phase 3 study of Axovant's lead product candidate, RVT-101. Axovant also announced that the company and the U.S. Food and Drug Administration (FDA) have agreed to a Special Protocol Assessment (SPA) supporting this phase 3 program.

MINDSET is an international, multi-center, double blind, placebo-controlled study designed to evaluate the safety, tolerability and efficacy of RVT-101 in patients with mild-to-moderate Alzheimer's disease. The 24-week trial will compare 35-mg, once-daily oral doses of RVT-101 to placebo in approximately 1,150 patients with mild-to-moderate Alzheimer's disease on a stable background of donepezil therapy. The primary efficacy evaluations are the Alzheimer's Disease Assessment Scale – cognitive subscale (ADAS-cog) and the Alzheimer's Disease Cooperative Study – Activities of Daily Living scale (ADCS-ADL), each of which have been used as endpoints to obtain regulatory approval of currently-marketed Alzheimer's disease treatments in the United States and Europe.

The MINDSET trial is designed to confirm the results of a 684-patient international, multi-center, double-blind placebo-controlled study in which patients on a stable background of donepezil therapy receiving 35 mg RVT-101 demonstrated statistically significant improvements on the ADAS-cog and ADCS-ADL as compared to patients receiving donepezil alone.

"I am grateful for the unwavering efforts of the entire development team that has so rapidly advanced RVT-101 into this final stage of the drug development process," said Axovant Chief Development Officer Dr. Lawrence Friedhoff, who is leading the RVT-101 development program and previously led the development program for donepezil (brand name Aricept®), the most widely used Alzheimer's treatment.

"No new compounds have been approved for Alzheimer's disease in over a decade, and physicians are scrambling to do more for their patients," said Dr. Gary Small, President of the American Association for Geriatric Psychiatry. "We need well-tolerated, once-daily oral treatments that provide clinically meaningful benefits. The start of the MINDSET study is an important milestone for the field of Alzheimer's drug development."

If the MINDSET study is successful, Axovant intends to submit a New Drug Application to FDA by the end of 2017.

To learn more about enrolling in the MINDSET study, please email mindset@axovant.com.

About a Special Protocol Assessment (SPA)

A Special Protocol Assessment (SPA) from the FDA is a binding agreement that the design and planned analysis of a study adequately address the objectives necessary to support a regulatory submission. More information about the FDA's Special Protocol Assessment process is available at http://www.fda.gov/downloads/Drugs/.../Guidances/ucm080571.pdf.

About RVT-101

RVT-101 is an orally administered, potent antagonist of the 5-HT6 serotonin receptor. Antagonism of the 5-HT6 receptor is a novel mechanism of action that promotes the release of acetylcholine and other neurotransmitters thought to improve cognition and function in patients suffering from Alzheimer's disease and other forms of dementia.

RVT-101 is currently being studied in the confirmatory Phase 3 MINDSET study under a Special Protocol Assessment agreement with the FDA. RVT-101 has previously been studied in 13 clinical trials and dosed in over 1,250 human subjects with a favorable safety and tolerability profile. In a 684-patient multinational double-blind placebo-controlled study, RVT-101 was observed to provide statistically significant benefits in cognition and function to patients with mild-to-moderate Alzheimer's disease.

RVT-101 is an investigational new drug candidate and is not approved for any indication in any markets.

About Axovant

Axovant Sciences Ltd. is a leading clinical-stage biopharmaceutical company focused on the acquisition, development and commercialization of novel therapeutics for the treatment of dementia, a condition characterized by significant decline in mental capacity and impaired daily function. Axovant intends to develop a pipeline of product candidates to comprehensively address the cognitive, behavioral and functional components of dementia, including Alzheimer's disease.

Forward Looking Statements

This press release contains forward-looking statements, including statements regarding Axovant's expectations about the planned Phase 3 registration program for RVT-101 and other elements of its clinical development and regulatory strategy, including timing and potential for the submission of an NDA for RVT-101. 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 our product candidate RVT-101; and increased regulatory requirements. These statements are subject to the risk that further

analyses of the phase 2b data which may lead to different (including less favorable) interpretations of the data than the analyses conducted to date and/or may identify important implications of the phase 2b data that are not reflected in these statements. 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 phase 2b data. There can be no assurance that the clinical program for RVT-101 will be successful in demonstrating safety and/or efficacy, that we will not encounter problems or delays in clinical development, or that RVT-101 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 our quarterly report on Form 10-Q filed with the Securities and Exchange Commission on August 11, 2015, and other filings that Axovant makes with the SEC from time to time. These forward-looking statements speak only as of the date of this release. Axovant disclaims any obligation to update these forward-looking statements, except as may be required by law.

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To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/axovant-sciences-announces-start-of-confirmatory-phase-3-mindset-study-and-special-protocol-assessment-spa-agreement-with-fda-300154619.html

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