Axovant's RVT-101 Phase 2b Completer Analysis Demonstrates Statistically Significant Benefits in Cognition and Function Through 48 Weeks

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HAMILTON, Bermuda, July 22, 2015 /PRNewswire/ -- Axovant Sciences Ltd. (NYSE: AXON), a leading clinical-stage biopharmaceutical company focused on the treatment of dementia, announces new results today from a 684-subject phase 2b clinical trial evaluating Axovant's lead product candidate, RVT-101, as an adjunctive therapy to donepezil in patients with mild-to-moderate Alzheimer's disease.

In Axovant's analysis of all patients with complete data at each study visit, patients receiving 35 mg RVT-101 in combination with donepezil demonstrated statistically significant improvements in cognition and function at 12, 24, 36, and 48 weeks as compared to patients receiving donepezil alone. Cognition was measured by the Alzheimer's Disease Assessment Scale – cognitive subscale (ADAS-cog) and function was measured by the Alzheimer's Disease Cooperative Study – Activities of Daily Living scale (ADCS-ADL). ADAS-cog and ADCS-ADL have each been used as endpoints to obtain regulatory approval of currently-marketed Alzheimer's disease treatments.

Axovant's completer analysis reinforces the protocol-specified analysis of the intent-to-treat (ITT) population from this same study, published in *Alzheimer's & Dementia*, where patients receiving 35 mg RVT-101 plus donepezil also demonstrated statistically significant improvements on the ADAS-cog and ADCS-ADL compared to patients receiving donepezil alone. These results also suggested a consistent dose response across placebo, 15mg RVT-101, and 35mg RVT-101, the only doses that were tested.

Axovant presents these results today at the 2015 Alzheimer's Association International Conference (AAIC) in Washington, D.C.

"These phase 2b results demonstrate that RVT-101 has the potential to be a significant advance in the treatment of mild-to-moderate Alzheimer's disease," said Dr. Lawrence Friedhoff, Chief Development Officer of Axovant Sciences, Inc., who formerly led the development of Aricept (donepezil) for Alzheimer's disease. "Statistically significant improvements were seen consistently across multiple analyses on the ADAS-cog and ADCS-ADL, endpoints historically used by the FDA and EMA to approve new Alzheimer's drugs."

RVT-101 was well-tolerated by subjects in all 13 clinical trials conducted to date.

"Based on the phase 2b data, I believe RVT-101 has the potential to safely improve cognition and function in Alzheimer's disease patients who are in need of new therapeutic options," said Dr. Atul Pande, who previously oversaw the development of RVT-101 at GlaxoSmithKline as SVP, Neurosciences and currently serves on Axovant's Board of Directors. "The data to date warrant advancement of RVT-101 into the upcoming confirmatory phase 3 study. I am especially encouraged to see the experienced development team at Axovant advance this program under the leadership of Dr. Friedhoff."

Axovant intends to commence a confirmatory phase 3 program in the fourth quarter of 2015.

Completer Analysis

The table below presents improvements on the ADAS-cog and ADCS-ADL seen in the completer analysis by patients that received 35 mg RVT-101 in combination with donepezil, as compared to patients that received donepezil alone. 684 patients were randomized in the study. The completer analysis includes only patients with complete data at each study visit, rather than using estimated values for patients with incomplete data. Statistically significant benefits on both endpoints were observed through 48 weeks, and the magnitude of those benefits generally tended to increase with time.

Week	ADAS-cog benefit relative to placebo	ADCS-ADL benefit relative to placebo
12	1.29 (p=.008)	1.72 (p=.016)
24	1.63 (p=.007)	2.11 (p=.016)
36	1.35 (p=.039)	2.20 (p=.029)
48	1.82 (p=.018)	2.34 (p=.048)

On a third endpoint measured, the Clinical Dementia Rating – Sum of Boxes (CDR-SB), the 35 mg RVT-101 treatment group demonstrated numerical improvements over the control group which were statistically significant at week 12, but not at other time points. The CDR-SB has not been used to obtain the approval of any currently marketed Alzheimer's disease treatment.

No drug-related serious adverse events were observed in the RVT-101 treatment groups at 24 or 48 weeks. There was no statistically significant difference in withdrawals and adverse events between the treatment groups and control group, with a slightly lower percentage of withdrawals and drug-related adverse events, including falls, in the 35 mg treatment group than in the control group.

ITT Analysis

The completer analysis reinforces the results of the previously published ITT analysis. ITT analyses use estimated values for patients who had incomplete data. The results of the ITT analysis showed the 35 mg treatment group had statistically significant improvements relative to placebo of ADAS-cog at 12, 24, and 48 weeks and ADCS-ADL at 12, 24, and 36 weeks. The table below presents those results:

Week	ADAS-cog benefit relative to placebo	ADCS-ADL benefit relative to placebo
12	1.30 (p=.006)	1.72 (p=.019)
24	1.50 (p=.013)	2.00 (p=.024)
36	1.21 (p=.057)	1.93 (p=.038)
48	1.64 (p=.024)	1.94 (p=.088)

Phase 3 Program Design

Axovant plans to commence a confirmatory phase 3 trial in Q4 2015, testing RVT-101 as an adjunctive therapy to donepezil in patients with mild-to-moderate Alzheimer's disease.

The trial is expected to randomize patients who are stable on donepezil therapy to receive either 35 mg RVT-101 or placebo. All patients will remain on donepezil therapy during the trial. Anticipated enrollment is over 500 subjects per arm. Co-primary endpoints will be the ADAS-cog and ADCS-ADL at Week 24.

Axovant also plans to evaluate RVT-101 as a potential treatment for other types of dementia later this year.

Investor Briefing & Live Webcast

Axovant will host an investor briefing and live webcast at 5:45 PM EDT today to further discuss Alzheimer's disease and the RVT-101 data. The briefing and webcast will include remarks from Dr. Friedhoff, Dr. Pande, and Dr. Gary Small, President of the American Association of Geriatric Psychiatry (AAGP). The webcast will be available through the Axovant website at http://investors.axovant.com/investors/events-and-presentation.aspx. An archived webcast will also be available for at least 30 days following the event.

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, glutamate and other neurotransmitters thought to improve cognition and function in patients suffering from Alzheimer's disease and other forms of dementia. RVT-101 has been studied in 13 clinical trials and dosed in over 1,250 human subjects with a favorable safety and tolerability profile. Axovant intends to commence a confirmatory phase 3 clinical study testing RVT-101 on a background of donepezil therapy in mild-to-moderate Alzheimer's disease patients in the fourth quarter of 2015.

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 participation in the Alzheimer's Association International Conference 2015 (AAIC), as well as the subject matter of Axovant's presentations at the conference, including the plans, strategies, and objectives of management for future operations, and clinical data. 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; amongst others. 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 the Companies' 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 Axovant's Prospectus filed with the Securities and Exchange Commission on June 11, 2015. These forward-looking statements speak only as of the date hereof. Axovant Sciences disclaims any obligation to update these

forward-looking statements.

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To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/axovants-rvt-101-phase-2b-completer-analysis-demonstrates-statistically-significant-benefits-in-cognition-and-function-through-48-weeks-300116866.html

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