Axovant Sciences Announces Launch of First-in-Class Clinical Programs for Lewy Body Dementia and Reports Financial Results for the Third Fiscal Quarter Ended December 31, 2015

February 9, 2016

- Positions Axovant as a global leader in Lewy body dementia with three clinical trials to address cognitive, behavioral and functional aspects of Lewy body dementia
- Successful completion of 70 mg safety study of RVT-101 to support higher dosing in HEADWAY-DLB Study
- Successful completion of Japanese PK Study to advance RVT-101 development in Japan
- Reaffirms expectation for MINDSET study results in the second half of 2017
- Strong balance sheet with $300 million of cash as of December 31, 2015 to support the Company's current development plans

HAMILTON, Bermuda, Feb. 9, 2016 /PRNewswire/ - Axovant Sciences Ltd. (NYSE: **AXON**), a leading clinical-stage biopharmaceutical company focused on the treatment of dementia, today announced further details of three new clinical trials to address cognitive, behavioral and functional aspects of Lewy body dementia, a disease affecting approximately 1.4 million people in the U.S. Two out of the three studies were recently initiated and the third is expected to start later this quarter. In addition, the Company reported financial results for the third fiscal quarter and nine months ended December 31, 2015.

“With the launch of three new studies this quarter, Axovant has emerged as the leading company pursuing treatments for Lewy body dementia, a debilitating disease with no approved therapies in the U.S. or EU and affecting over 1 million patients in the U.S. alone.” stated Vivek Ramaswamy, Chief Executive Officer of Axovant Sciences, Inc. “In addition, the longstanding attrition of late-stage drug candidates to address Alzheimer’s disease has only worsened over the last year, highlighting the desperate need for safe and effective new therapies. We believe that a potential approval for RVT-101 would position Axovant as a new global leader in the treatment of Alzheimer’s disease and other forms of dementia.”

“In getting these three innovative clinical studies started this quarter, our development team has moved at a pace seldom seen in the pharmaceutical industry,” said Lawrence Friedhoff, M.D., Ph.D., Chief Development Officer of Axovant Sciences, Inc. “This was only possible because of the close interaction and deep engagement we have had with the Lewy body disease clinical community and patient advocacy groups.”

RVT-101 in Dementia with Lewy Bodies (DLB) Program Initiation: The HEADWAY-DLB Study

Axovant has initiated a 24-week double-blind, randomized, placebo-controlled Phase 2b study of RVT- 101 as a potential treatment for DLB called The HEADWAY-DLB Study. Daily doses of 35 mg and 70 mg of RVT-101 will be evaluated and the study is designed to potentially serve as a pivotal trial. Target enrollment for the study is 240 patients and results are expected in calendar year 2017. If the results of this study are favorable, the Company believes that it, in combination with positive MINDSET study results, could serve as the basis for seeking approval of RVT-101 in DLB.

Nelotanserin in Lewy Body Dementia Subjects Experiencing Visual Hallucinations

Axovant has initiated a double-blind, randomized, placebo-controlled, cross-over Phase 2 study in patients with dementia with Lewy bodies or Parkinson's disease dementia suffering from visual hallucinations. The study is a pilot program with primary outcome measures focused on safety and secondary measures evaluating changes in the frequency and severity of visual hallucinations after 28 days of treatment. Target enrollment for the study is 20 patients and results are expected in the second half of calendar year 2016. The Company expects the results to inform a subsequent pivotal study design in this indication.

Nelotanserin in Dementia with Lewy Bodies Subjects Experiencing REM Behavior Disorder

Later this quarter Axovant plans to initiate a 4-week double-blind, randomized, placebo-controlled Phase 2 study in patients with dementia with Lewy bodies suffering from REM behavior disorder. The study is designed to potentially serve as a pivotal trial with primary measures focused on efficacy. Importantly, this study will utilize objective measures as assessed in a sleep-lab setting. Nelotanserin has previously shown statistically significant clinical results on objective measures of sleep maintenance and sleep architecture as measured in a sleep-lab setting. Target enrollment for the study is approximately 50 patients and results are expected in the first-half of calendar year 2017.

RVT-101 in Alzheimer's Disease Program Update: The MINDSET Study

Axovant reaffirmed its anticipated timeline for the completion of its global, multi-center, double-blind, placebo-controlled confirmatory Phase 3 study of RVT-101 for the treatment of mild-to-moderate Alzheimer’s disease called the MINDSET Study with results expected to be reported in the second-half of calendar year 2017. Axovant also announced the successful completion of a pharmacokinetic study of RVT-101 in Japanese subjects which will support its Japanese registration program.

Corporate Highlights since September 30, 2015

- **MINDSET initiation**: In October 2015 Axovant announced the first patients screened in MINDSET, its 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) did agree to a Special Protocol Assessment (SPA) supporting this phase 3 program.
- **Nelotanserin acquisition:** In October 2015 the Company exercised its option to acquire global rights to nelotanserin, a potential best-in-class 5HT2A inverse agonist, from its parent company, Roivant Sciences Ltd. (RSL). RSL had previously

acquired the global rights from Arena Pharmaceuticals, GmbH (Arena).

- **RVT-101 repeat dose safety and food effect study**: Axovant recently completed a successful study to investigate the safety and tolerability and to characterize the pharmacokinetics (PK) of RVT-101 at doses of 35 mg and 70 mg following repeat oral administration in healthy, elderly subjects. This study also evaluated the effect of food on the pharmacokinetics of RVT-101.
- **RVT-101 Japanese PK study**: Axovant recently observed successful preliminary results in a study to compare the pharmacokinetics (PK) of RVT-101 following repeat dose oral administration in healthy, Japanese subjects to matched Caucasian controls.
- **CTAD presentations**: The Company made three presentations at the Clinical Trials in Alzheimer's Disease (CTAD) Meeting held in November 2015. An oral presentation "RVT-101: review of the preclinical and clinical results and status of the development program" and two poster presentations: “PET studies with RVT-101 in healthy volunteers demonstrate high occupancy of the 5HT6 receptor" and "Safety of the 5HT6 antagonist, RVT-101 in patients with Alzheimer's Disease: summary of Phase 2 clinical trials".
- **LBDA presentations**: The Company made four presentations at the Lewy Body Dementia Association’s (LBDA) International Dementia with Lewy Bodies Conference held in December 2015. An oral presentation “Efficacy Results of a Phase 2b Study of RVT-101, a Neurotransmitter-Targeted Therapy, in Mild-to-Moderate Alzheimer’s Disease” and three poster presentations: “Safety of Nelotanserin in a Randomized Placebo-Controlled Phase 2 Study”, “Effect of RVT-101 and Donepezil on Extracellular Neurotransmitters in the Medial Prefrontal Cortex and Dorsal Hippocampus of Conscious Rats” and “Effect of Nelotanserin on Objective Sleep Parameters in a Phase 2 Study in Patients with Insomnia”.

Third-Quarter 2015 Financial Summary

For the third fiscal quarter ended December 31, 2015, research and development expenses for the period were $34.3 million, of which $14.6 million was attributable to non-cash, share-based compensation expense. General and administrative expenses for the third quarter of 2015 were $28.2 million, of which $24.5 million was attributable to non-cash, share-based compensation expense. Net loss for the third quarter of 2015 was $63.4 million, or $(0.64) per share.

Nine Months 2015 Financial Summary

For the nine months period ended December 31, 2015, research and development expenses were $53.2 million, of which $24.4 million was attributable to non-cash, share-based compensation expense. General and administrative expenses for the nine months ended December 31, 2015 were $49.4 million, of which $39.5 million was attributable to non-cash, share-based compensation expense. Net loss for the nine months ended December 31, 2015 was $103.5 million, or $(1.11) per share.

Axovant held cash of $300.0 million at December 31, 2015, and net cash used in operating activities was $30.1 million for the first nine months of 2015.

About Axovant

Axovant Sciences Ltd. is a leading clinical-stage biopharmaceutical company focused on acquiring, developing and commercializing novel therapeutics for the treatment of dementia. Axovant intends to develop a pipeline of product candidates to comprehensively address the cognitive, functional and behavioral components of dementia and related neurological disorders. Our vision is to become the leading company focused on the treatment of dementia by addressing all forms and aspects of this condition.

About MINDSET

MINDSET is a Phase 3 international, multi-center, double-blind, placebo-controlled clinical 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 Phase 2 international, multi-center, double-blind placebo-controlled study in which patients on a stable background of donepezil therapy receiving 35 mg of RVT-101 were observed to have statistically significant improvements in their ADAS-cog and ADCS-ADL scores as compared to patients receiving donepezil alone.

For more information please visit www.alzheimersglobalstudy.com, email mindset@axovant.com or call 646-495-8197.

About HEADWAY-DLB

HEADWAY-DLB is a Phase 2b international, multi-center, double-blind, placebo-controlled clinical study designed to evaluate the safety, tolerability and efficacy of RVT-101 in patients with dementia with Lewy bodies. The 24-week trial will compare 70 mg once-daily oral doses of RVT-101 to 35 mg once-daily oral doses of RVT-101 to placebo in subjects with probable dementia with Lewy bodies with or without existing background therapies. The primary efficacy evaluations are Clinician’s Interview-Based Impression of Change Plus Caregiver Input (CIBIC-Plus) and a computerized cognitive battery.

For more information please visit www.lewybodystudy.com or e-mail headwaydlb@axovant.com.

Forward-Looking Statements

This press release contains forward-looking statements, including statements regarding Axovant’s expectations about timing of the Phase 3 MINDSET

study of RVT-101 in patients with Alzheimer's disease, the Phase 2b HEADWAY-DLB study of RVT-101 in patients with dementia with Lewy bodies, the Phase 2 study of nelotanserin in patients with dementia with Lewy bodies or Parkinson's disease dementia suffering from visual hallucinations, the Phase 2 study of nelotanserin in patients with dementia with Lewy bodies suffering from REM behavior disorder and other elements of its clinical development and regulatory strategy. Forward-looking statements can be identified by the words “believe,” “anticipate,” “continue”, “estimate”, “project,” “expect,” “plan,” “potential,” “intends,” “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 our product candidates RVT-101 and nelotanserin; and increased regulatory requirements. These statements are 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. There can be no assurance that the clinical programs for RVT-101 or nelotanserin will be successful in demonstrating safety and/or efficacy, that we will not encounter problems or delays in clinical development, or that any of our 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 our quarterly report on Form 10-Q to be filed with the Securities and Exchange Commission on or about February 9, 2016, 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 release. Axovant disclaims any obligation to update these forward-looking statements, except as may be required by law.

AXOVANT SCIENCES LTD.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(in thousands, except share and per share data)
(unaudited)

	(Third Fiscal Quarter) Three Months Ended December 31, 2015	**Nine Months Ended December 31, 2015**	**Period From October 31, 2014 (Date Of Inception) To December 31, 2014**
Operating expenses:			
Research and development expenses (includes $14,599 and $24,421 of share-based compensation expense for the three and nine months ended December 31, 2015, respectively and $457 of share-based compensation expense for period from October 31, 2014 (Date of inception) to December 31, 2014)	$ 34,324	$ 53,209	$ 10,538
General and administrative expenses (includes $24,457 and $39,537 of share-based compensation expense for the three and nine months ended December 31, 2015, respectively and $146 of share-based compensation expense for period from October 31, 2014 (Date of inception) to December 31, 2014)	28,230	49,364	224
Total operating expenses	62,554	102,573	10,762
Loss before provision for income tax	(62,554)	(102,573)	(10,762)
Income tax expense	802	901	–
Net loss and comprehensive loss	$ (63,356)	$ (103,474)	$ (10,762)
Net loss per common share — basic and diluted	$ (0.64)	$ (1.11)	$ (1.08)
Weighted average common shares outstanding — basic and diluted	99,150,000	92,914,909	10,000,000

AXOVANT SCIENCES LTD.
Condensed Consolidated Balance Sheets
(in thousands)
(unaudited)

	December 31, 2015	March 31, 2015
Assets		
Current assets:		
Cash	$ 299,998	–
Prepaid expenses and other current assets	3,819	4
Deferred financing costs	–	1,104

Total current assets	303,817	1,108
Property, plant and equipment, net	61	9
Total assets	$ 303,878	$ 1,117
Liabilities and Shareholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 3,556	$ 403
Due to Roivant Sciences Ltd. and Roivant Sciences, Inc.	1,839	2,307
Accrued expenses	5,398	1,158
Contingent payment liability	5,000	–
Income tax payable	99	–
Total current liabilities	15,892	3,868
Contingent payment liability	–	5,000
Total liabilities	15,892	8,868
Total shareholders' equity (deficit)	287,986	(7,751)
Total liabilities and shareholders' equity (deficit)	$ 303,878	1,117

Source: Axovant Sciences Ltd.

Contact:
Jonathan Neely
Head, Investor Relations and Corporate Communications
Axovant Sciences, Inc.
(212) 634-9744