Axovant Announces Data to be Presented at the Annual Congress of the European Society of Gene and Cell Therapy

October 9, 2018

BASEL, Switzerland, Oct. 09, 2018 (GLOBE NEWSWIRE) -- Axovant Sciences (NASDAQ:AXON), a gene therapy company developing innovative treatments for debilitating neurologic and neuromuscular diseases, today announced upcoming presentations regarding its investigational gene therapy programs, AXO-Lenti-PD and AXO-AAV-OPMD, at the Annual Congress of the European Society of Gene and Cell Therapy (ESGCT) being held in Lausanne, Switzerland from October 16th to 19th, 2018.

Prosavin®, a dopamine gene therapy for advanced Parkinson's disease: 5 years phase I/II clinical update
Date: October 17, 2018
Time: 13:10-15:10 CEST
Location: Garden Floor, Poster session 1
Poster Number: P201

AXO-Lenti-PD for Parkinson's disease: endogenous dopamine production by intraputamenal three-gene transfer
Presented by: Fraser Wright, Chief Technology Officer of Axovant
During the lunch workshop on the use of lentiviral vectors for in vivo gene therapy
Date: October 18, 2018
Time: 13:15-14:15 CEST
Location: Garden Floor, Conference room 2/3

BB-301 (AXO-AAV-OPMD): a single "silence and replace" AAV-based vector for the treatment of oculopharyngeal muscular dystrophy (OPMD)
Date: October 18, 2018
Time: Poster presentation at 09:00-10:40 CEST, Poster session at 12:40-14:40 CEST
Location: Garden Floor, Conference room 2/3, Poster session 2
Poster Number: P392

About AXO-Lenti-PD
AXO-Lenti-PD, formerly OXB-102, is an investigational gene therapy for Parkinson's disease that delivers three genes encoding a critical set of enzymes required for dopamine synthesis in the brain and is designed to provide patient benefit for multiple years following a single administration. AXO-Lenti-PD is a next-generation gene therapy with a modified payload configuration of the predecessor product, ProSavin®, to further improve endogenous dopamine production. Oxford BioMedica has successfully completed a Phase 1/2 study for ProSavin®, which met its primary endpoint. The results, which were published in *The Lancet* in 2014, demonstrate favorable safety and tolerability and a statistically significant improvement of motor function as measured by the UPDRS Part III score at 6 and 12 months. This improvement was observed to be sustained in patients for up to six years despite the progressively degenerative nature of Parkinson's disease.

About AXO-AAV-OPMD
AXO-AAV-OPMD, formerly BB-301, is an investigational gene therapy for oculopharyngeal muscular dystrophy (OPMD) that utilizes an adeno-associated viral (AAV) vector to silence the mutant poly-A binding protein N1 (*PABPN1*) gene that causes OPMD and replace with a functional copy of *PABPN1*. The gene therapy will be delivered in a one-time, single administration directly into target muscle tissue with the goal of providing long-term correction of muscle pathology and restoration of function. Data from mouse models of OPMD showed AXO-AAV-OPMD provided up to 86% inhibition of PABPN1 expression, while restoring functional PABPN1 up to 63% of normal levels, which coincided with decreased muscle pathology and a restoration of muscle force and muscle weight to near wild-type levels. The U.S. Food & Drug Administration and European Commission have granted Orphan Drug Designation to the AXO-AAV-OPMD program for the treatment of OPMD.

About Axovant Sciences
Axovant is a clinical-stage neurological gene therapy company focused on developing a pipeline of clinical and nonclinical product candidates for debilitating diseases such as Parkinson's disease, oculopharyngeal muscular dystrophy, ALS, frontotemporal dementia, and other indications. Axovant is also developing small molecules for the treatment of Lewy body dementia and other neurology and psychiatry 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 AXO-AAV-OPMD program and expand its pipeline with additional gene therapy products, Axovant's expectations about timing of the results for its Phase 1/2 clinical study for AXO-Lenti-PD in Parkinson's disease, Axovant's license arrangements with Benitec and Oxford BioMedica, 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," "will," "would," "could," "aims", "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 Axovant's product candidates, including AXO-AAV-OPMD and

AXO-Lenti-PD; the ability to obtain issued patents; identify and in-license or acquire third party patents; and increased 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. 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 AXO-AAV-OPMD and AXO-Lenti-PD programs will be successful in demonstrating safety and/or efficacy, that Axovant will not encounter problems or delays in clinical development, 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 August 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, except as may be required by law.

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