Axovant Announces Global Licensing Agreement for AXO-AAV-OPMD Program for Treatment of Oculopharyngeal Muscular Dystrophy and Broader Platform Collaboration with Benitec Biopharma

July 9, 2018

- License grants Axovant worldwide rights to AXO-AAV-OPMD utilizing proprietary Silence-and-Replace technology, which suppresses mutant protein production while restoring expression of functional protein
- Plan to initiate placebo-controlled study of AXO-AAV-OPMD in patients with oculopharyngeal muscular dystrophy (OPMD) in 2019
- License also grants rights to five additional investigational gene therapy products for neurological conditions
- New agreement further demonstrates Axovant's commitment to the development and commercialization of transformational therapies for neurological diseases
- Conference call / webcast to be held July 9, 2018 at 8:30am EDT

BASEL, Switzerland, July 09, 2018 (GLOBE NEWSWIRE) -- Axovant Sciences (NASDAQ:AXON) today announced that it has licensed exclusive global rights to an investigational Silence-and-Replace gene therapy program from Benitec Biopharma for the treatment of oculopharyngeal muscular dystrophy (OPMD), and has also entered into a research collaboration for the development of five additional gene therapy products in neurological disorders. The Silence-and-Replace gene therapy technology is designed to deliver a combination of DNA-directed RNA interference (silence) along with a functional copy of the gene (replace) in a single vector construct. This approach is applicable to various genetic diseases, including autosomal dominant disorders caused by nucleotide repeat expansion.

The lead program, AXO-AAV-OPMD, is in preclinical development, and Axovant plans to initiate a placebo-controlled clinical study in 2019. OPMD is a neuromuscular disease that is caused by mutations in the gene coding for polyA-binding protein nuclear 1 (PABPN1), which can lead to formation of intranuclear inclusion bodies causing muscle cell pathology. Patients with OPMD may have swallowing difficulties with potentially life-threating consequences, including malnutrition and aspiration pneumonia. OPMD is estimated to affect at least 15,000 patients in North America and Europe, and there are no products approved for treatment of the disease. AXO-AAV-OPMD is an adeno-associated viral (AAV) vector gene therapy delivered via a one-time intramuscular administration, which both silences the mutant *PABPN1* gene and replaces it with a functional copy. The U.S. Food & Drug Administration and European Commission have granted Orphan Drug Designation to AXO-AAV-OPMD for the treatment of OPMD.

Under the terms of the agreement, Axovant will pay Benitec an upfront payment of $10 million for rights to the AXO-AAV-OPMD program and five additional investigational gene therapy products, as well as payments tied to development, regulatory and commercial sales milestones. In addition, Benitec will receive 30% of the net profits on worldwide sales of AXO-AAV-OPMD and tiered royalties on the other gene therapy products that result from this collaboration. The first additional investigational gene therapy product will target the *C9orf72* gene, which is associated with amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD).

"This expansion of Axovant's pipeline further demonstrates our commitment to advancing innovative gene therapies for serious neurological diseases," said Pavan Cheruvu, MD, Chief Executive Officer of Axovant. "OPMD is a debilitating, potentially fatal disease that affects adults in the prime of their careers, and no approved treatment options are currently available. Quality-of-life in patients with OPMD can be impaired due to proximal muscle weakness, swallowing difficulties, aspiration pneumonia and malnutrition, and no approved treatment options are currently available. AXO-AAV-OPMD directly targets the underlying genetic defect that causes this disease using Silence-and-Replace technology, and I am excited about the potential of our gene therapy program for patients suffering from OPMD."

"The Silence-and-Replace technology is a unique approach in gene therapy, using a single vector to suppress mutant protein production while also restoring expression of the functional protein, and could be an elegant solution to tackling autosomal dominant genetic disorders," stated Fraser Wright, PhD, Chief Technology Officer of Axovant. "I look forward to collaborating with the research and manufacturing teams at Benitec to advance the progress of the platform and bring additional therapies into the clinic."

Commenting on the agreement, Jerel Banks, MD PhD, Executive Chairman of Benitec Biopharma said: "This agreement with Axovant further demonstrates the importance of Benitec's core technology and our strategic focus on rapidly progressing these programs into the clinic. We believe Axovant is the ideal partner to take these programs forward, and look forward to working closely with them to develop AXO-AAV-OPMD and other neurological gene therapies."

Teleconference/Webcast Details

To participate in the live conference call on July 9, 2018 at 8:30am EDT, please dial 1-833-652-5918 from the U.S. and Canada or +1 409-767-9227 internationally, and use the passcode 8819919.

The live call is being webcast and can be accessed on the "Events and Presentations" page of the "Investors" section of the Company's website at http://investors.axovant.com. A replay of the webcast will be available for 30 days following the live event.

AXO-AAV-OPMD Program

The AXO-AAV-OPMD Program is an investigational gene therapy being developed as a one-time treatment for oculopharyngeal muscular dystrophy (OPMD). The Program utilizes an AAV vector to silence the mutant poly-A binding protein N1 (*PABPN1*) gene that causes OPMD and replace with a functional copy of the *PABPN1* gene. The Silence-and-Replace approach aims to knock down the expression of the mutant *PABPN1* gene through ddRNAi, while at the same time express a re-engineered copy of the *PABPN1* gene coding for the functional PABPN1 protein. The gene therapy will be delivered in a single administration directly into target muscle tissue to provide long-term correction of muscle pathology and restoration of function.

Data from mouse models of OPMD showed gene therapy from the AXO-AAV-OPMD Program provided up to 86% inhibition of *PABPN1* expression, while restoring functional *PABPN1* up to 63% of normal levels. The A17 mouse model is a well validated *in vivo* model that is designed to exhibit many of the key pathological features of OPMD patients. The levels of gene silencing and expression achieved in this model coincided with decreased muscle pathology and a restoration of muscle force and muscle weight to near wild-type levels.

Axovant expects to initiate a placebo-controlled clinical study for the investigational AXO-AAV-OPMD Program in 2019. 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.

Oculopharyngeal Muscular Dystrophy (OPMD)

Oculopharyngeal muscular dystrophy (OPMD) is a muscular disease that is inherited through a primarily autosomal dominant pattern. OPMD is estimated to affect approximately 15,000 people in North America and Europe. The disease generally presents between the ages of 40-70 years old and is characterized primarily by progressive swallowing difficulty (dysphagia), eyelid drooping (ptosis), and weakness of the proximal extremities. Swallowing difficulties can have life-threating consequences, including malnutrition and aspiration pneumonia. As the disease progresses, the dysphagia becomes more severe and other muscles may become involved. There are no products approved for the treatment of OPMD and therefore, treatment options available to patients are limited.

OPMD is caused by mutations in the gene coding for polyA-binding protein nuclear 1 (PABPN1), a ubiquitously expressed protein that regulates the processing of messenger RNAs. The normal PABPN1 protein contains ten copies of the amino acid alanine, which forms a polyalanine tract. In OPMD, the mutated *PABPN1* gene has an expansion of alanine-encoding trinucleotide repeats, resulting in an abnormally long polyalanine tract. The protein that forms from the mutated gene is prone to aggregating into insoluble nuclear inclusion bodies which leads to muscle cell pathology and disease progression.

About Axovant Sciences

Axovant is a clinical-stage biopharmaceutical company dedicated to advancing innovative treatments for patients with serious neurologic and neuropsychiatric conditions, and turning promising therapies into lasting solutions for patients. Axovant is committed to developing and commercializing a pipeline of product candidates by identifying and developing novel treatments for unmet needs in neurology and psychiatry.

Forward-Looking Statements and Information

This press release contains forward-looking statements, including statements regarding Axovant's plans to advance the development of AXO-AAV-OPMD and expand its pipeline with additional gene therapy products. 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 our intellectual property position including the ability to obtain issued patents, identify and in-license or acquire third-party patents and licenses, and associated costs; the success, cost, and timing of Axovant's product development activities, including the timing of the initiation and completion of preclinical and clinical trials, the timing of patient enrollment and dosing in clinical trials, and the timing of expected regulatory filings; and the clinical utility and potential attributes and benefits of AXO-AAV-OPMD Program and collaboration product candidates, including future out-and-in-license opportunities and reliance on collaboration partners, and the ability to procure additional sources of financing. 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on June 11, 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 release. Axovant disclaims any obligation to update these forward-looking statements, except as may be required by law.

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