Axovant Licenses Investigational Gene Therapy for Parkinson's Disease from Oxford BioMedica and Announces Key Leadership Team Addition

June 6, 2018

- Exclusive worldwide license to lentiviral vector gene therapy constitutes the first transaction of Axovant's 2018 pipeline expansion
- Fraser Wright, Co-Founder and former Chief Technology Officer of Spark Therapeutics, to join Axovant as CTO for gene therapy programs
- Axovant will receive $25 million equity financing from Roivant Sciences to support clinical development of AXO-Lenti-PD and additional business development
- Conference call / webcast today at 8:00am Eastern Time

BASEL, Switzerland, June 06, 2018 (GLOBE NEWSWIRE) -- Axovant Sciences (NASDAQ:AXON) today announced that it has licensed the exclusive worldwide rights to develop and commercialize OXB-102, now AXO-Lenti-PD, from Oxford BioMedica. AXO-Lenti-PD 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. Oxford BioMedica is a world leader in lentiviral vector product development and manufacturing, and will be the clinical and commercial supplier of AXO-Lenti-PD. Axovant expects to initiate a Phase 1/2 dose escalation study of AXO-Lenti-PD in patients with advanced Parkinson's disease by the end of 2018.

Under the terms of the license agreement with Oxford BioMedica, Axovant obtained rights to AXO-Lenti-PD, as well as its predecessor product ProSavin®, for an initial payment of $30 million in cash, $5 million of which will be applied as a credit against the process development work and clinical supply that Oxford BioMedica will provide to Axovant. Oxford BioMedica is also eligible to receive additional development, regulatory, and commercial milestone payments potentially in excess of $812 million, and tiered royalties on net sales of AXO-Lenti-PD, if approved. Roivant has agreed to purchase $25 million of Axovant common shares, which will support the clinical development of AXO-Lenti-PD and additional business development activities.

Fraser Wright, PhD, will join Axovant as Chief Technology Officer overseeing the company's gene therapy initiatives. Dr. Wright is the Co-Founder and former Chief Technology Officer of Spark Therapeutics and has over 20 years of leadership experience in the development of novel vector-based biologic products. At Spark he oversaw process development and clinical-stage manufacturing for LUXTURNA™. Prior to Spark, he was the founding Scientific Director of the Clinical Vector Core Laboratory at The Children's Hospital of Philadelphia, where he directed clinical core staff in gene therapy investigational product development, manufacture, and quality control testing for ten first-in-human viral vector investigational products including LUXTURNA™ and Kymriah®. He was also previously the Director of Development and Clinical Manufacturing at Avigen. Dr. Wright was formerly a Research Professor of Pathology and Laboratory Medicine at the University of Pennsylvania School of Medicine, and he is the lead inventor on numerous issued patents during his time in that role. He received his BSc and PhD in biochemistry from the University of Toronto, where he was also an assistant professor of biochemistry and medicine.

Pavan Cheruvu, MD, Chief Executive Officer of Axovant, stated: "Axovant remains committed to developing innovative treatments for serious neurodegenerative conditions such as Parkinson's disease, and we are excited to partner with Oxford BioMedica, a recognized global leader in cell and gene therapy. We are also pleased to welcome Fraser to our leadership team. He brings over two decades of experience in gene therapy manufacturing, and will be committed to building world-class gene therapy capabilities at Axovant. We will continue to pursue promising new therapeutic approaches based on transformative science, and will further expand our pipeline with high-quality assets like AXO-Lenti-PD. This is part of our long-term goal of building Axovant into a leader in the development and commercialization of innovative new medicines for neurological indications."

"This is an exciting time to join Axovant, and I look forward to the opportunity to work closely with Oxford BioMedica and help build gene therapy capabilities at Axovant," said Dr. Wright. "AXO-Lenti-PD is a strong foundation for Axovant's new pipeline, and I am excited to begin preparing the Phase 1/2 clinical study in advanced Parkinson's disease later this year."

Commenting on the announcement, John Dawson, Chief Executive Officer of Oxford BioMedica said: "We are delighted to sign this significant agreement which not only underlines our LentiVector®-enabled platform and product development strategy but further demonstrates Oxford BioMedica's ability to build multiple partnerships with leaders in their respective therapeutics fields. We believe Axovant's expertise and focus on neurological disorders, which includes Parkinson's disease, makes them an ideal development and commercialisation partner for this programme. Coupled with strong support and financial resources from parent company Roivant, we believe Axovant is well positioned to advance the development of AXO-Lenti-PD for the treatment of patients with Parkinson's, a disease which still has a high unmet need."

Teleconference/Webcast Details

To participate in the live conference call today, June 6, at 8:00 a.m. EDT, please dial 1-833-652-5918 from the U.S. and Canada or +1 409-767-9227 internationally, and use the passcode 8289429.

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.

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 sustained in most patients for up to four years despite the progressively degenerative nature of Parkinson's disease.

About Parkinson's Disease

Parkinson's disease is caused by degeneration of nerve cells in a portion of the brain called the *substantia nigra* which leads to a reduction in dopamine. Low dopamine causes nerve cells to activate without normal control. Characteristic Parkinson's disease symptoms include tremor, limb rigidity, slow physical movement, and gait and balance issues. Approximately one million Americans live with Parkinson's disease, with 60,000 diagnosed each year. The combined direct and indirect cost of Parkinson's disease, including treatment, Social Security payments, and lost income, is estimated to be nearly $25 billion per year in the United States alone.

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.

About Oxford BioMedica

Oxford BioMedica (LSE:OXB) is a leading gene and cell therapy group focused on developing life changing treatments for serious diseases. Oxford BioMedica and its subsidiaries (the "Group") have built a sector-leading lentiviral vector delivery platform (LentiVector®), which the Group leverages to develop *in vivo* and *ex vivo* products both in-house and with partners. The Group has created a valuable proprietary portfolio of gene and cell therapy product candidates in the areas of oncology, ophthalmology and CNS disorders. The Group has also entered into a number of partnerships, including with Novartis to manufacture Kymriah®, Bioverativ, Sanofi, GSK, Orchard Therapeutics, GC LabCell and Immune Design, through which it has long-term economic interests in other potential gene and cell therapy products. Oxford BioMedica is based across several locations in Oxfordshire, UK and employs more than 320 people.

About Roivant Sciences

Roivant Sciences is a global biopharmaceutical company focused on reducing the time and cost of the drug development process to improve the lives of patients and their families. Roivant partners with innovative biopharmaceutical companies and academic institutions to ensure that important medicines are rapidly delivered to patients.

Forward-Looking Statements and Information

This press release contains forward-looking statements, including statements regarding Axovant's plans to advance the development of AXO-Lenti-PD and expand its pipeline. 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 ability to identify and in-license or acquire product candidates, and the success, cost, and timing of Axovant's product development activities and any planned clinical trials. 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 (SEC) on February 9, 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.

Financial details regarding this transaction will be provided in Axovant's Form 8-K to be filed with the SEC. All trademarks are property of their respective owners.

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