Axovant Sciences Logo

Axovant Announces Presentation at J.P. Morgan Healthcare Conference and Anticipated 2019 Clinical Development Milestones

January 3, 2019

- *Axovant's CEO, Pavan Cheruvu, M.D., will be presenting at the J.P. Morgan Healthcare Conference at 8:00 a.m. PST on Thursday, January 10th, 2019*
- *Initial clinical data expected in first half of 2019 from the AXO-AAV-GM2 and AXO-Lenti-PD gene therapy programs*
- *Axovant assembles Scientific Advisory Board to provide strategic guidance across its gene therapy portfolio*

BASEL, Switzerland, Jan. 03, 2019 (GLOBE NEWSWIRE) -- Axovant Sciences (NASDAQ: AXON), a clinical-stage company developing innovative gene therapies, today announced that the company will be presenting at the 37th Annual J.P. Morgan Healthcare Conference. Pavan Cheruvu, M.D., chief executive officer, will present at 8:00 a.m. PST on Thursday, January 10th, 2019.

Over the course of the last year, Axovant has strategically built integrated capabilities for the development, manufacturing and commercialization of novel gene therapies, including:

- In-licensing and developing a pipeline of innovative, clinical-stage product candidates in partnership with well-recognized gene therapy organizations
- Assembling a team with decades of gene therapy expertise spanning regulatory affairs, clinical development, vector optimization and delivery, manufacturing and commercialization
- Assembling a world-class Scientific Advisory Board to provide strategic guidance across its gene therapy development programs
- Raised over $55 million in 2018, including a $30 million equity financing in December 2018 led by Deerfield Management Company, Sphera Funds Management and Roivant Sciences that will enable the company to reach certain key clinical milestones in 2019

"Building on our progress in 2018, Axovant is harnessing innovations in gene therapy to address the unmet needs of patients with debilitating neurological diseases," said Pavan Cheruvu, M.D., CEO of Axovant. "We anticipate that 2019 will highlight Axovant's steadfast commitment to becoming a leader in the development of novel gene therapies. We are eager to accelerate our pipeline of product candidates through important clinical milestones and data readouts in 2019."

Upcoming Milestones Anticipated in 2019

Axovant expects to advance its pipeline of innovative gene therapy programs through multiple milestones in 2019:

- **AXO-AAV-GM1:** First patient expected to be dosed in the first half of 2019; initial data expected in second half of 2019
- **AXO-AAV-GM2:** Initial data expected in the first quarter of 2019
- **AXO-Lenti-PD:** Initial data expected in March 2019
- **AXO-AAV-OPMD:** Initiation of clinical program expected in the second half of 2019

Formation of Scientific Advisory Board

In December 2018, Axovant assembled a Scientific Advisory Board (SAB), under the leadership of Michael Hayden, M.B. Ch.B., Ph.D., F.R.S.C., to advance its gene therapy strategy. This SAB meeting brought together thought leaders in gene therapy and neurodegenerative disorders to provide guidance related to scientific, clinical and technical topics of strategic importance to Axovant. The SAB shared their expertise on advancements in lentiviral and AAV gene therapy technology, gene therapy approaches to neurodegenerative conditions, and the future of gene editing. Members of the Scientific Advisory Board include:

- **Michael Hayden, M.B. Ch.B., Ph.D., F.R.S.C.** - chair of the Scientific Advisory Board and senior scientific advisor to Axovant, former chief scientific officer and president of global R&D at Teva
- **Beverly Davidson, Ph.D.** - chief scientific strategy officer, Arthur V. Meigs Chair in Pediatrics and director of the Raymond G. Perelman Center for Cellular and Molecular Therapeutics at Children's Hospital of Philadelphia (CHOP); professor, department of pathology and laboratory medicine at University of Pennsylvania; scientific co-founder of Spark Therapeutics
- **R. Jude Samulski, Ph.D.** - professor, department of pharmacology at University of North Carolina School of Medicine and director of the University of North Carolina Gene Therapy Center; scientific founder of Bamboo Therapeutics
- **Nicole Déglon, Ph.D.** - research director and deputy director of the Molecular Imaging Research Center (MIRCen) and associate professor in Clinical Neurosciences at the Lausanne University Hospital (CHUV)
- **Jeffrey Kordower, Ph.D.** - the Alla V. and Solomon Jesmer Professor of Neurological Sciences, director of the Research Center for Brain Repair and section head of neuroscience at Rush University Medical Center
- **Luigi Naldini, M.D., Ph.D.** - professor of cell and tissue biology and of gene and cell therapy at the San Raffaele University School of Medicine and scientific director of the San Raffaele Telethon Institute for Gene Therapy (SR-TIGET)
- **Guy Rouleau, Ph.D.** - director of the Montreal Neurological Institute and Hospital and chair of the Department of

Neurology and Neurosurgery at McGill University

"It was a pleasure to bring together these pre-eminent leaders in gene therapies and neurodegenerative disorders to discuss the latest developments in their fields," said Dr. Hayden, chair of the Scientific Advisory Board and senior scientific advisor to Axovant. "Insights gained from these discussions will help advance Axovant's strategy as the company embarks on an active year with a deep pipeline of gene therapies."

Webcast Information for J.P. Morgan Healthcare Conference

A live audio webcast of the presentation 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 following the live event.

About Axovant

Axovant Sciences is a clinical-stage gene therapy company focused on developing a pipeline of innovative product candidates for debilitating neurological and neuromuscular diseases. The company's current pipeline of gene therapy candidates targets GM1 gangliosidosis, Tay-Sachs and Sandhoff diseases, Parkinson's disease, oculopharyngeal muscular dystrophy (OPMD), amyotrophic lateral sclerosis (ALS) and frontotemporal dementia. Axovant is focused on accelerating product candidates into and through clinical trials with a team of experts in gene therapy development and through external partnerships with leading gene therapy organizations.

In December 2018, Axovant added two novel gene therapy programs to address a set of rare and fatal pediatric disorders, GM1 gangliosidosis, Tay-Sachs and Sandhoff diseases. These diseases are characterized by rapid neurodegeneration and reduce life expectancy to less than two to four years of age in severe forms, with no current disease-modifying treatments. AXO-AAV-GM1 and AXO-AAV-GM2, licensed from University of Massachusetts Medical School, are each designed to introduce functional copies of the respective genes encoding the critical enzymes impacted in GM1 gangliosidosis, Tay-Sachs and Sandhoff diseases, with an aim to enable children to reach key developmental milestones and improve survival.

AXO-Lenti-PD, licensed from Oxford Biomedica in June 2018, is an investigational gene therapy for Parkinson's disease that delivers the three genes that encode a set of critical enzymes required for dopamine synthesis in the brain via a single lentiviral vector. AXO-Lenti-PD is expected to provide patient benefit for many years following a single administration.

AXO-AAV-OPMD, licensed from Benitec Biopharma in July 2018, is a gene therapy utilizing novel silence-and-replace technology to restore normal muscle function in patients with oculopharyngeal muscular dystrophy (OPMD). OPMD is a progressive, potentially fatal disease that causes muscle weakness in the throat, face and proximal limbs. There are limited treatment options for OPMD, and no approved therapies to treat the underlying cause of the disease.

For more information, visit www.axovant.com.

Forward-Looking Statements and Information

This press release contains forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995 and other federal securities laws. The use of words such as "may," "might," "will," "expect," "plan," "anticipate," "believe," "intend," "future," or "continue" and other similar expressions are intended to identify forward-looking statements. For example, all statements Axovant makes regarding the potential efficacy of its product candidates; initiation, timing, progress, and reporting of results of its preclinical programs, clinical trials, and research and development programs; its ability to advance its product candidates into and successfully initiate, enroll, and complete clinical trials; and the timing or likelihood of its regulatory filings and approvals, are forward-looking. All forward-looking statements are based on estimates and assumptions by Axovant's management that, although Axovant believes to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that Axovant expected. Such risks and uncertainties include, among others, the initiation and conduct of preclinical studies and clinical trials; the availability of data from clinical trials; the expectations for regulatory submissions and approvals; the continued development of its product candidates and platforms; Axovant's scientific approach and general development progress; and the availability or commercial potential of Axovant's product candidates. These statements are also subject to a number of material risks and uncertainties that are described in Axovant's most recent Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2018, filed with the Securities and Exchange Commission on November 7, 2018, as updated by its subsequent filings with the Securities and Exchange Commission. Any forward-looking statement speaks only as of the date on which it was made. Axovant undertakes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise.

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