



Axovant Gene Therapies to Host Virtual Parkinson's Disease R&D Day on October 30, 2020

October 22, 2020

- Agenda to feature presentations from leading clinical researchers in Parkinson's disease and the Michael J. Fox Foundation
- Individual patient-level data from Cohort 2 of the Phase 2 SUNRISE-PD study to be shared

NEW YORK, Oct. 22, 2020 (GLOBE NEWSWIRE) -- Axovant Gene Therapies Ltd. (Nasdaq: AXGT), a clinical-stage company developing innovative gene therapies, today announced that it will host a virtual R&D Day on Friday, October 30, 2020 at 11:30 AM Eastern time, to discuss the Company's AXO-Lenti-PD gene therapy for Parkinson's disease.

Axovant's Parkinson's disease R&D Day will be moderated by Chief R&D Officer, **Gavin Corcoran, M.D.**, and will feature presentations on the current treatment landscape and unmet medical need for people living with Parkinson's disease from the following key opinion leaders:

- **Charles Adler, M.D., Ph.D.**, Professor at Mayo Clinic College of Medicine in Arizona
- **Jamie Eberling, Ph.D.**, Vice President of Research Programs at the Michael J. Fox Foundation
- **Stéphane Palfi, M.D., Ph.D.**, Professor of Neurosurgery and Head of the Neurosurgery Department at Henri Mondor Medical Center, Paris University (UPEC)

In addition, the Company will present data from the second cohort of the Phase 2 SUNRISE-PD trial for AXO-Lenti-PD including:

- Summary of available data from the second dose cohort at the 6-month timepoint following one-time dosing with AXO-Lenti-PD gene therapy
- Individual patient-level data at 6-months after dosing:
 - Hauser diary "Good ON time" and "OFF time" changes from baseline for all 4 patients
 - Levodopa-equivalent daily dose (LEDD) changes from baseline for all 4 patients
 - UPDRS Part II and Part III "OFF" scores for the 2 evaluable patients in the cohort
- 12-month evaluation for the first patient treated in Cohort 2, who was assessed remotely

Drs. Adler, Palfi, and Eberling will be joined by Dr. Corcoran to answer questions following the formal presentations.

AXO-Lenti-PD is the only investigational gene therapy for Parkinson's disease that delivers three genes via a lentiviral vector to encode a set of critical enzymes required for endogenous dopamine synthesis, with the goal of improving motor function and restoring steady, tonic levels of dopamine in the brain. The gene therapy aims to provide patient benefit for years following a single administration.

To register for the R&D webcast, please click [here](#).

A live audio webcast of the R&D Day can be accessed through the Events & Presentations section of the company's website at investors.axovant.com. An archived replay of the webcast will be available on the company's website following the event.

Biographies of R&D Day Panelists:

- **Charles H. Adler, M.D., Ph.D.** is currently Consultant and Professor in the Department of Neurology at Mayo Clinic College of Medicine in Arizona. He is also Co-Principal Investigator at both Arizona Parkinson's Disease Consortium and Arizona Study of Aging and Neurodegenerative Disorders in Scottsdale, Arizona. Dr. Adler received his Ph.D. in pharmacology and his medical degree, from the New York University School of Medicine. He did his neurology residency at the University of Pennsylvania and a fellowship in movement disorders at Graduate Hospital/University of Pennsylvania in Philadelphia. He then joined the staff at Mayo Clinic in Scottsdale, Arizona.

Dr. Adler has received numerous grants to investigate experimental treatments for Parkinson's disease, essential tremor, dystonia, restless legs syndrome, and chronic traumatic encephalopathy (CTE). He serves as an advisory member to many different international medical societies such as the International Parkinson and Movement Disorder Society, MDS Industry Education and Services Committee, and the American Academy of Neurology Section of Movement Disorders. Dr. Adler has a commitment to education having trained residents, 14 fellows and graduate students, and has given many invited lectures. Dr. Adler's main research interests are investigating tissue diagnostic tests for Parkinson's disease, biomarkers for an early diagnosis of Parkinson's disease and PD with dementia, and identification of new treatments for PD and PD with dementia. He also has been investigating essential tremor, restless legs syndrome, and dystonia. He has published over 400 research papers and reviews, and edited a book entitled Parkinson's Disease and Movement Disorders: Diagnosis and

Treatment Guidelines for the Practicing Physician. In 2006, Dr. Adler was awarded the Mayo Clinic Distinguished Investigator of the Year Award.

- **Stéphane Palfi, M.D., Ph.D.** is a Professor of Neurosurgery and Head of the Neurosurgery Department at Henri Mondor Medical Center, Paris University (UPEC). His interests are in developmental therapeutics for Parkinson's disease, Huntington's disease, tremor, dystonia, and psychiatric disorders. He has worked extensively in the area of electrical neuromodulation of the brain in movement disorders, gene therapy for Parkinson's disease, cell grafting for Huntington's and Parkinson's disease as well as primate models of neurodegenerative disorders.

Dr. Palfi has published extensively on trophic factor- and enzyme-based gene therapy in Parkinson's disease and Huntington's disease. He is a principal investigator on numerous preclinical and clinical studies and has been involved in studies of many novel agents including implanted brain devices, optogenetic, homeoprotein, trophic factors GDNF, CNTF and dopamine lentiviral vectors.

- **Jamie Eberling, Ph.D.** joined the Michael J. Fox Foundation (MJFF) in 2009. As Vice President, Research Programs, Dr. Eberling stays closely linked to the Parkinson's research community in order to develop an aggressive and innovative agenda for accelerating research and drug development for Parkinson's disease. This ensures that MJFF research priorities reflect and best serve the ultimate needs of patients. Dr. Eberling regularly meets with academic and industry researchers around the world to identify promising proposals to support, providing troubleshooting and ongoing management of projects as they go forward. She also supports the Foundation's priority interests in translating promising new therapies into the clinic. Jamie's primary responsibilities are managing the PET imaging program, including alpha-synuclein tracer development, the non-motor symptomatic program aimed at developing new therapies for non-motor symptoms, and the clinical trials that are funded by MJFF.

Dr. Eberling earned undergraduate and graduate degrees in biological psychology from the University of California at Berkeley, later moving to the Lawrence Berkeley National Laboratory where she developed expertise in neuroimaging techniques and gene therapy approaches for Parkinson's disease.

- **Gavin Corcoran, M.D.** joined Axovant as Chief R&D Officer. In his career, he has overseen successful drug development across multiple therapeutic areas, including neurology and psychiatry, and was previously Chief Medical Officer of Allergan plc and Actavis. Dr. Corcoran was Executive Vice President for Global Medicines Development at Forest Laboratories prior to the acquisition of Forest Laboratories by Actavis. Dr. Corcoran also served as Head of Late Stage Clinical Development for Inflammation and Immunology at Celgene, and as Chief Scientific Officer and head of R&D at Stiefel Laboratories. Earlier in his career he held leadership roles in clinical development and regulatory affairs at Amgen, Schering-Plough, and Bayer. He received his MB BCh from the University of the Witwatersrand in South Africa and completed his clinical training in internal medicine and infectious diseases at the University of Texas Health Science Center at San Antonio.

About Axovant Gene Therapies

Axovant Gene Therapies is a clinical-stage gene therapy company focused on developing a pipeline of innovative product candidates for debilitating neurodegenerative diseases. Our current pipeline of gene therapy candidates target GM1 gangliosidosis, GM2 gangliosidosis (also known as Tay-Sachs disease and Sandhoff disease), and Parkinson's disease. 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. For more information, visit www.axovant.com.

Contacts:

Investors

Parag Meswani
Axovant Gene Therapies Ltd.
(212) 547-2523
investors@axovant.com

Media

Josephine Belluardo, Ph.D.
LifeSci Communications
(646) 751-4361
jo@lifescicomms.com
media@axovant.com

