



Sio Gene Therapies Announces Four Upcoming Oral Presentations at the 24th Annual Meeting of the American Society of Gene and Cell Therapy

April 28, 2021

NEW YORK, and RESEARCH TRIANGLE PARK, N.C., April 28, 2021 (GLOBE NEWSWIRE) -- Sio Gene Therapies Inc. (NASDAQ: SIOX), a clinical-stage company focused on developing gene therapies to radically transform the lives of patients with neurodegenerative diseases, today announced four upcoming oral presentations at the 24th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT), to be held virtually between May 11th to May 14th, 2021.

The AXO-AAV-GM1 presentation will include a review of patient-level data on safety and efficacy at 6 months follow up from the low-dose cohort of the Company's ongoing clinical study. Additionally, Dr. Cynthia Tiff, the lead investigator for the study, will present 6-month biomarker data from cerebrospinal fluid (CSF) in the 5 children who received intravenous AAV9 gene therapy.

Oral Presentation Details:

Presentation Title: AXO-AAV-GM1 for the Treatment of GM1 Gangliosidosis: Preliminary Results from a Phase I-II trial

Abstract Number: 162

Session: Clinical Trials and Advanced Preclinical Studies for Neurologic Diseases

Presenting Author: Cynthia Tiff, MD, PhD, Deputy Clinical Director, National Human Genome Research Institute

Presentation Date and Time: Thursday, May 13, 2021 6:15 PM – 6:30 PM EDT

Presentation Title: AXO-Lenti-PD gene therapy for Parkinson's disease: efficacy, safety, and tolerability data from the second cohort in open-label dose evaluation study SUNRISE-PD at 6 months post administration

Abstract Number: 163

Session: Clinical Trials and Advanced Preclinical Studies for Neurologic Diseases

Presenting Author: Gavin Corcoran, MD, Chief R&D Officer

Presentation Date and Time: Thursday, May 13, 2021 6:30 PM – 6:45 PM EDT

Presentation Title: Immune Modulation Preceding AAV9-GLB1 Gene Therapy Preserves the Possibility for Re-Dosing in Children with GM1 Gangliosidosis

Abstract Number: 179

Session: Immunotherapy and Vaccines

Presenting Author: Precilla D'Souza, DNP, MSN, CRNP, National Human Genome Research Institute

Presentation Date and Time: Thursday, May 13, 2021 7:00 PM – 7:15 PM EDT

Presentation Title: A GLP Safety and Biodistribution Study of AXO-Lenti-PD Manufactured via Two Processes Delivered at a Higher Volume and Flow Rate

Abstract Number: 256

Session: Pharmacology/Toxicology Studies or Assay Development

Presenting Author: Thomas Pack, PhD, Sio Gene Therapies

Presentation Date and Time: Friday, May 14, 2021 from 1:45 PM – 2:00 PM EDT

About AXO-AAV-GM1

AXO-AAV-GM1 delivers a functional copy of the *GLB1* gene via an adeno-associated viral (AAV) vector, with the goal of restoring β -galactosidase enzyme activity for the treatment of GM1 gangliosidosis. The gene therapy is delivered intravenously, which has the potential to broadly transduce the central nervous system and treat peripheral manifestations of the disease as well. Preclinical studies in murine and a naturally-occurring feline model of GM1 gangliosidosis have supported AXO-AAV-GM1's ability to improve β -galactosidase enzyme activity, reduce GM1 ganglioside accumulation, improve neuromuscular function, and extend survival.

AXO-AAV-GM1 has received both Orphan Drug Designation and Rare Pediatric Disease Designation from the Food and Drug Administration and is the only gene therapy in clinical development for both Type I and Type II GM1 gangliosidosis.

In 2018, Sio licensed exclusive worldwide rights from the University of Massachusetts Medical School for the development and commercialization of gene therapy programs for GM1 gangliosidosis and GM2 gangliosidosis, including Tay-Sachs and Sandhoff diseases.

About AXO-Lenti-PD

AXO-Lenti-PD is an investigational gene therapy for the treatment of Parkinson's disease that is designed to deliver three genes (tyrosine hydroxylase, cyclohydrolase 1, and aromatic L-amino acid decarboxylase) via a single lentiviral vector to encode a set of critical enzymes required for dopamine synthesis, with the goal of reducing variability and restoring steady levels of dopamine in the brain. The investigational gene therapy aims to provide patient benefit for years following a single administration. Axovant expects to dose the first patient in EXPLORE-PD, a randomized, sham controlled study in 2021.

About Sio Gene Therapies

Sio Gene Therapies combines cutting-edge science with bold imagination to develop genetic medicines that aim to radically improve the lives of

patients. Our current pipeline of clinical-stage candidates includes the first potentially curative AAV-based gene therapies for GM1 gangliosidosis and Tay-Sachs/Sandhoff diseases, which are rare and uniformly fatal pediatric conditions caused by single gene deficiencies. We are also expanding the reach of gene therapy to highly prevalent conditions such as Parkinson's disease, which affects millions of patients globally. Led by an experienced team of gene therapy development experts, and supported by collaborations with premier academic, industry and patient advocacy organizations, Sio is focused on accelerating its candidates through clinical trials to liberate patients with debilitating diseases through the transformational power of gene therapies. For more information, visit www.sioctx.com.

Forward-Looking Statements

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 "believe," "estimate," "may be" and other similar expressions are intended to identify forward-looking statements. For example, all statements Sio makes regarding costs associated with its operating activities, funding requirements and/or runway to meet its upcoming clinical milestones, and timing of its upcoming clinical milestones are forward-looking. All forward-looking statements are based on estimates and assumptions by Sio's management that, although Sio 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 Sio expected. Such risks and uncertainties include, among others, the impact of the Covid-19 pandemic on our operations; the actual funds and/or runway required for our clinical and product development activities and anticipated upcoming milestones; actual costs related to our clinical and product development activities and our need to access additional capital resources prior to achieving any upcoming milestones; the initiation and conduct of preclinical studies and clinical trials; the availability of data from clinical trials; the development of a suspension-based manufacturing process for Axo-Lenti-PD; the scaling up of manufacturing, the expectations for regulatory submissions and approvals; the continued development of our gene therapy product candidates and platforms; Sio's scientific approach and general development progress; and the availability or commercial potential of Sio's product candidates. These statements are also subject to a number of material risks and uncertainties that are described in Sio's most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on February 9, 2021, 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. Sio undertakes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

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