Sio Gene Therapies to Present New Data at the European Society of Gene and Cell Therapy Virtual Congress 2021

October 4, 2021

Oral presentation to discuss new data updates from the high- and low-dose cohorts from the ongoing Phase 1/2 trial of AXO-AAV-GM1 for the treatment of GM1 gangliosidosis

NEW YORK, and DURHAM, N.C., Oct. 04, 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 that it will present new clinical and preclinical data in two oral presentations and one poster presentation at the upcoming European Society of Gene & Cell Therapy (ESGCT) Virtual Congress 2021, to be held virtually from October 19-22, 2021.

Oral presentations will include an update on the Phase 1/2 trial of AXO-AAV-GM1, the company's adeno-associated viral vector (AAV)9-based gene therapy for the treatment of Type I (early infantile onset) and Type II (late infantile and juvenile onset) GM1 gangliosidosis. Presentation will include new data from the low- and high-dose cohorts. The Company will also present a poster review of patient-level data up to 24 months from the Phase 1/2 study of AXO-Lenti-PD gene therapy for the treatment of Parkinson's disease.

Oral Presentation Details:

Presentation Title: Phase 1/2 Trial of AXO-AAV-GM1 Gene Therapy for the Treatment of Infantile- and Juvenile-onset GM1 Gangliosidosis
Presentation Number: OR28
Session: Session 4a: CNS & Sensory II
Presenting Author: Erica De Boever, Ph.D., DDS, MPH, Vice President of Clinical Development at Sio Gene Therapies
Presentation Date and Time: Thursday October 21, 2021; 9:00-11:00 AM CEST

Presentation Title: Bicistronic AAV Gene Therapy for Tay-Sachs and Sandhoff Diseases in a Sheep Model
Presentation Number: OR30
Session: Session 4a: CNS & Sensory II
Presenting Author: Toloo Taghian, Ph.D., University of Massachusetts
Presentation Date and Time: Thursday, October 21, 2021; 9:00-11:00 AM CEST

Poster Presentation Details:

Presentation Title: Phase 1/2 Open-label Dose Evaluation Study of AXO-Lenti-PD Gene Therapy for Parkinson's Disease: Efficacy, Safety, and Tolerability Data up to 24 Months
Poster Number: P254
Presenting Author: Gavin Corcoran, MD, Chief R&D Officer of Sio Gene Therapies

Copies of the presentation materials will be made available under the Events and Presentations section of Sio's website.

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 all pediatric forms of 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-AAV-GM2
AXO-AAV-GM2 is an investigational gene therapy for GM2 gangliosidosis (also known as Tay-Sachs and Sandhoff diseases), a set of rare and fatal pediatric neurodegenerative genetic disorders caused by defects in the HEXA (leading to Tay-Sachs disease) or HEXB (leading to Sandhoff disease) genes that encode the two subunits of the β-hexosaminidase A (HexA) enzyme. These genetic defects lead to progressive neurodegeneration and shortened life expectancy. AXO-AAV-GM2 aims to restore HexA function by introducing a functional copy of the HEXA and HEXB genes via delivery of two co-administered AAVrh8 vectors.

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.

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.siogtx.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 "expect," "estimate," "may" 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 and outcome of its upcoming clinical and manufacturing 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 August 12, 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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