Sio Gene Therapies Announces Dosing of First GM1 Gangliosidosis Early Infantile (Type I) Patient in Ongoing Phase 1/2 Study of AXO-AAV-GM1 Gene Therapy

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- *Study has now enrolled a total of nine patients across low-dose and high-dose cohorts*
- *On track to report 12-month topline safety, biomarker, and efficacy data from Type II low-dose AXO-AAV-GM1 cohort in October 2021*

NEW YORK, and DURHAM, N.C., Sept. 09, 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 dosing of the first Type I, or early infantile, patient in the low-dose cohort of its ongoing Phase 1/2 study of AXO-AAV-GM1, its adeno-associated viral vector (AAV)9-based gene therapy candidate for the treatment of GM1 gangliosidosis. No serious adverse events considered by the investigators to be related to AXO-AAV-GM1 have been observed following the administration of GM1 gene therapy, and additional screening and enrollment of Type I patients is ongoing.

“Dosing the first Type I patient represents a major milestone for the AXO-AAV-GM1 program and for the GM1 gangliosidosis community. The efforts by both Sio and our trial partners at the NIH are the culmination of our dedication to patients,” said Gavin Corcoran, M.D., Chief R&D Officer of Sio Gene Therapies. “We are excited to expand the age range of patients that have been dosed to address this more severe form of GM1 gangliosidosis, and we expect to report 12-month topline safety, biomarker, and clinical outcomes data from the Type II low-dose cohort of AXO-AAV-GM1 in October 2021.”

Dr. Cynthia Tifft, Deputy Clinical Director of the National Human Genome Research Institute (NHGRI) and study Principal Investigator added, “GM1 gangliosidosis patients and their families are faced with the diagnosis of a terminal illness with no available treatments. This milestone brings patients, now including those suffering from Type I early infantile disease, one step closer to the first approved gene therapy for this devastating pediatric lysosomal storage disorder.”

The clinical study (NCT03952637) is designed to evaluate the safety, tolerability, and potential efficacy of AXO-AAV-GM1 delivered intravenously in children with early infantile, or Type I, and late infantile and juvenile, or Type II, GM1 gangliosidosis. Stage 1 is a dose-escalation study in which the low-dose cohort is evaluating $1.5x10^{13}$ vg/kg and the high-dose cohort is evaluating a dose of $4.5x10^{13}$ vg/kg in both disease sub-types. Target enrollment of eight late infantile and juvenile-onset (Type II) patients across the low- and high-dose cohorts was recently completed and up to three Type I patients each in the low-dose and high dose cohorts are expected to be enrolled.

GM1 gangliosidosis is a progressive and fatal pediatric lysosomal storage disorder caused by mutations in the *GLB1* gene that cause impaired production of the β-galactosidase enzyme. Currently, there are no FDA-approved treatment options for GM1 gangliosidosis.

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 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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