



## Sio Gene Therapies to Present at the 18th Annual WORLDSymposium™ 2022

February 2, 2022

NEW YORK and DURHAM, N.C., Feb. 02, 2022 (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 data in an oral platform presentation and two poster presentations at the 18<sup>th</sup> Annual WORLDSymposium™ 2022, to be held February 7-11, 2022.

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. A comprehensive data update is still expected in the first half of this year. The Company is also collaborating with Invitae, who will present a poster on the Detect LSD program.

### Oral Platform Presentation Details:

**Presentation Title:** Phase 1/2 trial of AXO-AAV-GM1 (AAV9-GLB1) gene therapy for infantile- and juvenile-onset GM1 gangliosidosis

**Session:** Platform Presentations, Clinical Research

**Presenting Author:** Cynthia Tift, MD, PhD, National Human Genome Research Institute, National Institutes of Health (NIH)

**Presentation Date and Time:** Wednesday, February 9, 2022; 1:00 PM PST

### Poster Presentation Details:

**Presentation Title:** Phase 1/2 trial of AXO-AAV-GM1 (AAV9-GLB1) gene therapy for infantile- and juvenile-onset GM1 gangliosidosis

**Poster Number:** 302

**Presenting Author:** Cynthia Tift, MD, PhD, National Human Genome Research Institute, National Institutes of Health (NIH)

**Presentation Date and Time:** Wednesday, February 9, 2022; 3:00-5:00 PM PST

**Presentation Title:** Detect Lysosomal Storage Diseases: A no-charge, sponsored, testing program that enables access to genetic testing, treatment, and clinical trials for individuals with suspected lysosomal disorders

**Poster Number:** 194

**Presenting Author:** Heather McLaughlin, PhD, FACMG, Invitae

**Presentation Date and Time:** Thursday, February 10, 2022; 3:00-5:00 PM PST

Presentations can be accessed from the WORLDSymposium™ website at [www.worldsymposia.org](http://www.worldsymposia.org).

### 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  $\beta$ -galactosidase enzyme activity for the treatment of GM1 gangliosidosis. The gene therapy is delivered intravenously, which has the potential to achieve a broad central and peripheral biodistribution. Preclinical studies in murine and a naturally-occurring feline model of GM1 gangliosidosis have supported AXO-AAV-GM1's ability to increase  $\beta$ -galactosidase enzyme activity, reduce GM1 ganglioside accumulation, improve neuromuscular function, and extend survival.

AXO-AAV-GM1 has received Orphan Drug Designation, Rare Pediatric Disease Designation, and Fast Track Designation from the FDA and is the only gene therapy in clinical development for all pediatric forms of GM1 gangliosidosis.

In 2018, Sio licensed exclusive worldwide rights from UMass Chan 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 is comprised of 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. 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.sioqtx.com](http://www.sioqtx.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," "expect," "intend," "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, expected cash burn runway, expectations regarding licensing and commercial agreements, 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 Sio's operations; the actual funds and/or runway required for

Sio's clinical and product development activities and anticipated upcoming milestones; actual costs related to Sio's clinical and product development activities and Sio's 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 occurrence of adverse safety events during our current and future trials; the scaling up of manufacturing; the outcome of interactions with regulatory agencies and 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 November 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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