Sio Gene Therapies Announces Corporate Updates and Fiscal First Quarter 2021 Financial Results

August 12, 2021

– *Completed targeted enrollment of Type II patients in ongoing dose-escalation study of AXO-AAV-GM1 in GM1 gangliosidosis*

– *On track to report 12-month topline safety, biomarker, and clinical outcomes data from low-dose cohort of AXO-AAV-GM1 in late Q3 or early Q4 2021*

– *Strong cash position with $111 million of cash and cash equivalents as of June 30, 2021, providing cash runway into Q4 2022*

NEW YORK and RESEARCH TRIANGLE PARK, N.C., Aug. 12, 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 provided a corporate update and financial results for its fiscal first quarter ended June 30, 2021.

“We are enormously grateful for the support of the patient community and our clinical investigators as we mark a milestone in the GM1 gangliosidosis gene therapy program with the enrollment of the targeted eight Type II children in the dose-escalation stage of our clinical trial of AXO-AAV-GM1,” said Pavan Cheruvu, M.D., Chief Executive Officer of Sio Gene Therapies. “We are focused on continued clinical execution and look forward to presenting 12-month data from the low-dose cohort of this study in the second half of this year. With this additional data in hand, as well as emerging data from the high-dose cohort, we expect to meet with the FDA in the first half of 2022 to discuss the registrational pathway in GM1 gangliosidosis.”

Dr. Cheruvu continued, “The deep expertise and commitment of the Sio team, paired with our strong balance sheet, leaves us well positioned for continued execution to advance our pipeline of transformative gene therapies that address rare pediatric and adult neurodegenerative disease.”

Key Highlights and Development Updates

AXO-AAV-GM1 gene therapy for GM1 gangliosidosis

- Completed enrollment and dosing of eight late infantile and juvenile-onset (Type II) patients across the low-dose (n=5) and high-dose (n=3) cohorts, achieving the targeted enrollment of Type II patients in Stage 1 (dose-escalation) of the trial. The Company continues to collect information from additional Type II patients for potential enrollment in Stage 2 of the trial.
- Enrollment and screening of Type I (infantile-onset) patients for the low- and high-dose cohorts has been initiated.
- Cerebrospinal fluid (CSF) biomarker data presented at the American Society of Gene and Cell Therapy (ASGCT) 24th Annual Meeting demonstrated reductions in CSF GM1 ganglioside in 4 out of 5 children treated with the lowest dose of AXO-AAV-GM1 at 6-months follow up, providing the first evidence of a biochemical effect in the CNS following intravenous delivery.
- Upcoming milestones:
  - 12-month topline safety, biomarker, and efficacy data from the low-dose cohort in late Q3 or early Q4 2021.
  - Meeting with the U.S. Food and Drug Administration (FDA) to discuss the registrational pathway for AXO-AAV-GM1 in the first half of 2022.

AXO-AAV-GM2 gene therapy for Tay-Sachs and Sandhoff diseases

- Dosed first two patients in the Phase 1/2 trial investigating AXO-AAV-GM2 in Tay-Sachs and Sandhoff diseases, including one patient at the starting dose and one patient at the low dose.
- Expect continued patient identification, screening, and enrollment in Stage 1 of the dose-ranging trial throughout 2021.

AXO-Lenti-PD gene therapy for Parkinson’s disease

- Two GMP batches have been manufactured using an updated suspension-based process and have now completed fill and finish. Sio is awaiting final testing of these batches to support certification of at least one batch for use as clinical trial material by a Qualified Person in Q4 2021.
- Pending review and approval of an IMPD amendment by the MHRA in the U.K., we expect to resume enrollment of patients in the AXO-Lenti-PD clinical program in 2022.

Fiscal First Quarter Financial Summary

For the fiscal first quarter ended June 30, 2021, research and development expenses were $8.1 million, an increase of $2.9 million compared to the fiscal first quarter ended June 30, 2020. The current period increase was primarily related to increases in:

(i) AXO-AAV-GM1 clinical trial material manufacturing expenses for the planned enrollment of infantile patients in the high dose cohort, as well as clinical trial expenses due to the ongoing enrollment of juvenile patients in the high dose cohort and for the planned enrollment of infantile patients in

the low dose cohort;

(ii) AXO-AAV-GM2 clinical trial expenses associated with the ongoing enrollment of juvenile patients in the low dose cohort and for the planned enrollment of patients in the low-to-mid dose cohorts, as well as clinical trial material manufacturing expenses for the planned enrollment of patients in the mid-to-high dose cohorts (versus the prior year period, when this program was on clinical hold); and

(iii) personnel-related costs primarily due to increased headcount.

These increases were partially offset by a $1.1 million decrease in AXO-Lenti-PD costs. The delays in the development of a reliable suspension-based manufacturing process at Oxford Biomedica have resulted in lower than expected manufacturing expenses and also have delayed the initiation of further clinical studies of AXO-Lenti-PD. Additionally, early development programs were completed in the prior year period and as a result, development expenses have also decreased in the current year period.

General and administrative expenses for the fiscal first quarter ended June 30, 2021 were $3.9 million, a decrease of $0.7 million compared to the fiscal first quarter ended June 30, 2020, primarily related to decreased rent expense due to the downsizing of our New York office footprint, as well as reductions in accounting, auditing and tax fees, resulting, in part, from the simplification of our corporate structure and the domestication of the parent entity from Bermuda to Delaware that occurred in the prior year period.

The net loss for the fiscal first quarter ended June 30, 2021 was $11.9 million, or $0.16 per share, compared to a net loss of $8.6 million, or $0.20 per share, in the fiscal first quarter ended June 30, 2020. The prior year period net loss was partially offset by a gain of $2.2 million on our long-term investment in Arvelle Therapeutics B.V. ("Arvelle"). For the fiscal first quarter ended June 30, 2021, net cash used in operating activities was $12.7 million and net cash provided by investing activities of $4.2 million included $4.3 million of proceeds received from the sale of our long-term investment in Arvelle.

As of June 30, 2021, we had $111.0 million of cash and cash equivalents. We hold no short-term or long-term debt on the balance sheet. We estimate that our current cash and cash equivalents will sustain our operations into Q4 2022, beyond the expected dates of major upcoming milestones for our AXO-AAV-GM1 gene therapy program for the treatment of GM1 gangliosidosis.

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 Annual 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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SIO GENE THERAPIES INC.
Condensed Consolidated Statements of Operations
(Unaudited, in thousands, except share and per share amounts)

	Three Months Ended June 30,	
	2021	**2020**
Operating expenses:		
Research and development expenses (includes stock-based compensation expense of $432 and $563 for the three months ended June 30, 2021 and 2020, respectively)	$ 8,058	$ 5,194
General and administrative expenses (includes stock-based compensation expense of $889 and $1,027 for the three months ended June 30, 2021 and 2020, respectively)	3,859	4,640
Total operating expenses	11,917	9,834
Other (income) expenses:		
Interest expense	1	796
Other income	(20)	(2,066)
Loss before income tax (benefit) expense	(11,898)	(8,564)
Income tax (benefit) expense	(28)	30
Net loss	$ (11,870)	$ (8,594)
Net loss per share of common stock — basic and diluted	$ (0.16)	$ (0.20)
Weighted-average shares of common stock outstanding — basic and diluted	72,861,870	43,287,222

SIO GENE THERAPIES INC.
Condensed Consolidated Balance Sheets
(Unaudited, in thousands, except share and per share amounts)

	June 30, 2021	March 31, 2021
Assets		
Current assets:		
Cash and cash equivalents	$ 110,976	$ 118,986
Receivable from sale of long-term investment	—	4,343
Prepaid expenses and other current assets	6,276	7,348
Income tax receivable	1,681	1,656
Total current assets	118,933	132,333
Long-term restricted cash	1,184	1,184
Operating lease right-of-use assets	1,103	1,152
Property and equipment, net	595	478
Total assets	$ 121,815	$ 135,147
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 937	$ 1,341
Accrued expenses	6,389	9,196
Current portion of operating lease liabilities	269	311
Total current liabilities	7,595	10,848
Operating lease liabilities, net of current portion	921	932
Total liabilities	8,516	11,780
Stockholders' equity:		
Common stock, par value $0.00001 per share, 1,000,000,000 shares authorized, 69,639,509 and 69,377,567 issued and outstanding at June 30, 2021 and March 31, 2021, respectively	1	1
Additional paid-in capital	915,900	914,100
Accumulated deficit	(802,939)	(791,069)
Accumulated other comprehensive income	337	335
Total stockholders' equity	113,299	123,367
Total liabilities and stockholders' equity	$ 121,815	$ 135,147

Source: Sio Gene Therapies