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

February 11, 2022

- *Company prioritizing industry-leading clinical-stage programs, AXO-AAV-GM1 and AXO-AAV-GM2, the first potential disease-modifying therapies for GM1 gangliosidosis and Tay-Sachs/Sandhoff disease*

- *$81.9 million of cash and cash equivalents as of December 31, 2021, providing expected cash runway into calendar H2 2023 beyond the expected dates of major upcoming milestones for the AXO-AAV-GM1 and AXO-AAV-GM2 gene therapy programs*

NEW YORK and DURHAM, N.C., Feb. 11, 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 provided corporate updates and financial results for its fiscal third quarter ended December 31, 2021.

“As we recently announced, I’m excited to lead Sio as we enhance our focus on rare genetic diseases and implement measures to extend our expected cash runway into the second half of 2023,” said David Nassif, J.D., interim Chief Executive Officer of Sio. “While the relentless nature of GM1 and GM2 gangliosidosis drives us to move with urgency every day in order to potentially transform the lives of these patients and their families, we believe our GM1 and GM2 programs also bring meaningful value for our shareholders. These programs represent a critical opportunity, where the life-changing value for families desperately in need and the market value for our shareholders perfectly overlap and provide the motivation to keep our team moving with a sense of purpose for all stakeholders. I look forward to sharing continued updates across each of these programs as they reach meaningful clinical milestones.”

Key Developments and Priorities

- *AXO-AAV-GM1*
  - Presented updated safety data from the most advanced GM1 gene therapy program in the industry, at the 18th Annual WORLD*Symposium* ™ 2022, held from February 7-11, 2022
    - Ten patients across all pediatric subtypes (early infantile, late infantile, and juvenile) of GM1 gangliosidosis have received AXO-AAV-GM1 gene therapy to date
    - AXO-AAV-GM1 remains generally well tolerated in all ten patients at both low and high doses to date
      - There have been no reported drug-related serious adverse events
      - The majority of adverse events were considered mild to moderate, and no safety signals have been identified
  - The totality of the data to date have demonstrated a favorable risk: benefit profile and a dose-dependent improvement in key biomarkers of disease activity (β-galactosidase enzyme activity in the serum and GM1 ganglioside activity in the CSF) across the low- and high-dose cohorts
  - Based on developmental and mobility assessments, there was no clinical evidence of disease progression in 4 out of 5 low-dose subjects at 12 months or in the high-dose cohort at 6 months
  - Strategic Priorities:
    - Calendar 1H 2022: Present a data update from Stage 1 of the Phase 1/2 study, including a first look at Type I (early infantile) patients treated in the low-dose cohort and longer-term data from the Type II (late infantile to juvenile) patient cohort at future scientific conferences
    - Calendar 2022: Intend to engage with the FDA to review Stage 1 data and discuss next steps for clinical development
- *AXO-AAV-GM2*
  - Dosed first four 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 three patients at the low dose
  - Strategic Priorities:
    - 2022: Expect continued patient identification, screening, and enrollment in the mid-dose cohort (n= ~3) of the dose-ranging trial

Fiscal Third Quarter Financial Summary

Research and development expenses were $21.3 million for the three months ended December 31, 2021 and $6.4 million for the three months ended December 31, 2020, increasing by $14.9 million. The $14.9 million increase was primarily related to:

(i) increased AXO-AAV-GM1 program expenses primarily related to clinical trial material manufacturing expenses for the planned enrollment of

infantile patients in the high-dose cohort, as well as a $1.5 million license fee milestone due in December 2021 under the terms of our exclusive license agreement with the University of Massachusetts Medical School ("UMMS" and collectively, the "UMMS Agreement");

(ii) increased AXO-AAV-GM2 program expenses primarily related to a $1.5 million license fee milestone due in December 2021 under the terms of the UMMS Agreement, as well as non-GMP and GMP manufacturing expenses;

(iii) increased AXO-Lenti-PD program expenses, primarily related to clinical trial material manufacturing expenses resulting from Qualified Person certification of GMP batches and a $2.0 million nonrecurring development milestone achieved upon the successful completion of the updated suspension-based manufacturing process for the AXO-Lenti-PD program in the fourth calendar quarter of 2021; and

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

General and administrative expenses were $4.1 million for the three months ended December 31, 2021 and $4.2 million for the three months ended December 31, 2020. The decrease of $0.1 million was primarily related to decreases totaling $1.0 million for (i) rent, depreciation and overhead expenses due to the downsizing of our New York office footprint, and (ii) tax, legal, auditing and accounting fees resulting primarily from the simplification of our corporate structure and the domestication of Sio Gene Therapies Inc. from Bermuda to Delaware that was completed in November 2020. These decreases were partially offset by an increase of $0.7 million of stock-based compensation expense primarily associated with certain equity instruments of our affiliate, Roivant Sciences Ltd. ("RSL"), held by our former CEO (the "RSL Equity Instruments"), who resigned as our CEO in January 2022. Expensing of the RSL Equity Instruments commenced upon the liquidity event vesting condition being met upon the closing of RSL's business combination with Montes Archimedes Acquisition Corp. on September 30, 2021.

The net loss for the fiscal third quarter ended December 31, 2021 was $25.5 million, or $0.35 per share, compared to a net loss of $10.5 million, or $0.20 per share, in the fiscal third quarter ended December 31, 2020.

Nine-Months Financial Summary

Research and development expenses were $40.8 million for the nine months ended December 31, 2021 and $16.7 million for the nine months ended December 31, 2020, increasing by $24.1 million. The $24.1 million increase was primarily related to:

(i) increased AXO-AAV-GM1 program expenses primarily related to clinical trial material manufacturing expenses for the planned enrollment of infantile patients in the high-dose cohort, as well as a $1.5 million license fee milestone due in December 2021 under the terms of the UMMS Agreement;

(ii) increased AXO-AAV-GM2 program expenses primarily related to non-GMP and GMP manufacturing expenses, clinical trial expenses associated with the ongoing enrollment of patients in the low-dose cohort and for the planned enrollment of patients in the mid-dose cohort, as well as a $1.5 million license fee milestone due in December 2021 under the terms of the UMMS Agreement;

(iii) increased AXO-Lenti-PD program expenses, primarily related to clinical trial material manufacturing expenses resulting from Qualified Person certification of GMP batches and a $2.0 million nonrecurring development milestone achieved upon the successful completion of the updated suspension-based manufacturing process for the AXO-Lenti-PD program in the fourth calendar quarter of 2021, which were partially offset by decreased analytical development costs; and

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

General and administrative expenses were $17.7 million for the nine months ended December 31, 2021 and $13.3 million for the nine months ended December 31, 2020. The increase of $4.4 million was primarily related to $6.7 million of stock-based compensation expense primarily associated with the RSL Equity Instruments recorded during the nine months ended December 31, 2021. This increase was partially offset by decreases totaling $2.7 million for (i) rent, depreciation and overhead expenses due to the downsizing of our New York office footprint, and (ii) tax, legal, auditing and accounting fees resulting primarily from the simplification of our corporate structure and the domestication of Sio Gene Therapies Inc. from Bermuda to Delaware that was completed in November 2020.

The net loss for the nine months ended December 31, 2021 was $58.6 million, or $0.80 per share, compared to a net loss of $29.1 million, or $0.61 per share, in the nine months ended December 31, 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 nine months ended December 31, 2021, net cash used in operating activities was $42.5 million and net cash provided by investing activities of $4.0 million included $4.3 million of proceeds received from the sale of our long-term investment in Arvelle.

As of December 31, 2021, we had $81.9 million of cash and cash equivalents. We hold no short-term or long-term debt on the balance sheet. As a result of our decision to terminate the AXO-Lenti-PD gene therapy program for the treatment of Parkinson's disease that is expected to become effective by March 31, 2022, we estimate that our current cash and cash equivalents are sufficient to support operations into the second half of calendar year 2023, including beyond the expected dates of major upcoming milestones for our AXO-AAV-GM1 and AXO-AAV-GM2 gene therapy programs.

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.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 “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 February 11, 2022, 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.

Contacts:

Media

Josephine Belluardo, Ph.D.
LifeSci Communications
(646) 751-4361
jo@lifescicomms.com
info@siogtx.com

Investors and Analysts

David W. Nassif
Sio Gene Therapies Inc.
Interim Chief Executive Officer; Chief Financial Officer and General Counsel
david.nassif@siogtx.com

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

	Three Months Ended December 31,		Nine Months Ended December 31,	
	2021	2020	2021	2020
Operating expenses:				
Research and development expenses (includes stock-based compensation expense of $130 and $259 for the three months ended December 31, 2021 and 2020, respectively, and $1,051 and $1,280 for the nine months ended December 31, 2021 and 2020, respectively)	$ 21,287	$ 6,407	$ 40,793	$ 16,659
General and administrative expenses (includes stock-based compensation expense of $1,268 and $617 for the three months ended December 31, 2021 and 2020, respectively, and $8,966 and $2,294 for the nine months ended December 31, 2021 and 2020, respectively)	4,086	4,198	17,693	13,329
Total operating expenses	25,373	10,605	58,486	29,988
Other expenses (income):				
Interest expense	7	1	19	798
Other expense (income)	76	98	86	(1,388)
Loss before income tax benefit	(25,456)	(10,704)	(58,591)	(29,398)
Income tax benefit	—	(188)	(28)	(304)
Net loss	$ (25,456)	$ (10,516)	$ (58,563)	$ (29,094)
Net loss per share of common stock — basic and diluted	$ (0.35)	$ (0.20)	$ (0.80)	$ (0.61)
Weighted-average shares of common stock outstanding — basic and diluted	73,335,279	52,679,816	73,046,889	47,581,795

SIO GENE THERAPIES INC.

Condensed Consolidated Balance Sheets

(Unaudited, in thousands, except share and per share amounts)

	December 31, 2021	March 31, 2021
Assets		
Current assets:		
Cash and cash equivalents	$ 81,910	$ 118,986
Restricted cash	1,184	—
Receivable from sale of long-term investment	—	4,343
Prepaid expenses and other current assets	5,244	7,348
Income tax receivable	1,732	1,656
Total current assets	90,070	132,333
Long-term restricted cash	—	1,184
Operating lease right-of-use assets	2,613	1,152
Property and equipment, net	623	478
Total assets	$ 93,306	$ 135,147
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 6,611	$ 1,341
Accrued expenses	7,762	9,196
Current portion of operating lease liabilities	765	311
Total current liabilities	15,138	10,848
Operating lease liabilities, net of current portion	1,903	932
Total liabilities	17,041	11,780
Stockholders' equity:		
Common stock, par value $0.00001 per share, 1,000,000,000 shares authorized, 73,697,110 and 69,377,567 issued and outstanding at December 31, 2021 and March 31, 2021, respectively	1	1
Additional paid-in capital	925,558	914,100
Accumulated deficit	(849,632)	(791,069)
Accumulated other comprehensive income	338	335
Total stockholders' equity	76,265	123,367
Total liabilities and stockholders' equity	$ 93,306	$ 135,147

Source: Sio Gene Therapies