Sio Gene Therapies Announces Corporate Updates and Financial Results for Second Fiscal Quarter Ended September 30, 2020

November 13, 2020

- *Continued progress across pipeline of gene therapy programs, including recent IND clearance for AXO-AAV-GM2 in Tay-Sachs/Sandhoff diseases*
- *Completed rebranding to Sio Gene Therapies as part of corporate transformation aligning corporate structure and governance with current and future business activity*
- *Company had $63.2 million of cash and cash equivalents as of September 30, 2020, with sufficient cash runway into Q4 2021*

NEW YORK and RESEARCH TRIANGLE PARK, N.C., Nov. 13, 2020 (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 financial results for its second fiscal quarter ended September 30, 2020.

“In recent months, we have taken significant strides forward with our AAV-based gene therapy programs in GM1 gangliosidosis and Tay-Sachs/Sandhoff diseases. We obtained rare pediatric disease designation for both programs, and following IND clearance of AXO-AAV-GM2 from the FDA, we’re delivering on our goal of advancing the first potentially curative gene therapy clinical development programs for both GM1 and GM2 gangliosidosis,” said Pavan Cheruvu, M.D., Chief Executive Officer of Sio Gene Therapies. “We also advanced AXO-Lenti-PD in the SUNRISE-PD dose-escalation study and presented detailed patient-level data from the mid-dose cohort at our Parkinson’s disease focused R&D Day last month. Our rebranding as Sio Gene Therapies signifies a new beginning for the company – with a scientific focus, management team, Board of Directors, and portfolio strategy that is wholly committed to developing disease-modifying and curative genetic medicines on behalf of patients in need.”

Key Highlights and Development Updates

AXO-AAV-GM1 gene therapy for GM1 gangliosidosis

- On-track to report 6-month topline data, with a focus on safety and tolerability, from 5 children in the low-dose juvenile cohort (Type II) by year-end 2020.
- The U.S. Food and Drug Administration (FDA) granted Rare Pediatric Disease Designation for AXO-AAV-GM1 in GM1 gangliosidosis.
- Expect to complete dosing of juvenile (Type II) patients in the high-dose cohort of the ongoing AXO-AAV-GM1 clinical study before year-end 2020.

AXO-AAV-GM2 gene therapy for Tay-Sachs/Sandhoff disease

- FDA cleared Company-sponsored Investigational New Drug (IND) application for AXO-AAV-GM2 in Tay-Sachs and Sandhoff diseases.
- FDA granted Rare Pediatric Disease Designation for AXO-AAV-GM2.

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

- Hosted an R&D Day on October 30, 2020, during which individual patient-level 6-month follow up data were presented from the second cohort of the SUNRISE-PD dose escalation trial. In addition, key opinion leaders in Parkinson’s disease clinical research and the Michael J. Fox Foundation discussed the treatment landscape and the potential role of AXO-Lenti-PD gene therapy in the treatment paradigm.
- Reported positive 6-month follow-up data from the second cohort of the SUNRISE-PD Phase 2 trial
  - AXO-Lenti-PD was observed to be well-tolerated with no treatment related serious adverse events at 6 months
  - Greater than 2-hour improvement from baseline in both diary “good ON time” and diary OFF time assessments observed across all four patients in Cohort 2
  - Reported a 21-point mean improvement in UPDRS Part III “OFF” score in the two patients with evaluable data, a 40% improvement from baseline
  - Totality of individual patient outcomes across cohort demonstrate consistency of treatment benefit
- Based on new information received from our manufacturing partner, Oxford Biomedica, in mid-October regarding delays in CMC data and third-party fill/finish issues, the development of a suspension-based manufacturing process for AXO-Lenti-PD will take longer than expected. As a result, the Company believes that it is unlikely that its planned randomized, sham-controlled trial of AXO-Lenti-PD will enroll patients by the end of calendar year 2021. Manufacturing of

several GMP batches is now underway and planned at Oxford Biomedica with a goal of generating material for use in future clinical trials as soon as possible. The Company expects to provide an update in the first quarter of 2021 or as program timelines are clarified.

Corporate Updates

- Continued corporate transformation activities, including:
  - Company name change to Sio Gene Therapies. In connection with the name change, the Company's ticker on the NASDAQ exchange will change to "SIOX" and will be effective at market open on November 13, 2020
  - Appointment of Kristiina Vuori, M.D., Ph.D, as a new director, establishing a majority independent Board of Directors
  - No longer being a majority-owned and controlled public company
  - Completed redomiciliation to Delaware
- Signed strategic gene therapy development and manufacturing partnership with Viralgen, an AskBio subsidiary, securing access to cGMP capacity and resources to support the development and commercialization of AAV gene therapy programs in GM1 gangliosidosis and Tay-Sachs/Sandhoff diseases.
- Opening of new laboratory space in Research Triangle Park, North Carolina, focused on in-house preclinical and analytical development activities.
- Promoted Parag V. Meswani, Pharm.D. to Chief Commercial Officer to support Axovant's commercialization efforts across the clinical-stage pipeline.

Fiscal Second Quarter Financial Summary

For the second fiscal quarter ended September 30, 2020, research and development expenses were $5.1 million, a decrease of $1.8 million compared to the prior year quarter, primarily due to (i) lower AXO-Lenti-PD clinical expenses of approximately $0.9 million as the enrollment of Cohort 2 was completed in February 2020, (ii) reduced costs of $0.7 million while awaiting FDA clearance of the IND for the AXO-AAV-GM2 program, and (iii) a $0.5 million reversal of an accrual for manufacturing development services for our AXO-AAV-GM1 and AXO-AAV-GM2 programs under an agreement that was terminated.

General and administrative expenses for the second fiscal quarter ended September 30, 2020 were $4.5 million, a decrease of $0.6 million compared to the prior year quarter, primarily due to reductions in personnel costs (including severance) attributable to reduced headcount.

The net loss for the second fiscal quarter ended September 30, 2020 was $10.0 million, or $0.21 per share, compared to a net loss of $13.9 million, or $0.61 per share, in the prior year quarter.

Fiscal First-Half Financial Summary

For the six months ended September 30, 2020, research and development expenses were $10.3 million, a decrease of $17.7 million compared to the six months ended September 30, 2019. Excluding the net amount of $13.0 million due to Oxford for a development milestone achieved in the prior year period as well as a decrease of $2.1 million of expenses associated with our discontinued legacy AXO-AAV-OPMD program that was terminated in September 2019, research and development expenses decreased by $2.6 million in the current year period. The current period decrease was primarily due to (i) reduced costs of $1.0 million while awaiting FDA clearance of the IND for the AXO-AAV-GM2 program, (ii) a $0.8 million payment in the prior year period to our licensor, University of Massachusetts Medical School, for reaching a manufacturing milestone for the AXO-AAV-GM1 program, and (iii) a $0.5 million reversal of an accrual for manufacturing development services for our AXO-AAV-GM1 and AXO-AAV-GM2 programs under an agreement that was terminated.

General and administrative expenses for the six months ended September 30, 2020 were $9.1 million, a decrease of $2.4 million compared to the six months ended September 30, 2019, primarily related to reductions in (i) personnel costs (including severance) of $1.3 million and stock-based compensation expense of $0.2 million attributable to reduced headcount, and (ii) pharmaceutical market research expenses of $0.6 million.

The net loss for the six months ended September 30, 2020 was $18.6 million, or $0.41 per share, compared to a net loss of $41.9 million, or $1.84 per share, in the six months ended September 30, 2019. Net cash used in operating activities was $25.3 million for the six months ended September 30, 2020.

As of September 30, 2020, we had $63.2 million of cash and cash equivalents. The Company holds no short-term or long-term debt on the balance sheet. We expect the cash and cash equivalents to sustain our operations into the fourth calendar quarter of 2021.

On October 2, we filed a prospectus supplement with the SEC pertaining to a $50 million at-the-market equity financing facility. No sales under the facility occurred prior to our press release on October 6 and no sales have occurred since October 9. Approximately 1.2 million shares for total proceeds of $5.1 million, net of brokerage fees, were sold under the facility during this period.

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 “will,” “expect,” “believe,” “estimate,” and other similar expressions are intended to identify forward-looking statements. For example, all statements Sio makes regarding costs associated with its operating activities 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 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 November 13, 2020, 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.

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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 September 30,		Six Months Ended September 30,	
	2020	**2019**	**2020**	**2019**
Operating expenses:				
Research and development expenses (includes stock-based compensation expense of $458 and $409 for the three months ended September 30, 2020 and 2019 and $1,021 and $1,130 for the six months ended September 30, 2020 and 2019, respectively)	$ 5,058	$ 6,833	$ 10,252	$ 27,923
General and administrative expenses (includes stock-based compensation expense of $650 and $482 for the three months ended September 30, 2020 and 2019 and $1,677 and $1,896 for the six months ended September 30, 2020 and 2019, respectively)	4,491	5,051	9,131	11,519
Total operating expenses	9,549	11,884	19,383	39,442
Other (income) expenses:				
Interest expense	1	1,313	797	2,871
Other expense (income)	580	560	(1,486)	(537)
Loss before income tax (benefit) expense	(10,130)	(13,757)	(18,694)	(41,776)
Income tax (benefit) expense	(146)	127	(116)	165
Net loss	$ (9,984)	$ (13,884)	$ (18,578)	$ (41,941)
Net loss per common share — basic and diluted	$ (0.21)	$ (0.61)	$ (0.41)	$ (1.84)

Weighted-average common shares outstanding — basic and diluted	46,731,666	22,783,182	45,018,855	22,781,657

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

	September 30, 2020	March 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 63,171	$ 80,752
Prepaid expenses and other current assets	5,406	2,971
Income tax receivable	1,747	1,707
Total current assets	70,324	85,430
Long-term investment	8,055	5,871
Other non-current assets	169	46
Operating lease right-of-use assets	663	1,532
Property and equipment, net	560	801
Total assets	$ 79,771	$ 93,680
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	$ 2,172	$ 4,412
Accrued expenses	7,837	11,319
Current portion of operating lease liabilities	34	889
Current portion of long-term debt	—	15,423
Total current liabilities	10,043	32,043
Operating lease liabilities, net of current portion	55	79
Total liabilities	10,098	32,122
Stockholders' equity:		
Common stock, par value $0.00001 per share, 1,000,000,000 shares authorized, 47,249,729 and 39,526,299 issued and outstanding at September 30, 2020 and March 31, 2020, respectively	—	—
Additional paid-in capital	846,558	820,257
Accumulated deficit	(777,222)	(758,644)
Accumulated other comprehensive loss	337	(55)
Total stockholders' equity	69,673	61,558
Total liabilities and stockholders' equity	$ 79,771	$ 93,680