Sangamo Therapeutics Reports Recent Business Highlights and Second Quarter 2025 Financial Results

August 7, 2025

Announced positive topline results from registrational STAAR study in Fabry disease, including positive mean annualized estimated glomerular filtration rate (eGFR) slope at 52-weeks across all dosed patients in the study, which U.S. Food and Drug Administration (FDA) has agreed will serve as primary basis of approval.

First clinical site initiated for Phase 1/2 STAND study in chronic neuropathic pain. Expect to dose first patient in fall of 2025, with preliminary proof of efficacy data anticipated in late 2026.

Held productive meeting with Medicines and Healthcare products Regulatory Agency (MHRA) to discuss prion disease study ahead of anticipated Clinical Trial Application (CTA) submission.

RICHMOND, Calif., Aug. 07, 2025 (GLOBE NEWSWIRE) -- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today reported recent business highlights and second quarter 2025 financial results.

"I'm proud of the progress achieved across our pipeline this quarter. The announcement of positive topline results from our registrational STAAR study in Fabry disease represented a significant step forward on our path towards commercialization for this important program," said Sandy Macrae, Chief Executive Officer of Sangamo Therapeutics. "This quarter we also became a clinical-stage neurology company, with the initiation of our first clinical site in the Phase 1/2 STAND study in chronic neuropathic pain. We are excited to soon dose the first patient in this study and look forward to sharing clinical data by the end of 2026."

Recent Business Highlights

Corporate Updates

- Raised approximately $21 million in net proceeds from an underwritten registered equity offering.

Fabry Disease

- Announced positive topline results from the registrational Phase 1/2 STAAR study evaluating isaralgagene civaparvovec, or ST-920, a wholly owned investigational gene therapy for the treatment of adults with Fabry disease.
- Following a single dose of isaralgagene civaparvovec, a positive mean annualized eGFR slope of 1.965 mL/min /1.73m^2/year (95% confidence interval (CI): -0.153, 4.083) at 52-weeks was observed across all 32 dosed patients in the study, which the FDA has agreed will serve as an intermediate clinical endpoint under the Accelerated Approval pathway.
- Furthermore, a mean annualized eGFR slope at Week 104 of 1.747 mL/min/1.73m^2/year (95% CI: -0.106, 3.601) was observed for the 19 patients who have achieved 104-weeks of follow-up.
- Key secondary endpoints in the study were also positive. Elevated expression of alpha-galactosidase A (α-Gal A) activity was maintained for up to 4.5 years for the longest treated patient. Plasma lyso-Gb3 levels remained generally stable following Enzyme Replacement Therapy (ERT) withdrawal. A stabilization in cardiac endpoints was also observed, including cardiac function, morphological and biomarker data in the 32 patients with 52 weeks of follow-up.
- Patients demonstrated a range of other clinical benefits, including improvements in disease severity reported in the Fabry Outcome Survey adaptation of the Mainz Severity Score Index (FOS-MSSI) age-adjusted score and statistically and clinically significant improvements in the short form-36 (SF-36) quality of life scores at week 52 compared to baseline. Statistically significant improvements in the gastrointestinal symptoms rating scale (GSRS) compared to baseline were also observed.
- Furthermore, following a single administration of isaralgagene civaparvovec, additional clinical benefits were observed in some patients, such as the reduction or elimination in pain medication usage and the resumption of sweating, that has enabled these patients to perform physical tasks and exercise.
- Isaralgagene civaparvovec demonstrated a favorable safety and tolerability profile in the study, without the requirement for preconditioning. The majority of adverse events were grade 1-2 in nature.
- We believe these data support the potential for isaralgagene civaparvovec to be a one-time, durable treatment for Fabry disease that can improve patient outcomes and will form the basis for an anticipated Biologics License Application (BLA) submission under the Accelerated Approval pathway as early as the first quarter of 2026.
- Sangamo plans to present additional clinical data at the 15th International Congress of Inborn Errors of Metabolism (ICIEM2025), September 2-6, 2025 in Kyoto, Japan.
- Sangamo continues to engage with the FDA ahead of the planned BLA submission for isaralgagene civaparvovec, and continues to engage in business development negotiations for a potential Fabry commercialization agreement.

Core Neurology Pipeline

Chronic Neuropathic Pain – ST-503

- Nine clinical sites selected to date for the Phase 1/2 STAND study evaluating ST-503, an investigational epigenetic regulator for the treatment of intractable pain due to idiopathic small fiber neuropathy (iSFN), a type of chronic neuropathic pain.
- First clinical site has been initiated and patient identification is in progress.
- Expect to dose first patient in the fall of 2025, with preliminary proof of efficacy data anticipated in Q4 2026.
- Plan to present updated nonclinical data at the 9th International Congress on Neuropathic Pain, taking place September 4-6, 2025 in Berlin, Germany.

Prion Disease – ST-506

- CTA-enabling activities continue to advance for ST-506, an investigational epigenetic regulator for the treatment of prion disease, leveraging STAC-BBB.
- Held productive meeting with the MHRA, including alignment on nonclinical safety studies and clinical study design.
- Presented in the prestigious Presidential Symposium at the 28th American Society of Gene & Cell Therapy (ASGCT) Annual Meeting to showcase the potent combination of epigenetic regulation and capsid delivery technology for the treatment of prion disease in animal models, including a profound survival extension observed in disease mouse models.
- Completed ST-506 dose range finding study and advancing preparations for good laboratory practice (GLP) toxicology study.
- A CTA submission for ST-506 is expected as early as mid-2026.

Second Quarter 2025 Financial Results

Consolidated net loss for the second quarter ended June 30, 2025 was $20.0 million, or $0.08 per share, compared to a net loss of $36.1 million, or $0.18 per share, for the same period in 2024.

Revenues

Revenues for the second quarter ended June 30, 2025 were $18.3 million, compared to $0.3 million for the same period in 2024.

The increase of $18.0 million in revenues was primarily attributable to our receipt of an upfront license payment under our capsid license agreement with Eli Lilly and Company.

GAAP and Non-GAAP Operating Expenses

	Three months ended June 30,		Six Months Ended June 30,	
(In millions)	**2025**	**2024**	**2025**	**2024**
Research and development	$ 27.1	$ 24.2	$ 53.1	$ 60.1
General and administrative	9.1	12.0	19.1	23.8
Impairment of long-lived assets	-	1.2	-	5.5
Total operating expenses	36.2	37.4	72.2	89.4
Impairment of long-lived assets	-	(1.2)	-	(5.5)
Depreciation and amortization	(1.0)	(1.2)	(2.0)	(2.6)
Stock-based compensation	(2.2)	(3.1)	(4.8)	(5.8)
Non-GAAP operating expenses	$ 33.0	$ 31.9	$ 65.4	$ 75.5

Total operating expenses on a GAAP basis for the second quarter ended June 30, 2025 were $36.2 million, compared to $37.4 million for the same period in 2024. Non-GAAP operating expenses, which exclude impairment charges, depreciation and amortization, and stock-based compensation expense, for the second quarter ended June 30, 2025 were $33.0 million, compared to $31.9 million for the same period in 2024.

The decrease in total operating expenses on a GAAP basis was primarily driven by cost reductions resulting from the strategic realignment of the business, which included a lower headcount due to the restructuring of operations and corresponding reductions in workforce, lower impairment charges recorded in the current year, contract termination costs recorded in 2024 relating to a manufacturing-related supplier arrangement, and a decrease in facilities and infrastructure costs. These decreases were partially offset by an increase in clinical and manufacturing expenses due to BLA readiness activities for our Fabry disease program.

Cash and Cash Equivalents

Cash and cash equivalents as of June 30, 2025 were $38.3 million, compared to cash and cash equivalents of $41.9 million as of December 31, 2024. Based on our current operating plan, we believe that our cash and cash equivalents as of June 30, 2025, together with the proceeds from sales of common stock under our at-the-market offering program since June 30, 2025, will be sufficient to fund our planned operations into the fourth quarter of

2025.

Financial Guidance for 2025 Reiterated

On a GAAP basis, we continue to expect total operating expenses in the range of approximately $135 million to $155 million in 2025, which includes estimated non-cash stock-based compensation expense, and depreciation and amortization.

We continue to expect non-GAAP total operating expenses, excluding estimated non-cash stock-based compensation expense of approximately $7 million, and estimated depreciation and amortization of approximately $3 million, in the range of approximately $125 million to $145 million in 2025, consistent with 2024. This reflects our intention to operate a lean neurology-focused business and to continue advancing isaralgagene civaparvovec towards a potential BLA submission, while continuing to engage in business development negotiations for a potential Fabry commercialization agreement.

Upcoming Events

Sangamo plans to participate in the following events:

Investor Conferences

- Cantor Global Healthcare Conference 2025, September 3-5, 2025
- Wells Fargo Healthcare Conference, September 3-5, 2025

Access links for available webcasts for investor conferences will be available on the Sangamo website in the Investors and Media section under Events. Available materials will be found on the Sangamo website after the event under Presentations.

Conference Call

The Sangamo management team will hold a corporate call to further discuss program and financial updates on Thursday, August 7, at 4:30pm Eastern Time.

Participants should register for, and access, the call using this link. While not required, it is recommended you join 10 minutes prior to the event start. Once registered, participants will be given the option to either dial into the call with the number and unique passcode provided or to use the dial-out option to connect their phone instantly.

An updated corporate presentation is available in the Investors and Media section under Presentations.

The link to access the live webcast can also be found on the Sangamo website in the Investors and Media section under Events. A replay will be available following the conference call, accessible at the same link.

About Sangamo Therapeutics

Sangamo Therapeutics is a genomic medicine company dedicated to translating ground-breaking science into medicines that transform the lives of patients and families afflicted with serious neurological diseases who do not have adequate or any treatment options. Sangamo believes that its zinc finger epigenetic regulators are ideally suited to potentially address devastating neurological disorders and that its capsid discovery platform can expand delivery beyond currently available intrathecal delivery capsids, including in the central nervous system. Sangamo's pipeline also includes multiple partnered programs and programs with opportunities for partnership and investment. To learn more, visit www.sangamo.com and connect with us on LinkedIn and X.

Forward-Looking Statements

This press release contains forward-looking statements regarding our current expectations. These forward-looking statements include, without limitation, statements relating to: Sangamo's cash runway and ability to continue to operate as a going concern; the therapeutic and commercial potential and value of Sangamo's product candidates, including the durability of therapeutic effects, the therapeutic and commercial potential and value of technologies used by Sangamo in its product candidates, including the potential for isaralgagene civaparvovec to be a one-time, durable treatment for Fabry disease that can improve patient outcomes; expectations concerning regulatory approval and commercialization of isaralgagene civaparvovec, including the potential for isaralgagene civaparvovec to qualify for the FDA's Accelerated Approval program, the adequacy of data generated in the Phase 1/2 STAAR study to support FDA approval, and plans for a potential BLA submission for isaralgagene civaparvovec and the timing thereof; Sangamo's plans and ability to establish and maintain collaborations and strategic partnerships and realize the expected benefits of such arrangements, including its plans to secure a commercialization partner for its Fabry disease program; the anticipated plans and timelines for conducting, and presenting clinical data from, clinical trials; the advancement of Sangamo's preclinical neurology programs, including plans related to initiation of patient dosing for ST-503 and announcement of preliminary proof of efficacy data, and the anticipated prion disease CTA submission and announcement of related preliminary clinical data, and in each case the timing thereof; Sangamo's estimates regarding the sufficiency of its cash resources and its expenses, capital requirements and need for substantial additional financing; Sangamo's 2025 financial guidance; plans to participate in industry and investor conferences; and other statements that are not historical fact. These statements are not guarantees of future performance and are subject to certain risks and uncertainties that are difficult to predict. Factors that could cause actual results to differ include, but are not limited to, risks and uncertainties related to Sangamo's lack of capital resources and need for substantial additional funding to execute its operating plan and to continue to operate as a going concern, including the risk that Sangamo will be unable to obtain substantial additional funding on acceptable terms or at all or collaboration partners necessary to advance its preclinical and clinical programs, in particular for its Fabry disease program and to otherwise operate as a going concern, in which case Sangamo may be required to cease operations entirely, liquidate all or a portion of its assets and/or seek protection under the U.S. Bankruptcy Code; the potential for collaborators and licensees to breach or terminate their agreements with Sangamo; the potential for Sangamo to fail to realize its expected benefits from its collaboration and license agreements; the uncertain and costly research and development process, including the risk that preclinical results may not be indicative of results in any future clinical trials; the effects of macroeconomic factors or financial challenges, including as a result of the ongoing overseas conflicts, tariffs, geopolitical instability, inflation and fluctuations in interest rates, on the global business environment, healthcare systems and business and operations of Sangamo and its collaborators, including the initiation and operation of clinical trials; the impacts of clinical trial delays, pauses and holds on clinical

trial timelines and commercialization of product candidates; the uncertain timing and unpredictable nature of clinical trial results, including risk that the therapeutic effects observed in the latest preliminary clinical data from the Phase 1/2 STAAR study will not be durable in patients and that final clinical trial data from the study will not validate the safety and efficacy of isaralgagene civaparvovec, including that the 104-week data from such study will not verify the clinical benefit of isaralgagene civaparvovec or support FDA approval, and that the patients withdrawn from ERT will remain off ERT; the unpredictable regulatory approval process for product candidates across multiple regulatory authorities; reliance on results of early clinical trials, which results are not necessarily predictive of future clinical trial results, including the results of any registrational trial of Sangamo's product candidates; the potential for technological developments that obviate technologies used by Sangamo; Sangamo's reliance on collaborators and its potential inability to secure additional collaborations, and Sangamo's ability to achieve expected future operating results.

All forward-looking statements about Sangamo's future plans and expectations, including Sangamo's financial guidance and development plans for its product candidates, are subject to Sangamo's ability to secure adequate additional funding. There can be no assurance that Sangamo and its collaborators will be able to develop commercially viable products or that Sangamo will earn any milestone or royalty payments under its collaboration agreements. Actual results may differ materially from those projected in these forward-looking statements due to the risks and uncertainties described above and other risks and uncertainties that exist in the operations and business environments of Sangamo and its collaborators. These risks and uncertainties are described more fully in Sangamo's Securities and Exchange Commission, or SEC, filings and reports, including in Sangamo's Annual Report on Form 10-K for the year ended December 31, 2024 and Quarterly Report on Form 10-Q for the quarter ended June 30, 2025, and subsequent filings and reports that Sangamo makes from time to time with the SEC. Forward-looking statements contained in this announcement are made as of this date, and Sangamo undertakes no duty to update such information except as required under applicable law.

Non-GAAP Financial Measures

To supplement our financial results and guidance presented in accordance with GAAP, we present non-GAAP operating expenses, which excludes depreciation and amortization, stock-based compensation expense and impairment of long-lived assets from GAAP operating expenses. We believe that this non-GAAP financial measure, when considered together with our financial information prepared in accordance with GAAP, can enhance investors' and analysts' ability to meaningfully compare our results from period to period and to our forward-looking guidance, and to identify operating trends in our business. We have excluded depreciation and amortization, and stock-based compensation expense because they are non-cash expenses that may vary significantly from period to period as a result of changes not directly or immediately related to the operational performance for the periods presented, and we have excluded impairment of long-lived assets to facilitate a more meaningful evaluation of our current operating performance and comparisons to our operating performance in other periods. This non-GAAP financial measure is in addition to, not a substitute for, or superior to, measures of financial performance prepared in accordance with GAAP. We encourage investors to carefully consider our results under GAAP, as well as our supplemental non-GAAP financial information, to more fully understand our business.

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SELECTED CONSOLIDATED FINANCIAL DATA
(unaudited; in thousands, except per share data)

Statement of Operations Data:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	**2024**	**2025**	**2024**
Revenues	$ 18,306	$ 356	$ 24,743	$ 837
Operating expenses:				
Research and development	27,084	24,223	53,090	60,114
General and administrative	9,077	12,045	19,136	23,812
Impairment of long-lived assets	-	1,172	-	5,521
Total operating expenses	36,161	37,440	72,226	89,447
Loss from operations	(17,855)	(37,084)	(47,483)	(88,610)
Interest income	386	416	695	867
Other (expense) income, net	(2,490)	614	(3,649)	2,698
Loss before income taxes	(19,959)	(36,054)	(50,437)	(85,045)
Income tax expense	27	74	146	172
Net loss	$ (19,986)	$ (36,128)	$ (50,583)	$ (85,217)
Basic and diluted net loss per share	$ (0.08)	$ (0.18)	(0.21)	(0.44)
Shares used in computing basic and diluted net loss per share	256,950	203,946	238,711	194,049

Selected Balance Sheet Data:

	June 30, 2025	December 31, 2024
Cash and cash equivalents	$ 38,344	$ 41,918
Total assets	$ 97,558	$ 101,635
Total stockholders' equity	$ 19,602	$ 22,770

Source: Sangamo Therapeutics, Inc.