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

May 12, 2025

Announced capsid license agreement with Lilly to deliver genomic medicines for up to five central nervous system (CNS) disease targets. Received $18 million upfront license fee for first target and eligible to earn up to $1.4 billion in additional licensed target fees and milestone payments, plus tiered royalties on potential net sales.

Announced derisking milestones in pathway to anticipated biologics license application (BLA) submission for isaralgagene civaparvovec in Fabry disease, including all patients having passed one-year milestone required by U.S. Food and Drug Administration (FDA) for Accelerated Approval regulatory pathway, and productive Type B Chemistry, Manufacturing and Controls (CMC) meeting with FDA.

Announced participation at 28th American Society of Gene & Cell Therapy (ASGCT) Annual Meeting, including platform presentation in prestigious Presidential Symposium to showcase nonclinical proof of concept in prion disease.

Announced pricing of $23 million underwritten registered direct equity offering.

RICHMOND, Calif.--(BUSINESS WIRE)--May 12, 2025-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today reported recent business highlights and first quarter 2025 financial results.

“This quarter we continued to advance our promising neurology genomic medicine pipeline and are pleased to have signed our third STAC-BBB license agreement, reinforcing that Sangamo is a collaborator of choice for neurotropic capsids,” said Sandy Macrae, Chief Executive Officer of Sangamo Therapeutics. “We achieved significant clinical and regulatory derisking milestones in our Fabry disease program and raised additional capital through business development and other means, to provide additional runway to secure a potential Fabry partner. With our neuropathic pain program ready to enter the clinic, we look forward to dosing the first patients with our epigenetic regulation technology, which we hope will usher in a new era in chronic pain treatment.”

Recent Business Highlights

Corporate Updates

- Announced in April a capsid license agreement with Eli Lilly and Company (Lilly) to deliver genomic medicines for diseases of the CNS. Agreement grants Lilly a worldwide exclusive license to Sangamo’s novel proprietary neurotropic adeno-associated virus (AAV) capsid, STAC-BBB, for up to five potential disease targets. Received an $18 million upfront license fee from Lilly and eligible to earn up to $1.4 billion in additional licensed target fees and milestone payments across all five potential neurology disease targets, as well as tiered royalties on potential net sales.
- Announced pricing of a $23 million underwritten registered direct offering of equity securities, which is expected to close on May 14, 2025, subject to customary closing conditions.

Fabry Disease

- All dosed patients in the Phase 1/2 STAAR study evaluating isaralgagene civaparvovec, an investigational gene therapy for the treatment of Fabry disease, have now completed at least 52-weeks of follow-up, a key milestone required by the FDA for an Accelerated Approval regulatory pathway.
- Preliminary analysis of clinical data collected as of the April 2025, 52-week milestone, date across all 32 dosed patients indicates that the mean estimated glomerular filtration rate (eGFR) slope continued to remain positive since the last clinical update, which had a data cutoff date of September 12, 2024, presented at the WORLD*Symposium* in February 2025.
- The product candidate continues to be well tolerated, and a pivotal data readout is expected by the end of the second quarter of 2025.
- In April 2025, held a productive Type B meeting with the FDA, providing Sangamo with a clear CMC pathway to a planned BLA submission as early as the first quarter of 2026, including clarity on plans for process validation, path to commercial specifications and the commercial launch manufacturing site.
- This BLA submission timeline would facilitate a potential approval and commercial launch as early as the second half of 2026.
- Sangamo is advancing BLA preparation activities for isaralgagene civaparvovec, while continuing to engage in business development negotiations for a potential Fabry commercialization agreement.

Core Neurology Pipeline

Chronic Neuropathic Pain – ST-503

- Advanced preparations for the Phase 1/2 study of ST-503, an investigational epigenetic regulator for the treatment of intractable pain due to iSFN, a type of chronic neuropathic pain.

- Expect to commence patient enrollment and dosing in mid-2025, with preliminary proof of efficacy data anticipated in Q4 2026.

Prion Disease – ST-506

- Clinical Trial Authorisation (CTA) enabling activities continue to advance for ST-506, an investigational epigenetic regulator for the treatment of prion disease, leveraging STAC-BBB.
- A CTA submission is expected in Q1 2026, with preliminary clinical data anticipated in Q4 2026.

Novel AAV Capsid Delivery Technology

- Continue to engage in business development discussions with new potential collaborators for STAC-BBB for use in delivering intravenously administered genomic medicines for certain specified neurological diseases.

ASGCT Annual Meeting

- Nine Sangamo abstracts accepted for presentation at ASGCT on May 13-17, 2025, in New Orleans, Louisiana, demonstrating advances in its neurology pipeline, including platform presentations detailing the chronic neuropathic pain and prion disease programs, and posters outlining the latest innovations in capsid delivery engineering and modular integrase technologies.
- One abstract accepted as a platform presentation in the prestigious Presidential Symposium showcasing 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.

First Quarter 2025 Financial Results

Consolidated net loss for the first quarter ended March 31, 2025 was $30.6 million, or $0.14 per share, compared to a net loss of $49.1 million, or $0.27 per share, for the same period in 2024.

Revenues

Revenues for the first quarter ended March 31, 2025 were $6.4 million, compared to $0.5 million for the same period in 2024.

The increase in revenues was primarily attributed to $5.0 million in revenue relating to our collaboration agreement with Pfizer upon transfer of a specified sublicense and an increase of $1.0 million in revenue relating to our license agreement with Sigma-Aldrich Corporation.

GAAP and Non-GAAP Operating Expenses

(In millions)	Three Months Ended March 31, 2025	2024
Research and development	$ 26.0	$ 35.9
General and administrative	10.1	11.8
Impairment of long-lived assets	-	4.3
Total operating expenses	36.1	52.0
Impairment of long-lived assets	-	(4.3)
Depreciation and amortization	(1.0)	(1.4)
Stock-based compensation expense	(2.6)	(2.7)
Non-GAAP operating expenses	$ 32.5	$ 43.6

Total operating expenses on a GAAP basis for the first quarter ended March 31, 2025 were $36.1 million compared to $52.0 million for the same period in 2024. Non-GAAP operating expenses, which exclude depreciation and amortization, stock-based compensation expense, and impairment charges for the first quarter ended March 31, 2025 were $32.5 million, compared to $43.6 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. Additionally, the decrease reflects reprioritization of research and development investments, with a shift toward neurology programs. Other contributing factors included lower preclinical and clinical expenses due to program deferrals, lower impairment charges recorded in the current year, a decrease in restructuring charges related to the 2023 restructuring of operations, and a decrease in external professional services, facilities and infrastructure costs.

Cash and Cash Equivalents

Cash and cash equivalents as of March 31, 2025 were $25.2 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 March 31, 2025, together with the $18.0 million upfront license fee received from Lilly in April 2025, the anticipated net proceeds from the underwritten offering announced today, and our at-the-market

offering program since March 31, 2025, will be sufficient to fund our planned operations into late in the third quarter of 2025.

Financial Guidance for 2025 Reiterated (initial guidance provided on March 17, 2025)

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 engaging in business development negotiations for a potential Fabry commercialization agreement.

In addition, we expect to implement additional cost saving measures over the near term to further reduce our operating expenses and maximize the efficiency of the go-ahead neurology-focused genomic medicine company.

Upcoming Events

Sangamo plans to participate in the following events:

Investor Conferences

- H.C. Wainwright 3rd Annual BioConnect Investor Conference at Nasdaq, May 20, 2025
- Jefferies Global Healthcare Conference, June 3-5, 2025
- H.C. Wainwright 6th Annual Neuro Perspectives Hybrid Conference, June 16-17, 2025
- 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 Monday, May 12, at 6:30pm 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, including our expectations concerning the closing of the announced underwritten offering and the receipt of proceeds therefrom; 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; the potential of its AAV capsid delivery platform; the potential for isaralgagene civaparvovec to qualify for the FDA's Accelerated Approval program, including the adequacy of data generated in the Phase 1/2 STAAR study to support any such approval, expectations concerning the timing of the pivotal data readout and the availability of additional data to support a potential BLA submission for isaralgagene civaparvovec, and the timing of such submission; expectations concerning Sangamo's regulatory pathway for isaralgagene civaparvovec, including potential regulatory approval and commercial launch of isaralgagene civaparvovec and the timing thereof; Sangamo's plans to seek a potential commercialization partner for isaralgagene civaparvovec; Sangamo's ability to realize the expected benefits of the license agreement with Lilly, including the potential for Sangamo to receive licensed target fees and milestone payments and royalties; Sangamo's ability to establish and maintain collaborations and strategic partnerships and realize the expected benefits of such arrangements, including its ability to secure a commercialization partner for its Fabry disease program and additional collaborations with respect to Sangamo's STAC-BBB capsid delivery platform and epigenetic regulation capabilities; the anticipated plans and timelines for conducting, and presenting clinical data from clinical trials; the advancement of Sangamo's preclinical neurology programs, including plans to initiate patient enrollment and dosing for ST-503 and announcement of preliminary proof of efficacy data, and 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; risks and uncertainties related to completion of the underwritten offering on the anticipated terms or at all 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 52-week data from the Phase 1/2 STAAR study will not support a BLA submission and/or 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 March 31, 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 goodwill, indefinite-lived intangible assets and 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 goodwill, indefinite-lived intangible assets and 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.

SELECTED CONSOLIDATED FINANCIAL DATA

(unaudited; in thousands, except per share data)

Statement of Operations Data:

	Three months ended March 31,	
	2025	**2024**
Revenues	$ 6,437	$ 481
Operating expenses:		
Research and development	26,006	35,891
General and administrative	10,059	11,767
Impairment of long-lived assets	-	4,349
Total operating expenses	36,065	52,007
Loss from operations	(29,628)	(51,526)
Interest income	309	451
Other (expense) income, net	(1,159)	2,084
Loss before income taxes	(30,478)	(48,991)
Income tax expense	119	98
Net loss	$ (30,597)	$ (49,089)

Basic and diluted net loss per share	$ (0.14)	$ (0.27)
Shares used in computing net loss per share	220,269	180,342

Selected Balance Sheet Data:

	March 31, 2025	December 31, 2024
Cash and cash equivalents	$ 25,180	$ 41,918
Total assets	$ 86,166	$ 101,635
Total stockholders' equity	$ 4,906	$ 22,770

View source version on businesswire.com: https://www.businesswire.com/news/home/20250512153155/en/

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