



Sangamo Therapeutics Reports Recent Business Highlights and Fourth Quarter and Full Year 2024 Financial Results

March 17, 2025

Continued to advance our prioritized neurology genomic medicine pipeline towards the clinic.

Announced two neurology license agreements with blue-chip pharma companies, including a global epigenetic regulation and capsid delivery license agreement with Genentech in August 2024 to develop novel genomic medicines for neurodegenerative diseases and a capsid license agreement with Astellas in December 2024 to deliver genomic medicines for up to five neurological disease targets.

Raised over \$100 million in funding in 2024 through non-dilutive license fees and milestone payments, as well as equity financing.

Investigational new drug (IND) application cleared by U.S. Food and Drug Administration (FDA) for ST-503 for treatment of intractable pain due to idiopathic small fiber neuropathy (iSFN), a type of chronic neuropathic pain. Expect to commence patient enrollment and dosing in mid-2025.

Demonstrated nonclinical proof of concept in prion disease. A single intravenous infusion of Sangamo's zinc finger repressor (ZFR) significantly reduced expression of prion mRNA and protein in the mouse brain and extended mouse survival.

Aligned with FDA on clear regulatory pathway to Accelerated Approval for isargalgene civaparvovec in Fabry disease, using expected data from ongoing Phase 1/2 STAAR study, avoiding requirement for additional registrational study and accelerating estimated time to potential approval by approximately three years.

Announced updated Phase 1/2 STAAR study data that showed sustained benefit, improvements in kidney function and favorable safety profile.

RICHMOND, Calif.--(BUSINESS WIRE)--Mar. 17, 2025-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today reported recent business highlights and fourth quarter and full year 2024 financial results.

"I am pleased with Sangamo's pipeline progress since the start of 2024. We advanced our two prioritized neurology therapies towards the clinic, securing our first ever neurology IND; we showed we are a collaborator of choice for neurotropic capsids, with the announcement of two blue-chip pharma agreements for our STAC-BBB capsid, with negotiations advancing for a third potential agreement; and we have a clear regulatory pathway to Accelerated Approval in Fabry disease, which could reduce the time to potential approval by approximately three years," said Sandy Macrae, Chief Executive Officer of Sangamo Therapeutics. "We believe our neurology pipeline represents important potential value. In addition, we continue to engage in Fabry business development negotiations, in an effort to capitalize the business for the future. This will be an important year for the Company as we plan to begin patient enrollment and dosing in mid-2025 for our clinical study in iSFN, which we believe has the potential to transform the chronic neuropathic pain landscape, and as we prepare for an anticipated BLA submission in Fabry disease in the second half of the year."

Recent Business Highlights

Corporate Updates

- Announced in December a capsid license agreement with Astellas Gene Therapies, Inc. (Astellas) to deliver genomic medicines for neurological diseases. Agreement grants Astellas a worldwide exclusive license to STAC-BBB for up to five potential neurological disease targets. Received a \$20 million upfront license fee from Astellas and eligible to earn up to \$1.3 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 in December that Sangamo is scheduled to regain full rights to giroctocogene fitelparvovec, an investigational gene therapy product candidate for the treatment of adults with moderately severe to severe hemophilia A that it has co-developed with, and licensed to, Pfizer, Inc. (Pfizer), following a decision by Pfizer to terminate the global collaboration and license agreement between the parties. Sangamo continues to explore how to maximize the value of the SB-525 program, including a search for a potential new collaboration partner.

Core Neurology Pipeline

Chronic Neuropathic Pain – ST-503

- IND application cleared by the FDA for ST-503, an investigational epigenetic regulator for the treatment of intractable pain due to iSFN, a type of chronic neuropathic pain.
- Preparing for a Phase 1/2 study of ST-503 to assess the safety, tolerability and preliminary efficacy of a one-time dose administered intrathecally to patients with intractable pain due to iSFN.
- Expect to commence patient enrollment and dosing in mid-2025, with preliminary proof of efficacy data anticipated in Q4 2026.

Prion Disease

- Clinical Trial Authorisation (CTA) enabling activities continue to advance for Sangamo's product candidate to treat prion disease, leveraging STAC-BBB.
- Published a manuscript in *bioRxiv* titled, "Zinc Finger Repressors mediate widespread prion depletion from the nonhuman primate brain and profoundly extend survival in prion disease mice" demonstrating nonclinical proof of concept for this approach. A single intravenous infusion of Sangamo's ZFR significantly reduced expression of prion mRNA and protein in the mouse brain, extended mouse survival and improved an array of molecular, histological, biomarker and behavior readouts – even when administered post-symptomatically to mice with prion disease. In addition, a single intravenous administration of the prion ZFR, delivered via STAC-BBB to nonhuman primates, resulted in potent and widespread reduction of prion expression in transduced neurons throughout the brain.
- A CTA submission is expected in Q1 2026, with preliminary clinical data anticipated in Q4 2026.

Novel Adeno-Associated Virus (AAV) Capsid Delivery Technology

- Actively engaged in advanced contract negotiations with a potential collaborator for a third STAC-BBB license agreement for use in delivering intravenously administered genomic medicines for certain specified neurological diseases.

Clinical – Fabry Disease

- Presented updated Phase 1/2 STAAR study data at the 21st Annual *WORLD Symposium* in San Diego, CA in February 2025 showing sustained benefit, improvements in kidney function and a favorable safety profile, following a single administration of isaralgagene civaparvovec in 33 adults with Fabry disease.
- Elevated expression of alpha-galactosidase A (α -Gal A) activity maintained for nearly four years for the longest treated patient as of the September 12, 2024 data cutoff date.
- Positive mean estimated glomerular filtration rate (eGFR) slope of 3.061 mL/min/1.73m²/year (95% confidence interval: 0.863, 5.258) was observed in the 23 patients who had reached at least one-year follow-up, indicating notable improvements in renal function.
- All 18 patients who began the study on enzyme replacement therapy (ERT) have been withdrawn from, and remain off, ERT as of March 17, 2025.
- Significant improvements continued to be observed in the short form-36 (SF-36) quality of life (QoL) scores reported, with a mean change in General Health score of 10.6. Significant improvements in physical component, bodily pain, physical, vitality, social function, and emotional SF-36 scores were also observed.
- Enrollment and dosing are complete in the Phase 1/2 STAAR study.
- The FDA has provided a clear regulatory pathway to Accelerated Approval for isaralgagene civaparvovec, agreeing that data from the ongoing Phase 1/2 STAAR study can serve as the primary basis for approval under the Accelerated Approval Program, using eGFR slope at 52 weeks across all patients as an intermediate clinical endpoint.
- The 52-week eGFR slope data from all enrolled patients in the Phase 1/2 STAAR study will be available in the first half of 2025. A potential Biologics License Application (BLA) submission is anticipated in the second half of 2025.
- Sangamo is advancing BLA preparation activities for isaralgagene civaparvovec, while continuing to engage in business development negotiations for a potential Fabry commercialization agreement.

Fourth Quarter and Full Year 2024 Financial Results

Consolidated net loss for the fourth quarter ended December 31, 2024 was \$23.4 million, or \$0.11 per share, compared to consolidated net loss of \$60.3 million, or \$0.34 per share, for the same period in 2023. For the year ended December 31, 2024, consolidated net loss was \$97.9 million, or \$0.49 per share, compared to consolidated net loss of \$257.8 million, or \$1.48 per share, for the year ended December 31, 2023.

Revenues

Revenues for the fourth quarter ended December 31, 2024 were \$7.6 million, compared to \$2.0 million for the same period in 2023.

The increase of \$5.5 million in revenues was primarily attributed to \$6.5 million and \$0.8 million in revenues relating to our license agreements with Astellas Gene Therapies, Inc., or Astellas, and Genentech, Inc., respectively, partially offset by revenue decreases in other collaborations.

Revenues were \$57.8 million in 2024, compared to \$176.2 million in 2023.

The decrease of \$118.4 million in revenues in 2024 compared to 2023 was primarily attributed to decreases of \$134.8 million and \$12.2 million in revenues relating to our collaboration agreements with Biogen and Novartis, respectively, due to the termination of these collaboration agreements in June 2023, a decrease of \$20.5 million in revenue relating to our collaboration agreement with Kite, which expired pursuant to its terms in April 2024, a decrease of \$4.7 million in revenue relating to our license agreements with Sigma and Ligand, and a decrease of \$2.7 million in revenue relating to our other license agreements. These decreases were partially offset by \$50.0 million in revenue relating to our license agreement with Genentech and \$6.5 million in revenue relating to our license agreement with Astellas.

GAAP and Non-GAAP Operating Expenses

**Three Months Ended
December 31,**

**Year Ended
December 31,**

(In millions)	2024	2023	2024	2023
Research and development	\$ 23.6	\$ 50.7	\$ 111.5	\$ 234.0
General and administrative	9.9	13.1	44.8	61.2
Impairment of long-lived assets	-	0.3	5.5	65.5
Impairment of goodwill and indefinite-lived intangible assets	-	-	-	89.5
Total operating expenses	33.5	64.1	161.8	450.2
Impairment of long-lived assets	-	(0.3)	(5.5)	(65.5)
Impairment of goodwill and indefinite-lived intangible assets	-	-	-	(89.5)
Depreciation and amortization	(1.2)	(1.8)	(5.1)	(15.1)
Stock-based compensation expense	(3.3)	(6.1)	(12.4)	(27.4)
Non-GAAP operating expenses	\$ 29.0	\$ 55.9	\$ 138.8	\$ 252.7

Total operating expenses on a GAAP basis for the fourth quarter ended December 31, 2024 were \$33.5 million compared to \$64.1 million for the same period in 2023. Non-GAAP operating expenses, which exclude impairment charges, depreciation and amortization and stock-based compensation expense as shown in the reconciliation table above, for the fourth quarter ended December 31, 2024 were \$29.0 million, compared to \$55.9 million for the same period in 2023.

Total operating expenses on a GAAP basis in 2024 were \$161.8 million compared to \$450.2 million in 2023. Non-GAAP operating expenses, which exclude impairment charges, depreciation and amortization and stock-based compensation expense as shown in the reconciliation table above, were \$138.8 million in 2024 compared to \$252.7 million in 2023.

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 announced during 2023. Additionally, the decrease reflects intentional reprioritization of research and development investments, with a focused shift toward advancing the neurology pipeline. Other contributing factors included lower impairment charges recorded in the current year, lower preclinical and clinical expenses due to program deferrals, a decrease in restructuring charges related to the 2023 restructuring of operations, a decrease in depreciation due to reduced carrying values as a result of impairment charges recorded in 2023, and a decrease in external professional services, facilities, and infrastructure-related costs.

Cash and Cash Equivalents

Cash and cash equivalents as of December 31, 2024 were \$41.9 million, compared to cash, cash equivalents and marketable securities of \$81.0 million as of December 31, 2023. Based on our current operating plan, we believe that our cash and cash equivalents as of December 31, 2024, together with \$10.1 million generated to date through our at-the-market offering program in 2025 and the \$5.0 million payment expected from Pfizer by the end of March, will be sufficient to fund our planned operations into the middle of the second quarter of 2025.

Financial Guidance for 2025

- 2025 operating expenses on a non-GAAP basis are expected to be roughly in line with 2024, reflecting our intention to operate a lean neurology-focused business and to continue advancing isaralgagene civaparvec towards a potential BLA submission, while engaging in business development negotiations for a potential Fabry commercialization agreement.
- On a GAAP basis, we 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 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 the prior year.

Upcoming Events

Sangamo plans to participate in the following events:

- Jefferies Global Healthcare Conference, June 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, March 17, 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; the potential of its adeno-associated virus 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 availability of additional data to support a potential BLA submission for isaralgagene civaparvovec, and the timing of such submission, and the potential to accelerate the expected timeline to approval; the anticipated advancement of isaralgagene civaparvovec to registration, including Sangamo's plans to seek a potential commercialization partner; Sangamo's ability to realize the expected benefits of the license agreements with Genentech and Astellas, 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 hemophilia A program, STAC-BBB capsid delivery platform and epigenetic regulation capabilities; anticipated revenues from existing and new collaborations and the timing thereof; the anticipated plans and timelines of Sangamo and its collaborators in conducting our ongoing and potential future clinical trials and presenting clinical data from such clinical trials; the advancement of Sangamo's preclinical neurology programs, including the potential of ST-503 to transform the chronic neuropathic pain landscape, plans to initiate patient enrollment and dosing for ST-503 and announcement of such preliminary proof of efficacy data, and anticipated prion disease CTA submission and announcement of related preliminary clinical data; 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 related to GAAP and non-GAAP total operating expenses, impairments and non-cash stock-based compensation expense, depreciation and amortization; 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 funding or partnerships, in particular for its Fabry disease program, or additional collaboration partners necessary to advance its preclinical and clinical programs 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 the risk that therapeutic effects in the Phase 3 AFFINE trial will not be durable in patients as well as the 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, 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 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 amounts)

Statement of Operations Data:

	Three months ended		Year Ended	
	December 31,		December 31,	
	2024	2023	2024	2023
Revenues	\$ 7,551	\$ 2,042	\$ 57,800	\$ 176,232
Operating expenses:				
Research and development	23,675	50,706	111,521	234,057
General and administrative	9,866	13,099	44,727	61,167
Impairment of long-lived assets	-	296	5,521	65,528
Impairment of goodwill and indefinite-lived intangible assets	-	-	-	89,485
Total operating expenses	33,541	64,101	161,769	450,237
Loss from operations	(25,990)	(62,059)	(103,969)	(274,005)
Interest and other income, net	2,167	1,491	5,861	11,102
Loss before income taxes	(23,823)	(60,568)	(98,108)	(262,903)
Income tax benefit	(427)	(272)	(167)	(5,072)
Net loss	\$ (23,396)	\$ (60,296)	\$ (97,941)	\$ (257,831)
Basic and diluted net loss per share	\$ (0.11)	\$ (0.34)	\$ (0.49)	\$ (1.48)
Shares used in computing basic and diluted net loss per share	210,185	177,619	201,699	174,444

Selected Balance Sheet Data:

	December 31,	December 31,
	2024	2023
Cash, cash equivalents and marketable securities	\$ 41,918	\$ 81,002
Total assets	\$ 101,635	\$ 165,320
Total stockholders' equity	\$ 22,770	\$ 82,887

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