



Sangamo Therapeutics Reports Recent Business Highlights and Fourth Quarter and Full Year 2023 Financial Results

March 13, 2024

- *Data from novel proprietary neurotropic adeno-associated virus (AAV) delivery capsid, STAC-BBB, demonstrated industry-leading blood-brain barrier (BBB) penetration in non-human primates (NHPs) following intravenous administration, with capsid-enabled delivery of zinc finger payloads targeting prion disease and tauopathies resulting in potent and widespread repression of target genes.*
- *Chronic neuropathic pain and prion disease preclinical programs advance, with investigational new drug (IND) and clinical trial authorization (CTA) submissions expected in the fourth quarters of 2024 and 2025, respectively.*
- *Announced U.S. Food and Drug Administration (FDA) alignment on an abbreviated pathway to potential approval, and grant by European Medicines Agency (EMA) of priority medicines (PRIME) eligibility for isaralgagene civaparvovec in Fabry disease.*
- *Pfizer anticipates Biologics License Application (BLA) and marketing authorization application (MAA) submissions for Hemophilia A collaboration by early 2025 if the pivotal readout is supportive.*

RICHMOND, Calif.--(BUSINESS WIRE)--Mar. 13, 2024-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicines company, today reported recent business highlights and fourth quarter and full year 2023 financial results, including meaningful data to support advancement of its neurology pipeline.

"In 2023, Sangamo announced the prioritization of its pipeline programs that support our focus as a neurology-focused genomic medicine company," said Sandy Macrae, Chief Executive Officer of Sangamo. "With the meaningful preclinical data announced today, we believe our ability to combine potent zinc finger epigenetic regulation payloads with exciting new industry-leading capsid delivery technology could unlock significant potential for the treatment of devastating neurological diseases, indications for which delivery of treatments to the central nervous system has historically proved challenging. In the near-term, we are also seeking to create value by partnering our Fabry disease program, for which we aligned with the FDA on a potentially abbreviated and more cost-effective timeline and received EMA PRIME eligibility. We look forward to advancing our pipeline into the clinic to develop therapies designed to target neurological diseases with high unmet medical needs."

Recent Business Highlights

Prioritized Neurology Pipeline

Sangamo is developing epigenetic regulation therapies to treat serious neurological diseases and novel proprietary AAV capsids designed to deliver our therapies to the intended neurological targets, including across the blood-brain barrier.

Novel AAV Capsid Delivery Technology – *Data demonstrated industry-leading BBB penetration and brain transduction in NHPs, with capsid-enabled delivery of zinc finger payloads targeting prion disease and tauopathies resulting in potent and widespread repression of target genes.*

- *Novel STAC-BBB capsid demonstrated robust penetration of BBB with 700-fold higher transgene expression in neurons compared to the benchmark capsid AAV9 and outperformed all other known published capsid variants evaluated in the study.*
- *STAC-BBB mediated robust expression of zinc finger cargo in neurons, with potent and widespread repression of prion and tau genes observed across key brain regions, demonstrating the potential for modification of disease progression in prion disease and various tauopathies.*
- *Visualization of gene expression in individual brain cells by RNAscope revealed highly potent repression of tau in neurons expressing the zinc finger cargo, across multiple brain regions.*
- *Capsid biodistribution profile is optimal for the treatment of neurological diseases with AAV-based treatments, highlighted by the observed enrichment in the CNS and de-targeting from the liver, dorsal root ganglia (DRG) and other peripheral organs.*
- *STAC-BBB was well tolerated in NHPs, with no notable treatment related pathological findings in brain, spinal cord or peripheral tissues.*
- *We believe that STAC-BBB is manufacturable at commercial scale using standard cell culture and purification processes, is soluble using known excipients, and can be characterized using available analytics.*

Chronic Neuropathic Pain – *Preclinical data demonstrated potent and specific repression of Nav1.7 expression without impacting other sodium channels and that zinc finger repressors (ZFRs) were well tolerated in NHPs; IND submission expected in fourth quarter of 2024.*

- *IND-enabling toxicology studies are nearing completion.*
- *An IND submission is expected in the fourth quarter of 2024.*

Prion Disease – *Preclinical data demonstrated that AAV-delivered ZFRs significantly reduced expression of the prion protein in the brain, extended*

lifespan and limited formation of toxic prion aggregates in mice; CTA expected in the fourth quarter of 2025.

- CTA-enabling studies continue to advance.
- Prion-targeted zinc finger repressor, delivered via an intravenous administration of the STAC-BBB novel capsid, resulted in a dose-dependent repression of prion genes in NHPs.
- A CTA submission is expected in the fourth quarter of 2025.

Tauopathies – *Preclinical data demonstrated that AAV-delivered ZFRs significantly reduced expression of tau mRNA in brain of NHPs; intend to resume development of this program, leveraging STAC-BBB; IND submission expected as early as the fourth quarter of 2025.*

- Intend to resume development of our previously paused tau program, leveraging the newly identified STAC-BBB capsid variant, subject to additional funding.
- Tau clinical-lead zinc finger repressor, delivered via an intravenous administration of the STAC-BBB novel capsid, resulted in a dose-dependent repression of tau genes in NHPs. Visualization of gene expression in individual brain cells by RNAscope revealed highly potent repression of tau in neurons expressing the zinc finger cargo across multiple brain regions.
- We expect the IND submission could occur as early as the fourth quarter of 2025.

Other Programs

Fabry Disease – *Dosed total of 32 patients in Phase 1/2 STAAR study, with updated clinical data presented at 20th Annual WORLDSymposium showing sustained benefit and differentiated safety profile; announced FDA alignment on abbreviated pathway to potential approval; actively seeking collaboration partner to advance asset toward potential registration and commercialization.*

- Dosed seven additional patients in the dose expansion phase of the Phase 1/2 STAAR study evaluating isaralgagene civaparvovec, our wholly owned gene therapy product for the treatment of Fabry disease, for a total of 32 patients dosed to date.
- Presented updated clinical data at the 20th Annual WORLDSymposium in San Diego, CA in February 2024, showing that elevated levels of α -Gal A activity were sustained in all 24 patients evaluated as of the September 19, 2023 data cutoff date and accompanied by the reduction and/or long-term stabilization of lyso-Gb3 levels, with the largest reductions in plasma lyso-Gb3 seen in patients with the highest levels at baseline.
- All 13 patients who were withdrawn from Enzyme Replacement Therapy (ERT) remain off ERT as of March 12, 2024.
- In the 13 patients followed for 12-months or more after treatment, renal function remained stable, and significant improvements in overall disease severity, quality of life, and gastrointestinal symptoms compared to baseline were reported.
- Aligned with the FDA that data from a single, adequate, and well-controlled study may form the primary basis of approval of a BLA for isaralgagene civaparvovec, enabling a potentially abbreviated and more cost-effective pathway to BLA submission than originally anticipated. The study would enroll up to 25 patients, both male and female, without the need for a control arm. A head-to-head comparison with ERT is not part of the proposed study design deemed acceptable by the FDA.
- Granted PRIME eligibility from the EMA for isaralgagene civaparvovec, which aims to enhance support for the development of medicines that target an unmet medical need and is intended to optimize development plans and expedite review and approval processes.
- Granted Innovative Licensing and Access Pathway (ILAP) for isaralgagene civaparvovec from U.K. Medicines and Healthcare products Regulatory Agency (MHRA) which aims to accelerate time to market and facilitate access to medicines. Isaralgagene civaparvovec has already received Orphan Medicinal Product designation from the EMA as well as Orphan Drug, Fast Track and Regenerative Medicine Advanced Therapy (RMAT) designations from the FDA.
- Completed screening and enrollment in the Phase 1/2 STAAR study and expect to complete dosing of remaining patients in the first half of 2024.
- Deferring additional investments in planning for a potential registrational trial until a collaboration partnership or financing for this program is secured.

Hemophilia A (Pfizer) – *Pivotal data read-out in Phase 3 AFFINE trial expected in mid-2024; BLA and MAA submissions anticipated by early 2025 if pivotal readout is supportive.*

- A pivotal readout is expected in mid-2024 in the Phase 3 AFFINE trial of giroctocogene fitelparvovec, an investigational gene therapy we are developing with Pfizer for patients with moderately severe to severe hemophilia A.
- Pfizer anticipates BLA and MAA submissions by early 2025 if the pivotal readout is supportive.
- Presented updated data with Pfizer from the Phase 1/2 ALTA study of giroctocogene fitelparvovec in an oral presentation at the 65th American Society for Hematology Annual Meeting and Exposition in December 2023.
- Eligible to earn from Pfizer up to \$220.0 million in milestone payments upon the achievement of certain regulatory and commercial milestones for giroctocogene fitelparvovec and product sales royalties of 14% - 20% if giroctocogene fitelparvovec is approved and commercialized, subject to certain reductions.

CAR-Tregs – In alignment with previously announced strategic transformation, announced winddown of the Company's French research and manufacturing operations and a corresponding reduction in workforce; continuing to seek a potential collaboration partner or external investment in the CAR-Treg cell therapy programs.

- Announced a winddown of Sangamo's French operations and a corresponding reduction in workforce, including closure of Sangamo's cell therapy manufacturing facility and research labs in Valbonne, France, which is expected to commence in April 2024 and be complete by end of year. We expect this restructuring to result in the elimination of all roles in France (approximately 93).
- Dosed two additional patients in the Phase 1/2 STEADFAST study evaluating TX200, our wholly owned autologous CAR-Treg cell therapy treating patients receiving an HLA-A2 mismatched kidney from a living donor, including the first patient in the new fourth, highest dose cohort.
- Expect to dose up to two additional patients in the fourth highest-dose level cohort by the end of the second quarter of 2024.
- Plan to continue seeking a potential collaboration partner or external investment in our autologous CAR-Treg cell therapy programs.

Fourth Quarter and Full Year 2023 Financial Results

Consolidated net loss for the fourth quarter ended December 31, 2023 was \$60.3 million, or \$0.34 per share, compared to a net loss of \$52.0 million, or \$0.32 per share, for the same period in 2022. For the year ended December 31, 2023, consolidated net loss was \$257.8 million, or \$1.48 per share, compared to consolidated net loss of \$192.3 million, or \$1.25 per share, for the year ended December 31, 2022.

Revenues

Revenues for the fourth quarter ended December 31, 2023 were \$2.0 million, compared to \$27.2 million for the same period in 2022.

The decrease of \$25.2 million in revenues was primarily attributed to a decrease of \$17.5 million in revenue relating to our collaboration agreement with Kite, reflecting a reduction in level of our research and development services, and a decrease of \$10.3 million in revenue relating to our collaboration agreement with Novartis, due to the impact of termination of the collaboration agreement. These decreases were partially offset by an increase of \$2.6 million in revenues from other licensing agreements.

Revenues were \$176.2 million in 2023, compared to \$111.3 million in 2022.

The increase of \$64.9 million in revenues in 2023 compared to 2022, was primarily attributed to an increase of \$106.4 million in revenue relating to our collaboration agreement with Biogen, primarily due to the impact of termination of the collaboration agreement in June 2023, which resulted in an increase in the measure of proportional cumulative performance on this collaboration, an increase of \$3.8 million in revenue relating to our license agreement with Sigma-Aldrich Corporation, and an increase of \$3.6 million in revenues from other licensing agreements. These increases were partially offset by a decrease of \$27.5 million in revenue relating to our collaboration agreement with Novartis due to the termination of the collaboration agreement in June 2023, a decrease of \$18.1 million in revenue relating to our collaboration agreement with Kite due to a reduction in the estimated future level of our research and development services related to this collaboration, and a decrease of \$3.3 million in revenue relating to our collaboration agreement with Sanofi due to the termination of the collaboration agreement in June 2022.

GAAP and Non-GAAP Operating Expenses

(In millions)	Three Months Ended		Year Ended	
	December 31,		December 31,	
	2023	2022	2023	2022
Research and development	\$ 50.7	\$ 66.2	\$ 234.0	\$ 249.9
General and administrative	13.1	16.4	61.2	62.7
Impairment of goodwill and indefinite-lived intangible assets	-	-	89.5	-
Impairment of long-lived assets	0.3	-	65.5	-
Total operating expenses	64.1	82.6	450.2	312.6
Impairment of goodwill and indefinite-lived intangible assets	-	-	(89.5)	-
Impairment of long-lived assets	(0.3)	-	(65.5)	-
Depreciation and amortization	(1.8)	(3.3)	(15.1)	(12.1)
Stock-based compensation expense	(6.1)	(8.3)	(27.4)	(31.7)
Non-GAAP operating expenses	\$ 55.9	\$ 71.0	\$ 252.7	\$ 268.8

Total operating expenses on a GAAP basis for the fourth quarter ended December 31, 2023 were \$64.1 million compared to \$82.6 million for the same period in 2022. Non-GAAP operating expenses, which exclude impairment charges, depreciation and amortization, and stock-based compensation expense, for the fourth quarter ended December 31, 2023 were \$55.9 million, compared to \$71.0 million for the same period in 2022.

The decrease in total operating expenses on a GAAP and non-GAAP basis was primarily attributable to lower compensation and other personnel costs mainly due to a reduction in the bonus expense and lower headcount as a result of restructuring of operations and a corresponding reduction in workforce announced during the year, and a decrease in research and clinical expenses due to deferral and reprioritization of certain programs. These decreases were partially offset by restructuring charges related to the reductions in workforce announced in April and November 2023, and the France restructuring. The expense related to the France restructuring was recorded in the fourth quarter of 2023 as the payouts are based on an ongoing post-employment benefit plan, and the payments were probable and could be estimated as of December 31, 2023.

Total operating expenses on a GAAP basis in 2023 were \$450.2 million compared to \$312.6 million in 2022. Non-GAAP operating expenses, which exclude impairment charges, depreciation and amortization, and stock-based compensation expense, were \$252.7 million in 2023 compared to \$268.8 million in 2022.

Operating expenses on a GAAP basis included non-cash charges relating to impairment of goodwill and indefinite-lived intangible assets of \$89.5 million, and impairment of long-lived assets of \$65.5 million, which were a result of continued decline in our stock price and related market capitalization and termination of our collaboration agreements with Biogen and Novartis. The decrease in total operating expenses on a non-GAAP basis was primarily attributable to a reduction in the bonus expense and lower headcount as a result of restructurings of operations and corresponding reductions in workforce announced during the year, and a decrease in preclinical and clinical expenses due to the termination of collaboration agreements with Biogen and Novartis and deferral and reprioritization of certain programs. These decreases were partially offset by restructuring charges related to the reductions in workforce announced in April and November 2023, and the France restructuring.

Cash, Cash Equivalents and Marketable Securities

Cash, cash equivalents and marketable securities as of December 31, 2023 were \$81.0 million, compared to \$307.5 million as of December 31, 2022. During the year ended December 31, 2023, we raised approximately \$15.1 million in net proceeds under our at-the-market offering program. We believe that our available cash, cash equivalents and marketable securities as of December 31, 2023, in combination with potential future cost reductions, will be sufficient to fund our planned operations into the third quarter of 2024, excluding any additional capital raised. We are actively pursuing opportunities to raise additional capital.

Financial Guidance for 2024

On a GAAP basis, we expect total operating expenses in the range of approximately \$145 million to \$165 million in 2024, which includes non-cash stock-based compensation expense and depreciation and amortization, subject to additional funding.

We expect non-GAAP total operating expenses, excluding estimated non-cash stock-based compensation expense of approximately \$13 million, and depreciation and amortization of approximately \$7 million, in the range of approximately \$125 million to \$145 million in 2024, subject to additional funding.

Upcoming Events

Sangamo plans to participate in the following events:

Investor Conferences

- RBC Capital Markets Global Healthcare Conference, May 14-15, 2024
- Jefferies Global Healthcare Conference, June 5-6, 2024
- H.C. Wainwright 5th Annual Neuro Perspectives Virtual Conference, June 27, 2024

Access links for available webcasts for these 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 advancements and financial updates on Wednesday, March 13 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's zinc finger epigenetic regulators are ideally suited to potentially address devastating neurological disorders and Sangamo's capsid discovery platform is expanding 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 [Twitter/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: the therapeutic and commercial potential of Sangamo's product candidates and its engineered capsids, including the ability of STAC-BBB to unlock potential for the treatment of various neurological diseases, the anticipated plans and timelines of Sangamo and its collaborators dosing patients in and conducting our ongoing and potential future clinical trials and presenting clinical data from our clinical trials, including expectations regarding the conclusion of dosing in our Phase 1/2 STAAR study, plans for patient dosing in the STEADFAST study, the anticipated advancement of Sangamo's product candidates to late-stage development, including plans to seek a potential partner or additional financing to proceed with potential future Phase 3 trials of isaralgagene civaparvovec and the design and timing thereof, the timeline to present data from the Phase 3 AFFINE trial and to make BLA and MAA submissions for giroctocogene fitelparvovec, the potential to earn milestone payments and receive product sales royalties if giroctocogene fitelparvovec is approved and commercialized, expectations regarding advancement of Sangamo's

preclinical neurology programs, including announcement of data from, and anticipated IND and CTA submissions related to, such programs, plans to seek a partner for or investor in Sangamo's CAR-Treg program, expectations concerning Sangamo's announced winddown of our French research and manufacturing operations and a corresponding reduction in workforce and the expected charges and cost savings associated with such restructuring; future potential cost reductions, Sangamo's expected cash runway, Sangamo's 2024 financial guidance related to GAAP and non-GAAP total operating expenses, impairments and stock-based compensation, plans to participate in industry and investor conferences, efforts to secure additional funding, 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 to fully develop, obtain regulatory approval for and commercialize its product candidates, including the ability to secure the funding or partnerships required to advance its preclinical and clinical programs; Sangamo's ability to execute its restructurings as currently contemplated; the actual charges associated with the restructurings being higher than anticipated or changes to the assumptions on which the estimated charges associated with the restructurings are based; Sangamo's ability to achieve projected cost savings in connection with the restructurings and to further reduce operating expenses; unintended consequences from the restructuring that impact Sangamo's business; Sangamo's need for substantial additional funding to execute our operating plan and to continue to operate as a going concern, including the risk that Sangamo will be unable to obtain the funding 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 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 conflict, current or potential future bank failures, inflation and rising 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, 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 our future plans and expectations, including our financial guidance, are subject to our ability to secure adequate additional funding. There can be no assurance that Sangamo and its collaborators will be able to develop commercially viable products. 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, 2023, 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 December 31,		Year Ended December 31,	
	2023	2022	2023	2022
Revenues	\$ 2,042	\$ 27,230	\$ 176,232	\$ 111,299
Operating expenses:				
Research and development	50,706	66,179	234,057	249,898
General and administrative	13,099	16,443	61,167	62,682
Impairment of goodwill and indefinite-lived intangible assets	-	-	89,485	-
Impairment of long-lived assets	296	-	65,528	-
Total operating expenses	64,101	82,622	450,237	312,580
Loss from operations	(62,059)	(55,392)	(274,005)	(201,281)
Interest and other income, net	1,491	3,678	11,102	9,432

Loss before income taxes	(60,568)	(51,714)	(262,903)	(191,849)
Income tax (benefit) expense	(272)	259	(5,072)	429
Net loss	<u>\$ (60,296)</u>	<u>\$ (51,973)</u>	<u>\$ (257,831)</u>	<u>\$ (192,278)</u>
Basic and diluted net loss per share	\$ (0.34)	\$ (0.32)	\$ (1.48)	\$ (1.25)
Shares used in computing basic and diluted net loss per share	177,619	164,717	174,444	154,345

Selected Balance Sheet Data:

	<u>December 31, 2023</u>	<u>December 31, 2022</u>
Cash, cash equivalents, and marketable securities	\$ 81,002	\$ 307,477
Total assets	\$ 165,320	\$ 562,509
Total stockholders' equity	\$ 82,887	\$ 294,958

View source version on [businesswire.com](https://www.businesswire.com/news/home/20240313945090/en/): <https://www.businesswire.com/news/home/20240313945090/en/>

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