# Sangamo Therapeutics Reports Recent Business Highlights and Second Quarter 2024 Financial Results

August 6, 2024

*Announced global epigenetic regulation and capsid delivery license agreement with Genentech to develop novel genomic medicines for neurodegenerative diseases. Expect to receive $50 million in near-term upfront license fees and milestone payments and eligible to earn up to $1.9 billion in milestone payments, plus tiered royalties on net sales.*

*Reported Pfizer's announcement of positive topline results from Phase 3 AFFINE trial evaluating giroctocogene fitelparvovec, an investigational Hemophilia A gene therapy that Sangamo is co-developing with and licensing to Pfizer.*

*Continue to amass encouraging safety and efficacy data from the Phase 1/2 STAAR study of isaralgagene civaparvovec for Fabry disease, including preliminary evidence of improved kidney function and withdrawal from enzyme replacement therapy (ERT) by seventeen of eighteen patients.*

*Announced data demonstrating progression of neurology-focused preclinical pipeline, including additional data from novel delivery capsid, STAC ™BBB, that demonstrated industry-leading blood-brain barrier (BBB) penetration in non-human primates (NHPs) following intravenous administration.*

RICHMOND, Calif.--(BUSINESS WIRE)--Aug. 6, 2024-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today reported business highlights and second quarter 2024 financial results.

"Sangamo has demonstrated important progress over the last few months, with the announcement of a significant business development agreement with Genentech earlier today; positive topline results from the Phase 3 AFFINE trial in Hemophilia A we are developing with Pfizer; and highly encouraging Phase 1/2 data continuing to accumulate from our wholly-owned Fabry disease program – all of which have the potential to meaningfully extend our cash runway as we continue to advance our wholly owned neurology epigenetic regulation pipeline," said Sandy Macrae, Chief Executive Officer of Sangamo. "Our license agreement with Genentech – the first in what we hope could be multiple capsid collaborations to come with other potential partners – reinforces the potential of our gene editing and STAC-BBB capsid delivery platforms. Our core, and highly focused, neurology pipeline continues to advance as planned and we look forward to providing further updates."

**Recent Business Highlights**

***Corporate Updates***

*Genentech License Agreement*

- Announced global epigenetic regulation and capsid delivery license agreement with Genentech, a member of the Roche Group, to develop novel genomic medicines for neurodegenerative diseases.
- Granted an exclusive license to Genentech for Sangamo's proprietary zinc finger repressors that are directed to the tau gene, which is critically involved in Alzheimer's disease and other tauopathies, as well as a second undisclosed neurology target. Agreed to also exclusively license STAC-BBB to Genentech for tau and the second neurology target.
- Expect to receive from Genentech $50.0 million in near-term upfront license fees and milestone payments.
- Eligible to earn up to $1.9 billion in development and commercial milestones spread across multiple potential products under the agreement and tiered royalties on net sales of such products, subject to certain specified reductions.

***Clinical Programs***

*Hemophilia A*

- Pfizer announced positive topline results from the Phase 3 AFFINE trial evaluating giroctocogene fitelparvovec, an investigational gene therapy that Sangamo is co-developing with and licensing to Pfizer for the treatment of adults with moderately severe to severe hemophilia A.
- Sangamo is 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.
- Pfizer reported that the AFFINE trial achieved its primary objective of non-inferiority, as well as superiority, of total annualized bleeding rate (ABR) from Week 12 through at least 15 months of follow up post-infusion compared with routine Factor VIII (FVIII) replacement prophylaxis treatment. Following a single 3e13 vg/kg dose, giroctocogene fitelparvovec demonstrated a statistically significant reduction in mean total ABR compared to the pre-infusion period.
- Key secondary endpoints as defined by the trial protocol were met and also demonstrated superiority compared to prophylaxis. 84% of participants maintained FVIII activity >5% at 15 months post-infusion with the majority of participants having FVIII activity ≥15%. The mean treated ABR showed a statistically significant 98.3% reduction post-infusion.
- In the AFFINE trial, giroctocogene fitelparvovec was generally well tolerated.

- Pfizer reported that analyses of the full Phase 3 dataset from the AFFINE trial are ongoing and additional data will be presented at upcoming medical meetings.
- Pfizer reported that it will discuss these data with regulatory authorities in the coming months.

*Fabry Disease*

- Dosing is complete in the Phase 1/2 STAAR study of isaralgagene civaparvovec, an investigational gene therapy for the treatment of Fabry disease, with a total of 33 patients dosed.
- Three additional patients were withdrawn from ERT, resulting in a total of 17 patients withdrawn from ERT to date. All 17 patients remain off ERT as of August 6. The one remaining patient dosed since February 2024 who began the study on ERT has plans in place to withdraw ERT treatment at the appropriate time.
- With the longest-treated patient having achieved four years of follow-up, Sangamo continues to amass encouraging clinical data, including evidence of improvements in kidney function. In the 18 patients treated for more than one year, a statistically significant rise in both mean and median eGFR levels was observed in male and female treated patients, based on preliminary findings. Anticipate sharing updated clinical data in the coming months.
- Held a productive meeting in June 2024 with the European Medicines Agency (EMA) on proposed pathway to potential approval for isaralgagene civaparvovec in Europe, with members of the U.S. Food and Drug Administration (FDA) in attendance.
- Engaged in ongoing business development discussions with potential collaborators for the Fabry disease program.

***Prioritized Neurology Pipeline***

*Neurology Epigenetic Regulation Programs*

- Investigational new drug (IND) enabling activities continue to advance in the Nav1.7 program to treat chronic neuropathic pain.
- Clinical trial authorization (CTA) enabling activities continue to advance for Sangamo's program to treat prion disease, leveraging the novel STAC-BBB capsid.
- Showcased 10 poster presentations at the 27$^{th}$ ASGCT Annual Meeting demonstrating advances in epigenetic regulation for the treatment of various neurological diseases, including prion disease and tauopathies.
- Engaged in ongoing business development discussions with potential collaborators for zinc finger neurology epigenetic regulation programs.

*Novel Adeno-Associated Virus (AAV) Capsid Delivery Technology*

- Continued to advance work on STAC-BBB, our novel neurotropic AAV capsid that demonstrated industry-leading BBB penetration in 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.
- Presented two platform presentations and four poster presentations at ASGCT outlining developments in AAV capsid delivery capabilities developed through Sangamo's SIFTER capsid engineering platform.
- Presented promising initial findings for a possible mechanism supporting how STAC-BBB may cross the blood-brain barrier and continued to successfully advance STAC-BBB manufacturing activities.
- Engaged in ongoing business development discussions with new potential collaborators for STAC-BBB.

*Next-Generation Genome Engineering*

- Presented one platform and three poster presentations at ASGCT showcasing Sangamo's next-generation genome engineering capabilities, including the discovery of the Modular Integrase (MINT) platform, a versatile, protein-guided genome editing method designed to integrate large sequences of DNA into the genome to potentially treat – with a single medicine – patients who have unique mutations in the same gene.
- Published a manuscript in *bioRxiv* titled, "Systematic Development of Reprogrammed Modular Integrases Enables Precise Genomic Integration of Large DNA Sequences" further detailing how the MINT platform builds on the strength of Sangamo's structural protein-DNA engineering capabilities derived from its zinc finger platform to target Bxb1, a serine recombinase. This technology is intended to integrate entire genes into the genome, to avoid double-stranded DNA breaks and the need for assistance from ancillary genome editing or DNA-repair modulating cargo.
- Presented MINT platform developments in June 2024 at the Federation of American Societies for Experimental Biology (FASEB), Genome Engineering: Research and Applications Conference.
- The MINT platform could be deployed internally for neurology-focused indications, and could provide potential partnering opportunities, both for human disease and in agricultural biotech settings.
- Engaged in ongoing business development discussions for Sangamo's modular integrase capabilities with potential collaborators.

**Second Quarter 2024 Financial Results**

Consolidated net loss for the second quarter ended June 30, 2024 was $36.1 million, or $0.18 per share, compared to a net loss of $114.5 million, or $0.66 per share, for the same period in 2023.

*Revenues*

Revenues for the second quarter ended June 30, 2024 were $0.3 million, compared to $6.8 million for the same period in 2023.

The decrease of $6.5 million in revenues was primarily attributed to decreases of $2.4 million and $2.2 million in revenues relating to our prior collaboration agreements with Biogen and Novartis, respectively, due to the termination of those collaboration agreements in June 2023, a decrease of $1.2 million in revenue relating to our collaboration agreement with Kite which expired pursuant to its terms in April 2024, and a decrease of $0.5 million in revenue relating to our license agreements with Sigma-Aldrich Corporation and Ligand Pharmaceuticals Inc.

*GAAP and Non-GAAP Operating Expenses*

| (In millions) | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2024 | 2023 | 2024 | 2023 |
| Research and development | $ 24.2 | $ 63.0 | $ 60.1 | $ 126.3 |
| General and administrative | 12.0 | 16.1 | 23.8 | 34.1 |
| Impairment of long-lived assets | 1.2 | - | 5.5 | 20.4 |
| Impairment of goodwill and indefinite-lived intangible assets | - | 51.3 | - | 89.5 |
| Total operating expenses | 37.4 | 130.4 | 89.4 | 270.3 |
| Impairment of long-lived assets | (1.2) | - | (5.5) | (20.4) |
| Impairment of goodwill and indefinite-lived intangible assets | - | (51.3) | - | (89.5) |
| Depreciation and amortization | (1.2) | (4.2) | (2.6) | (7.7) |
| Stock-based compensation expense | (3.1) | (6.8) | (5.8) | (15.1) |
| Non-GAAP operating expenses | $ 31.9 | $ 68.1 | $ 75.5 | $ 137.6 |

Total operating expenses on a GAAP basis for the second quarter ended June 30, 2024 were $37.4 million compared to $130.4 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 second quarter ended June 30, 2024 were $31.9 million, compared to $68.1 million for the same period in 2023.

The decrease in total operating expenses on a non-GAAP basis was primarily attributable to lower compensation and other personnel costs mainly due to lower headcount as a result of restructuring of operations and a corresponding reductions in workforce announced during 2023, a decrease due to restructuring expenses recorded in the second quarter of 2023 related to a reduction in workforce announced in April 2023, a decrease in preclinical and clinical expenses due to termination of collaboration agreements, and deferral and reprioritization of certain research and development programs, a decrease in external professional services, and a decrease in facilities and infrastructure related expenses. These decreases were partially offset by an increase relating to a terminated manufacturing-related supplier arrangement for costs that will be incurred without economic benefit to Sangamo.

*Cash and Cash Equivalents*

Cash and cash equivalents as of June 30, 2024 were $27.8 million, compared to cash, cash equivalents and marketable securities of $81.0 million as of December 31, 2023. On August 2, 2024, we entered into a global epigenetic regulation and capsid delivery license agreement with Genentech, a member of the Roche Group, under which we expect to receive an aggregate of $50.0 million in near-term upfront license fees and milestone payments. We believe that our available cash and cash equivalents as of June 30, 2024, in combination with the $50.0 million in expected near-term license fees and milestone payments from Genentech, will be sufficient to fund our planned operations into the first quarter of 2025.

*Financial Guidance for 2024*

- On a GAAP basis, we expect total operating expenses in the range of approximately $150 million to $170 million in 2024, which includes non-cash stock-based compensation expense, impairment expense, and depreciation and amortization.
- We expect non-GAAP total operating expenses, excluding estimated non-cash stock-based compensation expense of approximately $13 million, impairment expense of approximately $6 million, and depreciation and amortization of approximately $7 million, in the range of approximately $125 million to $145 million in 2024.

**Upcoming Events**

Sangamo plans to participate in the following events:

- Wells Fargo Healthcare Conference, September 4-6, 2024
- H.C. Wainwright 26th Annual Global Investment Conference, September 9-11, 2024

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 advancements and financial updates on Tuesday, August 6, 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 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 impact of business development and clinical advancements on Sangamo's cash runway and ability to continue to operate as a going concern, the therapeutic and commercial potential of Sangamo's product candidates, including the durability of therapeutic effects, the therapeutic and commercial potential of technologies used by Sangamo in its product candidates, including its gene therapy and cell therapy technologies and , zinc finger platform, the potential of its adeno-associated virus capsid delivery platform and its novel next-generation Modular Integrase (MINT) platform, Sangamo's ability to realize the expected benefits of the license agreement with Genentech, including but not limited to the receipt and timing of the upfront license fee and Sangamo's completion of the requisite technology transfer in order to receive an expected near-term milestone payment, the potential for Genentech to complete clinical development, regulatory interactions, manufacturing and global commercialization of any resulting products, the potential for Sangamo to receive development and commercial 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 find a collaboration partner for its Fabry disease gene therapy program and additional collaborations with respect to Sangamo's STAC-BBB capsid delivery platform and epigenetic regulation capabilities, and Pfizer's continued advancements of the giroctocogene fitelparvovec program, including the potential for Pfizer to complete clinical development, regulatory interactions, manufacturing and global commercialization of any resulting products, 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 anticipated advancement of Sangamo's product candidates to late-stage development, the timeline to present data from the Phase 3 AFFINE trial and Pfizer's discussions of data with applicable regulatory authorities, advancement of Sangamo's preclinical neurology programs, including announcement of data from, and anticipated IND and CTA submissions. the potential of the MINT platform to enable large-scale genomic engineering, Sangamo's estimates regarding the sufficiency of its cash resources and its expenses, capital requirements and need for substantial additional financing, 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, Sangamo's efforts and ability to secure additional funding, including plans to seek partners for certain of Sangamo's programs and the discussions related thereto, 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 the potential for Genentech to breach or terminate its agreement with Sangamo; and the potential for Sangamo to fail to realize its expected benefits from the Genentech agreement, including but not limited to further validating the importance of the zinc finger platform to support the development of therapeutics for neurodegenerative diseases; 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 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; Sangamo's ability to execute its restructurings as currently contemplated; 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, current or potential future bank failures, inflation and high 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 or that Sangamo will earn any milestone or royalty payments under the Genentech agreement or obtain regulatory approvals for product candidates arising from this agreement. 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, as supplemented by Sangamo's Quarterly Reports on Form 10-Q for the quarters ended March 31, 2024 and June 30, 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 data)

**Statement of Operations Data:**

| | Three months ended June 30, | | Six months ended June 30, | |
|---|---|---|---|---|
| | **2024** | **2023** | **2024** | **2023** |
| Revenues | $ 356 | $ 6,835 | $ 837 | $ 164,792 |
| Operating expenses: | | | | |
| Research and development | 24,223 | 63,046 | 60,114 | 126,262 |
| General and administrative | 12,045 | 16,014 | 23,812 | 34,150 |
| Impairment of long-lived assets | 1,172 | - | 5,521 | 20,433 |
| Impairment of goodwill and indefinite-lived intangible assets | - | 51,347 | - | 89,485 |
| Total operating expenses | 37,440 | 130,407 | 89,447 | 270,330 |
| Loss from operations | (37,084) | (123,572) | (88,610) | (105,538) |
| Interest and other income, net | 1,030 | 2,802 | 3,565 | 6,095 |
| Loss before income taxes | (36,054) | (120,770) | (85,045) | (99,443) |
| Income tax expense (benefit) | 74 | (6,264) | 172 | (6,070) |
| Net loss | $ (36,128) | $ (114,506) | $ (85,217) | $ (93,373) |
| Basic and diluted net loss per share | $ (0.18) | $ (0.66) | $ (0.44) | $ (0.54) |
| Shares used in computing basic and diluted net loss per share | 203,946 | 174,325 | 194,049 | 171,445 |

**Selected Balance Sheet Data:**

| | **June 30, 2024** | **December 31, 2023** |
|---|---|---|
| Cash and cash equivalents | $ 27,786 | $ 81,002 |
| Total assets | $ 93,014 | $ 165,320 |
| Total stockholders' equity | $ 23,690 | $ 82,887 |

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