Sangamo Therapeutics Reports Recent Business Highlights and First Quarter 2024 Financial Results

May 9, 2024

Showcased 20 presentations at the 27th American Society of Gene & Cell Therapy (ASGCT) Annual Meeting 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.

Announced discovery of novel next-generation modular integrase (MINT) platform that allows targeting of a serine recombinase engineered to enable large-scale genome editing.

Completed dosing in Phase 1/2 STAAR study of isaralgagene civaparvovec for Fabry disease and engaged in active discussions with potential Fabry collaboration partners.

Pfizer anticipates biologics license application (BLA) and marketing authorization application (MAA) submissions for Hemophilia A collaboration in early 2025 if mid-2024 pivotal readout is supportive.

Raised approximately $24 million in gross proceeds from a registered direct offering with institutional investors.

RICHMOND, Calif.--(BUSINESS WIRE)--May 9, 2024-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicines company, today reported business highlights and first quarter 2024 financial results.

“Sangamo continues to progress its neurology-focused genomic medicine pipeline as most recently showcased at the 27 th ASGCT Annual Meeting, with foundational advances across our epigenetic regulation programs and capsid engineering platform, supported by recent discovery of the neurotropic capsid STAC-BBB, and our novel next-generation integrase technology,” said Sandy Macrae, Chief Executive Officer of Sangamo. “We are pleased with the progress being made in business development discussions across our portfolio, including our Fabry disease program. We are building on the momentum achieved in the first quarter and look forward to achieving meaningful potential milestones throughout the rest of 2024 and beyond.”

Recent Business Highlights

Corporate Updates

- Raised approximately $24 million in gross proceeds from a registered direct offering with institutional investors.

Prioritized Neurology Pipeline

Neurology Epigenetic Regulation Programs

- Investigational new drug (IND) enabling toxicology studies are nearing completion in the Nav1.7 program to treat chronic neuropathic pain. An IND submission is expected in the fourth quarter of 2024.
- Clinical trial authorization (CTA) enabling activities are advancing for Sangamo’s program to treat prion disease, leveraging the novel STAC-BBB capsid. A CTA submission is expected in the fourth quarter of 2025.
- Intend to resume development of previously paused zinc finger repressor program addressing tauopathies, including Alzheimer’s disease, which leverages STAC-BBB. IND submission could occur as early as the fourth quarter of 2025.
- Showcased 10 poster presentations at the ASGCT Annual Meeting demonstrating advances in epigenetic regulation for the treatment of various neurological diseases, including prion disease and tauopathies.

Novel Adeno-Associated Virus (AAV) Capsid Delivery Technology

- Announced preclinical data from novel AAV capsid, STAC-BBB, 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.
- Engaged in ongoing business development discussions with 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.
- 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.

Other Programs

Fabry Disease

- Dosed the final patient in the Phase 1/2 STAAR study of isaralgagene civaparvovec, an investigational gene therapy for the treatment of Fabry disease, resulting in a total of 33 patients dosed in the study. Screening, enrollment and dosing are now complete.
- One additional patient was withdrawn from enzyme replacement therapy (ERT), resulting in a total of 14 patients withdrawn from ERT to date. The four patients dosed since February 2024 who began the study on ERT have plans in place to withdraw ERT treatment at the appropriate time.
- Presented updated clinical data in February 2024 at the 20th Annual WORLD*Symposium* in San Diego, CA, showing sustained benefit and differentiated safety profile for isaralgagene civaparvovec.
- Aligned with U.S. Food and Drug Administration (FDA) on an abbreviated pathway to potential approval. Granted PRIME eligibility from the European Medicines Agency (EMA) and Innovative Licensing and Access Pathway (ILAP) from U.K. Medicines and Healthcare products Regulatory Agency (MHRA) for isaralgagene civaparvovec.
- Engaged in active discussions with potential collaboration partners for the Fabry disease program. Deferring additional investments, including planning for a potential registrational trial, until a collaboration partnership or financing is secured.

Hemophilia A

- Pivotal readout 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 in early 2025 if the pivotal readout is supportive.
- 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.

First Quarter 2024 Financial Results

Consolidated net loss for the first quarter ended March 31, 2024 was $49.1 million, compared to a net income of $21.1 million, for the same period in 2023.

Basic and diluted net loss per share for the first quarter ended March 31, 2024 was $0.27, compared to basic net income per share of $0.13 per share and diluted net income per share of $ 0.12 per share for the same period in 2023.

Revenues

Revenues for the first quarter ended March 31, 2024 were $0.5 million, compared to $158.0 million for the same period in 2023.

The decrease of $157.5 million in revenues was primarily attributed to decreases of $132.3 million and $9.8 million in revenues relating to our collaboration agreements with Biogen and Novartis, respectively, due to the termination of collaboration agreements in June 2023, a decrease of $12.3 million in revenue relating to our collaboration agreement with Kite which expired pursuant to its terms in April 2024, and a decrease of $3.1 million in revenue relating to our license agreements with Sigma-Aldrich Corporation and Ligand Pharmaceuticals Inc.

GAAP and Non-GAAP Operating Expenses

	Three Months Ended March 31,	
(In millions)	**2024**	**2023**
Research and development	$ 35.9	$ 63.2
General and administrative	11.8	18.1
Impairment of long-lived assets	4.3	20.4
Impairment of goodwill	-	38.1
Total operating expenses	52.0	139.8
Impairment of long-lived assets	(4.3)	(20.4)
Impairment of goodwill	-	(38.1)

Depreciation and amortization	(1.4)	(3.5)
Stock-based compensation expense	(2.7)	(8.3)
Non-GAAP operating expenses	$ 43.6	$ 69.5

Total operating expenses on a GAAP basis for the first quarter ended March 31, 2024 were $52.0 million compared to $139.8 million for the same period in 2023. Non-GAAP operating expenses, which exclude impairment charges, depreciation and amortization, and stock-based compensation expense, for the first quarter ended March 31, 2024 were $43.6 million, compared to $69.5 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 a reduction in the bonus expense and lower headcount as a result of restructuring of operations and a corresponding reduction in workforce announced during 2023 and the first quarter of 2024, and decrease in preclinical and clinical expenses due to termination of collaboration agreements, and deferral and reprioritization of certain research and development programs.

Cash and Cash Equivalents

Cash and cash equivalents as of March 31, 2024 were $54.4 million, compared to cash, cash equivalents and marketable securities of $81.0 million as of December 31, 2023. In March, we completed a registered direct offering of common stock, for net proceeds of approximately $21.8 million after deducting placement agents' fees and estimated offering expenses payable by us. We believe that our available cash and cash equivalents as of March 31, 2024, in combination with the cost savings expected from the restructurings, workforce reduction and other potential cost reductions, will be sufficient to fund our planned operations into the third quarter of 2024.

Financial Guidance for 2024 Reiterated (initial guidance provided on March 13, 2024)

- On a GAAP basis, we continue to 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 continue to 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, week of June 24, 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 Thursday, May 9 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 therapeutic and commercial potential of Sangamo's product candidates and its engineered capsids and the potential of its next generation genome engineering technology, 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, 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, the potential of the MINT platform to enable large-scale genomic engineering, 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, 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 Sangamo's lack of capital resources to fully develop, obtain regulatory approval for and commercialize its product candidates and/or technologies, 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; Sangamo's 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 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, as supplemented by Sangamo's Quarterly Report on Form 10-Q for the quarter ended March 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 data)

Statement of Operations Data:

	Three months ended March 31,	
	2024	**2023**
Revenues	$ 481	$ 157,957
Operating expenses:		
Research and development	35,891	63,216
General and administrative	11,767	18,136
Impairment of long-lived assets	4,349	20,433
Impairment of goodwill	-	38,138
Total operating expenses	52,007	139,923
(Loss) income from operations	(51,526)	18,034
Interest and other income, net	2,535	3,293

(Loss) income before income taxes	(48,991)	21,327
Income tax expense	98	194
Net (loss) income	$ (49,089)	$ 21,133
Net (loss) income per share	-	-
Basic	$ (0.27)	$ 0.13
Diluted	$ (0.27)	$ 0.12
Shares used in computing net (loss) income per share	-	-
Basic	180,342	168,533
Diluted	180,342	169,181

Selected Balance Sheet Data:

	March 31, 2024	December 31, 2023
Cash and cash equivalents	$ 54,417	$ 81,002
Total assets	$ 128,961	$ 165,320
Total stockholders' equity	$ 56,697	$ 82,887

View source version on businesswire.com: https://www.businesswire.com/news/home/20240509815876/en/

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