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

November 12, 2024

Announced clear regulatory pathway to Accelerated Approval from U.S. Food and Drug Administration (FDA) for isaralgagene civaparvovec in Fabry disease, using 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.

Pfizer plans to present detailed data from Phase 3 AFFINE trial evaluating giroctocogene fitelparvovec, an investigational Hemophilia A gene therapy that Sangamo has co-developed with and licensed to Pfizer, via a platform presentation at the 66th American Society for Hematology (ASH) Annual Meeting and Exposition.

Received $50 million in upfront license fees and milestone payments in connection with previously announced global epigenetic regulation and capsid delivery license agreement with Genentech to develop novel genomic medicines for neurodegenerative diseases, and eligible to earn up to $1.9 billion in additional milestone payments, plus tiered royalties on net sales.

Submitted investigational new drug (IND) application to FDA for ST-503 for the treatment of intractable pain due to idiopathic small fiber neuropathy (iSFN), a type of chronic neuropathic pain.

RICHMOND, Calif.--(BUSINESS WIRE)--Nov. 12, 2024-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today reported business highlights and third quarter 2024 financial results.

“This is a pivotal time for Sangamo as we build upon our recent strong momentum with news of a clear regulatory pathway to Accelerated Approval in the U.S. for ST-920 in Fabry disease, accelerating time to potential approval by approximately three years,” said Sandy Macrae, Chief Executive Officer of Sangamo Therapeutics. “Alongside Pfizer’s Hemophilia A program activities, and against a backdrop of our recent epigenetic regulation and capsid license agreement with Genentech, these developments create a strong foundation for our core neurology pipeline, which continues to advance. We were thrilled to submit our first neurology IND application this quarter for ST-503 for the treatment of intractable pain due to idiopathic small fiber neuropathy and expect to be in the clinic in mid-2025. We are proud of our scientific and strategic execution so far this year and are continuing to work diligently to secure additional funding to further advance our wholly owned neurology programs, capsid delivery technology and next-generation genome engineering capabilities.”

Recent Business Highlights

Corporate Updates

- Announced in August a global epigenetic regulation and capsid delivery license agreement with Genentech to develop novel genomic medicines for neurodegenerative diseases.
- Received $50.0 million from Genentech in upfront license fees and milestone payments.
- Eligible to earn up to $1.9 billion from Genentech in additional 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

Fabry Disease

- Announced in October the outcome of a successful interaction with the FDA, providing a clear regulatory pathway to Accelerated Approval for isaralgagene civaparvovec, or ST-920, an investigational gene therapy for the treatment of Fabry disease.
- The FDA has agreed in a Type B interaction that data from the ongoing Phase 1/2 STAAR study can serve as the primary basis for approval under the Accelerated Approval Program, using estimated glomerular filtration rate (eGFR) slope as an intermediate clinical endpoint.
- Sangamo engaged with the FDA on alternative pathways to potential approval following analysis of clinical data from the Phase 1/2 STAAR study showing encouraging safety and efficacy data, including promising preliminary evidence of improved kidney function. In the 18 male and female patients treated with isaralgagene civaparvovec with more than one year of follow-up data, a statistically significant positive mean annualized eGFR slope was observed.
- Based on these latest data, the FDA agreed that eGFR slope at 52 weeks can serve as an intermediate clinical endpoint to support a potential Accelerated Approval. The FDA also advised that eGFR slope at 104 weeks may be assessed to verify clinical benefit.
- The complete dataset to support an Accelerated Approval pathway will be available in the first half of 2025. This approach unlocks a potential Biologics License Application (BLA) submission in the second half of 2025, three years ahead of previous estimates, and avoids the requirement for an additional, costly registrational study to establish clinical efficacy.

- Dosing was completed in the Phase 1/2 STAAR study in April 2024, with a total of 33 patients dosed. The longest treated patient recently achieved four years of follow-up.
- The 18th and final patient who started the study on Enzyme Replacement Therapy (ERT), was successfully withdrawn from ERT in September 2024, and all 18 patients remain off ERT as of today.
- Sangamo has begun to execute BLA readiness activities for isaralgagene civaparvovec, while continuing to advance ongoing business development discussions with potential collaboration partners.

Hemophilia A

- Pfizer will be presenting detailed data from the Phase 3 AFFINE trial of giroctocogene fitelparvovec, an investigational gene therapy that Sangamo has co-developed with and licensed to Pfizer for the treatment of adults with moderately severe to severe hemophilia A, in an oral presentation at the 66th ASH Annual Meeting and Exposition on December 9, 2024.
- The ASH abstract confirmed that the AFFINE trial met its primary endpoint of non-inferiority and superiority, with a statistically significant decrease in 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 (mean total ABR estimates, 1.24 vs 4.73).
- Key secondary endpoints as defined by the trial protocol – the percentage of participants with FVIII activity >5% (chromogenic assay) at 15 months and ABR for treated bleeds – were met, and also demonstrated superiority compared to prophylaxis. At Month 15, 84% of participants had FVIII activity >5%, with 82.8% of participants continuing to maintain FVIII activity >5% at 2-years post-infusion.
- Treated ABR during Week 12 through ≥15 months post-infusion was significantly reduced compared to prophylaxis (mean treated ABR estimates, 0.07 vs. 4.08), demonstrating superiority.
- In the AFFINE trial, giroctocogene fitelparvovec was generally well tolerated with no study discontinuations.
- Pfizer is discussing these data with regulatory authorities.
- 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.

Neurology Pipeline

Chronic Neuropathic Pain

- Submitted IND application to the FDA for ST-503, an investigational epigenetic regulator for the treatment of intractable pain due to iSFN, a type of chronic neuropathic pain.
- Assuming clearance of this IND by the FDA, we expect to start the Phase 1/2 study in the middle of 2025.
- Published a manuscript in *bioRxiv* titled, “Potent and selective repression of SCN9A by engineered zinc finger repressors (ZFRs) for the treatment of neuropathic pain” demonstrating that ZFRs can selectively and potently reduce the expression of Nav1.7 sodium channels in sensory neurons in animal models, following a single intrathecal administration of ST-503.

Prion Disease

- Clinical trial authorization (CTA) enabling activities continue to advance for Sangamo’s program to treat prion disease, leveraging our novel neurotropic AAV capsid known as STAC-BBB, that has demonstrated industry-leading blood brain barrier penetration in nonhuman primates (NHPs) following intravenous administration.
- Presented updated data at Prion 2024 Conference in October 2024, showing the potency of Sangamo’s ZFR in a disease mouse model at multiple dose levels. The ZFR significantly reduced expression of prion mRNA and protein in the brain, extended mouse survival and limited the formation of toxic prion aggregates.
- Additionally, we presented NHP data at the Prion 2024 Conference, showing that a single intravenous administration of the prion ZFR, delivered via STAC-BBB, resulted in potent and widespread repression of the prion gene in transduced neurons.
- A CTA submission is expected in the fourth quarter of 2025.

Novel Adeno-Associated Virus (AAV) Capsid Delivery Technology

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

Third Quarter 2024 Financial Results

Consolidated net income available for common stockholders for the third quarter ended September 30, 2024 was $10.7 million, or $0.04 per share on a fully diluted basis, compared to a net loss of $104.2 million, or $0.59 per share, for the same period in 2023.

Revenues

Revenues for the third quarter ended September 30, 2024 were $49.4 million, compared to $9.4 million for the same period in 2023.

The increase of $40.0 million in revenues was primarily attributed to $49.2 million in revenue relating to our collaboration agreement with Genentech, Inc. This increase was partially offset by a decrease of $5.5 million in revenue relating to our collaboration agreement with Kite Pharma, Inc. which expired pursuant to its terms in April 2024, and a decrease of $3.7 million in revenue relating to our other license agreements.

GAAP and Non-GAAP Operating Expenses

(In millions)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Research and development	$ 27.7	$ 57.1	$ 87.8	$ 183.4
General and administrative	11.1	13.9	34.9	48.1
Impairment of long-lived assets	-	44.8	5.5	65.2
Impairment of goodwill and indefinite-lived intangible assets	-	-	-	89.5
Total operating expenses	38.8	115.8	128.2	386.2
Impairment of long-lived assets	-	(44.8)	(5.5)	(65.2)
Impairment of goodwill and indefinite-lived intangible assets	-	-	-	(89.5)
Depreciation and amortization	(1.3)	(5.5)	(3.9)	(13.2)
Stock-based compensation expense	(3.3)	(6.2)	(9.1)	(21.3)
Non-GAAP operating expenses	$ 34.2	$ 59.3	$ 109.7	$ 197.0

Total operating expenses on a GAAP basis for the third quarter ended September 30, 2024 were $38.8 million compared to $115.8 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 third quarter ended September 30, 2024 were $34.2 million, compared to $59.3 million for the same period in 2023.

The decrease in total operating expenses on a non-GAAP basis was primarily attributable to a decrease in preclinical and clinical expenses due to deferral and reprioritization of certain research and development programs, 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 in external professional services costs, and a decrease in facilities and infrastructure related expenses.

Cash and Cash Equivalents

Cash and cash equivalents as of September 30, 2024 were $39.2 million, compared to cash, cash equivalents and marketable securities of $81.0 million as of December 31, 2023. We believe that our available cash and cash equivalents as of September 30, 2024, together with the $10.0 million milestone payment we received 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 $6 million, in the range of approximately $125 million to $145 million in 2024.

Upcoming Events

Sangamo plans to participate in the following event:

- Jefferies London Healthcare Conference, November 19-21, 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, November 12, 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: 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 technologies and zinc finger platform, 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; 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 collaboration partner; Sangamo's ability to realize the expected benefits of the license agreement with Genentech, including 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 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, advancement of Sangamo's preclinical neurology programs, including announcement of such data, and anticipated CTA submission, 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 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, 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 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, 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 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 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, 2023, as supplemented by Sangamo's Quarterly Report on Form 10-Q for the quarter ended September 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 amounts)

Statement of Operations Data:

	Three months ended September 30,		Nine months ended September 30,	
	2024	**2023**	**2024**	**2023**
Revenues	$ 49,412	$ 9,398	$ 50,249	$ 174,190
Operating expenses:				
Research and development	27,732	57,089	87,846	183,351
General and administrative	11,049	13,918	34,861	48,068
Impairment of long-lived assets	-	44,799	5,521	65,232
Impairment of goodwill and indefinite-lived intangible assets	-	-	-	89,485
Total operating expenses	38,781	115,806	128,228	386,136
Income (loss) from operations	10,631	(106,408)	(77,979)	(211,946)
Interest and other income, net	129	3,515	3,694	9,610
Income (loss) before income taxes	10,760	(102,893)	(74,285)	(202,336)
Income tax expense (benefit)	88	1,270	260	(4,800)
Net income (loss)	10,672	(104,163)	(74,545)	(197,536)
Net income allocated to participating securities	1,287	-	-	-
Net income (loss) available to common stockholders	$ 9,385	$ (104,163)	$ (74,545)	$ (197,536)
Net income (loss) per share				
Basic	$ 0.05	$ (0.59)	$ (0.37)	$ (1.14)
Diluted	$ 0.04	$ (0.59)	$ (0.37)	$ (1.14)
Shares used in computing net income (loss) per share				
Basic	208,345	177,171	198,849	173,375
Diluted	214,325	177,171	198,849	173,375

Selected Balance Sheet Data:

	September 30, 2024	**December 31, 2023**
Cash, cash equivalents, and marketable securities	$ 39,201	$ 81,002
Total assets	$ 111,263	$ 165,320
Total stockholders' equity	$ 39,134	$ 82,887

View source version on businesswire.com: https://www.businesswire.com/news/home/20241112955936/en/

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Source: Sangamo Therapeutics, Inc.