Sangamo Therapeutics Reports Recent Business Highlights and Second Quarter 2023 Financial Results

August 8, 2023

Conference call and webcast scheduled for Tuesday, August 8, 4:30 p.m. Eastern Time.

BRISBANE, Calif.--(BUSINESS WIRE)--Aug. 8, 2023-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicines company, today reported recent business highlights and second quarter 2023 financial results.

“I am extremely proud of our significant progress this quarter. We are pleased to have executed several business development deals, demonstrating the excitement in Sangamo’s science and platform,” said Sandy Macrae, Chief Executive Officer of Sangamo. “We continue to make progress in our clinical programs, with productive written feedback received from the FDA on our Fabry Phase 3 strategy, and initial approvals received on our amended TX200 protocol to accelerate dose escalation. The impressive data we presented this quarter on our preclinical neurology and AAV capsid delivery capabilities reinforces our scientific capabilities, which continue to lay the foundation for the company’s future.”

Recent Business Highlights

Corporate Updates

- Announced research evaluation and option agreement with Prevail Therapeutics, a wholly owned subsidiary of Eli Lilly and Company, for novel engineered cerebrospinal fluid-administered capsids with enhanced nervous system delivery. We have granted Prevail rights to evaluate our proprietary adeno-associated virus (AAV) capsids developed through our AAV capsid engineering platform, SIFTER, in exchange for an upfront payment. If Prevail exercises the option to license the capsids, then we will become eligible to earn exercise fees and potential development and commercial milestones of up to $1.2 billion and tiered royalties on potential sales of products using the capsids.
- Announced research evaluation and option agreement with Chroma Medicine to explore zinc finger proteins (ZFPs) for epigenetic editing. We have granted Chroma rights to evaluate our novel ZFPs in specified targets outside of the central nervous system in exchange for an upfront payment. If Chroma exercises the option to license the ZFPs, then we will become eligible to earn exercise fees, potential development and commercial milestone payments and tiered royalties on potential sales of products using the ZFPs.

Program Highlights

Fabry Disease *– Dosed total of 22 patients in Phase 1/2 STAAR study; received Fast Track Designation from U.S. FDA; received productive written FDA feedback on proposed Phase 3 trial strategy; expect to submit proposed Phase 3 protocol to the FDA as early as the end of 2023.*

- Dosed 2 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 22 patients dosed to date.
- Received U.S. FDA Fast Track Designation for isaralgagene civaparvovec, which aims to expedite the review of new therapeutics that are intended to treat serious or life-threatening conditions.
- Received productive written feedback from U.S. FDA on proposed Phase 3 trial strategy that seeks to maximize the potential patient population for ST-920 across two studies. Based on feedback received, we do not expect a head-to-head comparison with enzyme replacement therapy to be required in the naïve and pseudo-naïve patient study. Requesting specific additional clarifications before submitting a proposed Phase 3 protocol to the FDA, which we expect to be as early as the end of 2023.

Renal Transplant Rejection *– Safety Monitoring Committee endorsed moving to the second dose level based on encouraging safety data to date; successfully manufactured dose for the first patient in the second dose cohort; received first approvals from regulatory authorities for protocol amendment to accelerate dose escalation.*

- A total of six study sites across four countries are now open and enrolling patients.
- Based on encouraging safety data, the Safety Monitoring Committee endorsed moving to cohort 2 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.
- The product candidate continues to be generally well tolerated in all three patients dosed to date.
- Completed manufacturing of dose for the first patient in the second cohort, who recently received a kidney transplant. Dosing of this fourth patient is expected in the third quarter of 2023.
- Received first full country approval for amended protocol to accelerate dose escalation.
- Intend to share initial data by the end of 2023.

Neurology Epigenetic Regulation Programs *– Presented first preclinical data evaluating zinc finger transcriptional repressors targeting Nav1.7; presented updated animal model data in prion disease; presented additional data describing advances being made in AAV capsid delivery.*

- Presented comprehensive preclinical data package evaluating zinc finger repressors (ZF-Rs) targeting the Nav1.7 gene at the American Society for Cell and Gene Therapy (ASGCT) 26th Annual Meeting. The data demonstrated potent and specific repression of Nav1.7 expression, without impacting other sodium channels, and that the ZF-Rs are well tolerated in non-human primates. Continue to expect an IND submission in 2024.
- Presented updated animal model data at ASGCT showing that our ZF-Rs significantly reduce expression of the prion protein in the brain, extend lifespan, and limit formation of toxic prion aggregates. Continue to expect an IND submission in 2025.
- Entered into a license agreement with Voyager Therapeutics for Voyager's intravenous-administered capsid, which has been shown in animal models to achieve the specific central nervous system coverage we believe to be required for our prion disease program.
- Continued to advance identification and selection of engineered AAV capsids for enhanced central nervous system delivery, with additional data presented at ASGCT describing the identification of multiple novel AAV capsids exhibiting characteristics consistent with enhanced blood brain barrier transit.

Hemophilia A (Pfizer) *– Phase 3 AFFINE trial continues to progress; pivotal data read-out expected in mid-2024; BLA and MAA submissions anticipated in second half of 2024.*

- 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, continues to progress following the dosing of all patients required to support primary analysis.
- A pivotal readout is expected in mid-2024, with Pfizer anticipating BLA and MAA submissions in the second half of 2024.

Second Quarter 2023 Financial Results

Consolidated net loss for the second quarter ended June 30, 2023 was $114.5 million, or $0.66 per share, compared to a net loss of $43.2 million, or $0.29 per share, for the same period in 2022. This was primarily due to higher non-cash charges relating to impairment of indefinite-lived intangible assets of $51.3 million, which was a result of continued decline in our market capitalization reflecting the decline in equity values in the biotechnology industry, and a decrease in revenues of $22.5 million as a result of termination of certain collaboration agreements.

Revenues

Revenues for the second quarter ended June 30, 2023 were $6.8 million, compared to $29.4 million for the same period in 2022.

The decrease of $22.5 million in revenues was primarily attributed to decreases of $8.5 million, $7.8 million, and $1.8 million of revenues relating to our collaboration agreements with Novartis, Biogen and Sanofi, respectively, due to the termination of these collaboration agreements. Additionally, revenues relating to our collaboration agreement with Kite decreased by $5.1 million, reflecting a reduction in collaboration activities during the quarter.

GAAP and Non-GAAP Operating Expenses

	Three Months Ended June 30,		Six Months Ended June 30,	
(In millions)	**2023**	**2022**	**2023**	**2022**
Research and development	$ 63.0	$ 60.0	$ 126.3	$ 118.6
General and administrative	16.1	15.1	34.1	30.0
Impairment of goodwill and indefinite-lived intangible assets	51.3	-	89.5	-
Impairment of long-lived assets	-	-	20.4	-
Total operating expenses	130.4	75.1	270.3	148.6
Impairment of goodwill and indefinite-lived intangible assets	(51.3)	-	(89.5)	-
Impairment of long-lived assets	-	-	(20.4)	-
Stock-based compensation expense	(6.8)	(7.9)	(15.1)	(15.6)
Non-GAAP operating expenses	$ 72.3	$ 67.2	$ 145.3	$ 133.0

Total operating expenses on a GAAP basis for the second quarter ended June 30, 2023 were $130.4 million, compared to $75.1 million for the same period in 2022. GAAP operating expenses for the second quarter ended June 30, 2023 include a full write-down of indefinite-lived intangible assets of $51.3 million, which was a result of continued decline in our market capitalization reflecting the decline in equity values in the biotechnology industry. Non-GAAP operating expenses, which exclude impairment charges and stock-based compensation expense, for the second quarter ended June 30, 2023 were $72.3 million, compared to $67.2 million for the same period in 2022.

The increase in total operating expenses on a non-GAAP basis was primarily attributable to higher compensation and other personnel costs mainly due to restructuring-related charges, higher facilities, infrastructure-related costs, and professional services as we advance our preclinical and clinical pipeline. These increases were partially offset by reimbursement of certain research and development expenses by Sanofi under the termination and transition agreement.

Cash, Cash Equivalents and Marketable Securities

Cash, cash equivalents and marketable securities as of June 30, 2023 were $182.1 million, compared to $307.5 million as of December 31, 2022. We have raised approximately $15.1 million in net proceeds under our at-the-market offering program since January 1, 2023. We believe that our available cash, cash equivalents and marketable securities as of June 30, 2023, in combination with other potential cost reductions, will be sufficient to fund our planned operations for at least the next 12 months.

Updated Financial Guidance for 2023

- GAAP operating expenses, including impairment of goodwill, indefinite-lived intangible assets, and long-lived assets, and stock-based compensation expense, are now estimated to be in the range of approximately $378 million to $398 million, reflecting the additional non-cash impairment charges. The previous GAAP operating expenses guidance provided on April 26, 2023 was in the range of approximately $315 million to $335 million.
- We continue to estimate non-GAAP operating expenses to remain unchanged from the last update on April 26, 2023 and to be in the range of approximately $240 million to $260 million. Estimated non-GAAP operating expenses exclude estimated impairment of goodwill of $38.1 million, impairment of indefinite-lived intangible assets of $51.3 million, impairment of long-lived assets of $20.4 million and stock-based compensation expense of $28 million.

Upcoming Events

Sangamo plans to participate in the following events:

Investor Conferences

- 2023 Wells Fargo Healthcare Conference, September 6-8, 2023
- Truist Securities BioPharma Symposium, November 8-9, 2023
- Jefferies London Healthcare Conference, November 14-16, 2023

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 to Discuss Second Quarter 2023 Results

The Sangamo management team will discuss these results on a conference call today, Tuesday, August 8, 2023, at 4:30 p.m. 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 clinical-stage biopharmaceutical company with a robust genomic medicines pipeline. Using ground-breaking science, including our proprietary zinc finger genome engineering technology and manufacturing expertise, Sangamo aims to create new genomic medicines for patients suffering from diseases for which existing treatment options are inadequate or currently don't exist. To learn more, visit www.sangamo.com and connect with us on LinkedIn and Twitter.

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 our product candidates, the anticipated plans and timelines of Sangamo and our collaborators for screening, enrolling and 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, preparations and plans for patient dosing in the STEADFAST study, the potential for acceleration of the study timeline and the availability of data therefrom, the anticipated advancement of our product candidates to late-stage development, including potential future Phase 3 trials of isaralgagene civaparvovec and the timing thereof, the anticipated submission to the FDA of a proposed Phase 3 trial protocol, the expectation that a head-to-head comparison with existing enzyme replacement therapy will not be required in the naïve and pseudo-naïve patient study, the timeline to present data from the Phase 3 AFFINE trial and make BLA and MAA submissions for giroctocogene fitelparvovec, expectations regarding advancement of our preclinical neurology programs, including announcement of data from, and anticipated IND submissions related to, such programs, the potential for Chroma and Prevail to exercise their options and for Sangamo to receive exercise fees, development and commercial milestone payments and royalties under the Chroma and Prevail agreements, our expected cash runway, potential cost reductions, our 2023 financial guidance related to GAAP and non-GAAP total operating expenses, impairments and stock-based compensation, our plans to participate in industry and investor conferences, and other statements that are not historical fact. These statements are not guarantees of future performance and are subject to certain risks and uncertainties that are difficult to predict. Factors that could cause actual results to differ include, but are not limited to, risks and uncertainties related the effects of macroeconomic factors or financial challenges, including as a result of the ongoing conflict between Russia and Ukraine 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 our collaborators, including the initiation and operation of clinical trials; the research and development process, including the enrollment, operation and results of clinical trials and the presentation of clinical data; 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, including data from kidney biopsies, 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 Phase 3 trial of our product candidates; our limited experience manufacturing biopharmaceutical products, including the risks that we may be unable to maintain compliant manufacturing facilities, build additional facilities and manufacture our product candidates as intended; the potential for Chroma, Prevail or Voyager to breach or terminate their agreements with Sangamo; the potential for Sangamo to fail to realize its expected benefits from the Chroma, Prevail or Voyager agreements; the potential for technological developments that obviate technologies used by Sangamo; our lack of capital resources to fully develop, obtain regulatory approval for and commercialize our product candidates, including our ability to secure the funding required to initiate a potential Phase 3 trial of isaralgagene civaparvovec in a timely manner or at all; our need for substantial additional funding to execute our operating plan and to continue to operate as a going concern; our reliance on collaborators and our potential inability to secure additional collaborations, and our ability to achieve expected future financial performance.

There can be no assurance that we and our 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 our collaborators. These risks and uncertainties are described more fully in our Securities and Exchange Commission, or SEC, filings and reports, including in our Annual Report on Form 10-K for the year ended December 31, 2022, as supplemented by our Quarterly Report on Form 10-Q for the quarter ended June 30, 2023 to be filed with the SEC, and future 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 we undertake 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 total operating expenses, which excludes stock-based compensation expense and impairment of goodwill, indefinite-lived intangible assets and long-lived assets from GAAP total 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 stock-based compensation expense because it is a non-cash expense 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,	
	2023	**2022**	**2023**	**2022**
Revenues	$ 6,835	$ 29,378	$ 164,792	$ 57,609
Operating expenses:				
Research and development	63,046	60,019	126,262	118,603
General and administrative	16,014	15,093	34,150	30,001
Impairment of goodwill and indefinite-lived intangible assets	51,347	-	89,485	-
Impairment of long-lived assets	-	-	20,433	-
Total operating expenses	130,407	75,112	270,330	148,604
Loss from operations	(123,572)	(45,734)	(105,538)	(90,995)
Interest and other income, net	2,802	2,643	6,095	3,985
Loss before income taxes	(120,770)	(43,091)	(99,443)	(87,010)
Income tax (benefit) expense	(6,264)	82	(6,070)	140
Net loss	$ (114,506)	$ (43,173)	$ (93,373)	$ (87,150)
Basic and diluted net loss per share	$ (0.66)	$ (0.29)	$ (0.54)	$ (0.59)
Shares used in computing basic and diluted net loss per share	174,325	148,158	171,445	147,194

Selected Balance Sheet Data:

	June 30, 2023	**December 31, 2022**
Cash, cash equivalents, and marketable securities	$ 182,138	$ 307,477
Total assets	$ 325,074	$ 562,509
Total stockholders' equity	$ 233,642	$ 294,958

View source version on businesswire.com: https://www.businesswire.com/news/home/20230808479265/en/

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