Sangamo Therapeutics Reports Recent Business Highlights and Fourth Quarter and Full Year 2022 Financial Results

February 22, 2023

Conference Call and Webcast Scheduled for 4:30 p.m. Eastern Time

BRISBANE, Calif.--(BUSINESS WIRE)--Feb. 22, 2023-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicines company, today reported recent business highlights and fourth quarter and full year 2022 financial results.

“2022 was a year of important clinical and non-clinical milestones across our pipeline. We strengthened that momentum today by releasing compelling data from our Phase 1/2 Fabry disease study, supporting a potential best-in-class product profile,” said Sandy Macrae, Chief Executive Officer of Sangamo. “In 2023, wise resource allocation is our top priority, as we focus on advancing our wholly owned Fabry program, CAR-Treg portfolio and epigenetic regulation programs in the central nervous system, alongside progression of our Zinc Finger platform and AAV delivery capabilities. We look forward to sharing additional pipeline and delivery milestones in 2023 as we continue to strive to deliver for patients in need.”

Fourth Quarter Updates and Recent Business Highlights

Fabry disease *– Reported compelling updated preliminary Phase 1/2 data showing extended clinical benefit; reported kidney biopsy data demonstrating 78% globotriaosylceramide (Gb3) substrate clearance at 6-months; Phase 3 trial anticipated to start by end of 2023.*

- Presented updated preliminary data as of the October 20, 2022 cutoff date from the Phase 1/2 STAAR study evaluating isaralgagene civaparvovec, our wholly owned gene therapy product candidate for the treatment of Fabry disease at the 19th Annual WORLD*Symposium*.
- Sustained, elevated expression of alpha-galactosidase A (α-Gal A) activity observed in 13 patients for over two years for the longest treated patient as of the November 15, 2022 supplemental cutoff date.
- No prophylactic corticosteroids or other immune modulating agents have been administered.
- All five patients who began the dose escalation phase on enzyme replacement therapy (ERT) had been successfully withdrawn from ERT and remain off ERT today.
- Announced first available kidney biopsy data demonstrating 78% Gb3 substrate clearance at 6-months and 77% reduction in urine podocyte loss in a treated patient with high baseline plasma globotriaosylsphingosine (lyso-Gb3) levels.
- Reported a clinically meaningful and statistically significant increase in mean general health scores measured across all patients treated in dose escalation phase, as measured by the SF-36 General Health Survey.
- As of the October 20, 2022 cutoff date, naïve and pseudo-naïve patients treated in the dose expansion and escalation phases with baseline lyso-Gb3 levels above 80 ng/mL experienced a 40-65% reduction. For the first time, and at the highest dose, a 54% reduction in lyso-Gb3 levels was observed where baseline levels started below 25 ng/mL.
- As of the October 20, 2022 cutoff date, isaralgagene civaparvovec remained generally well tolerated with no treatment-related adverse events greater than Grade 2 and no treatment-related serious adverse events.
- Since the October 20, 2022 cutoff date, four additional patients have been dosed in the expansion phase to achieve a total of 17, and a further two patients have been withdrawn from ERT.
- Dosing of remaining patients in the expansion phase of the Phase 1/2 STAAR study is ongoing, with a total of 20 sites active and recruiting. We expect dosing to conclude by the end of 2023.
- Preparations for a potential Phase 3 trial actively progress, with a trial start anticipated by the end of 2023, depending on regulatory interactions. Dosing of the first patient is expected to start as early as the first part of 2024.

Renal Transplant Rejection *– Progressed manufacturing and clinical activities ahead of anticipated Q2 dosing; potential dose escalation acceleration scenarios in discussion with regulators.*

- Progressed manufacturing and clinical activities 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 both patients dosed to date.
- Completed manufacturing of the dose for the final patient in the first cohort, who recently received a kidney transplant. Dosing is expected early in the second quarter of 2023.
- Progressed clinical and manufacturing activities for the first patient in the second cohort, with dosing expected in the summer of 2023.
- Opportunities to accelerate dose escalation are being explored with regulators.

Sickle cell disease *– Presented promising 6-month data for first patient dosed with a product candidate manufactured using improved methods; discontinuing additional material investments in Phase 3 planning in order to prioritize capital allocation; intend to seek a potential collaboration partner.*

- Presented promising data from the Phase 1/2 PRECIZN-1 study of BIVV003, a zinc finger nuclease gene-edited cell therapy candidate for the treatment of sickle cell disease at the 64th American Society of Hematology (ASH) Annual Meeting. The first patient to receive a product candidate manufactured using improved methods achieved fetal hemoglobin levels of 45% and total hemoglobin of 12.4 g/dL at week 26.
- Progressed clinical and manufacturing activities for patient 7.
- Agreed on Phase 3 trial design, Chemistry, Manufacturing and Controls (CMC) package and other requirements with the FDA.
- Progressed additional manufacturing improvements which we believe have the potential to further strengthen clinical outcomes and reduce manufacturing costs in a potential Phase 3 trial.
- Announced strategic decision to halt further material investments in the program beyond completion of Phase 1/2 study, to redeploy resources to Fabry and TX200 programs, and to seek a potential collaboration partner to progress this asset to a potential Phase 3 trial.

Hemophilia A *– Dosing in Phase 3 AFFINE trial expected to be complete by end of Q1 2023; pivotal data read-out expected in first half of 2024; BLA submission anticipated in second half of 2024.*

- Dosing progressed 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.
- Dosing to support primary analysis is expected to be complete by the end of Q1 2023.
- A pivotal readout is expected in the first half of 2024, with Pfizer anticipating a BLA submission in the second half of 2024.

Preclinical Programs *– Progressed preclinical programs in CAR-Tregs and epigenetic regulation of the central nervous system (CNS), alongside advancements in the zinc finger platform and AAV delivery capabilities.*

- By the end of 2023, we expect to announce, and reveal data from, our next wholly owned CNS epigenetic regulation program.
- By the end of 2024, we anticipate submitting two new IND applications from our second wave CAR-Treg and CNS programs.

Fourth Quarter and Full Year 2022 Financial Results

Consolidated net loss for the fourth quarter ended December 31, 2022 was $52.0 million, or $0.32 per share, compared to net loss of $37.5 million, or $0.26 per share, for the same period in 2021. For the year ended December 31, 2022, consolidated net loss was $192.3 million, or $1.25 per share, compared to consolidated net loss of $178.3 million, or $1.23 per share, for the year ended December 31, 2021.

Revenues

Revenues for the fourth quarter ended December 31, 2022 were $27.2 million, compared to $28.0 million for the same period in 2021.

The decrease of $0.8 million in revenues was primarily attributed to decreases of $12.4 million and $1.8 million in revenues related to our collaboration agreements with Biogen and Sanofi, respectively. These decreases were partially offset by increases of $12.6 million and $1.3 million in revenues related to our collaboration agreements with Kite and Novartis, respectively.

Revenues were $111.3 million in 2022, compared to $110.7 million in 2021.

The increase of $0.6 million in revenues was primarily attributed to increases of $13.1 million and $1.8 million in revenues related to our collaboration agreements with Kite and Novartis, respectively. These increases were partially offset by decreases of $13.9 million in revenue related to our collaboration agreement with Biogen.

GAAP and Non-GAAP operating expenses

	Three Months Ended December 31,		Year Ended December 31,	
(In millions)	**2022**	**2021**	**2022**	**2021**
Research and development	$ 66.2	$ 51.8	$ 249.9	$ 230.8
General and administrative	16.4	16.1	62.7	63.2
Total operating expenses	82.6	67.9	312.6	294.0
Stock-based compensation expense	(8.3)	(8.1)	(31.7)	(33.0)
Non-GAAP operating expenses	$ 74.3	$ 59.8	$ 280.9	$ 261.0

Total operating expenses on a GAAP basis for the fourth quarter ended December 31, 2022 were $82.6 million, compared to $67.9 million for the same period in 2021. Non-GAAP operating expenses, which exclude stock-based compensation expense, for the fourth quarter ended December 31, 2022 were $74.3 million, compared to $59.8 million for the same period in 2021.

The increase in total operating expenses on a GAAP basis was primarily attributable to increased spending on our internal infrastructure and external services as we progress our clinical trials coupled with higher headcount related personnel costs, and an adjustment in 2021 related to dissolution of the repayment obligation of a grant from California Institute for Regenerative Medicine with the discontinuation of the ST-400 program. These increases were partially offset by reimbursement of certain research and development expenses by Sanofi under the termination agreement.

Total operating expenses on a GAAP basis in 2022 were $312.6 million compared to $294.0 million in 2021. Non-GAAP operating expenses, which exclude stock-based compensation expense, were $280.9 million and $261.0 million in 2022 and 2021, respectively.

The increase in total operating expenses in the full year on a GAAP basis was primarily driven by increased spending on our internal infrastructure and external services as we progress our clinical trials coupled with higher headcount related personnel costs and our ongoing collaborations, and an adjustment in 2021 related to dissolution of the repayment obligation of a grant from California Institute for Regenerative Medicine with the discontinuation of the ST-400 program. These increases were partially offset by reimbursement of certain research and development expenses by Sanofi under the termination agreement.

Cash, cash equivalents and marketable securities

Cash, cash equivalents and marketable securities as of December 31, 2022 were $307.5 million, compared to $464.7 million as of December 31, 2021. We have raised approximately $90.6 million in net proceeds under our at-the-market offering program since January 1, 2022.

Initial Financial Guidance for 2023

On a GAAP basis, we expect total operating expenses in the range of approximately $310 million to $330 million in 2023, which includes non-cash stock-based compensation expense.

We expect non-GAAP total operating expenses, excluding estimated non-cash stock-based compensation expense of approximately $35 million, in the range of approximately $275 million to $295 million in 2023.

Upcoming Events

Sangamo plans to participate in the following events:

Scientific / Medical Conferences

- 19th Annual WORLD*Symposium*, February 22-24, 2023. Platform presentation taking place Friday February 24, during a session from 8:00-9:00am Eastern Time and poster session 3:00-4:00pm Eastern Time (Poster Ref: 169).

Investor Conferences

- Cowen 43rd Annual Health Care Conference, March 6, 2023
- Barclays Global Healthcare Conference, March 15, 2023
- 2023 Bank of America Global Healthcare Conference, May 9-11, 2023
- 2023 RBC Global Healthcare Conference, May 16-17, 2023
- Stifel 2023 Tailoring Genes: Genetic Medicines Day, May 30, 2023

Access links for available webcasts for these investor conferences will be available on the Sangamo Therapeutics website in the Investors and Media section under Events. Available materials will be found on the Sangamo Therapeutics website after the event under Presentations.

Conference Call to Discuss Fourth Quarter and Full Year 2022 Results

The Sangamo management team will discuss these results on a conference call today, Wednesday, February 22, 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 Therapeutics 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.

Our Annual Report on Form 10-K summarizing the updated preliminary results from the Phase 1/2 STAAR clinical study in more detail will be filed by Sangamo, and this press release is subject to the further detail provided in the form 10-K.

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 and the potential for acceleration of the study timeline, updates regarding the PRECIZN-1 study, including the dosing of additional patients, the use of improved manufacturing methods and the potential impacts thereof, and plans to halt further investment in the BIVV003 and seek a potential partner for the program, the anticipated advancement of our product candidates to late-stage development, including potential future Phase 3 trials of isaralgagene civaparvovec and BIVV003, plans and timing regarding the completion of dosing in the phase 3

AFFINE trial, the availability and presentation of data from such trial, and plans for a BLA submission for giroctocogene fitelparvovec, expectations regarding advancement of our preclinical programs, including announcement of, and data from, such programs, as well as anticipated IND submissions related to such programs, our 2023 financial guidance related to GAAP and non-GAAP total operating expenses 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 to the effects of the COVID-19 pandemic and the impacts of the pandemic and other macroeconomic factors, including as a result of the ongoing conflict between Russia and Ukraine, 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 and the Phase 1/2 PRECIZN-1 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 or BIVV003, 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 technological developments that obviate technologies used by Sangamo; the potential for Sangamo to cease development of the BIVV003 program, whether due to its inability to secure a partner to advance the program or otherwise; our lack of resources to fully develop, obtain regulatory approval for and commercialize our product candidates; 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, 2021, as supplemented by our Quarterly Report on Form 10-Q for the quarter ended September 30, 2022, and future filings and reports that Sangamo makes from time to time with the SEC, including Sangamo's Annual Report on Form 10-K for the year ended December 31, 2022. 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 Measure

To supplement our financial results and guidance presented in accordance with GAAP, we present non-GAAP total operating expenses, which exclude stock-based compensation expense 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. This non-GAAP financial measure is in addition to, not a substitute for, or superior to, measures of financial performance prepared in accordance with GAAP. We encourage investors to carefully consider our results under GAAP, as well as our supplemental non-GAAP financial information, to more fully understand our business.

SELECTED CONSOLIDATED FINANCIAL DATA

(unaudited; in thousands, except per share data)

Statement of Operations Data:

	Three months ended December 31,		Year ended December 31,	
	2022	**2021**	**2022**	**2021**
Revenues	$ 27,230	$ 27,986	$ 111,299	$ 110,701
Operating expenses:	-	-	-	-
Research and development	66,179	51,801	249,898	230,819
General and administrative	16,443	16,084	62,682	63,219
Total operating expenses	82,622	67,885	312,580	294,038
Loss from operations	(55,392)	(39,899)	(201,281)	(183,337)
Interest and other income, net	3,678	2,336	9,432	5,346
Loss before income taxes	(51,714)	(37,563)	(191,849)	(177,991)
Income tax expense (benefit)	259	(67)	429	306
Net loss	$ (51,973)	$ (37,496)	$ (192,278)	$ (178,297)
Net loss attributable to non-controlling interest	-	-	-	(11)
Net loss attributable to Sangamo Therapeutics, Inc. stockholders	$ (51,973)	$ (37,496)	$ (192,278)	$ (178,286)
Basic and diluted net loss per share attributable to Sangamo Therapeutics, Inc. stockholders	$ (0.32)	$ (0.26)	$ (1.25)	$ (1.23)
Shares used in computing basic and diluted net loss per share attributable to Sangamo Therapeutics, Inc. stockholders	164,717	145,740	154,345	144,568

Selected Balance Sheet Data:

	December 31, 2022	December 31, 2021
Cash, cash equivalents, and marketable securities	$ 307,477	$ 464,717
Total assets	$ 562,509	$ 721,923
Total stockholders' equity	$ 294,958	$ 375,343

View source version on businesswire.com: https://www.businesswire.com/news/home/20230222005678/en/

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Source: Sangamo Therapeutics, Inc.