Sangamo Therapeutics Reports Fourth Quarter and Full Year 2021 Financial Results and Recent Business Highlights

February 24, 2022

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

BRISBANE, Calif.--(BUSINESS WIRE)--Feb. 24, 2022-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicines company, today reported fourth quarter and full year 2021 financial results and recent business highlights.

"In 2021, we continued to advance the development of genomic medicines for patients across multiple therapeutic areas using our innovative technologies," said Sandy Macrae, Chief Executive Officer of Sangamo. "We along with our partners presented compelling clinical data in our three lead programs and advanced development of preclinical product candidates using our second-generation technologies of CAR-Treg cell therapy for autoimmune diseases and engineered zinc finger transcription factors for neurological disorders. In addition, we completed and brought online our cell therapy manufacturing facilities in Brisbane and Valbonne and now have operational AAV and cell therapy facilities in-house. We believe this progress positions us well to generate long-term value for our shareholders."

Fourth Quarter Updates and Recent Business Highlights

Fabry disease – Updated preliminary Phase 1/2 data show continued safety, tolerability and elevated α-Gal A enzyme activity; Phase 3 planning initiated

- We presented updated preliminary clinical data from the Phase 1/2 STAAR study evaluating isaralgagene civaparvovec, or ST-920, our wholly owned gene therapy product candidate for the treatment of Fabry disease at the 18th Annual WORLD*Symposium* earlier this month. As of the November 9, 2021 cutoff date:
  - The four patients in Cohorts 1 and 2 all exhibited above normal α-Gal A activity, ranging from 3-fold to 15-fold above mean normal at last measurement.
  - The two patients in Cohort 1 maintained elevated α-Gal A activity for one year and are now in the long-term follow-up study.
  - The first patient in Cohort 3 exhibited α-Gal A activity within mean normal range by week 2.
  - Lyso-Gb3 levels remained significantly reduced in the patient who exhibited the highest baseline levels of this biomarker.
  - The gene therapy candidate continued to be generally well tolerated in the five treated patients.
- The sixth patient in the STAAR study, who is the second patient in Cohort 3, was dosed after the cutoff date. We expect to provide updated data in the second half of 2022.
- Based on the Phase 1/2 data, we have initiated Phase 3 planning.

Sickle cell disease – Updated preliminary Phase 1/2 proof-of-concept safety, tolerability and efficacy results

- We presented updated preliminary proof-of-concept clinical data from the Phase 1/2 PRECIZN-1 study of SAR445136, a zinc finger nuclease gene-edited cell therapy candidate in development with Sanofi, at the 63rd American Society for Hematology Annual Meeting and Exposition (ASH 2021). As of the September 22, 2021 cutoff date:
  - No adverse events related to SAR445136 were reported.
  - All four treated patients experienced increases in total hemoglobin, fetal hemoglobin and percent F cells.
  - None of the patients required blood transfusions post engraftment.
- We expect that the next four patients treated in the study will be dosed with a product candidate manufactured using improved methods, which have been shown in internal experiments to increase long-term progenitor cells. We expect to complete dosing of these patients in the third quarter of this year.
- We and Sanofi are collaborating on an orderly transition of Sanofi's rights and obligations under the program to Sangamo on June 28, 2022, while we explore options to advance the program, including seeking a potential new partner.

Hemophilia A – Updated Phase 1/2 results show sustained bleeding control in highest dose cohort through two years following giroctocogene fitelparvovec gene therapy

- With our collaborator Pfizer, we presented updated follow-up data from the Phase 1/2 Alta study of giroctocogene fitelparvovec, an investigational gene therapy for patients with moderately severe to severe hemophilia A, at ASH 2021. As of the October 1, 2021 cutoff date:
  - At 104 weeks, the five patients in the highest dose 3e13 vg/kg cohort had mean factor VIII (FVIII) activity of 25.4% via chromogenic clotting assay.
  - In this cohort, mean annualized bleeding rate was 0.0 in the first year post-infusion and was 1.4 throughout the

total duration of follow-up. All bleeding events occurred after week 69 post-infusion. Two patients experienced bleeding events necessitating treatment with exogenous FVIII. No participants in the highest dose cohort had resumed prophylaxis.
    - Giroctocogene fitelparvovec continued to be generally well-tolerated.
- Regarding the Phase 3 AFFINE trial of giroctocogene fitelparvovec, Pfizer has announced that it hopes to obtain agreements from health authorities to resume the AFFINE trial and to begin to reopen trial sites in the first half of 2022. This trial was previously paused when some of the patients treated in this trial experienced FVIII activity greater than 150% following treatment. Pfizer has announced that it currently is in the process of submitting a protocol amendment to health authorities in the countries where this trial is being conducted and preparing responses to the U.S. FDA clinical hold. Over 50% of the patients have been enrolled in the Phase 3 AFFINE trial.

Renal Transplant Rejection – First patient enrolled and expected to be dosed soon in Phase 1/2 study

- The first patient has been enrolled and is expected to be dosed soon in our Phase 1/2 STEADFAST study evaluating TX200, our wholly owned autologous HLA-A2 CAR Treg cell therapy product candidate treating patients receiving an HLA-A2 mismatched kidney from a living donor. We expect the second patient in this study to be dosed by the middle of 2022. We continue to open study sites and screen patients.

Manufacturing – AAV and cell therapy cGMP manufacturing facilities fully operational

- We completed and brought online our in-house cell therapy manufacturing facilities in our Brisbane, California headquarters and in our Valbonne, France facilities in 2021, in addition to the in-house AAV manufacturing facilities we brought online in Brisbane in 2020.

Fourth Quarter and Full Year 2021 Financial Results

Consolidated net loss for the fourth quarter ended December 31, 2021 was $37.5 million, or $0.26 per share, compared to net loss of $40.7 million, or $0.29 per share, for the same period in 2020. For the year ended December 31, 2021, consolidated net loss was $178.3 million, or $1.23 per share, compared to consolidated net loss of $121.1 million, or $0.90 per share, for the year ended December 31, 2020.

Revenues

Revenues for the fourth quarter ended December 31, 2021 were $28.0 million, compared to $25.8 million for the same period in 2020.

The increase of $2.2 million in revenues was primarily due to increase in recognition of upfront license fees and research revenue of $3.7 million under our collaboration agreement with Novartis. The increase was partially offset by a decrease of $1.4 million in revenue related to the termination of our licensing agreement with Dow AgroSciences LLC in July 2021.

Revenues were $110.7 million in 2021, compared to $118.2 million in 2020. The decrease in revenues was primarily due to a decrease of $47.4 million of milestone fees and recognition of upfront license fees related to our giroctocogene fitelparvovec and C9ORF72 collaboration agreements with Pfizer resulting from the completion of our activities in 2020 and a decrease of $5.4 million from our collaboration agreements with Kite and Sanofi. These decreases were partially offset by higher revenues of $32.7 million and $14.4 million related to our collaboration agreements with Novartis and Biogen, respectively.

GAAP and Non-GAAP operating expenses

(In millions)	Three Months Ended December 31,		Year Ended December 31,	
	2021	2020	2021	2020
Research and development	$ 51.8	$ 52.4	$ 230.8	$ 180.6
General and administrative	16.1	16.8	63.2	67.1
Total operating expenses	67.9	69.2	294.0	247.7
Stock-based compensation expense	(8.1)	(6.6)	(33.0)	(25.7)
Non-GAAP operating expenses	$ 59.8	$ 62.6	$ 261.0	$ 222.0

Total operating expenses on a GAAP basis for the fourth quarter ended December 31, 2021 were $67.9 million, compared to $69.2 million for the same period in 2020. Non-GAAP operating expenses, which exclude stock-based compensation expense, for the fourth quarter ended December 31, 2021 were $59.8 million, compared to $62.6 million for the same period in 2020.

The decrease in total operating expenses in the fourth quarter on a GAAP basis was primarily due to a reduction of research and development expenses by $5.2 million related to dissolution of the repayment obligation of a grant from California Institute for Regenerative Medicine associated with the discontinuation of the ST-400 program. This decrease was partially offset by an increase of $4.6 million in research and development expenses due to increased headcount to support the advancement of our clinical trials and our ongoing collaborations, and an increase in manufacturing and overhead costs as we ramped up our internal manufacturing operations.

Total operating expenses on a GAAP basis in 2021 were $294.0 million compared to $247.7 million in 2020. Non-GAAP operating expenses, which exclude stock-based compensation expense, were $261.0 million and $222.0 million in 2021 and 2020, respectively.

The increase in total operating expenses in the full year on a GAAP basis was primarily driven by our higher clinical and manufacturing supply

expenses along with our increased headcount to support the advancement of our clinical trials and our ongoing collaborations and an increase in manufacturing and overhead costs as we ramped up our internal manufacturing operations.

Cash, cash equivalents and marketable securities

Cash, cash equivalents and marketable securities as of December 31, 2021 were $464.7 million, compared to $692.0 million as of December 31, 2020.

Initial Financial Guidance for 2022

On a GAAP basis, we expect total operating expenses in the range of approximately $320 million to $350 million in 2022, 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 $40 million, in the range of approximately $280 million to $310 million in 2022.

Conference Call

Sangamo will host a conference call today, February 24, 2022, at 4:30 p.m. Eastern Time, which will be open to the public. The call will also be webcast with live Q&A and can be accessed via a link on the Sangamo Therapeutics website in the Investors and Media section under Events and Presentations.

The conference call dial-in numbers are (877) 377-7553 for domestic callers and (678) 894-3968 for international callers. The conference ID number for the call is 2235808. Participants may access the live webcast via a link on the Sangamo Therapeutics website in the Investors and Media section under Events and Presentations. A conference call replay will be available for one week following the conference call. The conference call replay numbers for domestic and international callers are (855) 859-2056 and (404) 537-3406, respectively. The conference ID number for the replay is 2235808.

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. For more information about Sangamo, visit www.sangamo.com.

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 the presentation of updated clinical data from and the selection of a dose for cohort expansion in the Phase 1/2 STAAR study, the use of improved manufacturing methods in the PRECIZN-1 study and the potential impacts thereof, as well as the expectations for completion of dosing in such study, plans and timing for the transition of the SAR445136 program from Sanofi to Sangamo, Sangamo's plans to explore options to advance the SAR445136 program, including a potential new collaboration partner, the expected timeline for dosing patients in the STEADFAST study, the anticipated advancement of our product candidates to late-stage development including potential future Phase 3 trials, plans and timing regarding the ability to obtain approval from health authorities to resume the Phase 3 AFFINE trial and to begin to reopen trial sites, and expectations regarding the patient population and analysis represented in, and the anticipated timing of, data readouts for the Phase 3 AFFINE trial; our initial 2022 financial guidance related to GAAP and non-GAAP total operating expenses and stock-based compensation; 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 evolving COVID-19 pandemic and the impacts of the pandemic 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 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 updated 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 SAR445136; the unpredictable regulatory approval process for product candidates across multiple regulatory authorities, including the potential that health authorities will not permit the resumption of the Phase 3 AFFINE trial in a timely manner, or at all; 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 isaralgagene civaparvovec; 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 Sanofi and Sangamo to fail to come to agreement on appropriate transition agreements or to execute an orderly transition under their collaboration agreement; the potential that Sangamo will not be able to identify and secure options or new collaborators for the SAR445136 program; the potential for Sangamo to cease development of the SAR445136 program, whether due to its inability to secure options 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 filings and reports, including in our Annual Report on Form 10-K for the year ended December 31, 2021. 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,	
	2021	**2020**	**2021**	**2020**
Revenues	$ 27,986	$ 25,800	$ 110,701	$ 118,192
Operating expenses:				
Research and development	51,801	52,358	230,819	180,647
General and administrative	16,084	16,874	63,219	67,097
Total operating expenses	67,885	69,232	294,038	247,744
Loss from operations	(39,899)	(43,432)	(183,337)	(129,552)
Interest and other income, net	2,336	2,865	5,346	8,775
Loss before income taxes	(37,563)	(40,567)	(177,991)	(120,777)
Income tax (benefit) expense	(67)	108	306	345
Net loss	$ (37,496)	$ (40,675)	$ (178,297)	$ (121,122)
Net loss attributable to non-controlling interest	-	(71)	(11)	(126)
Net loss attributable to Sangamo Therapeutics, Inc. stockholders	$ (37,496)	$ (40,604)	$ (178,286)	$ (120,996)
Basic and diluted net loss per share attributable to Sangamo Therapeutics, Inc. stockholders	$ (0.26)	$ (0.29)	$ (1.23)	$ (0.90)
Shares used in computing basic and diluted net loss per share attributable to Sangamo Therapeutics, Inc. stockholders	145,740	141,508	144,568	134,449

Selected Balance Sheet Data:

	December 31, 2021	**December 31, 2020**
Cash, cash equivalents, and marketable securities	$ 464,717	$ 691,953
Total assets	$ 721,923	$ 938,550
Total stockholders' equity	$ 375,343	$ 497,366

View source version on businesswire.com: https://www.businesswire.com/news/home/20220224005163/en/

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