



## Sangamo Therapeutics Reports Recent Business and Clinical Highlights and Third Quarter 2021 Financial Results

November 4, 2021

*Conference Call and Webcast Scheduled for 9:15 a.m. Eastern Time*

BRISBANE, Calif.--(BUSINESS WIRE)--Nov. 4, 2021-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today reported third quarter financial results and provided business and clinical highlights.

"We are delighted to share clinical data and business updates across several programs demonstrating that Sangamo has three important assets progressing toward late-stage development. Our gene therapy portfolio is advancing with accumulating safety and efficacy data in our Fabry and hemophilia A programs, and preliminary proof-of-concept data demonstrate the clinical potential of our zinc finger genome engineering technology in sickle cell disease. These data readouts show the progression of our first-generation genomic medicine pipeline and potentially pave the way for new treatments. Our next generation programs focus on genome regulation and allogeneic CAR-Treg cell therapy, where we have a robust preclinical pipeline in neurological and autoimmune diseases. We are energized by this momentum and look forward to continued execution of our corporate strategy," said Sandy Macrae, Chief Executive Officer of Sangamo.

### Recent Clinical and Business Highlights

*Fabry Disease – First four patients dosed exhibited above normal  $\alpha$ -Gal A activity; Phase 3 planning initiated*

- Earlier today, we announced preliminary clinical data from the first four patients treated in our Phase 1/2 STAAR study evaluating isaralgagene civaparvovec, or ST-920, our wholly owned Fabry disease gene therapy product candidate. Data as of the September 17, 2021 cutoff date from the four patients in the first two dose cohorts showed that isaralgagene civaparvovec was generally well tolerated. All four patients exhibited above normal alpha-galactosidase A ( $\alpha$ -Gal A) activity, which was maintained for up to one year for the first patient treated and through 14 weeks for the most recently treated patient. Activity of 2-fold to 15-fold above mean normal was observed at last measurement as of the cutoff date. Withdrawal from enzyme replacement therapy (ERT) has taken place for one patient and is planned for the other patient on ERT, based on the stability of their  $\alpha$ -Gal A activity following treatment.
- The fifth patient in the STAAR study, who is the first patient in the third cohort (3e13vg/kg), was dosed after the cutoff date. The sixth patient is currently in screening also for the third dose cohort. We expect to provide updated data throughout 2022 and present these results at a medical meeting.
- Based on the STAAR study results to date, we have initiated planning for a Phase 3 Fabry disease clinical trial.

*Sickle Cell Disease – Preliminary-proof-of-concept data will be presented at ASH as clinical program advances*

- Preliminary proof-of-concept results from the Phase 1/2 PRECIZN-1 study investigating SAR445136, formerly BIVV003, an investigational zinc finger nuclease gene edited cell therapy, in patients with severe sickle cell disease (SCD) will be presented at the 63rd Annual Meeting of the American Society of Hematology (ASH) on December 12, 2021. Results as of the June 25, 2021 cutoff date show that all four treated patients did not require blood transfusions post engraftment and had no adverse or serious adverse events related to SAR445136 through 65 weeks of follow-up for the longest treated patient. The four treated patients all experienced increases in total hemoglobin, fetal hemoglobin and percent F cells.
- We and Sanofi continue to advance the sickle cell disease program. We recently obtained manufacturing requirements guidance from FDA in preparation for further potential clinical studies. Separately, we and Sanofi made the business decision to cease development of the beta thalassemia indication in order to focus resources on the sickle cell disease program. ST-400 for beta thalassemia was developed with the support of a grant from the California Institute for Regenerative Medicine (CIRM).

*Hemophilia A – Four patients at highest dose experienced mean FVIII activity of 30.9% at week 104*

- Updated follow-up results from the Phase 1/2 Alta study of giroctocogene fitelparvovec gene therapy in patients with severe hemophilia A will be presented at ASH on December 12, 2021. For the four patients in the highest dose 3e13vg/kg cohort who have reached 104 weeks of follow-up as of the May 19, 2021 cutoff date, mean Factor VIII (FVIII) activity was 30.9% at week 104 as measured by chromogenic assay. In this cohort, the annualized bleeding rate was zero for the first year after treatment and 0.9 throughout total duration of follow-up. Giroctocogene fitelparvovec was generally well tolerated.
- We and Pfizer also announced that some of the patients treated in the Phase 3 AFFINE trial of giroctocogene fitelparvovec experienced FVIII activity greater than 150% following treatment. None of these patients have experienced thrombotic events and some have been treated with direct oral anticoagulants to reduce thrombotic risk. Pfizer voluntarily paused

screening and dosing of additional patients in the trial to implement a protocol amendment intending to provide guidance regarding the management of patients with FVIII levels that exceed 150%. On November 3, 2021, Pfizer was informed that the FDA has put this trial on clinical hold. The next step is to share the proposed protocol amendment with health authorities and respond to the clinical hold, after which the Companies will be able to provide updated timing for the trial.

*Renal Transplant – First patient enrolled, expect two patients to be dosed by mid-2022*

- The first patient has been enrolled 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 first two patients in this study to be dosed by the middle of 2022 following kidney transplantation. We continue to open study sites and screen patients.

*Research, Manufacturing, and Corporate Updates*

- Biogen announced type 1 myotonic dystrophy (DM1) as the previously undisclosed neuromuscular preclinical target in our collaboration.
- We recently completed and brought online our in-house cell therapy manufacturing facility in our Brisbane, California headquarters and remain on track to complete our in-house cell therapy manufacturing facility in Valbonne, France by year-end.
- We appointed D. Mark McClung as Chief Operating Officer, an important organizational step to support the multiple advancing wholly owned and partnered programs.

### Third Quarter 2021 Financial Results

Consolidated net loss attributable to Sangamo for the third quarter ended September 30, 2021 was \$47.7 million, or \$0.33 per share, compared to a net loss attributable to Sangamo of \$1.6 million, or \$0.01 per share, for the same period in 2020.

#### *Revenues*

Revenues for the third quarter ended September 30, 2021, were \$28.6 million, compared to \$57.8 million for the same period in 2020, a decrease of \$29.2 million.

The reduction in revenue was primarily due to a \$39.3 million decrease related to our giroctocogene fitelparvovec and C9ORF72 collaboration agreements with Pfizer, resulting from the completion of our activities in 2020, and a \$2.3 million decrease related to our collaboration agreement with Sanofi. These decreases were partially offset by higher revenues of \$11.5 million and \$1.3 million related to our collaboration agreements with Novartis and Biogen, respectively.

#### *GAAP and Non-GAAP operating expenses*

(In millions)	Three Months Ended		Nine Months Ended	
	September 30, 2021	2020	September 30, 2021	2020
Research and development	\$ 62.5	\$ 45.3	\$ 179.0	\$ 128.3
General and administrative	14.5	16.2	47.1	50.2
Total operating expenses	77.0	61.5	226.1	178.5
Stock-based compensation expense	(7.9)	(6.7)	(24.9)	(19.1)
Non-GAAP operating expenses	\$ 69.1	\$ 54.8	\$ 201.2	\$ 159.4

Total operating expenses on a GAAP basis for the third quarter ended September 30, 2021 were \$77.0 million compared to \$61.5 million for the same period in 2020. Non-GAAP operating expenses, which exclude stock-based compensation expense, for the third quarter ended September 30, 2021 were \$69.1 million compared to \$54.8 million for the same period in 2020.

The increase in total operating expenses 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.

#### *Cash, cash equivalents and marketable securities*

Cash, cash equivalents and marketable securities as of September 30, 2021 were \$519.0 million compared to \$692.0 million as of December 31, 2020.

#### *Revised Financial Guidance for 2021*

We are revising our full-year operating expense guidance initially provided on February 24, 2021 and reiterated most recently on August 5, 2021 as follows:

	Initially Provided February 24, 2021;	
	Reiterated May 4, 2021	
(in millions)	and August 5, 2021	Updated on November 4, 2021

Estimated GAAP Operating Expenses	\$285 to \$305	\$300 to \$310
Estimated Non-GAAP Operating Expenses	\$255 to \$275*	\$265 to \$275**
*excludes estimated stock-based compensation of \$30 million		
**excludes estimated stock-based compensation of \$35 million		

## Conference Call

Sangamo will host a conference call today, November 4, 2021, at 9:15 a.m. Eastern Time, which will be open to the public. The call and live Q&A will be webcast.

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 5178059. Participants may access the live webcast via a link on the Sangamo Therapeutics website in the Investors and Media section under [Events and Presentations](#). 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 5178059.

## 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](http://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, the anticipated advancement of our product candidates to late-stage development including potential future Phase 3 trials, anticipated implementation of a protocol amendment for the Phase 3 AFFINE clinical trial of giroctocogene fitelparvovec and the resumption of the dosing of additional patients in the trial; our revised 2021 financial guidance related to GAAP and non-GAAP total operating expenses and stock-based compensation; our continued execution of our corporate strategy; the anticipated completion of our in-house cell therapy manufacturing facility in Valbonne, France; 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 trials and clinical trial results, including the risk that any protocol amendment for the Phase 3 AFFINE trial of giroctocogene fitelparvovec may not be accepted by the relevant review bodies in a timely manner, or at all, or that the FDA may not lift its clinical hold on the Phase 3 AFFINE trial in a timely manner, or at all, each of which could further delay or preclude further patient dosing in the trial as well as the risks that therapeutic effects observed in clinical trial results will not be durable in patients and that final clinical trial data will not validate the safety and efficacy of our product candidates; reliance on results of early clinical trials, which results are not necessarily predictive of future clinical trial results; 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; 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, 2020 as supplemented by our Quarterly Report on Form 10-Q for the quarter ended September 30, 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		Nine Months Ended	
September 30,		September 30,	
2021	2020	2021	2020

Revenues	\$	28,563	\$	57,763	\$	82,715	\$	92,392
Operating expenses:								
Research and development		62,498		45,287		179,018		128,289
General and administrative		14,501		16,177		47,135		50,223
Total operating expenses		<u>76,999</u>		<u>61,464</u>		<u>226,153</u>		<u>178,512</u>
Loss from operations		(48,436)		(3,701)		(143,438)		(86,120)
Interest and other income, net		834		2,430		3,010		5,910
Loss before taxes		(47,602)		(1,271)		(140,428)		(80,210)
Income tax expense		86		237		373		237
Net loss	\$	(47,688)	\$	(1,508)	\$	(140,801)	\$	(80,447)
Net income (loss) attributable to non-controlling interest		-		42		(11)		(55)
Net loss attributable to Sangamo Therapeutics, Inc. stockholders	\$	<u>(47,688)</u>	\$	<u>(1,550)</u>	\$	<u>(140,790)</u>	\$	<u>(80,392)</u>
Basic and diluted net loss per share attributable to Sangamo Therapeutics, Inc. stockholders	\$	<u>(0.33)</u>	\$	<u>(0.01)</u>	\$	<u>(0.98)</u>	\$	<u>(0.61)</u>
Shares used in computing basic and diluted net loss per share attributable to Sangamo Therapeutics, Inc. stockholders		<u>145,399</u>		<u>141,100</u>		<u>144,173</u>		<u>132,079</u>

**Selected Balance Sheet Data:**

	<u>September</u>	<u>December 31,</u>
	<u>30, 2021</u>	<u>2020</u>
Cash, cash equivalents and marketable securities	\$ 519,044	\$ 691,953
Total assets	\$ 772,398	\$ 938,550
Total stockholders' equity	\$ 406,016	\$ 497,366

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