# Sangamo Therapeutics Reports First Quarter 2017 Financial Results

May 10, 2017

RICHMOND, Calif., May 10, 2017 /PRNewswire/ --

Company Also Separately Announced Strategic Collaboration with Pfizer for Hemophilia A Phase 1/2 Gene Therapy Program

Conference Call and Webcast at 5:00 p.m. Eastern Time Today Will Discuss Structure and Terms of Collaboration with Pfizer



Sangamo Therapeutics, Inc. (NASDAQ: SGMO), the leader in therapeutic genome editing, today reported its first quarter 2017 financial results and recent accomplishments.

### **Recent Accomplishments**

#### Clinical

- Phase 1/2 clinical trials of three lead programs are now open for enrollment
  - o In vivo genome editing treatment SB-318 for Mucopolysaccharidosis (MPS) I
  - o In vivo genome editing treatment SB-913 for MPS II
  - o In vivo genome editing treatment SB-FIX for Hemophilia B
  - Phase 1/2 clinical trial of fourth lead program, SB-525 cDNA gene therapy, for Hemophilia A to open for enrollment this quarter

### Regulatory

- Received Fast Track designation from the U.S. Food and Drug Administration (FDA) for *in vivo* genome editing treatment SB-FIX for Hemophilia B. Sangamo's SB-FIX program has also previously received Orphan Drug Designation.
- Received Orphan Drug Designation (ODD) for SB-525 cDNA gene therapy for Hemophilia A. ODD is granted to drugs and biologics intended to treat rare diseases with a patient population less than 200,000 in the U.S. The program provides incentives to advance development and commercialization of rare disease drugs.
- Received Rare Pediatric Disease (RPD) Designation for *in vivo* genome editing treatment SB-318 for MPS I. Under the RPD program, a sponsor who receives approval for a new drug application or biologics license application may be eligible to receive a voucher for a priority review of a subsequent marketing application for a different product. The voucher may be used by the sponsor or sold or transferred. SB-318 also previously received ODD.
- Received ODD and RPD Designation for in vivo genome editing treatment SB-913 for MPS II

#### Research

- New human *in vitro* and animal model data demonstrating significant reduction of tau mRNA and tau protein expression using zinc finger protein transcription factor (ZFP-TF)-mediated gene regulation technology were presented at the 13<sup>th</sup> International Conference on Alzheimer's & Parkinson's Diseases.
- Sangamo scientists or collaborators will deliver ten oral and nine poster presentations during the 20<sup>th</sup>Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT) being held this week in Washington, D.C.

"Our team continues to make significant operational progress across all four lead clinical programs. Three Phase 1/2 *in vivo* genome editing clinical trials are now screening subjects, and the fourth trial evaluating SB-525, a cDNA gene therapy approach for Hemophilia A, will open later this quarter," said Dr. Sandy Macrae, CEO of Sangamo. "We are initiating multiple sites for each program over the next few months to ensure we can accrue patients rapidly into these studies. We remain on course and expect to announce data from each program late this year or in early 2018."

### First Quarter 2017 Financial Results

For the first quarter ended March 31, 2017, Sangamo reported a consolidated net loss of \$16.6 million, or \$0.23 per share, compared to a net loss of \$16.5 million, or \$0.23 per share, for the same period in 2016. As of March 31, 2017, the Company had cash, cash equivalents, marketable securities and interest receivable of \$132.7 million.

Revenues for the first quarter of 2017 were \$3.4 million, compared to \$3.9 million for the same period in 2016. First quarter 2017 revenues were generated from Sangamo's collaboration agreements with Bioverativ, Shire International (Shire) and Sigma-Aldrich, enabling technology agreements and research grants. The revenues recognized for the first quarter of 2017 consisted of \$3.3 million in collaboration agreements and approximately \$0.1 million in research grants, compared to \$3.7 million and approximately \$0.2 million, respectively, for the same period in 2016.

In the first quarter of 2017, Sangamo recognized \$1.7 million of revenues related to research services performed under the collaboration agreement with Bioverativ, and \$0.1 million of revenues related to research services performed under the collaboration agreement with Shire. Sangamo received upfront payments of \$13.0 million and \$20.0 million pursuant to the agreements entered into with Shire in 2012 and Biogen (the predecessor of Bioverativ) in 2014, respectively. The Shire payment is being recognized as revenue on a straight-line basis through approximately December 2017. Beginning in January 2017, the Biogen agreement was transferred to Bioverativ, and the remaining upfront payment is being recognized through approximately June 2020. The Company recognized \$0.6 million of the Shire upfront payment and \$0.4 million of the Bioverativ upfront payment as revenue during the first quarter of 2017.

Research and development expenses were \$12.9 million for the first quarter of 2017, compared to \$15.3 million for the same period in 2016. The decrease was primarily due to the completion of external GMP manufacturing expenses associated with the Company's 2017 clinical studies. General and administrative expenses were \$7.3 million for the first quarter of 2017, compared to \$5.4 million for the same period in 2016.

Total operating expenses for the first quarter of 2017 were \$20.2 million, compared to \$20.6 million for the same period in 2016.

#### **Conference Call**

Sangamo will host a conference call today, May 10, 2017, at 5:00 p.m. ET, which will be open to the public. The call will also be webcast live and can be accessed via a link on the Sangamo Therapeutics website in the Investors and Media section under Events and Presentations. A replay of the webcast will also be available for one week after the call. During the conference call, the Company will review these results, discuss other business matters and provide guidance with respect to 2017.

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 15225000. For those unable to listen in at the designated time, a conference call replay will be available for one week following the conference call, from approximately 8:00 p.m. ET on May 10, 2017 to 11:59 p.m. ET on May 17, 2017. 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 15225000.

### **About Sangamo**

Sangamo Therapeutics is focused on translating ground-breaking science into genomic therapies that transform patients' lives using the company's industry leading platform technologies in genome editing, gene therapy, gene regulation and cell therapy. The Company is advancing Phase 1/2 clinical programs in hemophilia A and hemophilia B, and lysosomal storage disorders MPS I and MPS II. Sangamo is engaged in strategic collaborations with several partners, including Bioverativ for hemoglobinopathies, such as beta thalassemia and sickle cell disease, and with Shire International to develop therapeutics for Huntington's disease. In addition, strategic partnerships have been established with companies for non-therapeutic applications of its technology, including Sigma-Aldrich Corporation and Dow AgroSciences. For more information about Sangamo, visit www.sangamo.com.

This press release contains forward-looking statements regarding Sangamo's current expectations. These forward looking statements include, without limitation, references to the collaboration agreement with Pfizer, expected timing of initiating clinical trials and program sites, presentation of clinical trial data and filling of INDs, the expected accomplishment in 2017, anticipated cash and investment balance, operating expenses, revenue and potential milestone and royalty payments under Sangamo's agreements with Shire and Bioverativ, and the research and development of ZFNs and ZFP TFs, clinical trials and therapeutic applications of Sangamo's ZFP technology. These statements are not guarantees of future performance and are subject to certain risks, uncertainties and assumptions that are difficult to predict. Factors that could cause actual results to differ include, but are not limited to, the dependence on the success of clinical trials of lead programs, the lengthy and uncertain regulatory approval process, uncertainties related to the timing of initiation and completion of clinical trials, whether clinical trial results will validate and support the safety and efficacy of Sangamo's therapeutics, and the ability to establish strategic partnerships. Further, there can be no assurance that the necessary regulatory approvals will be obtained or that Sangamo and its partners will be able to develop commercially viable gene-based therapeutics. Actual results may differ from those projected in forward-looking statements due to risks and uncertainties that exist in Sangamo's operations and business environments. These risks and uncertainties are described more fully in Sangamo's Annual Reports on Form 10-K and Quarterly Reports on Form 10-Q as filed with the Securities and Exchange Commission. 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.

## UNAUDITED SELECTED CONSOLIDATED FINANCIAL DATA

(UNAUDITED; IN THOUSANDS, EXCEPT PER SHARE DATA)

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Statement of Operations Data:	
	Three Months Ended
	March 31,
	2017 2016
Revenues:	
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Research grants	119		231	
Total revenues	3,425		3,942	
Operating expenses:				
Research and development	12,942		15,266	
General and administrative	7,275		5,357	
Total operating expenses	20,217		20,623	
Loss from operations	(16,792)		(16,681)	
Interest and other income, net	160		187	
Net loss	\$	(16,632)	\$	(16,494)
Basic and diluted net loss per common share	\$	(0.23)	\$	(0.23)

Shares used in computing basic and diluted net loss per common share 71,025

71,025 70,373

## March 31, 2017 December 31, 2016

## SELECTED BALANCE SHEET DATA

Cash, cash equivalents, marketable securities and interest receivable	\$	132,659	\$	142,759
Total assets	146,810		157,891	
Total stockholders' equity	125,6	683	136,195	

To view the original version on PR Newswire, visit: <a href="http://www.prnewswire.com/news-releases/sangamo-therapeutics-reports-first-quarter-2017-financial-results-300455560.html">http://www.prnewswire.com/news-releases/sangamo-therapeutics-reports-first-quarter-2017-financial-results-300455560.html</a>

SOURCE Sangamo Therapeutics, Inc.

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