Sangamo Therapeutics Logo

Sangamo BioSciences Reports Third Quarter 2016 Financial Results

October 26, 2016

Company Highlights Progress of Key Clinical Programs in Hemophilia and Lysosomal Storage Disorders

RICHMOND, Calif., Oct. 26, 2016 /PRNewswire/ -- Sangamo BioSciences, Inc. (NASDAQ: SGMO), the leader in therapeutic genome editing, today reported its third quarter 2016 financial results and provided an update on recent events and development timelines for its therapeutic programs.

"The third quarter of 2016 has been a pivotal time for Sangamo, as we worked to focus our efforts and execute on our prioritized therapeutic programs in hemophilia B, hemophilia A, MPS I and MPS II," said Sandy Macrae, M.B., Ch.B., Ph.D., Sangamo's president and chief executive officer. "I am pleased to announce that the Phase 1/2 clinical trial for SB-FIX, our *in vivo* genome editing program for hemophilia B, is open. We are also on track to file an IND application for our AAV cDNA Factor 8 gene therapy program for hemophilia A by the end of this year. In addition, we submitted the additional data package for our MPS I and MPS II programs to the FDA in September, and I am pleased to report that the FDA has cleared these programs for clinical development. Preparations are now underway to initiate Phase 1/2 clinical trials for these indications in early 2017."

Dr. Macrae continued, "We also made a number of organizational changes, including several key hires in our clinical and technical operations teams and instituted new procedures in order to position the company for clinical success. I am very encouraged by the commitment of our entire team and the progress we have made in the third quarter to drive these activities forward. I remain confident that we can demonstrate the value and therapeutic potential of our genome editing and gene therapy platforms and with reliable steps, make sensible progress and realize our vision of transforming Sangamo into a patient-focused therapeutics company."

Recent Highlights

- **Initiation of FIXtendz (SB-FIX-1501) Phase 1/2 clinical trial designed to assess safety, tolerability and potential efficacy of SB-FIX in adults with hemophilia B.** In October, Sangamo opened the first clinical study of an *in vivo* genome editing therapeutic, its Phase 1/2 clinical trial (FIXtendz, SB-FIX-1501). SB-FIX-1501 is an open-label, dose-escalation study in male subjects over eighteen years of age, with severe hemophilia B, who do not have inhibitors or hypersensitivity to recombinant Factor IX protein (rFIX). The study will enroll up to nine subjects in three dosing cohorts of two subjects per cohort, with additional subjects to be enrolled at the optimal therapeutic dose, and will evaluate the safety and potential efficacy of a single administration of SB-FIX.
- **U.S. Food and Drug Administration (FDA) grants orphan drug designation to SB-FIX, the first *in vivo* genome editing therapeutic in development.** In September, Sangamo announced that the FDA granted Orphan Drug Designation (ODD) to SB-FIX, the company's zinc finger nuclease (ZFN)-mediated *in vivo* genome editing therapeutic candidate for hemophilia B. Orphan drug designation is granted to investigational drugs and biologics that are intended to treat rare diseases that affect fewer than 200,000 people in the U.S. This designation helps facilitate drug development by providing several benefits to drug developers, including assistance with clinical study design and drug development, tax credits for qualified clinical trial costs, exemption from certain FDA application fees and seven years of market exclusivity upon regulatory product approval.
- **FDA clearance to initiate Phase 1/2 clinical trials for SB-318 (MPS I) and SB-913 (MPS II) therapeutic programs.** Sangamo submitted the additional *in vitro* studies requested by the FDA in September and recently received clearance to initiate Phase 1/2 clinical trials for the Mucopolysaccharidosis Type I (MPS I, Hurler syndrome) and Mucopolysaccharidosis Type II (MPS II, Hunter syndrome) programs based on its ZFN-mediated *in vivo* genome editing therapeutic platform. The company expects to initiate the clinical studies in early 2017.
- **Appointment of new head of technical operations.** In August, Sangamo appointed Mohammad El-Kalay, Ph.D., as Vice President, Technical Operations. Dr. El-Kalay brings over 25 years of operational management experience in the life sciences field, including expertise in process development and cGMP manufacturing operations at clinical scale with hematopoietic stem cells, T-cells and various other cell types. Dr. El-Kalay is responsible for process development and manufacturing of all biotherapeutics for Sangamo.

Third Quarter 2016 Results

For the third quarter ended September 30, 2016, Sangamo reported a consolidated net loss of $19.0 million, or $0.27 per share, compared to a net loss of $9.2 million, or $0.13 per share, for the same period in 2015. As of September 30, 2016, the Company had cash, cash equivalents, marketable securities and interest receivable of $155.4 million.

Revenues for the third quarter of 2016 were $2.8 million, compared to $8.6 million for the same period in 2015. Third quarter 2016 revenues were generated from the Company's collaboration agreements with Biogen and Shire International GmbH (Shire), enabling technology agreements and

research grants. The revenues recognized for the third quarter of 2016 consisted of $2.7 million from collaboration agreements and $0.1 million from research grants, compared to $8.4 million and $0.2 million, respectively, for the same period in 2015. The decrease in collaboration agreement revenues was a result of an amendment to the Company's collaboration and license agreement with Shire in the third quarter of 2015, which returned the rights to the hemophilia programs to Sangamo, as well as a decrease in revenues from the Biogen agreement as the initial research phase of these programs has matured and activities during this quarter were largely internal.

In the third quarter of 2016, Sangamo recognized $1.2 million of revenues related to research services performed under the collaboration agreement with Biogen, and $0.2 million of revenues related to research services performed under the collaboration agreement with Shire. In addition, Sangamo received upfront payments of $13.0 million and $20.0 million pursuant to the agreements entered into with Shire in 2012 and Biogen in 2014, respectively. The Shire payment is being recognized as revenue on a straight-line basis over the initial six-year research term. Beginning in January 2016, the Biogen payment is being recognized over approximately 42 months which reflects the revised service period related to Sangamo's deliverables under the Biogen agreement. The Company recognized $0.5 million of the Shire upfront payment and $0.6 million of the Biogen upfront payment as revenue for the third quarter of 2016.

Research and development expenses were $17.0 million for the third quarter of 2016, compared to $16.7 million for the same period in 2015. General and administrative expenses were $5.0 million for the third quarter of 2016, compared to $4.6 million for the same period in 2015.

Total operating expenses for the third quarter of 2016 were $22.0 million, compared to $21.3 million for the same period in 2015.

Nine Months Results
For the nine months ended September 30, 2016, the consolidated net loss was $62.0 million, or $0.88 per share, compared to a consolidated net loss of $26.7 million, or $0.38 per share, for the nine months ended September 30, 2015. Revenues were $10.5 million for the nine months ended September 30, 2016, compared to $30.4 million for the same period in 2015. The decrease in revenues was primarily related to the amendment of our collaboration and license agreement with Shire, as well as a decrease in revenues related to our agreements with Sigma and Biogen. Total operating expenses were $73.2 million for the nine months ended September 30, 2016, compared to $61.6 million for the same period in 2015 and reflect increased expenses related to salaries and benefits, including stock-based compensation expense, as well as professional fees, consulting services and other corporate costs.

Financial Guidance for 2016
The Company reiterates guidance as follows:

- **Cash and Investments:** Sangamo expects that its cash, cash equivalents and marketable securities will be at least $140 million at the end of 2016, inclusive of research funding from existing collaborators but exclusive of funds arising from any additional new collaborations or partnerships, equity financings or other new sources.
- **Revenues:** Sangamo expects that revenues will be in the range of $12 million to $17 million in 2016, inclusive of research funding from existing collaborations.
- **Operating Expenses:** Sangamo expects that operating expenses will be in the range of $85 million to $95 million for 2016.

Conference Call
Sangamo will host a conference call today, October 26, 2016, at 5:00 p.m. ET, which will be open to the public. The call will be webcast live and can be accessed via a link on the Sangamo BioSciences website in the Investor Relations section under Events and Presentations. A replay of the webcast will be available for two weeks after the call. During the conference call, the Company will review these results, discuss other business matters and provide guidance with respect to the remainder of 2016.

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 96289087. A conference call replay will be available for one week following the conference call, from approximately 8:00 p.m. ET on October 26, 2016 to 11:59 p.m. ET on November 2, 2016. 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 96289087.

About Sangamo
Sangamo BioSciences, Inc. is focused on Engineering Genetic Cures® for monogenic and infectious diseases by deploying its novel zinc finger DNA-binding protein technology, in therapeutic genome editing and gene regulation, and AAV-based gene therapy platforms. The Company's proprietary ZFN-mediated *in vivo* genome editing approach is focused on monogenic diseases, including hemophilia and lysosomal storage disorders, including MPS I and MPS II. Sangamo has initiated a Phase 1/2 clinical trial for hemophilia B, the first *in vivo* genome editing application cleared by the FDA. In addition, Sangamo has Phase 1/2 and Phase 2 clinical programs in HIV/AIDS (SB-728). The Company has also formed a strategic collaboration with Biogen Inc. for hemoglobinopathies, including sickle cell disease and beta-thalassemia, and with Shire International GmbH to develop therapeutics for Huntington's disease. It has established strategic partnerships with companies in non-therapeutic applications of its technology, including Dow AgroSciences and Sigma-Aldrich Corporation. For more information about Sangamo, visit the Company's website at www.sangamo.com.

ZFP Therapeutic® is a registered trademark of Sangamo BioSciences, Inc.

This press release contains forward-looking statements regarding Sangamo's current expectations. These forward looking statements include, without limitation, references to expected timing of initiating clinical trials, presentation of clinical trial data and submission of INDs, anticipated cash and investment balance, operating expenses, revenue and achievement of milestone and royalty payments under Sangamo's collaboration agreements, changes in internal organization, the research and development of ZFNs and ZFP TFs, and the focus on clinical trials and therapeutic applications of Sangamo's gene therapy and ZFP technology platforms. These statements are not guarantees of future performance and success 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 early stage of ZFP Therapeutic development, 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 ZFP Therapeutics, and the ability to maintain and 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.

SELECTED CONSOLIDATED FINANCIAL DATA

(unaudited; in thousands, except per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Statement of Operations Data:				
Revenues:				
Collaboration agreements	$ 2,728	$ 8,406	$ 10,031	$ 28,878
Research grants	95	163	436	1,540
Total revenues	2,823	8,569	10,467	30,418
Operating expenses:				
Research and development	17,008	16,694	51,728	47,292
General and administrative	5,021	4,560	21,468	14,309
Total operating expenses	22,029	21,254	73,196	61,601
Loss from operations	(19,206)	(12,685)	(62,729)	(31,183)
Interest and other income, net	238	101	668	406
Loss before taxes	(18,968)	(12,584)	(62,061)	(30,777)
Benefit from income taxes	3	3,339	27	4,087
Net loss	$ (18,965)	$ (9,245)	$(62,034)	$(26,690)
Basic and diluted net loss per common share	$ (0.27)	$ (0.13)	$ (0.88)	$ (0.38)
Shares used in computing basic and diluted net loss per common share	70,618	69,892	70,493	69,622

SELECTED BALANCE SHEET DATA

	September 30, 2016	December 31, 2015
Cash, cash equivalents, marketable securities and interest receivable	$ 155,398	$ 209,307
Total assets	164,978	217,235
Total stockholders' equity	143,836	192,439

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SOURCE Sangamo BioSciences, Inc.

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