Sangamo Therapeutics Logo

Sangamo BioSciences Reports Second Quarter 2016 Financial Results

August 3, 2016

Company to Host Conference Call and Webcast Today at 5:00 p.m. ET

CEO Sandy Macrae Will Provide an Update on Program Timelines

RICHMOND, Calif., Aug. 3, 2016 /PRNewswire/ -- Sangamo BioSciences, Inc. (NASDAQ: SGMO), the leader in therapeutic genome editing, today reported its second quarter 2016 financial results and updated on recent events and development timelines for its preclinical and clinical therapeutic programs.

"My mandate as CEO of Sangamo is to lead the Company in its transition from a research-focused organization to a clinical-stage product development company capable of building a versatile portfolio of therapeutics based on both our proprietary zinc finger nuclease (ZFN) and gene therapy platforms, and I am excited to be part of that evolution," said Sandy Macrae, M.B., Ch.B., Ph.D., Sangamo's president and chief executive officer.

Dr. Macrae continued, "In my first few months I have been reviewing programs and working with the team to understand how best to use our resources to position the Company for success. As part of this process, we are re-prioritizing some of our efforts. In 2016, our intention remains to file an investigational new drug (IND) application for our hemophilia A program which uses an adeno-associated virus (AAV) cDNA gene therapy approach and to begin a Phase 1/2 clinical trial of our *in vivo* genome editing program in hemophilia B. However, the initiation of our Phase 1/2 trials in MPS I and MPS II is delayed until 2017 and we expect that the timing of milestones related to some of our other programs will change as we refocus our efforts.

"I am confident that this review and future focusing of our efforts will be beneficial to the efficiency and performance of the organization in the long run. I am pleased to be leading Sangamo to a new stage in its development and excited to be a part of building a world class organization capable of developing innovative medicines for patients and creating value for its shareholders."

Summary of Therapeutic Development Program Updates
Sangamo has eight programs in therapeutic development in addition to ongoing clinical trials to evaluate its ZFN-mediated genome editing approach for HIV/AIDS in T-cells as well as hematopoietic stem and progenitor cells (HSPCs). As part of its review and evaluation process the Company is prioritizing efforts to enable development of its therapeutics and execution of it trials to the highest standards.

The current expected status of Sangamo's programs is summarized below. Additional detail will be provided during this afternoon's call.

- Sangamo expects to file an investigational new drug (IND) application for its AAV cDNA human Factor 8 (hF8) gene therapy program for the treatment of hemophilia A and to initiate the Phase 1/2 clinical trial of its *in vivo* genome editing approach for hemophilia B in 2016.
- The Company's proposed Phase 1/2 clinical trials for both Mucopolysaccharidosis Type I (MPS I) and MPS II are expected to begin in 2017 after Sangamo completes certain *in vitro* preclinical studies and discussions with the U.S. Food and Drug Administration (FDA).
- IND applications for Sangamo's collaborative programs with Biogen in beta-thalassemia and sickle cell disease are anticipated to be filed in 2017 after some additional preclinical work to optimize the programs. Sangamo will provide more information as it becomes available.
- Sangamo is evaluating the preclinical development plans for its *in vivo* genome editing approach for Fabry disease and Gaucher disease to inform the optimal path forward to IND application.
- The Company expects to present data in 2017 from its ongoing Phase 2 clinical trial in T-cells (SB-728-1101 Cohort 3*) and investigator-sponsored Phase 1/2 clinical study (SB-728mR-HSPC) in HSPCs, both of which are designed to evaluate Sangamo's ZFN-mediated genome-editing approach for HIV/AIDS.

Recent Events

- **Appointment of Sandy Macrae as new president and CEO.** In June, Sangamo announced the appointment of Alexander 'Sandy' Macrae, M.B., Ch.B., Ph.D., MRCP, as president and chief executive officer to succeed founder and former president and CEO, Edward Lanphier. Dr. Macrae, a physician scientist, joins Sangamo with deep experience in clinical development and global business strategy, most recently serving as global medical officer of Takeda Pharmaceuticals. Mr. Lanphier stepped down from his chief executive leadership role and, following the Company's 2016 annual meeting of stockholders on June 14, 2016, assumed chairmanship of Sangamo's Board of Directors.

- **Announcement of new gene therapy clinical development program for the treatment of hemophilia A.** Following the presentation of preclinical data at the World Federation of Hemophilia 2016 World Congress demonstrating supraphysiological levels of human factor VIII (hFVIII) expression from Sangamo's new proprietary AAV cDNA therapeutic, the Company announced that it is advancing the clinical development of this AAV cDNA approach (SB-525) for the treatment of hemophilia A. Sangamo also announced the goal of filing an IND application with the FDA in 2016 and, pending FDA clearance, initiating a Phase 1/2 clinical trial in adults with hemophilia A.
- **Presentation of new data and recent developments from multiple research and ZFP Therapeutic® programs at 2016 Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT).** At the 2016 ASGCT Annual Meeting, Sangamo presented new preclinical data from its proprietary ZFN-mediated *in vivo* genome editing approach for MPS I, providing histologic evidence of clearance of toxic glycosaminoglycans (GAGs) in various tissues after a single systemic treatment of the Company's therapeutic candidate. Data were also presented from Sangamo scientists and their collaborators highlighting technological advances in process development and therapeutic applications. Specifically:
    - Data demonstrating the capabilities of ZFNs as tools in the development of allogeneic T-cell therapies, including simultaneous high efficiency biallelic knock out of the T-cell receptor (TCR) and HLA Class I protein as well as highly efficient targeted integration into these sites.
    - Improved multipotency and long-term engraftment of ZFN-modified HSPCs after treatment with Valproic acid (VPA), a small-molecule epigenetic modifier.
    - The use of serum-free conditions to increase ZFN-mediated targeted integration efficiency in isolated T-cells.

Second Quarter 2016 Results

For the second quarter ended June 30, 2016, Sangamo reported a consolidated net loss of $26.6 million, or $0.38 per share, compared to a net loss of $12.1 million, or $0.17 per share, for the same period in 2015. As of June 30, 2016, the Company had cash, cash equivalents, marketable securities and interest receivable of $172.6 million.

Revenues for the second quarter of 2016 were $3.7 million, compared to $8.4 million for the same period in 2015. Second 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 second quarter of 2016 consisted of $3.6 million from collaboration agreements and $0.1 million from research grants, compared to $7.8 million and $0.6 million, respectively, for the same period in 2015. The decrease in collaboration agreement revenues was primarily 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.

In the second quarter of 2016, Sangamo recognized $1.9 million of revenues related to research services performed under the collaboration agreement with Biogen, and $0.4 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 second quarter of 2016.

Research and development expenses were $19.5 million for the second quarter of 2016, compared to $15.6 million for the same period in 2015. The increase was primarily due to increases in manufacturing and clinical trial expenses, consulting and personnel-related expenses.

General and administrative expenses were $11.1 million for the second quarter of 2016, compared to $5.0 million for the same period in 2015. The increase was primarily due to corporate costs and separation expenses associated with the CEO transition, the majority of which was non-cash, stock-based compensation expense.

Total operating expenses for the second quarter of 2016 were $30.5 million, compared to $20.6 million for the same period in 2015.

Six Months Results

For the six months ended June 30, 2016, the consolidated net loss was $43.1 million, or $0.61 per share, compared to a consolidated net loss of $17.4 million, or $0.25 per share, for the six months ended June 30, 2015. Revenues were $7.6 million for the first half of 2016, compared to $21.8 million for the same period in 2015. Total operating expenses were $51.2 million for the first half of 2016, compared to $40.3 million for the first half of 2015.

Financial Guidance for 2016

The Company updates 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.

The Company reiterates guidance as follows:

- **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, August 3, 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 52450915. A conference call replay will be available for one week following the conference call, from approximately 8:00 p.m. ET on August 3, 2016 to 11:59 p.m. ET on August 10, 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 52450915.

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 In Vivo Protein Replacement Platform™ (IVPRP) approach is focused on monogenic diseases, including hemophilia and lysosomal storage disorders. Based on its proprietary IVPRP approach, Sangamo is initiating a Phase 1/2 clinical trial for hemophilia B, the first *in vivo* genome editing application cleared by the FDA. In addition, Sangamo has a Phase 2 clinical program to evaluate the safety and efficacy of novel ZFP Therapeutics® for the treatment of HIV/AIDS (SB-728). The Company has also formed a strategic collaboration with Biogen Inc. for hemoglobinopathies, such as 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 potential milestone and royalty payments under Sangamo's agreements with Shire and Biogen, the research and development of ZFNs and ZFP TFs, clinical trials and therapeutic applications of Sangamo's ZFP technology platform and achievement of research milestones and objectives under collaboration agreements with Shire and Biogen. 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 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 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 June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
Statement of Operations Data:				
Revenues:				
Collaboration agreements	$ 3,592	$ 7,801	$ 7,303	$ 20,472
Research grants	110	557	341	1,377
Total revenues	3,702	8,358	7,644	21,849
Operating expenses:				
Research and development	19,454	15,618	34,720	30,598
General and administrative	11,090	5,017	16,447	9,749

Total operating expenses	30,544	20,635	51,167	40,347
Loss from operations	(26,842)	(12,277)	(43,523)	(18,498)
Interest and other income, net	243	151	430	305
Loss before taxes	(26,599)	(12,126)	(43,093)	(18,193)
Benefit from income taxes	24	-	24	748
Net loss	$ (26,575)	$ (12,126)	$(43,069)	$(17,445)
Basic and diluted net loss per common share	$ (0.38)	$ (0.17)	$ (0.61)	$ (0.25)
Shares used in computing basic and diluted net loss per common share	70,487	69,684	70,430	69,485

SELECTED BALANCE SHEET DATA

	June 30, 2016	December 31, 2015
	(Unaudited)	
Cash, cash equivalents, marketable securities and interest receivable	$ 172,575	$ 209,307
Total assets	180,906	217,235
Total stockholders' equity	160,023	192,439

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SOURCE Sangamo BioSciences, Inc.

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