Sangamo Therapeutics Logo

Sangamo Provides Clinical Development Update Including Early Phase 1/2 Beta Thalassemia Gene-edited Cell Therapy Data

April 2, 2019

– Conference call and webcast scheduled for 8:00 a.m. Eastern Time today

BRISBANE, Calif., April 2, 2019 /PRNewswire/ -- Sangamo Therapeutics, Inc. (NASDAQ: SGMO), a genomic medicine company, today announced recent progress across its clinical portfolio, including early data for ST-400, an *ex vivo* gene-edited beta thalassemia cell therapy developed in partnership with Sanofi, and next steps for its *in vivo* genome editing programs. Additionally, in separate announcements, Sangamo today also provided an update on its AAV manufacturing capacity and capabilities, and in a joint press release with partner Pfizer, reported interim data from the SB-525 hemophilia A gene therapy program. These updates will be discussed during a conference call and webcast scheduled for 8:00 a.m. Eastern Time this morning (details below).

"Over the last three years, we've built a solid foundation to support Sangamo's clinical organization and we believe this morning's updates demonstrate how the various components of our clinical development activities are coming together," said Sandy Macrae, Sangamo's Chief Executive Officer. "We are leveraging our technical, clinical, and manufacturing expertise along with our partnerships to create a genomic medicine company with an advancing pipeline of clinical development programs in gene therapy, *ex vivo* gene-edited cell therapy and *in vivo* genome editing."

ST-400 ex vivo gene-edited cell therapy for beta thalassemia: ST-400 is an autologous cell therapy that involves gene editing of a patient's own hematopoietic stem cells (HSCs) using non-viral delivery of zinc finger nuclease (ZFN) technology. The first patient treated with ST-400 in the Phase 1/2 THALES study has the most severe form of transfusion-dependent beta thalassemia (β^0/β^0). For the two years prior to treatment in the study, this patient received packed red blood cell (PRBC) transfusions every other week.

- During the ST-400 infusion, the patient experienced a serious adverse event, a transient allergic reaction considered related to the cryoprotectant present in the product. Thereafter, the post-transplant clinical course was routine.
- The patient demonstrated neutrophil and platelet recovery, within two and four weeks of infusion, respectively, indicating that ST-400 successfully reconstituted hematopoiesis following conditioning.
- Indels (small insertions or deletions generated at the targeted DNA sequence) have been detected in circulating white blood cells, indicating successful editing of the *BCL11A* gene and disruption of the *BCL11A* erythroid specific enhancer, which is intended to upregulate endogenous fetal hemoglobin production in red blood cells.
- At seven weeks post ST-400 infusion, total hemoglobin levels remained stable (~9 g/dL), and levels of fetal hemoglobin have continued to rise from approximately 1% of total hemoglobin at the time of infusion to 31% as of the most recent measurement.
- The patient received several PRBC transfusions for approximately two weeks after the ST-400 infusion. During the subsequent five weeks, the most recent data available, no further PRBC transfusions have been required.

"While these data are very early and will require confirmation in additional patients as well as longer follow-up to draw any clinical conclusion, they are promising. The detection of indels in peripheral blood with increasing fetal hemoglobin at seven weeks is suggestive of successful gene editing in this transfusion-dependent beta thalassemia patient," said Angela Smith, MD, Associate Professor in the Division of Pediatric Blood and Marrow Transplantation at the University of Minnesota. "These initial results are especially encouraging given the patient's β^0/β^0 genotype, a patient population which has proved to be difficult-to-treat and where there is high unmet medical need."

Enrollment in the THALES study is ongoing. Sangamo expects to present longer-term ST-400 data in Q4 2019, including results from additional patients. Until that time, Sangamo is not planning to report additional clinical data from the program.

In vivo genome editing programs: Sangamo today also provided an update on its *in vivo* genome editing programs: SB-913 (mucopolysaccharidosis type II, or MPS II), SB-318 (MPS I), and SB-FIX (hemophilia B).

The Phase 1/2 clinical trials evaluating *in vivo* genome editing product candidates SB-913 (the CHAMPIONS study for the treatment of MPS II), SB-318 (the EMPOWERS study for MPS I) and SB-FIX (the FIXtendz study for hemophilia B) are ongoing. Data will continue to accumulate throughout 2019, including in the recently treated expansion cohort patients in the CHAMPIONS study, and further updates on these studies are expected later this year. Sangamo expects that no additional patients will be treated at this time with first-generation ZFNs given that clinical benefit has not been demonstrated in analyses conducted to date in ongoing clinical trials and the expected near-term clinical development of second-generation ZFNs.

As previously announced, Sangamo plans to initiate a new clinical trial to evaluate second-generation ZFNs for SB-913 to treat MPS II. *In vitro* preclinical data presented last year showed three potential advantages of second-generation ZFNs: (1) improvements in efficiency and potency due to structural modifications to the ZFN architecture and expression vector; (2) the ability to function equally well in the patients with a single nucleotide polymorphism (SNP) in the target locus in the albumin gene (~20% of the population); (3) improvements in specificity.

The clinical trial of SB-913 using second-generation ZFNs is planned to begin in the second half of 2019. Sangamo expects to use data from this study to make a Phase III decision for the SB-913 program in 2020 and to define the next steps for the SB-318 and SB-FIX programs.

Conference Call
Sangamo will host a conference call today, April 2, 2019, at 8:00 a.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. The slides which accompany this webcast will also be available on Sangamo's website.

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 6063108. For those unable to listen in at the designated time, a conference call replay will be available for one week following the 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 6063108.

About Sangamo Therapeutics
Sangamo Therapeutics, Inc. is focused on translating ground-breaking science into genomic medicines with the potential to transform patients' lives using gene therapy, *ex vivo* gene-edited cell therapy, *in vivo* genome editing, and gene regulation. For more information about Sangamo, visit www.sangamo.com.

Forward-Looking Statements
This press release contains forward-looking statements based on Sangamo's current expectations. These forward-looking statements include, without limitation, statements related to the potential therapeutic applications for ST-400, SB-913, SB-318 and SB-FIX, the potential for ST-400 to treat beta thalassemia and for SB-913 to treat MPS II, the timing and nature of additional data from THALES study, the potential advantages of second-generation ZFNs, the expected near-term clinical development of second-generation ZFNs, including the planned clinical trial of SB-913 using second-generation ZFNs, anticipated next steps for the SB-913, SB-318 and SB-FIX programs, Sangamo's advancing pipeline of clinical development programs, and other statements that are not historical fact. 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, risks and uncertainties related to early data, including the risk that the very early data from the single patient in the THALES study may not be representative of the final results in the THALES study after all patients are treated in the study and all data are collected and analyzed and therefore, such early data should be considered carefully and with caution and not relied upon as indicative of future clinical results in the THALES study or in any other clinical trials; whether the final results from the THALES study will validate and support the early data reported and the overall safety and efficacy of ST-400, including the risk that the early data from the single patient in the THALES study to date may not be maintained or replicated in that patient or in any other patients; Sangamo's ability to complete the THALES study and to initiate clinical trials using second-generation ZFNs; Sangamo's dependence on the success of clinical trials; the lengthy and uncertain regulatory approval process; uncertainties related to the initiation, enrollment and completion of clinical trials; whether Sangamo will be able to effectively deliver its ZFNs to produce a beneficial therapeutic effect, including the risks that the second-generation ZFNs may not be successfully integrated into Sangamo's product candidates and even if successfully integrated, the second-generation ZFNs may not have any advantages over Sangamo's first-generation ZFNs or otherwise may not produce any beneficial therapeutic effect; Sangamo's reliance on partners and other third-parties to meet their clinical and manufacturing obligations; and Sangamo's ability to maintain strategic partnerships. Further, there can be no assurance that the necessary regulatory approvals for ST-400, SB-913, SB-318 and SB-FIX or any other product candidates will be obtained or that Sangamo and its partners will be able to develop commercially viable product candidates for the treatment of beta thalassemia, MPS II and other diseases. These risks and uncertainties are described more fully in Sangamo's Annual Report on Form 10-K for the year ended December 31, 2018 as filed with the Securities and Exchange Commission. Forward-looking statements contained in this press release are made as of this date, and Sangamo undertakes no duty to update such information except as required under applicable law.

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