Sangamo Announces Interim Results Of Phase 1/2 EMPOWERS Study Evaluating SB-318 Zinc Finger Nuclease (ZFN) In Vivo Genome Editing Demonstrating Increased Leukocyte IDUA Activity In Patients With MPS I

February 7, 2019

-- Administration of SB-318 was generally well tolerated in all 3 subjects across 2 dosing cohorts, up to a dose of 5e13 vg/kg (2 patients)

-- Clinical relevance of observed biochemical changes to be informed by additional data expected later this year

-- Conference call and webcast scheduled for 12:30 p.m. Eastern Time today

BRISBANE, Calif., Feb. 7, 2019 /PRNewswire/ -- Sangamo Therapeutics, Inc. (NASDAQ: SGMO) today presented interim data from the Phase 1/2 EMPOWERS Study evaluating the SB-318 zinc finger nuclease (ZFN) *in vivo* (inside the body) genome editing product candidate in patients with Mucopolysaccharidosis Type I (MPS I). These data, along with interim results of the CHAMPIONS Study evaluating SB-913 for MPS II, were presented today at the WORLD*Symposium* 2019 being held in Orlando, Florida. Sangamo believes data from these two studies provide complementary evidence supportive of a favorable safety profile and of the activity of the ZFN *in vivo* genome editing technology used in both SB-318 and SB-913. The two WORLD*Symposium* presentations have been made available on the Sangamo website.

"The results so far suggest a dose-dependent increase in leukocyte IDUA enzyme activity," said Dr. Paul Harmatz, a professor at UCSF Benioff Children's Hospital Oakland and a lead investigator on the study. "Leukocytes are an easily accessible target tissue for IDUA and therefore provide an estimate of tissue enzyme activity for patients with MPS I. Whether these observed increases will translate into clinical benefit from SB-318 is yet to be determined."

MPS I, also known as Hurler syndrome, is a rare genetic disorder caused by a deficiency of alpha-L-iduronidase (IDUA), a lysosomal enzyme which is required to break down or recycle the toxic buildup of glycosaminoglycans (GAGs). Without IDUA enzyme activity, GAGs accumulate in cells throughout the body, leading to widespread tissue and organ damage. The current standard-of-care treatment for MPS I is enzyme replacement therapy (ERT), given as weekly intravenous infusions. For severe MPS I patients, bone marrow transplant is also a common treatment. SB-318 is an investigational product candidate being evaluated to treat MPS I using ZFNs, which are designed to insert a normal copy of the IDUA gene into a precise location in the DNA of liver cells. The goal of SB-318 treatment is to enable a patient's liver to produce a continuous supply of functional IDUA enzyme.

"It is a tremendous responsibility to undertake the first *in vivo* genome editing clinical trials, and we are learning quickly about our technology and about these rare diseases," said Dr. Edward Conner, Chief Medical Officer of Sangamo. "While additional data will be critical in assessing the safety profile and potential therapeutic benefit of SB-318, these early data provide evidence of the progress that we are making toward a potential new treatment for MPS I using *in vivo* genome editing with ZFNs."

Interim EMPOWERS Study Results Presented at WORLD*Symposium*

The primary objective of the EMPOWERS Study is to determine the safety and tolerability of SB-318, and secondary objectives include evaluation of change from baseline in IDUA activity and urine GAG levels. Biochemical measurements of urinary GAGs, as well as plasma and leukocyte IDUA activity, are assessed at screening and baseline visits, and every two to four weeks during the initial phase of the trial.

Patients with mild MPS I receiving weekly ERT were enrolled in the study. One patient has been dosed with 1e13 vector genomes per kilogram body weight (vg/kg) of SB-318 and two patients have been dosed with 5e13 vg/kg of SB-318. None of the three patients enrolled in the study have received a bone marrow transplant.

Safety data were collected and analyzed for the three patients. Administration of SB-318 was generally well tolerated. No treatment related serious adverse events (SAEs) have been reported. Of the six total adverse events (AEs) reported, all were mild or moderate and consistent with ongoing MPS I disease, and none were considered related to SB-318 treatment.

The results suggest a dose-dependent increase in leukocyte IDUA activity, with activity levels rising above baseline and in the normal range (normal range is 6.0-71.4 nmol/hr/mg). Plasma IDUA activity was unchanged from baseline in all three patients.

Baseline urine GAG measurements for the three patients in the EMPOWERS Study were in a range considered to be at or slightly above normal. In the limited duration data set available at the time of the WORLD*Symposium* presentation, urine GAG measurements show no meaningful change.

The clinical relevance of the biochemical changes observed following administration of SB-318 will be assessed as clinical data and patient outcomes are analyzed following a trial of withdrawal from ERT. ERT withdrawal is expected for these patients later in 2019. Sangamo also expects to report analyses of liver biopsies later this year.

Sangamo has developed second-generation, potentially more potent ZFN constructs designed to increase editing efficiency. Preclinical data of these second-generation ZFNs were reviewed by U.S. regulators. The preclinical data showed three potential advantages for use in the clinic: (1) a 5- to 30-fold improvement in efficiency and potency due to structural changes; (2) the ability to function equally well in the patients who have a single nucleotide polymorphism (SNP) in the target locus in the albumin gene (approximately 20% of the population); (3) improved specificity. The second-generation ZFNs are already being manufactured and are expected to be ready for use in the clinic later this year. Additional data from Sangamo's *in vivo* genome editing programs will be assessed before potential integration plans for the second-generation ZFNs are finalized.

Conference Call

Sangamo will host a conference call today, February 7, 2019, at 12:30 p.m. Eastern Time, 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 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 4387585. 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 4387585.

About Sangamo Therapeutics

Sangamo Therapeutics, Inc. is focused on translating ground-breaking science into genomic medicines with the potential to transform patients' lives using the Company's platform technologies in genome editing, gene therapy, gene regulation and cell therapy. 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 relating to the potential therapeutic applications for SB-318 and SB-913, the potential for SB-318 to treat MPS I, including the potential for increased leukocyte IDUA activity to translate into clinical benefit and for SB-318 to potentially cause the liver to produce a continuous supply of functional IDUA enzyme, the timing and nature of additional data from SB-318, the potential advantages of second-generation ZFNs, the timing of the use of the second-generation ZFNs, Sangamo's belief that the second-generation ZFNs have the potential for greater benefit for patients, 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 early data from the EMPOWERS Study may not be representative of final results after all patients are treated in the study and all data are collected and analyzed; Sangamo's ability to complete the EMPOWERS Study and the CHAMPIONS Study; whether the final results from the EMPOWERS Study and CHAMPIONS Study will validate and support interim results and the overall safety and efficacy of SB-318 and SB-913, respectively, including the risk that the early efficacy data from the EMPOWERS Study and the CHAMPIONS Study may not be maintained or replicated; 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 its ability to maintain strategic partnerships. Further, there can be no assurance that the necessary regulatory approvals for SB-318 or SB-913 will be obtained or that Sangamo and its partners will be able to develop commercially viable product candidates for the treatment of MPS I, MPS II and other diseases. These risks and uncertainties are described more fully in Sangamo's Quarterly Report on Form 10-Q for the quarter ended September 30, 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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