RICHMOND, Calif., Feb. 6, 2018 /PRNewswire/ -- Sangamo Therapeutics, Inc. (Nasdaq: SGMO) today announced the presentation of initial safety data from the CHAMPIONS Study, the Phase 1/2 clinical trial evaluating SB-913 for the treatment of mucopolysaccharidosis (MPS) type II, a rare disease also known as Hunter syndrome. The data are being presented this afternoon at the 2018 WORLD Symposium medical congress being held in San Diego.

The poster presentation titled "Update on phase 1/2 clinical trials for MPS I and MPS II using ZFN-mediated in vivo genome editing" includes safety data collected and prepared as of December 27, 2017 and describes the first six weeks of experience of the first patient in the CHAMPIONS study, who was treated November 13, 2017 with a dose of 5.00E+12 vg/kg of SB-913. The patient tolerated the infusion well. Mild (Grade 1) adverse events related to the study drug were reported on the fourth day after dosing as dizziness, weakness, and frequent urination, all of which resolved within one day without treatment. No other adverse events related to the study drug were observed. Liver function tests have remained within normal limits for the patient since the infusion.

One serious adverse event has been reported and is classified as unrelated to SB-913. The subject developed an upper respiratory tract infection and was hospitalized for acute bronchitis. The subject, who recovered after receiving medical treatment, has chronic pulmonary disease and has previously been hospitalized for a similar respiratory illness.

A second patient was treated in the study in mid-January 2018, also at a dose of 5.00E+12 vg/kg.

"Two patients have thus far been treated in the CHAMPIONS Study, and both have tolerated the SB-913 infusions well, with no concerning safety issues related to study drug seen to date," said Paul Harmatz, M.D., a pediatric gastroenterologist and a principal investigator for the CHAMPIONS study at the UCSF Benioff Children's Hospital Oakland,
where the first subjects in the study were treated.

Dr. Edward Conner, Chief Medical Officer of Sangamo, also commented: "We are pleased with how the CHAMPIONS Study is progressing. We expect enrollment of remaining patients to proceed on schedule and to begin to report additional safety and initial efficacy data by mid-year."

*Sangamo and collaborators are presenting four abstracts at the 2018 WORLDsymposium:*

Tuesday, February 6, 4:30 – 6:30pm, Poster Presentations

#134 "Update on phase 1/2 clinical trials for MPS I and MPS II using ZFN-mediated *in vivo* genome editing." Presenter: Paul Harmatz, UCSF Benioff Childrens' Hospital Oakland

#158 "Liver-based expression of the human alpha-galactosidase A gene in a murine Fabry model results in continuous therapeutic levels of enzyme activity and effective substrate reduction." Presenter: Marshall Huston, Sangamo Therapeutics

Wednesday, February 7, 1:00pm, Translational Research Presentations

"ZFN-mediated *in vivo* genome editing of hepatocytes results in phenotypic correction in murine MPS I and MPS II models." Presenter: Thomas Wechsler, Sangamo Therapeutics

"ZFN-mediated *in vivo* genome editing results in therapeutic levels of alpha-galactosidase A and effective substrate reduction in Fabry knockout mice." Presenter: Silvere Pagant, Icahn School of Medicine at Mount Sinai

**About MPS II**
Mucopolysaccharidosis type II, also known as Hunter syndrome, is a rare progressive disorder that primarily affects males and is caused by mutations in the gene encoding the iduronate-2-sulfatase (IDS) enzyme. Children with MPS II begin showing symptoms of developmental delay by age 2 – 3 years. Depending on the severity of the mutation and degree of residual enzyme activity, affected individuals may experience delayed development and develop enlarged internal organs, cardiovascular disorders, stunted growth, skeletal abnormalities and hearing loss.

**About SB-913 and the CHAMPIONS Study**
SB-913 is designed as a single treatment strategy intended to provide stable, continuous production of the IDS enzyme for the lifetime of the patient. SB-913 makes use of Sangamo's zinc finger nuclease (ZFN) genome editing technology to insert a corrective gene into a precise location in the DNA of liver cells. To restrict editing to liver cells, the ZFNs and the corrective gene are delivered in a single intravenous infusion using AAV vectors that target the liver. The ZFNs enter the cells as inactive DNA instructions in a format designed only for liver cells to unlock. Once "unlocked", the ZFNs then identify, bind to and cut the DNA in a specific location within the albumin gene. Using the cells' natural DNA repair processes, liver cells can then insert the corrective gene for IDS at that precise location. The FDA has granted Orphan Drug, Fast Track and Rare Pediatric Disease designations to SB-913 for the treatment of MPS II.
The CHAMPIONS study is an open-label clinical study designed to assess the safety, tolerability and preliminary efficacy of three dose levels of the SB-913 investigational genome editing therapy in up to nine adult males with MPS II. The study is sponsored by Sangamo Therapeutics with participation from hospitals specializing in the care of patients with MPS II, including hospitals in Oakland, Chapel Hill, Chicago, Minneapolis, New York and Philadelphia.

About Sangamo Therapeutics
Sangamo Therapeutics, Inc. 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. For more information about Sangamo, visit www.sangamo.com.

Forward-Looking Statements
This press release may contain forward-looking statements based on Sangamo's current expectations. These forward-looking statements include, without limitation references to the progress of, the expected rate and timing of patient enrollment in, and anticipated schedule of reporting additional safety and initial efficacy data from, the CHAMPIONS STUDY, as well as the potential of Sangamo's technology to treat MPS II. Actual results may differ materially from these forward-looking statements due to a number of factors, including uncertainties relating to the safety and efficacy of SB-913, the initiation and completion of stages of our clinical trials, whether the clinical trials will validate and support the tolerability and efficacy of SB-913 Sangamo’s ability to develop commercially viable products for the treatment of MPS II and other diseases. For a more detailed discussion of these and other risks, please see Sangamo's SEC filings, including the risk factors described in its most recent Quarterly Report on Form 10-Q. Sangamo assumes no obligation to update the forward-looking information contained in this press release.

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