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Sangamo Announces Treatment Of First Patient In Phase 1/2 Clinical Trial Evaluating SB-318 Investigational In Vivo Genome Editing Therapy For MPS I

RICHMOND, Calif., July 23, 2018 /PRNewswire/ -- Sangamo Therapeutics, Inc. (Nasdaq: SGMO) today announced treatment of the first patient in the Phase 1/2 clinical trial evaluating SB-318, an investigational *in vivo* genome editing therapy for patients with mucopolysaccharidosis type I (MPS I, Hurler syndrome) (the "EMPOWERS Study").

The EMPOWERS Study is an open-label, ascending dose clinical trial designed to assess the safety, tolerability and preliminary efficacy of SB-318 in up to nine adult subjects with attenuated MPS I. The study is currently screening subjects at hospitals specializing in the care of patients with MPS I, including hospitals in Oakland, Gainesville, Atlanta, Minneapolis, New York and Cincinnati.

"We are pleased to announce the treatment of the first patient in the Empowers Study," said Sangamo Chief Medical Officer, Edward Conner, MD. "With this first patient in the Empowers Study of SB-318 for MPS I, and with five patients now treated in the CHAMPIONS Study of SB-913 for MPS II, we are making strong progress in the evaluation of our *in vivo* genome editing approach for these two rare inherited metabolic diseases. We remain on track to announce preliminary data from the MPS II CHAMPIONS Study in late summer."

Both SB-318 and SB-913 make use of Sangamo's zinc finger nuclease (ZFN) genome editing technology that is designed to insert a corrective copy of the *IDUA* (SB-318) or *IDS* (SB-913) gene into a precise location in the DNA of liver cells with the goal of enabling a patient's liver to produce a lifelong and stable supply of enzyme. 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 at that precise location.

The potential to permanently and precisely integrate a therapeutic gene into the DNA differentiates Sangamo's *in vivo* genome editing approach from conventional AAV cDNA gene therapy, which expresses the therapeutic gene episomally from the nucleus without integrating it into the cell's genome.

SB-318 has received Orphan Drug, Fast Track and Rare Pediatric Disease designations from the U.S. Food and Drug Administration (FDA), as well as Orphan Medical Product designation by the European Medicines Agency (EMA). In June, the Medicines and Healthcare Products Regulatory Agency (MHRA) granted the Clinical Trial Authorisation (CTA) for enrollment of subjects into the ongoing Phase 1/2 clinical trial evaluating SB-318 for MPS I, and Sangamo plans to initiate clinical trial sites in the U.K. later this year.

About Sangamo Therapeutics
Sangamo Therapeutics, Inc. is focused on translating ground-breaking science into genomic therapies that 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 may contain forward-looking statements based on Sangamo's current expectations. These forward-looking statements include, without limitation, references relating to being on track to announce preliminary data from the MPS II CHAMPIONS Study in late summer, the design of Sangamo's ZFN genome editing technology, potential to permanently and precisely integrate a therapeutic gene into the DNA, the potential of Sangamo's technology to treat lysosomal storage disorders, including MPS I and MPS II, Sangamo plan to initiate clinical trial sites in the U.K. later this yearand the impact of Sangamo's clinical trials on the field of genomic medicine. Actual results may differ materially from these forward-looking statements due to a number of factors, including uncertainties relating to substantial dependence on the clinical success of lead therapeutic programs, the initiation and completion of stages of our clinical trials, whether the clinical trials will validate and support the tolerability and efficacy of ZFNs, technological challenges, ability to manufacture product candidates for our clinical trials, Sangamo's ability to develop commercially viable products and technological developments by our competitors. 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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