June 4, 2018

Sangamo Announces U.K. Authorization Of Clinical Trials Evaluating Zinc Finger Nuclease In Vivo Genome Editing Treatments SB-318 For MPS I And SB-913 For MPS II

RICHMOND, Calif., June 4, 2018 /PRNewswire/ -- Sangamo Therapeutics, Inc. (Nasdaq: SGMO) today announced that the Medicines and Healthcare Products Regulatory Agency (MHRA) of the United Kingdom has granted the Clinical Trial Authorisation (CTA) for enrollment of subjects into ongoing Phase 1/2 clinical trials evaluating SB-318 and SB-913, zinc finger nuclease (ZFN) *in vivo* genome editing treatments for Mucopolysaccharidosis Type I (MPS I) and MPS II, respectively.

"Patients with MPS I and MPS II have very few treatment options, and we are excited to expand access to our clinical trials to the U.K.," said Dr. Edward Conner, Chief Medical Officer at Sangamo. "We are pleased with the MHRA's rapid action on our CTA applications and to be working closely with them to advance our evaluation of zinc finger nuclease genome editing treatments into younger patient populations for whom we believe the need and potential benefits are greatest."

The CTA for SB-913 allows for treatment of children as young as five years of age following a review of cumulative safety data from adult and adolescent cohorts. The SB-318 CTA application was based on the protocol of the ongoing Phase 1/2 clinical trial which includes only adult patients. Sangamo plans this year to request a protocol amendment for the SB-318 study to include younger patients.

Sangamo expects to initiate clinical trial sites in the U.K. later this year for the SB-318 and SB-913 Phase 1/2 clinical trials.

Sangamo's *In Vivo* Genome Editing Approach
Sangamo aims to treat patients with MPS I and MPS II using its proprietary ZFN genome editing technology to insert a corrective 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

the alpha-L-iduronidase (IDUA) and iduronate 2-sulfatase (IDS) enzymes for MPS I and MPS II, respectively.

To restrict editing to liver cells, the ZFNs and the corrective gene are delivered in a single intravenous infusion using an AAV vector that targets 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 located in liver cells. Using the cells' natural DNA repair processes, liver cells can then insert the corrective gene for IDUA (MPS I) or IDS (MPS II) at that precise location.

The potential to permanently and precisely integrate the therapeutic IDUA (MPS I) or IDS (MPS II) gene into the DNA differentiates Sangamo's *in vivo* genome editing approach from conventional AAV cDNA gene therapy and from lenti- or retroviral-based gene therapies that insert genes randomly into the genome.

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.

Forward-Looking Statements
This press release contains forward-looking statements regarding Sangamo's current expectations. These forward-looking statements include, without limitation, references to Sangamo's ability to open clinical sites in the U.K. this year and to begin enrolling patients; the Company's opportunity to enroll adolescents and children once preliminary safety and efficacy have been demonstrated in the ongoing SB-318 and SB-913 Phase 1/2 clinical trials in adults, and the Company's goal of enabling a patient's liver to produce a lifelong and stable supply of the alpha-L-iduronidase (IDUA) and iduronate 2-sulfatase (IDS) enzymes for MPS I and MPS II, respectively, the potential to permanently and precisely integrate the therapeutic IDUA (MPS I) or IDS (MPS II) gene into the DNA . 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 dependence on the success of the clinical trials of lead programs, the lengthy and uncertain regulatory approval process, uncertainties related to the initiation, enrollment and completion of clinical trials, whether clinical trial results will validate and support the safety and efficacy of Sangamo's product candidates, and the reliance on partners and other third-parties to meet their obligations. 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 Report 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.

releases/sangamo-announces-uk-authorization-of-clinical-trials-evaluating-zinc-finger-nuclease-in-vivo-genome-editing-treatments-sb-318-for-mps-i-and-sb-913-for-mps-ii-300658887.html

SOURCE Sangamo Therapeutics, Inc.