Sangamo Therapeutics Logo

Sangamo Announces Treatment of First Patient in Landmark Phase 1/2 Clinical Trial Evaluating In Vivo Genome Editing for MPS II

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RICHMOND, Calif., Nov. 15, 2017 /PRNewswire/ -- Sangamo Therapeutics, Inc. (Nasdaq: SGMO) today announced treatment of the first patient in the Phase 1/2 clinical trial ("the CHAMPIONS study") evaluating SB-913, an investigational *in vivo* genome editing therapy for people with mucopolysaccharidosis type II (MPS II), also known as Hunter syndrome.

"For the first time, a patient has received a therapy intended to precisely edit the DNA of cells directly inside the body. We are at the start of a new frontier of genomic medicine," said Dr. Sandy Macrae, CEO of Sangamo Therapeutics.

Sangamo aims to treat MPS II by using genome editing 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 an enzyme he or she currently lacks.

Without that enzyme, called iduronate-2-sulfatase (IDS), people with MPS II suffer debilitating buildup of toxic carbohydrates in cells throughout their body. Approximately one in 100,000 to one in 170,000 people are born with MPS II. Many people with MPS II receive weekly infusions of enzyme replacement therapy (ERT), the current standard-of-care treatment. Within a day of receiving ERT, however, IDS quickly returns to near undetectable levels in the blood.

"Even with regular infusions of ERT, which has markedly improved functional health outcomes, patients endure progressive damage to heart, bones and lungs. Many patients with MPS II die of airway obstruction, upper respiratory infection or heart failure before they reach the age of 20," 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 subject in the study was treated.

The CHAMPIONS study, which is also screening subjects at hospitals specializing in the care of patients with MPS II, including hospitals in Chapel Hill, Chicago, Minneapolis and Philadelphia, is an open-label clinical study designed to assess the safety, tolerability and preliminary efficacy of the SB-913 investigational genome editing therapy in up to nine adult males with MPS II.

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 ability to permanently and precisely integrate the therapeutic IDS 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.

Two additional clinical trials are underway in the United States to evaluate Sangamo's *in vivo* genome editing therapeutics for hemophilia B and MPS I, which is also known as Hurler or Hurler-Scheie syndrome. All three trials use ZFNs designed to edit liver cells at the same location in the albumin gene, but differ in delivering the corrective gene relevant to the respective disease.

"As a physician, I feel a real sense of responsibility toward the patients who are participating in these three clinical trials," said Ed Conner, M.D., chief medical officer of Sangamo. "We have been working closely with the FDA and the NIH Recombinant DNA Advisory Committee to make sure that we are thoroughly and prudently developing this new class of medicines."

All three of Sangamo's *in vivo* genome editing product candidates have received Fast Track and Orphan Drug designations from the U.S. Food and Drug Administration (FDA). Additionally, SB-318 for MPS I and SB-913 for MPS II have received Rare Pediatric Disease designations from the FDA.

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 relating to research and development of therapeutic applications of Sangamo's gene therapy and ZFP technology platforms, the potential of Sangamo's technology to treat hemophilia and lysosomal storage disorders, including MPS I and MPS II, and the impact of Sangamo's clinical trials on the field of genetic 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, 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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