Sangamo Therapeutics Logo

Sangamo Therapeutics Announces Special Regulatory Designations from the FDA for Three Clinical Programs

May 4, 2017

Rare Pediatric Disease Designation for SB-913 In Vivo Genome Editing Treatment for MPS II

Orphan Drug Designation for SB-525 cDNA Gene Therapy for Hemophilia A

Fast Track Designation for SB-FIX In Vivo Genome Editing Treatment for Hemophilia B

RICHMOND, Calif., May 4, 2017 /PRNewswire/ -- Sangamo Therapeutics, Inc. (NASDAQ: SGMO), the leader in therapeutic genome editing, announced today that the Company has received notice from the U.S. Food and Drug Administration (FDA) of special regulatory designations for three of the Company's clinical programs.

- **Rare Pediatric Disease (RPD) designation for SB-913 *in vivo* genome editing treatment for Mucopolysaccharidosis Type II (MPS II).** RPD provides incentives to develop drugs for the treatment of rare diseases primarily affecting children ages 18 years or younger. In addition, a sponsor who receives a new drug approval for a rare pediatric disease may be eligible to receive a priority review voucher for a subsequent marketing application for a different product. The voucher may be sold or transferred.

SB-913 has already received Orphan Drug designation from the FDA. FDA has cleared an Investigational New Drug Application (IND) for this program, and a Phase 1/2 clinical trial evaluating SB-913 in adults with MPS II is open and screening subjects for enrollment.

- **Orphan Drug designation for SB-525 cDNA gene therapy for Hemophilia A.** Orphan Drug designations are granted to drugs and biologics intended to treat rare diseases with a patient population less than 200,000 in the U.S. The designation provides incentives to advance development and commercialization of rare disease drugs.

FDA has cleared an IND for this program, and a Phase 1/2 clinical trial evaluating SB-525 in adults with Hemophilia A is expected to be opened and to begin screening subjects for enrollment later this quarter.

- **Fast Track designation for SB-FIX *in vivo* genome editing treatment for Hemophilia B.** Fast track is designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. Once a drug receives Fast Track designation, early and frequent communication with the FDA is encouraged throughout the development and review process, often leading to earlier drug approval and access by patients.

The SB-FIX program has already received Orphan Drug designation from the FDA. FDA has cleared an IND for this program, and a Phase 1/2 clinical trial evaluating SB-FIX in adults with Hemophilia B is open and screening subjects for enrollment.

"Our four early clinical programs are focused on rare diseases for which new treatments are sorely needed, and we are gratified to work closely with the FDA as we advance these novel genomic therapies in human studies," said Dr. Sandy Macrae, CEO of Sangamo.

Sangamo's fourth lead clinical program, SB-318 *in vivo* genome editing treatment for MPS I, has already received Orphan Drug and RPD designations. FDA has cleared an IND for this program, and a Phase 1/2 clinical trial evaluating SB-318 in adults with MPS I is open and screening subjects for enrollment.

Sangamo's *In Vivo* Genome Editing Approach
Sangamo's ZFN-mediated *in vivo* genome editing approach makes use of the endogenous albumin gene locus, a highly expressing and liver-specific site that can be edited with ZFNs to accept and express therapeutic genes. The approach is designed to enable the patient's liver to permanently produce circulating therapeutic levels of a corrective protein. The ability to permanently integrate the therapeutic gene in a highly specific, targeted fashion significantly differentiates Sangamo's *in vivo* genome editing approach from conventional AAV cDNA gene therapy. Ultimately, the target population for these programs will include pediatric patients, and it will be important in this population to be able to produce stable levels of therapeutic protein for the lifetime of the patient.

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. The Company is advancing Phase 1/2 clinical programs in hemophilia A and hemophilia B, and lysosomal storage disorders MPS I and MPS II. Sangamo has a strategic collaboration with Bioverativ Inc. for hemoglobinopathies, including beta thalassemia and sickle cell disease, and with Shire International GmbH to develop therapeutics for Huntington's disease. In addition, it has established strategic partnerships with companies in non-therapeutic applications of

its technology, including Sigma-Aldrich Corporation and Dow AgroSciences. For more information about Sangamo, visit the Company's website at 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, the expected timing of these clinical trials and the release of data from these trials, the impact of Sangamo's clinical trials on the field of genetic medicine and the benefit of orphan drug status, rare pediatric disease status and fast track status. 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 Annual Report on Form 10-K and its most recent Quarterly Report on Form 10-Q. Sangamo Therapeutics, Inc. assumes no obligation to update the forward-looking information contained in this press release.

SOURCE Sangamo Therapeutics, Inc.

Released May 4, 2017