Sangamo Therapeutics Announces Dosing of First Patient in Phase 1/2 Clinical Study of Investigational CAR-Treg Cell Therapy TX200 in Kidney Transplantation

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BRISBANE, Calif.--(BUSINESS WIRE)--Mar. 29, 2022-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced dosing of the first patient in the Phase 1/2 STEADFAST clinical study evaluating TX200, a wholly-owned autologous Chimeric Antigen Receptor Regulatory T Cell (CAR-Treg) cell therapy product candidate for the prevention of immune-mediated rejection in HLA-A2 mismatched kidney transplantation from a living donor.

“We are very grateful to the patient and investigator who participated in what we believe is the first-ever dosing of a human with a CAR-Treg cell therapy candidate,” said Sandy Macrae, CEO of Sangamo. “We believe that CAR-Tregs are the next frontier of cell therapy and represent a potentially transformational approach to solid organ transplantation as well as to many challenging autoimmune and inflammatory conditions. The STEADFAST Phase 1/2 study is an important milestone for genomic medicine that we anticipate will yield insights regarding CAR-Treg biology and could potentially help patients living with a donated HLA-A2 mismatched kidney from a living donor by promoting immunological tolerance to the graft.”

“This is an exciting time for the renal transplant community and more broadly for medicine. The hope is that CAR-Treg cell therapies reduce the need for lifelong immunosuppressive medications, which are known to have challenging side effects,” said Dr. Jan-Stephan Sanders of the University Medical Center Groningen in The Netherlands and investigator of the STEADFAST clinical study. “I look forward to the dosing of more patients and seeing data from the study to understand the potential of TX200.”

Kidney transplantation is the treatment of choice for patients with end-stage renal disease (ESRD) who must otherwise remain on long-term dialysis. Approximately 21-26% of transplanted kidneys are estimated to be HLA-A2 mismatched[1]. To prevent graft rejection, transplanted patients are treated with lifelong immunosuppressive therapy, which impacts the body’s immune system and is associated with multiple side effects, including an increased risk of severe life-threatening infections, malignancies, cardiovascular disease, and other drug-related toxicities, such as nephrotoxicity, which can impact the function and survival of the newly transplanted kidney.

TX200 was designed with the potential to prevent kidney rejection by reducing local inflammation and promoting immunological tolerance to the graft. TX200 is composed of autologous Treg cells engineered to express an HLA-A2 CAR. This investigational cell therapy is being assessed in HLA-A2 negative patients receiving a mismatched HLA-A2 positive kidney from a living donor. TX200 cells are expected to localize to the graft and activate upon binding to the HLA-A2 antigen. Through their ability to regulate the immune system, TX200 cells may protect the graft from immune-mediated rejection and reduce or eliminate the need for lifelong treatment with immunosuppressants.

In the STEADFAST clinical study, each patient undergoes a leukapheresis procedure to collect their white blood cells, after which their Treg cells are isolated, genetically engineered and then cryopreserved. The patient subsequently undergoes transplantation surgery to receive a kidney from a living donor. Following a recovery period, the patient receives their personalized TX200 investigational cell therapy. Dosing of patients therefore occurs several months after patient enrollment. Sangamo expects to dose a second patient in the STEADFAST study in the middle of 2022.

The STEADFAST study forms the first step in a pipeline of CAR-Treg programs. In addition to TX200, Sangamo is developing CAR-Treg cell therapy candidates in preclinical studies, including for potential use in treating multiple sclerosis and inflammatory bowel disorders.

About the STEADFAST Study

The STEADFAST study is a multicenter, open-label, single ascending dose, dose-ranging Phase 1/2 study for the prevention of immune-mediated rejection in HLA-A2 mismatched kidney transplantation from a living donor. Study sites are currently open and recruiting participants in Belgium, the Netherlands, and the United Kingdom. The primary objective of the STEADFAST study is to evaluate the safety and tolerability of TX200. Key secondary objectives include the evaluation of the effect of TX200 on acute graft-related outcomes and on long-term safety outcomes, as well as the evaluation of the pharmacodynamic and pharmacokinetic effects of TX200. Routine post-transplant biopsies will allow for the early detection of engineered CAR-Tregs in the kidney. The adjustment and tapering of standard immunosuppressive therapy will be at the discretion of the investigator.

About Sangamo Therapeutics

Sangamo Therapeutics is a clinical-stage biopharmaceutical company with a robust genomic medicines pipeline. Using ground-breaking science, including our proprietary zinc finger genome engineering technology and manufacturing expertise, Sangamo aims to create new genomic medicines for patients suffering from diseases for which existing treatment options are inadequate or currently don’t exist. For more information about Sangamo, visit www.sangamo.com.

1. Barocci et al. 2007; Middleton et al, 1985; Schnitzler et al, 1997.

Forward-Looking Statements

This press release contains forward-looking statements regarding Sangamo's current expectations. These forward-looking statements include, without limitation, statements relating to: the therapeutic potential of TX200, including its potential clinical benefit to patients receiving HLA-A2 mismatched kidney transplants from a living donor and patients with other autoimmune and inflammatory conditions, and its potential to promote immunological tolerance to a graft and reduce the need for immunosuppressants; the expected localization and activation of TX200 cells; Sangamo’s expectations for dosing additional patients in the Phase 1/2 STEADFAST study and the expected timing thereof; the design of the Phase 1/2 STEADFAST study; Sangamo’s development of other CAR-Treg therapies and the therapeutic potential thereof; and other statements that are not historical fact. These statements are not guarantees of future performance and are subject to risks and uncertainties that are difficult to predict. Sangamo’s actual results

may differ materially and adversely from those expressed. Factors that could cause actual results to differ include, but are not limited to, risks and uncertainties related to: the uncertain timing and unpredictable nature of clinical trials and clinical trial results, including the risks that Phase 1/2 STEADFAST study data will not validate the safety and efficacy of TX200; the research and development process, including the enrollment, operation and results of clinical trials and the presentation of clinical data; the effects of the evolving COVID-19 pandemic and the impacts of the pandemic on the global business environment, healthcare systems and business and operations of Sangamo, including the initiation and operation of clinical trials; the unpredictable regulatory approval process for product candidates across multiple regulatory authorities; the manufacturing of products and product candidates; the commercialization of approved products; the potential for technological developments that obviate technologies used by Sangamo in TX200; and the risks and uncertainties described in Sangamo's filings with the U.S. Securities and Exchange Commission, including its Annual Report on Form 10-K for the year ended December 31, 2021. The information contained in this release is as of March 29, 2022, and Sangamo undertakes no duty to update forward-looking statements contained in this release except as required by applicable laws.

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