European Orphan Medicinal Product Designation Granted to Sangamo Therapeutics Investigational CAR-Treg Cell Therapy TX200 for Solid Organ Transplantation

July 21, 2022

BRISBANE, Calif.--(BUSINESS WIRE)--Jul. 21, 2022-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced that the European Commission (EC) has granted Orphan Medicinal Product Designation (OMPD) to TX200, a wholly-owned autologous Chimeric Antigen Receptor Regulatory T Cell (CAR-Treg) cell therapy product candidate for treatment in solid organ transplantation.

“Patients who have received a solid organ transplant require lifelong surveillance and chronic immunosuppressive medications to manage the risk of transplant rejection,” said Rob Schott, MD, MPH, FACC, Head of Development for Sangamo. “Our goal with TX200 is to create a transformative therapy that reduces the risk of organ rejection, while reducing the patient burden from chronic immunosuppressive therapy. Achieving this important regulatory milestone takes us one step closer to realizing that goal for patients.”

Dosing of the first patient in the STEADFAST Phase 1/2 clinical study took place in March 2022, with the second patient dosing planned later this quarter.

The EC granted OMPD to TX200 following a positive opinion from the European Medicines Agency’s Committee for Orphan Medicinal Products. To qualify for orphan designation, a treatment must be intended for a life-threatening or chronically debilitating disease affecting fewer than 5 in 10,000 people. Importantly, no satisfactory method of treatment must exist, or if such a method exists, the treatment must be of significant benefit to patients.

The OMPD status offers a range of incentives to encourage the development of orphan medicines, including protocol assistance on study protocols, potential fee reductions, and 10 years of market exclusivity upon regulatory approval.

About TX200

TX200 is a cell therapy composed of autologous regulatory T cells (Tregs) engineered to express an HLA-A2 Chimeric Antigen Receptor (CAR), with the aim of preventing immune-mediated rejection in HLA-A2 mismatched kidney transplants from a living donor. TX200 is intended to reduce the risk of transplant rejection by suppressing local inflammation and promoting immunological tolerance to the graft. TX200 is being assessed in HLA-A2 negative patients receiving a mismatched HLA-A2 positive kidney from a living donor. TX200 CAR-Treg 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. The first patient in the ongoing STEADFAST Phase 1/2 study was dosed in March 2022.

About HLA-A2 mismatched kidney transplantation

Kidney transplantation is the treatment of choice for patients with end-stage renal disease who must otherwise remain on long-term dialysis. Approximately 21-26% of transplanted kidneys are estimated to be HLA-A2 mismatched. 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.

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.

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 potential benefits available to Sangamo as a result of the receipt of OMPD for TX200; the design of the Phase 1/2 STEADFAST study; 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 and its Quarterly Report on Form 10-Q for the quarter ended March 31, 2022. The information contained in this release is as of July 21, 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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