Case Western Reserve and Sangamo Therapeutics Announce $11 Million NIH Grant for Study of Gene-Edited T Cells for Viral Eradication of HIV

CLEVELAND and RICHMOND, Calif., Feb. 7, 2018 /PRNewswire/ -- Case Western Reserve University and Sangamo Therapeutics, Inc. (Nasdaq: SGMO) today announced the award of an $11 million grant from the National Institutes of Health for a planned study of gene-edited T cells designed to eradicate persistent HIV infection in patients receiving anti-retroviral therapy, a combination of medicines that slows the rate at which HIV replicates.

The grant will fund a clinical trial to test the hypothesis that treating patients with their own gene-edited T cells may lead to a sustained increase in T cell counts and eradication of latent HIV reservoirs. T cells (so-called because they develop in the thymus gland) are responsible for a variety of immune responses. Currently available treatments do not completely cure infected individuals due to the persistence of a latent HIV virus population. As a result, if treatment is stopped, the dormant virus rapidly emerges and reestablishes the infection.

The principal investigator of the new study will be Rafick-Pierre Sekaly, PhD, Richard J. Fasenmyer Professor of Immunopathogenesis at CWRU School of Medicine and one of the world's leading scientists in AIDS research, human immunology, and immunotherapy. Sangamo Therapeutics will be contributing materials, equipment, and manufacturing expertise for the study, which is expected to begin in 2018.

The new study is designed such that T cells from the blood of 20 subjects will have the CCR5 gene "knocked out" via zinc finger nuclease gene editing. The CCR5 gene allows HIV to enter host cells. In this process, zinc finger nucleases, which are engineered proteins akin to genetic "scissors," are designed to enable targeted editing of genes by creating double-strand breaks in DNA at precise locations identified by researchers. The newly-edited, "repaired" cell population would be expanded and infused back into the patients. A second set of ten patients will receive an infusion of unmodified T cells.
"For patients with HIV, new treatments to permanently eradicate latent HIV reservoir and increase CD4+ T cell counts – potentially leading to a cure – are an important unmet need," said Sekaly. "Although standard-of-care anti-retroviral HIV therapy does suppress viral replication, HIV infection persists, and patients must stay on treatment for life. Moreover, a large subset of HIV patients receiving the therapy fails to achieve sustained rebound in T cell counts, leading to chronic elevations in inflammation with increased risks of cancer and other diseases."

Sangamo has completed several earlier phase 1/2 studies evaluating CCR5 edited T cells (known as SB-728-T) in patients. These single arm studies (which did not have control arms): 1) demonstrated an ability to efficiently knock out the CCR5 gene in T cells by zinc finger nuclease-driven gene editing and grow the cells ex vivo (outside the body); 2) showed that a single infusion of SB-728-T led to proven engraftment and expansion of the T cells in vivo (in the body); 3) enabled long-term persistence of the gene-edited cells; and 4) generated sustained increases in CD4+ T cell counts and a significant and continuous decay of the HIV reservoir.

No SB-728-T product-related serious adverse events were reported in Sangamo's SB-728-T clinical trials, and there was no observation of development of anti-zinc finger nuclease antibodies.

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.

Sangamo's Forward-Looking Statements

This press release contains forward-looking statements, including, but not limited to, statements related to the potential of gene edited T cells to eradicate persistent HIV infection in patients; the planned study of gene edited T cells, including the ability of gene-edited T cells in the study to lead to a sustained increase in T cell counts and eradication of latent HIV reservoirs, the timing of such study and Sangamo's expected contributions therefor; the adequacy of the grant to fund the planned study of gene edited T cells; and other statements that are not historical facts. These forward-looking statements are based on Sangamo's current plans, objectives, estimates, expectations and intentions and inherently involve significant risks and uncertainties. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of these risks and uncertainties, which include, without limitation, risks and uncertainties associated with: gene-editing product candidate development and the inherent uncertainty of clinical success, including the risks that unanticipated toxicity or adverse events in the planned study may be observed and that the planned study may otherwise fail to validate and support the tolerability and efficacy of gene-edited T cells; the initiation, enrollment and completion of the stages of its clinical trials, including the potential inability to enroll the planned study in a timely manner or at all; technological challenges; the lengthy and uncertain regulatory approval process; technological developments by competitors and others in the genomic therapy field; and the potential
inability of Sangamo and its partners to obtain necessary regulatory approvals and/or develop commercially viable gene-based therapeutics. A more detailed discussion of these and other risks and uncertainties may be found under the caption "Risk Factors" and elsewhere in Sangamo's SEC filings and reports, including Sangamo's Quarterly Report on Form 10-Q for the quarter ended September 30, 2017 and future filings and reports by Sangamo. Sangamo assumes no obligation to update the forward-looking information contained in this press release.


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