Therapies Targeting Cancer, Deadly Immune Disorder and Life-Threatening Blood Condition Get Almost $32 Million Boost from Stem Cell Agency

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April 27, 2018, Oakland, CA – An innovative therapy that uses a patient's own immune system to attack cancer stem cells is one of three new clinical trials approved for funding by the Board of the California Institute for Regenerative Medicine (CIRM), the state's Stem Cell Agency.

Researchers at the Stanford University School of Medicine were awarded $11.9 million to test their Chimeric Antigen Receptor (CAR) T Cell Therapy in patients with B cell leukemias who have relapsed or are not responding after standard treatments, such as chemotherapy.

Researchers take a patient's own T cells (a type of immune cell) and genetically re-engineer them to recognize two target proteins on the surface of cancer cells, triggering their destruction. In addition, some of the T cells will form memory stem cells that will survive for years and continue to survey the body, killing any new or surviving cancer cells.

"When a patient is told that their cancer has returned it can be devastating news," says Maria T. Millan, MD, President & CEO of CIRM. "CAR T cell therapy is an exciting and promising new approach that offers us a way to help patients fight back against a relapse, using their own cells to target and destroy the cancer."

The CIRM Board also approved $8 million for Sangamo Therapeutics, Inc. to test a new therapy for beta-thalassemia, a severe form of anemia (lack of healthy red blood cells) caused by mutations in the beta hemoglobin gene. Patients with this genetic disorder require frequent blood transfusions for survival and have a life expectancy of only 30-50 years. The Sangamo team will take a patient's own blood stem cells and, using a gene-editing technology called zinc finger nuclease (ZFN), turn on a different hemoglobin gene (gamma hemoglobin) that can functionally substitute for the mutant gene. The modified blood stem cells will be given back to the patient, where they will give rise to functional red blood cells, and potentially eliminate the need for chronic transfusions and its associated complications. Sangamo is developing this new therapy in partnership with Bioverativ, a Sanofi Company, as part of a worldwide collaboration to develop new therapies for beta-thalassemia and sickle cell disease.

The third clinical trial approved is a $12 million grant to UC San Francisco for a treatment to restore the defective immune system of children born with severe combined immunodeficiency (SCID), a genetic blood disorder in which even a mild infection can be fatal. This condition is also called "bubble baby disease" because in the past children were kept inside sterile plastic bubbles to protect them from infection. This trial will focus on SCID patients who have mutations in a gene called Artemis, the most difficult form of SCID to treat using a standard bone marrow transplant from a healthy donor. The team will genetically modify the patient's own blood stem cells with a functional copy of Artemis, with the goal of creating a functional immune system.

CIRM has funded two other clinical trials targeting different approaches to different forms of SCID. In one, carried out by UCLA and Orchard Therapeutics, 50 children have been treated and all 50 are considered functionally cured.

This brings the number of clinical trials funded by CIRM to 48, 42 of which are active. There are 11 other projects in the clinical trial stage where CIRM funded the early stage research.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality. For more information go to www.cirm.ca.gov

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