California's Stem Cell Agency Invests in Stem Cell-Based Therapies Targeting Sickle Cell Disease and Cancer

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February 22, 2018 - Oakland, CA The California Institute for Regenerative Medicine (CIRM), California's Stem Cell Agency, today invested almost $10 million in stem cell research targeting sickle cell disease and cancer at its February Board meeting.

The CIRM Board awarded $5.74 million to the City of Hope to fund a Phase 1 clinical trial testing a stem cell-based therapy for adult patients with severe sickle cell disease (SCD). SCD refers to a group of inherited blood disorders that cause red blood cells to take on an abnormal, sickle shape. Sickle cells clog blood vessels and block the normal flow of oxygen-carrying blood to the body's tissues. Patients with SCD have a reduced life expectancy and experience various complications including anemia, stroke, organ damage, and bouts of excruciating pain.

"The current standard of treatment for SCD is a bone marrow stem cell transplant from a genetically matched donor, usually a close family member," explains Maria T. Millan, M.D., President and CEO of CIRM. "This treatment is typically reserved for children and requires high doses of toxic chemotherapy drugs to remove the patient's diseased bone marrow. Unfortunately, most patients do not have a genetically matched donor and are unable to benefit from this treatment. The City of Hope trial aims to address this unmet medical need for adults with severe SCD."

The treatment involves transplanting blood-forming stem cells from a donor into a patient who has received a milder, less toxic chemotherapy treatment that removes some but not all of the patient's diseased bone marrow stem cells. The donor stem cells are depleted of immune cells called T cells prior to transplantation. This approach allows the donor stem cells to engraft and create a healthy supply of non-diseased blood cells without causing an immune reaction in the patient.

"The City of Hope transplant program in SCD is one of the largest in the nation," says Joseph Rosenthal, M.D., Director of Pediatric Hematology and Oncology at the City of Hope and lead investigator on the trial. "CIRM funding will allow us to conduct a Phase 1 trial in six adult patients with severe SCD. We believe this treatment will improve the quality of life of patients while also reducing the risk of graft-versus-host disease and transplant-related complications. Our hope is that this treatment can be eventually offered to SCD patients as a curative therapy."

This is the second clinical trial for SCD that CIRM has funded - the first being a Phase 1 trial at UCLA treating SCD patients with their own genetically modified blood stem cells. CIRM is also currently funding research at Children's Hospital of Oakland Research Institute and Stanford University involving the use of CRISPR gene editing technologies to develop novel stem cell therapies for SCD patients.

The CIRM Board also awarded San Diego-based company Fate Therapeutics $4 million to further develop a stem cell-based therapy for patients with advanced solid tumors.

Fate is developing FT516, a Natural Killer (NK) cell cancer immunotherapy derived from an engineered human induced pluripotent stem cell (iPSC) line. NK cells are part of the immune system's first-line response to infection and diseases like cancer. Fate is engineering human iPSCs to express a novel form of a protein receptor, called CD16, and is utilizing these cells as a renewable source for generating a population of NK cells in quantities that support the treatment of many patients in an off-the-shelf manner. The company will use the engineered NK cells in combination with an anti-breast cancer drug called trastuzumab to augment the drug's ability to kill breast cancer cells.

"CIRM sees the potential in Fate's unique approach to developing cancer immunotherapies. Different cancers require different approaches that often involve a combination of treatments. Fate's NK cell product is distinct from the T cell immunotherapies that CIRM also funds and will allow us to broaden the arsenal of immunotherapies for incurable and devastating cancers," says Maria Millan.

Fate's NK cell product will be manufactured in large batches made from a master human iPSC line. This strategy would allow them to treat a large patient population with a characterized and uniform cell product.

The award Fate received is part of CIRM's late stage preclinical funding program, which aims to fund the final stages of research required to file an Investigational New Drug (IND) application with the US Food and Drug Administration. If the company is granted an

IND, it will be able to launch a clinical trial.

“Fate has more than a decade of experience in developing human iPSC-derived cell products,” said Scott Wolchko, President and CEO of Fate Therapeutics. “CIRM funding will enable us to complete our IND-enabling studies and the manufacturing of our clinical product. Our goal is to launch a clinical trial in 2019 using the City of Hope CIRM Alpha Stem Cell Clinic.”

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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