

Alzheimer's, Sickle Cell Anemia, Cartilage and Bone Disorders Get \$15 Million Research Boost from Stem Cell Agency

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Oakland, CA – Four research projects, focused on diseases that have an enormous impact on life expectancy and quality of life, have been awarded a total of \$15 million by the governing Board of the California Institute for Regenerative Medicine (CIRM).

The funding is part of the Translational Award program, which has a goal of moving the most promising projects out of the laboratory and into clinical trials in people.

Researchers at the Gladstone Institutes in San Francisco will use induced pluripotent stem cells (iPSCs) – mature human cells that have been genetically manipulated so they can become any other cell in the body – to generate brain cells to replace those damaged by Alzheimer's.

It's estimated that more than five million Americans have Alzheimer's and that number is expected to more than double in the next 30 years. It's a devastating disease that has no cure or long-term effective therapy.

"Our mission is to accelerate treatments to patients with unmet medical needs, and Alzheimer's clearly represents an unmet need," says C. Randal Mills, Ph.D., President & CEO of CIRM. "There's evidence this kind of stem cell can repair the damage caused by Alzheimer's in mice. We are hoping our funding will move the research along so that we can test this same approach in people."

At the Children's Hospital Research Institute in Oakland (CHORI), researchers want to use a new kind of genetic editing tool, CRISPR-Cas9, to help find a cure for sickle cell disease. This is a condition that affects around 100,000 people in the US, mostly African Americans, where red blood cells become distorted and break down, leading to intense pain, strokes and organ damage.

Sickle cell can be cured by a bone marrow transplant, but this is a risky procedure and most children don't have a sibling or bone marrow donor who is a good match for them. To get around that the researchers want to take the patient's own blood stem cells, use CRISPR-Cas9 to correct the genetic mutation causing the disease, then return those cells to the child. It's hoped the "corrected" blood stem cells will then multiply and create a new blood system, one free of sickle cell. The funding from CIRM will enable the CHORI team to do the preliminary testing and research needed to get this project ready for a clinical trial.

In the two other awards researchers at the University of Southern California hope to develop a universal, off-the-shelf stem cell product to repair damaged cartilage. Untreated cartilage problems can cause chronic pain, arthritis and often result in the need for joint replacement. And Ankasa Regenerative Therapeutics hopes to create a stem cell therapy for osteonecrosis, a painful, progressive disease that causes bones to rot and break.

"These Translational Awards highlight our goal of creating a pipeline of projects, moving through different stages of research with an ultimate goal of a successful treatment," says Jonathan Thomas, Ph.D., J.D., Chair of the CIRM Board. "We are hopeful these projects will be able to use our newly created Stem Cell Center to speed up their progress and pave the way for approval by the FDA for a clinical trial in the next few years."

APPLICATION	TITLE	INSTITUTION	ICOC COMMITTED FUNDING
TRAN1-09394	Human iPSC-derived GABAergic Progenitors for Alzheimer's Disease	Gladstone Institutes	\$5,944,681

TRAN1-09292	Curing Sickle Cell Disease with CRISPR-Cas9 Genome Editing	Children's Hospital Oakland Research Institute	\$4,463,435
TRAN1-09288	Pluripotent stem cell-derived chondrocytes for articular cartilage repair	University of Southern California	\$2,503,104
TRAN1-09270	An Autologous Somatic Stem Cell Therapy for the Treatment of Osteonecrosis	Ankasa Regenerative Therapeutics	\$2,088,780

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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