

California Stem Cell Agency Invests \$16.4 Million to Test Great New Ideas Targeting Cancer, Vision Loss and Parkinson's Disease.

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November 30, 2017 Oakland, CA Every life-saving therapy or advance in medicine begins with a scientist posing a simple question, "I wonder if...?" Today the governing Board of the California Institute for Regenerative Medicine (CIRM) invested more than \$16.4 million to help researchers find answers to questions that could one day lead to new therapies for leukemia, respiratory problems, and a devastating skin disease that affects children.

The CIRM Board approved \$3.02 million in funding for 14 projects in our Discovery Inception program. These are great new ideas that could have a big impact on the field of stem cell research but need some funding to help test those ideas and see if they work. The Board also invested \$13.4 million in funding for three projects at the Translation stage. These are programs that have already shown promise in early stage research. Under this award they can now do the work to advance it to the next level of development.

"This research is critically important in advancing our knowledge of stem cells and are the foundation for future therapeutic candidates and treatments," says Maria Millan, M.D., President and CEO of CIRM. "Exploring and testing new ideas increases the chances of finding treatments for patients with unmet medical needs. Without CIRM's support many of these projects might never get off the ground. That's why our ability to fund research, particularly at the earliest stage, is so important to the field as a whole."

The Translation stage projects include:

- \$5.56 million to Anthony Oro at Stanford to test a stem cell therapy to help people with a form of Epidermolysis bullosa, a painful, blistering skin disease that leaves patients with wounds that won't heal.
- \$5.15 million to Dan Kaufman at UC San Diego to produce natural killer (NK) cells from embryonic stem cells and see if they can help people with acute myelogenous leukemia (AML) who are not responding to treatment.
- \$2.7 million to Catriona Jamieson at UC San Diego to test a novel therapeutic approach targeting cancer stem cells in AML. These cells are believed to be the cause of the high relapse rate in AML and other cancers.

The Board also approved 14 projects in the Discovery Inception program, including one targeting Parkinson's disease. The project, by Zenobia Therapeutics, uses induced pluripotent stem cells (iPSCs) that have been turned into dopaminergic neurons – the kind of brain cell that is dysfunctional in Parkinson's disease. These iPSCs will then be used to screen drugs to see if any hold potential as a therapy for Parkinson's disease.

David Higgins, Ph.D., a patient advocate member for Parkinson's on the CIRM Board, said there is a real need for this research: "I'm a fourth generation Parkinson's patient and I'm taking the same medicine that my grandmother took. They work but not for everyone and not for long. People with Parkinson's need new treatment options and we need them now. That's why this project is worth supporting. It has the potential to identify some promising candidates that might one day lead to new treatments."

"We never know where the next great discovery or breakthrough in stem cell research is going to come from," says Dr. Millan. "What we do know is that if we don't fund these intriguing ideas and promising projects then nothing will happen. And we don't just provide money, we also provide support and guidance to the researchers when needed. We want to do everything we can to ensure all these projects reach their full potential."

The 14 projects funded under CIRM's Discovery Inception Award program are:

APPLICATION	TITLE	INSTITUTION	CIRM COMMITTED FUNDING
DISC1-10613	iPSC-Derived Smooth Muscle Progenitors for Treatment of Abdominal Aortic Aneurysm	Palo Alto Veterans Institute for Research (PAVIR)	\$172,621

DISC1-10475	Generation of human airway stem cells by direct transcriptional reprogramming for disease modeling and regeneration	U.C. San Francisco	\$238,408
DISC1-10643	IVD rejuvenation using iPSC-derived notochordal cells	Cedars-Sinai	\$241,992
DISC1-10598	Enhanced Branching Morphogenesis and Pluripotent Cell Lineage Differentiation for Pediatric Regenerative Therapies	U.C. Davis	\$235,800
DISC1-10583	Human Pancreatic Cancer Stem Cells: Developing a Novel Drug for Cancer Eradication	Human BioMolecular Research Institute	\$303,894
DISC1-10555	Optimizing self-renewal signaling kinetics to stabilize ex vivo hematopoietic stem cell expansion	Stanford	\$235,836
DISC1-10620	Bone Marrow Targeting of Hematopoietic Stem Cells Engineered to Overexpress 25-OH-VD ₃ 1- α -hydroxylase for Acute Myeloid Leukemia Therapy	Loma Linda University	\$178,967
DISC1-10513	Novel metabolic labeling method for tracking stem cells to irradiated salivary glands using PET	Stanford	\$235,613
DISC1-10522	Identification of antigenic neo-epitopes from in vitro reprogrammed human tissue precursors for regenerative therapy	U.C. San Diego	\$193,500
DISC1-10588	Targeting cancer stem cells with nanoparticle RNAi delivery to prevent recurrence and metastasis of ovarian cancer	Loma Linda University	\$172,870
DISC1-10721	An iPSC cell based model of macular degeneration for drug discovery.	U.C. San Diego	\$232,200
DISC1-10516	Development of treatments to improve healing of ischemic wounds	U.C. Davis	\$235,800
DISC1-10718	Gingival mesenchymal stem cells as a novel treatment modality for periodontal tissue regeneration	U.C. Los Angeles	\$194,483

DISC1-10674	A new phenotypic screening platform that identifies biologically-relevant targets and lead compounds for the treatment of Parkinson's disease	Zenobia Therapeutics	\$150,000

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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