

Stem Cell Agency Funds Clinical Trial Targeting Blood Cancers in Children and Young Adults

Posted: September 23, 2021

Oakland, CA – Hematologic malignancies, such as cancers of the blood, bone marrow and lymph nodes are the most common form of cancer in children and young adults. Current treatments can be effective but can also pose life-threatening health risks to the child. Now researchers at Stanford have developed a new approach and the Board of the California Institute for Regenerative Medicine (CIRM) voted today to support that approach in a clinical trial.

The Board approved investing \$11,996,634 in the study, which is the Stem Cell Agency's 76th clinical trial.

The current standard of care for cancers such as acute leukemias and lymphomas is chemotherapy and a bone marrow (also called HSCT) transplant. However, without a perfectly matched donor the risk of the patient's body rejecting the transplant is higher. Patients may also be at greater risk of graft vs host disease (GVHD), where the donor cells attack the patient's body. In severe cases GVHD can be life-threatening.

Dr. Maria Grazia Roncarolo and her team at Stanford will test an immunotherapy cell approach using a therapy that is enriched with specialized immune cells called type 1 regulatory T (Tr1) cells. These cells will be infused into the patient following the bone marrow transplant. Both the Tr1 cells and the bone marrow will come from the same donor. The hope is this will help provide the patient's immune system with these regulatory cells to combat life-threatening graft versus host disease and increase the success of treatment and bone marrow (HSCT) transplant.

"Every year around 500 children receive stem cell transplants in California, and while many children do well, too many experiences a rejection of the transplant or a relapse of the cancer," says Dr. Maria T. Millan, President and CEO of CIRM. "Finding an improved therapy for these children means a shorter stay in the hospital, less risk of the need for a second transplant, and a greater quality of life for the child and the whole family."

The CIRM Board also approved 18 programs, investing more than \$86 million, as part of the Agency's Research Training Grants program. The goal of the program is to create a diverse group of scientists with the knowledge and skill to lead effective stem cell research programs.

The awards provide up to \$5 million per institution over five years, to support the training of predoctoral graduate students, postdoctoral trainees, and/or clinical trainees.

This is a revival of an earlier Research Training program that ran from 2006-2016 and trained 940 "CIRM Scholars" including:

- 321 PhD students
- 453 Postdocs
- 166 MDs

The Research Training Grants go to:

AWARD	INSTITUTION	TITLE	AMOUNT
EDUC4-12751	Cedars-Sinai	<i>CIRM Training Program in Translational Regenerative Medicine</i>	\$4,999,333

EDUC4-12752	UC Riverside	<i>TRANSCEND – Training Program to Advance Interdisciplinary Stem Cell Research, Education, and Workforce Diversity</i>	\$4,993,115
EDUC4-12753	UC Los Angeles	<i>UCLA Training Program in Stem Cell Biology</i>	\$5 million
EDUC4-12756	University of Southern California	<i>Training Program Bridging Stem Cell Research with Clinical Applications in Regenerative Medicine</i>	\$5 million
EDUC4-12759	UC Santa Cruz	<i>CIRM Training Program in Systems Biology of Stem Cells</i>	\$4,913,271
EDUC4-12766	Gladstone Inst.	<i>CIRM Regenerative Medicine Research Training Program</i>	\$5 million
EDUC4-12772	City of Hope	<i>Research Training Program in Stem Cell Biology and Regenerative Medicine</i>	\$4,860,989
EDUC4-12782	Stanford	<i>CIRM Scholar Training Program</i>	\$4,974,073
EDUC4-12790	UC Berkeley	<i>Training the Next Generation of Biologists and Engineers for Regenerative Medicine</i>	\$4,954,238
EDUC4-12792	UC Davis	<i>CIRM Cell and Gene Therapy Training Program 2.0</i>	\$4,966,300
EDUC4-12802	Children's Hospital of Los Angeles	<i>CIRM Training Program for Stem Cell and Regenerative Medicine Research</i>	\$4,999,500

EDUC4-12804	UC San Diego	<i>Interdisciplinary Stem Cell Training Grant at UCSD III</i>	\$4,992,446	
EDUC4-12811	Scripps	<i>Training Scholars in Regenerative Medicine and Stem Cell Research</i>	\$4,931,353	
EDUC4-12812	UC San Francisco	<i>Scholars Research Training Program in Regenerative Medicine, Gene Therapy, and Stem Cell Research</i>	\$5 million	
EDUC4-12813	Sanford Burnham	<i>A Multidisciplinary Stem Cell Training Program at Sanford Burnham Prebys Institute, A Critical Component of the La Jolla Mesa Educational Network</i>	\$4,915,671	
EDUC4-12821	UC Santa Barbara	<i>CIRM Training Program in Stem Cell Biology and Engineering</i>	\$1,924,497	
EDUC4-12822	UC Irvine	<table border="1" data-bbox="797 1089 1162 1199"> <tr> <td><i>CIRM Scholars Comprehensive Research Training Program</i></td> </tr> </table>	<i>CIRM Scholars Comprehensive Research Training Program</i>	\$5 million
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EDUC4-12837	Lundquist Institute for Biomedical Innovation	<i>Stem Cell Training Program at the Lundquist Institute</i>	\$4,999,999	

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 \$5.5 billion in funding and more than 150 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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