Sickle Cell Disease Fact Sheet

CIRM funds many projects seeking to better understand sickle cell disease and to translate those discoveries into new therapies.

Description

Around 100,000 Americans have sickle cell disease and despite decades of research the average life expectancy has dropped from 42 in 1995 to 39 today. It is a disease that largely targets the African-American community and to a lesser degree the Hispanic community.

Sickle cell disease is a genetic disorder that causes red blood cells to assume a sickle shape under stress, clogging blood vessels and producing episodes of excruciating pain, called crises, and leading to progressive organ damage. By twenty years of age about 15 percent of people with sickle cell disease have had major strokes and by 40 almost half of the patients have significant mental dysfunction.

The most common recommendation for people with sickle cell disease is to stay hydrated. The more water a person drinks, the less likely it is that their abnormal blood cells will clog their blood vessels. Another effective treatment is a medication called hydroxyurea, which reduces crises by 50 percent and death by 40 percent, but most adults are not treated. The populations most effected by sickle cell disease also suffer from significant health care disparities, which lower the quality of care they receive for their disease.

Bone marrow transplants are used to treat children with the most severe cases of the disease. In fact one of CIRM's former Board members, the late Bert Lubin, MD, the President and CEO of Children's Hospital and Research Center Oakland, has been a leader in developing this therapy for kids with sickle cell disease (his bio is here). The replacement bone marrow cells generate an entirely new blood system for the patient. However, bone marrow transplants are extremely risky and require a matched sibling donor and even under the best conditions there is always the risk of rejection.

Research funded by California's stem cell agency focuses on making bone marrow transplants safer and more effective for treating people with sickle cell disease. In one project, the researchers intend to remove bone marrow from the patient and fix the genetic defect in the blood-forming stem cells. Then those cells can be reintroduced into the patient to create a new, healthy blood system. Because the cells come from the patient this technique avoids the issue of rejection. Other researchers are developing ways of making bone marrow transplants safer.

Clinical Stage Programs

University of California, Los Angeles

This team of researchers plans to remove bone marrow cells from people with sickle cell disease and fix the genetic mutation that causes the disease. The team will then reintroduce the new cells into the patient. Those cells will then generate new, healthy blood cells.

- Read more about this project

City of Hope, Duarte

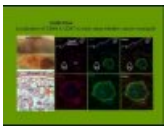
Scientists at the City of Hope are going to transplant blood-forming stem cells from a donor into a patient who has received a milder, less toxic chemotherapy treatment that removes some but not all of the patient's diseased bone marrow stem cells. This allows the donor stem cells to engraft and create a healthy supply of non-diseased blood cells without causing an immune reaction in the patient. The hope is that this treatment will cure patients with more severe forms of SCD who aren't able to benefit from currently available blood stem cell transplants that require the administration of more toxic chemotherapy drugs.

- Read more about this project

CIRM Grants Targeting Sickle Cell Disease

Researcher name	Institution	Grant Title	Grant Type	Award Amount	
Mark Walters	University of California, San Francisco	Curing Sickle cell Disease with CRISPR-Cas9 genome editing	Late Stage Preclinical Projects	$2,242,805	
Mark Walters	University of California, San Francisco	Curing Sickle cell Disease with CRISPR-Cas9 genome editing	Therapeutic Translational Research Projects	$60,635	
Pierre Caudrelier	ExCellThera Inc.	A Phase 1 Study of ECT-001 Expanded Cord Blood and Myeloablative Regimen with Reduced Toxicity in Patients with Severe Sickle Cell Disease.	Cure Sickle Cell Initiative Clinical Trial Stage Projects	$600,000	
David Williams	Boston Children's Hospital	Phase 2 Study of Hematopoietic Stem Cell Gene Transfer Inducing Fetal Hemoglobin in Sickle Cell Disease	Cure Sickle Cell Initiative Clinical Trial Stage Projects	$8,333,581	
Steven Mack	University of California, San Francisco	Development of a Noninvasive Prenatal Test for Beta-Hemoglobinopathies for Earlier Stem Cell Therapeutic Interventions	Diagnostic Translational Research Projects	$633,014	
Mark Walters	University of California, San Francisco	Transplantation of CRISPR-CAS9 Corrected Hematopoietic Stem Cells (CRISPR_SCD001) in Patients with Severe Sickle Cell Disease	Cure Sickle Cell Initiative Clinical Trial Stage Projects	$8,389,407	
Donald Kohn	University of California, Los Angeles	Stem Cell Gene Therapy for Sickle Cell Disease	Disease Team Research I	$8,833,695	
Donald Kohn	University of California, Los Angeles	Beta-Globin Gene Correction of Sickle Cell Disease in Hematopoietic Stem Cells	Early Translational IV	$1,651,884	
Donald Kohn	University of California, Los Angeles	Clinical Trial of Stem Cell Gene Therapy for Sickle Cell Disease	Disease Team Therapy Development III	$13,145,465	
Donald Kohn	Children's Hospital of Los Angeles	STEM CELL GENE THERAPY FOR SICKLE CELL DISEASE	Disease Team Planning	$12,131	
Mark Walters	UCSF Benioff Children's Hospital Oakland	Curing Sickle cell Disease with CRISPR-Cas9 genome editing	Therapeutic Translational Research Projects	$4,394,276	
Matthew Porteus	Stanford University	Genome Editing of Autologous Hematopoietic Stem Cells to Treat Sickle Cell Disease	Late Stage Preclinical Projects	$4,849,363	
Henry Erlich	UCSF Benioff Children's Hospital Oakland	Development of a Noninvasive Prenatal Test for Beta-Hemoglobinopathies for Earlier Stem Cell Therapeutic Interventions	Diagnostic Translational Research Projects	$1,074,177	
Joseph Rosenthal	City of Hope, Beckman Research Institute	Treatment of sickle cell disease by induction of mixed chimerism and immune tolerance using CD4+ T-depleted haploidentical blood stem cell transplant	Clinical Trial Stage Projects	$4,352,180	
					Total: $58,572,613.00

CIRM Blood Disease Videos

 **CURED: Stem Cell Clinical Trial Stories**	 **Defeating Sickle Cell Disease with Stem Cells + Gene Therapy**	 **Spotlight on Amyloidosis and Stem Cell Research: Robert Vescio MD - Cedars-Sinai**	 **Michael York: Amyloidosis and Stem Cell Research**
 **William Kim, UCLA - CIRM Stem Cell #SciencePitch**	 **Catriona Jamieson, UCSD - CIRM Stem Cell #SciencePitch**	 **Spotlight on Genomics: Understanding Our Genes**	 **Catriona Jamieson - UCSD | CIRM Spotlight on Genomics**
 **Spotlight on Genomics: Clinical Trial for Myelofibrosis that Targets Cancer Stem Cells**	 **Stem Cell Gene Therapy for Sickle Cell Anemia - Donald Kohn**	 **Spotlight on Basic Research: Irv Weissman**	 **Spotlight on Leukemia: Welcoming Remarks**
 **Spolight on Leukemia: Catriona Jamieson, M.D.**	 **Spotlight on Leukemia: Clinical Trial Participants**	 **Progress and Promise in Leukemia**	

Resources

- NIH: What is Sickle Cell Anemia?
- CDC: Sickle Cell Information
- Find a clinical trial near you: NIH Clinical Trials database
- Sickle Cell Disease Association of America
- Sickle Cell Disease Foundation of California
- American Sickle Cell Anemia Association

Find Out More:
Stem Cell FAQ | Stem Cell Videos | What We Fund

Source URL: https://www.cirm.ca.gov/our-progress/disease-information/sickle-cell-disease-fact-sheet