

## Leukemia Fact Sheet

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

### Description

Leukemia is a cancer of the blood cells. Although leukemia is among the most common childhood cancers, it most often occurs in older adults and is slightly more common in men than women. In 2016 there were an estimated 60,000 new cases with around 24,000 people dying of the disease.

The stem cells in the bone marrow normally form all cells of the blood system, including the red blood cells, platelets, and immune cells. In people with leukemia, the bone marrow stem cells create abnormal immune cells that aren't able to carry out their normal job of fighting infection.

Eventually, these abnormal cells crowd out the normal blood cells. Without a sufficient population of working blood cells, people with leukemia develop symptoms such as anemia, bleeding and infections.

Recent research has shown that in addition to these abnormal white cells, leukemia patients also have a small population of cells called leukemia stem cells. Scientists suspect that these cells evade treatments that kill leukemia cells and then later go on to cause a relapse. The goal of stem cell research for leukemia is to find ways of destroying these leukemia stem cells.

**Catriona Jamieson of the UC San Diego Moores Cancer Center discusses a clinical trial for a pre-leukemia condition that was based in part on CIRM funding**

### Clinical Stage Programs

#### University of California, San Diego

A team at UC San Diego is testing the safety of a monoclonal antibody called cirmtuzumab that targets cancer stem cells in a Phase 1

clinical trial. It's named after CIRM because we helped fund the research that led to its development. Cirmtuzumab is designed to attach to a protein, called ROR1, that is found on the surface of chronic lymphocytic leukemia (CLL) cells but is rarely found on healthy cells. The team hopes cirmtuzumab will target the cancer cells, blocking their ability to grow and/or survive.

- Read a summary of this project
- Learn more about this clinical trial

The same UCSD team is also testing cirmtuzumab in combination with an approved cancer fighting drug call Ibrutinib, to target cancer stem cells in a separate Phase 1 trial. The hope is that combining Cirmtuzumab with Ibrutinib will improve cancer remission and long-term cancer control in patients.

- Read a summary of this project
- Learn more about this clinical trial

## CIRM Grants Targeting Leukemia











Researcher name	Institution	Grant Title	Grant Type	Award Amount
Catriona Jamieson	University of California, San Diego	Preclinical development of a pan Bcl2 inhibitor for cancer stem cell directed therapy	Early Translational II	\$3,103,041
John Chute	University of California, Los Angeles	Niche-Focused Research: Discovery & Development of Hematopoietic Regenerative Factors	Research Leadership	\$4,645,297
Markus Muschen	University of California, San Francisco	Dual targeting of tyrosine kinase and BCL6 signaling for leukemia stem cell eradication	Early Translational II	\$2,756,536
David Baylink	Loma Linda University	Bone Marrow Targeting of Hematopoietic Stem Cells Engineered to Overexpress 25-OH-VD3 1- $\alpha$ -hydroxylase for Acute Myeloid Leukemia Therapy	Inception - Discovery Stage Research Projects	\$178,967
Dennis Carson	University of California, San Diego	Development of Highly Active Anti-Leukemia Stem Cell Therapy (HALT)	Disease Team Research I	\$19,999,826
Jacob Corn	University of California, Berkeley	Genome editing for causation and reversion of MPN-associated mutations in human hematopoietic stem cells	Inception - Discovery Stage Research Projects	\$235,800
Tannishtha Reya	University of California, San Diego	Targeting Cancer Stem Cells in Hematologic Malignancies	Quest - Discovery Stage Research Projects	\$1,960,560
Irving Weissman	Stanford University	Development of Therapeutic Antibodies Targeting Human Acute Myeloid Leukemia Stem Cells	Disease Team Research I	\$18,759,276
Dan Kaufman	University of California, San Diego	Targeted off-the-shelf immunotherapy to treat refractory cancers	Quest - Discovery Stage Research Projects	\$1,936,936
David Miklos	Stanford University	Phase 1 Study of CD19/CD22 Chimeric Antigen Receptor (CAR) T Cells in Adults with Recurrent or Refractory B Cell Malignancies	Clinical Trial Stage Projects	\$11,034,982

Catriona Jamieson	University of California, San Diego	Derivation and Characterization of Myeloproliferative Disorder Stem Cells from Human ES Cells	New Faculty II	\$3,065,572
John Chute	University of California, Los Angeles	Protein tyrosine phosphatase - sigma inhibitors for hematopoietic regeneration	Quest - Discovery Stage Research Projects	\$2,116,708
Emmanuelle Passegue	University of California, San Francisco	Mechanisms Underlying the Responses of Normal and Cancer Stem Cells to Environmental and Therapeutic Insults	New Faculty II	\$2,124,488
Paul Finnegan	Angiocrine Bioscience, Inc.	Development of AB-110: genetically-modified endothelial cells plus expanded cord blood hematopoietic stem cells as a transplantation therapy	Late Stage Preclinical Projects	\$3,797,117
Edward Kavalerschik	Angiocrine Bioscience, Inc.	AB-205-001 Phase 1b Trial and Related Activities to Support Clinical Development of AB-205	Clinical Trial Stage Projects	\$6,192,579
Hanna Mikkola	University of California, Los Angeles	Mechanisms of Hematopoietic stem cell Specification and Self-Renewal	New Faculty I	\$2,286,900
Markus Muschen	City of Hope, Beckman Research Institute	Lgr5-mediated self-renewal in B cell selection and leukemia-initiation	Quest - Discovery Stage Research Projects	\$2,186,520
Chong-Xian Pan	University of California, Davis	Combinatorial Chemistry Approaches to Develop Ligands against Leukemia Stem Cells	New Faculty I	\$2,386,409
Swapna Panuganti	Cellerant Therapeutics, Inc.	Development of CLT030-ADC, a Leukemic Stem Cell Targeting Antibody-Drug-Conjugate, for Treatment of Acute Myeloid Leukemia	Late Stage Preclinical Projects	\$6,863,755
Joseph Woodard	Immune-Onc Therapeutics	Phase 1 Clinical Development of IO-202, A First-in-Class Antibody Targeting LILRB4, for the Treatment of AML with Monocytic Differentiation and CMML	Clinical Trial Stage Projects	\$6,000,000
Mehrdad Abedi	University of California, Davis	Stem Cell Gene Therapy for HIV in AIDS Lymphoma Patients	Disease Team Therapy Planning I	\$66,880
Michael Cleary	Stanford University	Prostaglandin pathway regulation of self-renewal in hematopoietic and leukemia stem cells	Basic Biology IV	\$1,244,455
Edward Kavalerschik	Angiocrine Bioscience, Inc.	AB-110-001 Phase 1b Trial and Related Activities to Support Clinical Development of AB-110	Clinical Trial Stage Projects	\$5,000,000
Maria Grazia Roncarolo	Stanford University	Phase 1/1b study of T-allo10 infusion after HLA-partially matched abdepleted-HSCT in children and young adults with hematologic malignancies.	Clinical Trial Stage Projects	\$10,563,822
Ann Zovein	University of California, San Francisco	Human endothelial reprogramming for hematopoietic stem cell therapy.	New Faculty Physician Scientist	\$2,197,683

Thomas Kipps	University of California, San Diego	A Phase 1b/2a Study of the ROR1-Targeting Monoclonal Antibody, Cirmtuzumab, and the Bruton Tyrosine Kinase Inhibitor, Ibrutinib, in B-Cell Cancers	Clinical Trial Stage Projects	\$18,292,674
Ezra Cohen	University of California, San Diego	Late Stage Pre-Clinical Development of a Cirmtuzumab Based CAR T-cell for the Treatment of ROR1+ Hematological Malignancies	Late Stage Preclinical Projects	\$4,130,260
Thomas Kipps	University of California, San Diego	Therapeutic Eradication of Cancer Stem Cells with UC-961 (Cirmtuzumab)	Disease Team Therapy Development III	\$4,179,598
Colleen Delaney	Nohla Therapeutics Inc	A Phase 2 Open-Label, Multi-Center, Randomized, Controlled, Optimal Dose-Finding Study of DCC-UCB in Adults Receiving High Dose Chemotherapy for AML	Clinical Trial Stage Projects	\$4,310,000
Arun Wiita	University of California, San Francisco	CD72 nanoCARs for the treatment of refractory pediatric B-cell acute lymphoblastic leukemia	Therapeutic Translational Research Projects	\$3,330,801
Irving Weissman	Stanford University	Clinical Investigation of a Humanized Anti-CD47 Antibody in Targeting Cancer Stem Cells in Hematologic Malignancies and Solid Tumors	Disease Team Therapy Development III	\$6,505,568
Mark Chao	Forty Seven Inc.	Phase 1b Trial of Hu5F9-G4 Monotherapy or Hu5F9-G4 in Combination with Azacitidine in Patients with Acute Myeloid Leukemia and Myelodysplastic Syndrome	Clinical Trial Stage Projects	\$0
Julia Carnevale	University of California, San Francisco	Generating deeper and more durable BCMA CAR T cell responses in Multiple Myeloma through non-viral knockin/knockout multiplexed genome engineering	Quest - Discovery Stage Research Projects	\$1,463,368
Markus Muschen	Children's Hospital of Los Angeles	Dual targeting of tyrosine kinase and BCL6 signaling for leukemia stem cell eradication	Early Translational II	\$850,769
Michael Pulsipher	Children's Hospital of Los Angeles	Antiviral Cellular Therapy for Enhancing T-cell Reconstitution Before or After Hematopoietic Stem Cell Transplantation (ACES)	Clinical Trial Stage Projects	\$4,825,587
Catriona Jamieson	University of California, San Diego	Derivation and Characterization of Cancer Stem Cells from Human ES Cells	SEED Grant	\$616,305
Matthew Spear	Poseida Therapeutics, Inc.	Clinical Study of T stem cell memory (Tscm)-based CAR-T cells in Patients with Multiple Myeloma	Clinical Trial Stage Projects	\$19,813,407
Mehrdad Abedi	University of California, Davis	Stem Cell Gene Therapy for HIV Mediated by Lentivector Transduced, Pre-selected CD34+ Cells in AIDS lymphoma patients	Clinical Trial Stage Projects	\$8,414,265
Dan Kaufman	University of California, San Diego	Human Embryonic Stem Cell-Derived Natural Killer Cells for Cancer Treatment	Therapeutic Translational Research Projects	\$4,698,821

Lili Yang	University of California, Los Angeles	Stem Cell-Based iNKT Cell Therapy for Cancer	Therapeutic Translational Research Projects	\$6,956,775	
Karin Gaensler	University of California, San Francisco	Developing engineered autologous leukemia vaccines to target residual leukemic stem cells	Therapeutic Translational Research Projects	\$4,171,728	
Yvonne Chen	University of California, Los Angeles	BCMA/CS1 Bispecific CAR-T Cell Therapy to Prevent Antigen Escape in Multiple Myeloma	Therapeutic Translational Research Projects	\$3,176,805	
Ezra Cohen	University of California, San Diego	Development of ROR1 CAR-T cells to target cancer stem cells in advanced malignancies	Therapeutic Translational Research Projects	\$5,795,584	
Lili Yang	University of California, Los Angeles	HSC-Engineered Off-The-Shelf CAR-iNKT Cell Therapy for Multiple Myeloma	Therapeutic Translational Research Projects	\$5,281,199	
Catriona Jamieson	University of California, San Diego	A Splicing Modulator Targeting Cancer Stem Cells in Acute Myeloid Leukemia	Therapeutic Translational Research Projects	\$2,511,767	
					Total: \$230,029,390.00

## CIRM Leukemia Videos

 <p><b>Stem Cell Therapies for Leukemia: Marching Toward the Clinic</b></p>	 <p><b>Anica Sayoc, City of Hope - CIRM Stem Cell #SciencePitch</b></p>	 <p><b>Catriona Jamieson, UCSD - CIRM Stem Cell #SciencePitch</b></p>	 <p><b>Irving Weissman, Stanford - CIRM Stem Cell #SciencePitch</b></p>
 <p><b>Leukemia: Advancing Stem Cell Therapies - 2011 CIRM Grantee Meeting</b></p>	 <p><b>Spotlight on Basic Research: Irv Weissman</b></p>	 <p><b>Spotlight on Leukemia: Welcoming Remarks</b></p>	 <p><b>Spotlight on Leukemia: Catriona Jamieson, M.D.</b></p>
 <p><b>Spotlight on Leukemia: Clinical Trial Participants</b></p>	 <p><b>Progress and Promise in Leukemia</b></p>		

## News and Information

- CIRM Stem Cellar blogs on blood cancer

- Leukemia under the microscope (San Diego Union Tribune)
- Bad Seeds: Cancer's Ultimate Source (Stanford Medicine)
- The True Seeds of Cancer (Stanford Medicine)
- From Bench to Bedside in a Year (UC San Diego)
- Living with Leukemia: Theresa Blanda (CIRM)

## Resources

- NIH: Leukemia information
- Find a clinical trial near you: NIH Clinical Trials database
- Leukemia and Lymphoma Society
- Leukemia Research Foundation
- Stem Cell Network blood cancers page
- Family Caregiver Alliance
- National Family Caregivers Association

## Find Out More:

[Stem Cell FAQ](#) | [Stem Cell Videos](#) | [What We Fund](#)

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