Blindness Fact Sheet

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

Description

Over a million Americans are legally blind, with another 3.2 million suffering significant visual impairment [1]. While there are several causes of blindness, the leading cause of all visual impairment is age-related macular degeneration, which affects 1.7 million Americans.

California's stem cell agency funds research into potential therapies for three of the causes of blindness. All the research teams are seeking to use various forms of stem cells to rescue or replace cells in the eye damaged or threatened by the diseases. Several groups are working on ways to restore vision for people with age-related macular degeneration (AMD). Other projects are looking to preserve vision in patients with retinitis pigmentosa (RP), and to restore clarity to the surface of eyes impacted by corneal disease.

Macular Degeneration

AMD affects 8 million Americans and accounts for over 50% of vision loss cases in white Americans. In AMD, the layer of cells that support the photoreceptors is destroyed. Without this support system, the photoreceptors, the cells that actually allow us to sense light start to malfunction. CIRM-funded teams are looking at various methods of replacing this layer of support cells called RPE (retinal pigment epithelial) cells. Some are using embryonic stem cells as a starting point to generate new RPE cells. Others are using stem cells obtained by reprogramming adult cells to be like embryonic cells, which could potentially come from the patients' themselves.

Retinitis Pigmentosa

Retinitis pigmentosa (RP) is an inherited and progressive vision loss disease that has an incidence of 1:4000 and leaves most patients legally blind by mid-life. RP destroys the light-sensing photoreceptors in the retina. CIRM-funded researchers are seeking to use stem cells to rescue these photoreceptors from further damage and potentially replace them with new ones.

Limbal Stem Cell Deficiency

The cornea, the outer surface of the eye, is constantly refreshed by stem cells that reside in neighboring tissue. But some people just don't have enough of these stem cells, called limbal stem cells, to make enough new cornea cells. CIRM-funded researchers are trying to correct this condition, limbal stem cell deficiency, by retrieving the few existing limbal stem cells, and using various techniques to expand them in the laboratory until there are enough cells to rebuild a healthy cornea.

Clinical Stage Programs

University of Southern California

This team is using embryonic stem cells to produce the support cells, or RPE cells, needed to replace those lost in AMD. Because these cells exist in a thin sheet in the back of the eye, they are assembling these sheets in the lab by growing the RPE cells on synthetic scaffolds. These sheets are then surgically implanted into the eye. They are testing the human embryonic stem cell-derived RPE cells in a Phase 1/2a clinical trial to treat the advanced dry form of AMD.

- Read more about this clinical program
- Learn more about this trial on clinicaltrials.gov

University of California, Los Angeles

This team, led by Sophie Deng, is taking the patient's owns limbal stem cells and expanding their number in the laboratory, then returning them to the patient in the hope they will repair the damage caused by the disease and help restore vision or at least halt the progression of the disease. They are testing this in a Phase 1 clinical trial. They are also using novel diagnostic methods to assess the severity of the disease and the patient's response to treatment.

- Learn more about this clinical trial

University of California, Irvine

For retinitis pigmentosa, the team is using donor tissue to isolate cells that are part way down the path from neural stem cells to adult eye tissue. These retinal progenitor cells are grown in large quantities in the lab and then injected into the eye. The team suggests the cells could help in two ways. They may be able to protect the photoreceptors not yet damaged by the disease, and they may be able to form new photoreceptors to replace those already lost. The team tested the safety of transplanting human retinal progenitor cells into patients with RP in a phase 1/2 clinical trial that is now completed. CIRM is now funding a Phase 2 trial, sponsored by a jCyte, that is testing this treatment in a larger group of RP patients (see trial below).

- Read more about this clinical program
- Learn more about this trial on clinicaltrials.gov

jCyte

The same team from UC Irvine is now conducting a Phase 2b clinical trial for retinitis pigmentosa using the same stem cell derived retinal progenitor cell therapy. The trial, which is sponsored by the company jCyte, will test the treatment in a larger patient population to determine whether the treatment is effective at restoring some vision. After finishing patient enrollment, the team will conduct patient follow up studies and collect of all clinical outcome measures.

- Read more about this clinical program
- Learn more about this trial on clinicaltrials.gov

CIRM Grants Targeting Vision Loss

Researcher name	Institution	Grant Title	Grant Type	Award Amount	
Deepak Lamba	Buck Institute for Age Research	3D Modeling of Retina using Polymer Scaffolds for Understanding Disease Pathogenesis	Basic Biology IV	$1,212,553	
Steven Schwartz	University of California, Los Angeles	Clinical Translation of Autologous Regenerative Cell Therapy for Blindness	Therapeutic Translational Research Projects	$5,068,026	

Kang Zhang	University of California, San Diego	Generation of fibroblast cell lines in patients with common blinding eye diseases	Tissue Collection for Disease Modeling	$1,034,425
Theodore Leng	Stanford University	NeuBright, a purified allogeneic cell therapy product for treatment of Dry Age-related Macular Degeneration	Therapeutic Translational Research Projects	$4,235,758
Magdalene Seiler	University of California, Irvine	Restoring vision by sheet transplants of retinal progenitors and retinal pigment epithelium (RPE) derived from human embryonic stem cells (hESCs)	Early Translational IV	$3,998,948
Mark Humayun	University of Southern California	PRPE-SF, polarized hESC-derived RPE Soluble Factors, as a Therapy for Early Stage Dry Age-related Macular Degeneration	Therapeutic Translational Research Projects	$3,733,556
Mark Humayun	University of Southern California	Phase 1 Safety Assessment of CPCB-RPE1, hESC-derived RPE Cell Coated Parylene Membrane Implants, in Patients with Advanced Dry Age Related Macular Degeneration	Disease Team Therapy Development III	$16,339,827
Henry Klassen	jCyte, Inc	A Phase 2 Study of the Safety of Repeat Intravitreal Injection of Human Retinal Progenitor Cells (jCell) in Adult Subjects with Retinitis Pigmentosa	Clinical Trial Stage Projects	$6,608,592
Mark Humayun	University of Southern California	Stem cell based treatment strategy for Age-related Macular Degeneration (AMD)	Disease Team Planning	$3,088
Clive Svendsen	Cedars-Sinai Medical Center	Clinical Study to Assess Safety and Efficacy of Subretinal Injection of Human Neural Progenitor Cells for Treatment of Retinitis Pigmentosa	Clinical Trial Stage Projects	$10,444,063
Sophie Deng	University of California, Los Angeles	Regeneration of Functional Human Corneal Epithelial Progenitor Cells	Early Translational II	$697,507
Sophie Deng	University of California, Los Angeles	Safety and Feasibility of Cultivated Autologous Limbal Stem Cells for Limbal Stem Cell Deficiency	Clinical Trial Stage Projects	$10,301,486
David Schaffer	University of California, Berkeley	Engineered Biomaterials for Scalable Manufacturing and High Viability Implantation of hPSC-Derived Cells to Treat Neurodegenerative Disease	Tools and Technologies III	$1,239,276
Shaomei Wang	Cedars-Sinai Medical Center	IND-enabling study of subretinal delivery of human neural progenitor cells for the treatment of retinitis pigmentosa	Late Stage Preclinical Projects	$4,954,514
Karl Wahlin	University of California, San Diego	Microenvironment based optimization of retinal induction using CRISPR-CAS9 reporter pluripotent stem cells as an expandable source of retinal progenitors and photoreceptors.	Inception - Discovery Stage Research Projects	$232,200
Sophie Deng	University of California, Los Angeles	Regeneration of Functional Human Corneal Epithelial Progenitor Cells	Early Translational II	$1,524,947

Henry Klassen	University of California, Irvine	Human retinal progenitor cells as candidate therapy for retinitis pigmentosa	Early Translational II	$1,803,768	
Jeffrey Goldberg	Stanford University	Embryonic Stem Cells for Corneal Endothelial Degeneration	Inception - Discovery Stage Research Projects	$235,836	
Mark Humayun	University of Southern California	Stem cell based treatment strategy for Age-related Macular Degeneration (AMD)	Disease Team Research I	$18,904,916	
Sophie Deng	University of California, Los Angeles	Regeneration of a Normal Corneal Surface by Limbal Stem Cell Therapy	Late Stage Preclinical Projects	$4,244,211	
David Hinton	University of Southern California	Therapeutic potential of Retinal Pigment Epithelial cell lines derived from hES cells for retinal degeneration.	SEED Grant	$651,607	
Henry Klassen	jCyte, Inc	Phase 2b Clinical Study of Safety and Efficacy of Intravitreal Injection of Retinal Progenitor Cells (jCell) for Treatment of Retinitis Pigmentosa	Clinical Trial Stage Projects	$8,295,750	
Martin Friedlander	Scripps Research Institute	Autologous Retinal Pigmented Epithelial Cells Derived from Induced Pluripotent Stem Cells for the Treatment of Atrophic Age Related Macular Degeneration	Early Translational I	$5,806,321	
Biju Thomas	University of Southern California	A Novel Tissue Engineering Technique to Repair Degenerated Retina	Inception - Discovery Stage Research Projects	$215,133	
Gabriel Travis	University of California, Los Angeles	Development of a Stem Cell-based Transplantation Strategy for Treating Age-related Macular Degeneration	Early Translational I	$5,487,136	
Karl Wahlin	University of California, San Diego	An IPSC cell based model of macular degeneration for drug discovery.	Inception - Discovery Stage Research Projects	$232,200	
Peter Coffey	University of California, Santa Barbara	Development of Cellular Therapies for Retinal Disease	Research Leadership	$4,690,963	
Magdalene Seiler	University of California, Irvine	Morphological and functional integration of stem cell derived retina organoid sheets into degenerating retina models	Therapeutic Translational Research Projects	$4,769,039	
Henry Klassen	University of California, Irvine	Retinal progenitor cells for treatment of retinitis pigmentosa	Disease Team Therapy Development - Research	$17,144,825	
Jonathan Lin	University of California, San Diego	Small Molecule Proteostasis Regulators to Treat Photoreceptor Diseases	Quest - Discovery Stage Research Projects	$1,160,648	
					Total: $145,271,119.00

CIRM Videos about Vision Loss

 Stem Cell Clinical Trial for Retinitis Pigmentosa: Rosie's Story	 Eyeing Stem Cell Therapies for Vision Loss	 Masayo Takahashi - 2015 Winner of Ogawa-Yamanaka Stem Cell Prize	 A Stem Cell-Based Clinical Trial for Retinitis Pigmentosa: Henry Klassen, UC Irvine
 A Stem Cell-Based Therapy for Retinitis Pigmentosa: The Patient's Perspective	 Hossein Nazari, USC - CIRM Stem Cell #SciencePitch	 Jacqueline Ward, UCSD - CIRM Stem Cell #SciencePitch	 Mark Humayun, USC - CIRM Stem Cell #SciencePitch
Webinar: Focus on the Eye	 Blindness: Advancing Stem Cell Therapies - 2011 CIRM Grantee Meeting	 Cures through Collaboration: Funding a Team Approach to Disease Research	 Video - Spotlight on Stem Cell Research: Devic's Disease (NMO) - Introduction
 Video - Spotlight on Stem Cell Research: Devic's Disease (NMO) - Victoria Jackson	 Video - Spotlight on Stem Cell Research: Devic's Disease (NMO) - Michael Yeaman	 Video - Spotlight on Stem Cell Research: Devic's Disease (NMO) - Benjamin Greenberg	 Video - Spotlight on Stem Cell Research: Devic's Disease (NMO) - Candace Coffee
 Progress and Promise in Macular Degeneration	 Spotlight on Macular Degeneration: Welcoming Remarks	 Spotlight on Macular Degeneration: Seminar by David Hinton, M.D.	 Spotlight on Macular Degeneration: Seminar by Mark Humayun, M.D., Ph.D.
 Spotlight on Macular Degeneration: Seminar by Sharon Hayes	 Stem Cell Based Therapies for Blindness: David Hinton - CIRM Science Writer's Seminar		

News and Information

- *The Stem Cellar*'s entries on blindness
- Stories of Hope: Macular Degeneration
- Sights on a Cure: Stem cell scientists have macular degeneration in the crosshairs (CIRM)
- Living with Macular Degeneration: Sharon Hayes (CIRM)

Resources

- National Eye Institute: Macular Degeneration Facts
- National Eye Institute: Retinitis Pigmentosa
- Find a clinical trial near you: NIH Clinical Trials database
- Macular Degeneration Association
- American Macular Degeneration Foundation
- The Macula Foundation
- Foundation Fighting Blindness
- Lighthouse for the Blind
- Family Caregiver Alliance
- National Family Caregivers Association

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

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