

Stem Cell Agency Funded Therapy for Blinding Disease Treats First Patients

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San Francisco, CA – A clinical trial using stem cells to treat people who have lost their vision due to retinitis pigmentosa (RP) has treated its first four patients. CIRM, California's stem cell agency, is funding the trial, which has FDA consent.

"We are delighted to be moving into the clinic after many years of bench research," says Dr. Henry Klassen, the project director at the University of California, Irvine (UCI).

The four patients are legally blind because of retinitis pigmentosa, a degenerative disease that slowly destroys cells in the retina, the light sensitive area in the back of the eye that is essential for vision. The patients were each given a single injection of retinal progenitor cells. It's hoped these cells will help protect the photoreceptors in the retina that have not yet been damaged by RP, and even revive those that have been damaged but not yet destroyed by the disease.

"This is an important step for Dr. Klassen and his team, and hopefully an even more important one for people battling this devastating disease," says Jonathan Thomas, Ph.D., J.D., Chair of the Board of CIRM. "Our mission at CIRM is to accelerate the development of stem cell therapies for patients with unmet medical needs, and this certainly fits that bill. That's why we have invested almost \$19 million in helping this therapy reach this point."

Altogether 16 patients will be enrolled in this first phase. All will receive a single injection of retinal progenitor cells into the most affected eye. The patients will then be followed for 12 months to determine if the therapy is safe and if it has any beneficial effects on vision compared to the untreated eye.

The treatments are being administered at the Gavin Herbert Eye Institute in Irvine and at Retina Vitreous Associates in Los Angeles. This is one of the first clinical trials being carried out as part of CIRM's new Alpha Stem Cell Clinic Network, which brings together experts in developing and delivering these new therapies to patients.

Worldwide almost 1.5 million people suffer from RP. It is the leading cause of inherited blindness in the developed world. There is no cure and no effective treatment.

To learn more about the study or to enroll contact the UCLA-UCI Alpha Stem Cell Clinic at 949-824-3990 or by email at stemcell@uci.edu. Or for more information go here: <https://www.stemcell.ucla.edu/cirm-ucla-uci-alpha-stem-cell-clinic>

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 to act with a sense of urgency commensurate with 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 over 280 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of medicine closer to reality.

For more information visit www.cirm.ca.gov

About the University of California, Irvine: Currently celebrating its 50th anniversary, UCI is the youngest member of the prestigious Association of American Universities. The campus has produced three Nobel laureates and is known for its academic achievement, premier research, innovation and anteater mascot. Led by Chancellor Howard Gillman, UCI has more than 30,000 students and offers 192 degree programs. It's located in one of the world's safest and most economically vibrant communities and is Orange County's second-largest employer, contributing \$4.8 billion annually to the local economy. For more on UCI, visit www.uci.edu.

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