

NEWS



Research to
Prevent Blindness

FALL 2022

An RPB Awardee Tackles Inherited Retinal Diseases

Many inherited diseases—across all areas of the body—are caused by gene mutations that are passed down from generation to generation as part of a person's DNA. RPB Stein Innovation Awardee **Krzysztof Palczewski, PhD**, develops and applies cutting-edge gene editing techniques to challenging genetic conditions.

Dr. Palczewski, who is the Donald Bren Professor, Irving H. Leopold Chair of Ophthalmology, and Distinguished Professor at the University of California, Irvine, School of Medicine, has advanced a new generation of gene editing technology, referred to as base editing, as a therapeutic approach for Leber congenital amaurosis (LCA). LCA is the most common cause of inherited retinal degeneration in children, which results in early visual impairment. Dr. Palczewski and his team have made incredible progress since receiving his RPB grant in 2020.

Dr. Palczewski's research has recently been published in *Cell*,



Dr. Krzysztof Palczewski (center) and his lab members Elliot Choi and Susie Suh pioneered new gene editing techniques for inherited retinal diseases.

Nature Biomedical Engineering, *PNAS*, and *Nature Communications*, highly respected and ranked scientific journals.

"It is our belief, that with some further development, base editing will provide a new paradigm for the treatment of numerous inherited ocular diseases caused by different modes of inheritance," said Dr. Palczewski. "If scientists align behind this approach, there is a chance that in 10 years, all inherited retinal diseases could be treatable."

RPB is proud to support this exciting and field-changing work.

Congratulations to RPB-supported researchers at Oregon Health & Science University and their collaborators for publishing **new research on retinopathy of prematurity (ROP)**, a condition that affects premature babies and can lead to blindness.

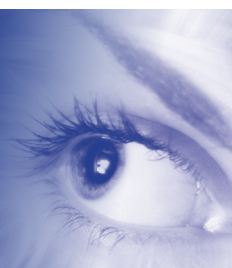
Timely diagnosis of this condition is essential to initiate treatment, but diagnosis can be subtle and subjective. The researchers found that an advanced imaging technology may provide an objective and quantitative biomarker for clinical diagnosis and longitudinal monitoring of the condition.

The Oregon research team included RPB Career Advancement Awardee **Yifan Jian, PhD**; RPB Career Development Awardee **J. Peter Campbell, MD, MPH**; and RPB Special Scholar Awardee **Yali Jia, PhD**. **Michael Chiang, MD**, Director of the National Eye Institute, former faculty at Oregon, and a prior recipient of an RPB Career Development Award, also contributed to the research.



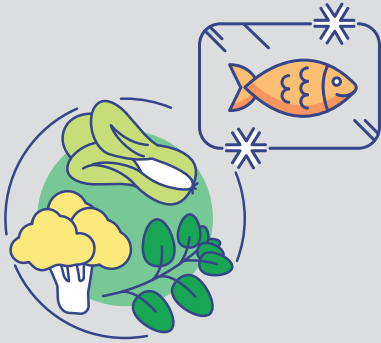
Hear Dr. Palczewski talk about his research on Leber congenital amaurosis and its implications for retinitis pigmentosa and other inherited retinal diseases in a free RPB virtual event.

Access the video at bit.ly/RPBvideoRP



Did You Know?

Eating green leafy vegetables and fatty fish can reduce your risk for age-related macular degeneration (AMD).



To learn more about AMD, view our Fact Sheets on AMD and Dry AMD & Geographic Atrophy at bit.ly/RPBfactsheets

A GIFT TO RPB CAN SAVE SIGHT

Research to Prevent Blindness, Inc. (RPB) is the only public foundation supporting research aimed at treating, preventing or curing *all* diseases that damage and destroy vision. Your support is critical to the success of our efforts!

Contributions totaling up to \$1 million within a calendar year are matched through a fund established by RPB's founder, Dr. Jules C. Stein. All gifts and bequests are tax deductible.

RPB is recognized by the U.S. Internal Revenue Service as a publicly supported tax exempt organization under section 501(c)(3) of the Internal Revenue Code.

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Researchers Revive Cells in Organ Donor Eyes



Frans Vinberg, PhD, (left) and Fatima Abbas, PhD, collaborate at the John A. Moran Eye Center at the University of Utah in the Vinberg lab. Photo Credit: John A. Moran Eye Center at the University of Utah.

Researchers have long been constrained by the models that they are able to use for research into human diseases; sometimes, research based on models does not translate well to human therapies. But recently, researchers from the John A. Moran Eye Center at the University of Utah made an important breakthrough for research into age-related macular degeneration (AMD) and other neurodegenerative diseases. RPB grantee **Frans Vinberg, PhD**, and collaborators from Scripps Research, published research in *Nature* showing that they have revived light-sensing cells in organ donor eyes.

According to the University of Utah, the researchers have restored communication between

photoreceptors—the cells that die off in AMD, causing vision loss—as part of a series of discoveries that stand to transform both vision and brain research. In order to achieve this cell interaction, the researchers built a new type of organ donation carrier that maintains the flow of oxygen and nutrients to sensitive retinal cells.

This new approach will allow researchers to move beyond animal models, studying neurodegenerative diseases in human cells.

RPB is proud to have supported this groundbreaking work with Dr. Vinberg's RPB / Dr. H. James and Carole Free Career Development Award and an Unrestricted Grant to the University of Utah Department of Ophthalmology and Visual Sciences.

Glaucoma Disease Tip

The frequency of glaucoma testing is usually related to age (although personal risk factors could increase the need for screening):

- Under the age of 40, every 2 to 5 years
- Ages 40 to 54, every 1 to 3 years
- Ages 55 to 64, every 1 to 2 years
- Ages 65 and older, every 1 to 2 years



Visit your eye doctor regularly to stay on top of glaucoma testing and other important screenings that can help you maintain healthy vision far into the future!