

WINTER 2023



New Center Brightens the Future for People with Inherited Retinal Disease

Growing new organs in the laboratory is a longtime goal of medical research. The option of replacing diseased or damaged organs with healthy human tissue could change countless lives. Supported by philanthropy, Casey Eye Institute's Retinal Stem Cell Center brings us a few steps closer to making that dream a reality for people with inherited retinal diseases (IRDs). IRDs are rare, no treatments are available to date, and these diseases eventually lead to vision loss.

Creating "mini-retinas"

Starting with blood samples donated by Casey patients living with inherited retinal diseases (IRDs), the Retinal Stem Cell Center's scientists are creating stem cells that grow into tiny retinas in about 240 days. Called retinal organoids, they are being used to study IRDs and develop new treatments.

"It's incredible. We give them these cues, and the cells do everything themselves. They assemble themselves into three-dimensional spheres and then keep growing and maturing," says Kathleen Chirco, Ph.D., assistant professor of ophthalmology at Casey Eye Institute. Donor investment is essential to this project, both because IRDs are rare and because Casey scientists are using such revolutionary techniques. Stem cell research is less likely than some other projects to receive funding from major granting agencies such as the National Institutes of Health. Up to now, this has meant little hope for people with IRDs. However, Casey researchers have never hesitated to take on big tasks if it means saving sight.

A solid investment

Casey Eye Institute has the facilities, staff and technology needed to develop treatments for complex conditions. It also has a long history of research into genetic and rare eye diseases. Years ago, the Center for Ophthalmic Genetics was developed into a leading center for gene replacement, stem cell therapy trials and other innovations, including several clinical trials that were the first ever done in humans.

Today, Casey researchers conduct more gene therapy clinical trials than almost any location worldwide. Mark Pennesi, M.D., Ph.D., head of the Paul H. Casey



Dear Friends,

2023 was an exceptional year for Casey. I'm humbled by the scientific advances we've seen, the level of education we provide, and our growing ability to deliver expert, compassionate care to more Oregonians. We've never been more committed to reaching our goal to end preventable blindness in Oregon and beyond.

Our extraordinary faculty have garnered serious recognition this year -a testament to our investments in building research capacity and recruiting top-

tier people. Portland Monthly named fifteen of our physicians as top doctors, including every member of our pediatric ophthalmology team (except a new hire). We are also very proud of Dr. David Huang, who received significant national recognition this fall for co-inventing OCT: The White House's National Medal of Technology and Innovation Award and the Lasker-DeBakey Clinical Medical Research Award.

As we continue to invest in innovation, our clinical trials enterprise is gaining momentum. The Elks Children's Eye Clinic houses the ideal, state-of-the-art clinical trial spaces that enabled our doctors and vision researchers to achieve several research firsts, such as performing a new surgical technique for gene therapy treatment to reverse the inherited retinal disease X-linked retinoschisis. It also frees up space at the original Casey facility, making it possible for yet more teams to develop ambitious research programs. Our ophthalmic plastic surgery division is now a leading recruiter for a national clinical trial, for example, and our glaucoma and neuro-ophthalmology specialists are ramping up more research projects than ever before.

Casey is also leading a revolution in how eye care is delivered, with an expanding array of community programs focused on bringing expert care to everyone in the state — not just patients who arrive at our door. We've been pursuing this ideal for decades, thanks in large part to supporters like the Oregon State Elks Association, who have partnered with Casey to provide free vision screenings for preschoolers all over the state for the past 20 years.

In May, Casey established the Oregon Vision Health Network, which empowers communities across the state to provide high-quality eye care by training local healthcare workers and establishing a telehealth program. Come January, our second mobile eye clinic will be hitting the road, doubling our capacity to bring vision screening, referrals, and prescription glasses to patients across the state. Philanthropic investment was key – the new network received significant support from The Roundhouse Foundation, Heather Killough, S. Page Evans, the Theodore Rutherford Lilley Fund of Oregon Community Foundation and community partners around the state.

In closing, I'll say that I'm incredibly grateful for our Casey community and the people whose technical skills, imagination, fortitude, and compassion brings us closer to realizing our audacious goals every day.

Sincerely,

Judnes Klauer mo

Andreas K. Lauer, M.D. Director, Casey Eye Institute Chair & Professor, Department of Ophthalmology Margaret Thiele-Petti and August Petti Endowed Chair Oregon Health & Science University Ophthalmic Genetics Division, and his colleagues helped gain approval from the US Food and Drug Administration for a drug called voretigene neparvovec-rzyl (Luxturna®), which can restore sight in people with a specific genetic eye disease. The Retinal Stem Cell Center's work with organoids could lead to many more sight-saving treatments.

"The thing that amazes me is the ingenuity that these researchers demonstrate, and their dedication to coming up with new and better ways to help humanity," says Andreas K. Lauer, M.D., director of the OHSU Casey Eye Institute, chair and professor for the Department of Ophthalmology.

A scaled-up approach to research

In the Retinal Stem Cell Center, hundreds of miniretinas can be developed at once. Working on a larger scale increases researchers' efficiency. It is also safer and less costly than traditional research methods.

"Treating patients in clinical trials is very expensive," says Paul Yang, M.D., Ph.D., an ophthalmic geneticist at Casey Eye Institute. "We can only treat a small number of patients, using therapies that are a sure bet, because we don't want to do any harm. But with hundreds of retinal organoids, we can potentially try hundreds of different drugs. The organoids extend our ability to try new things with no risk to patients."

They also reduce the need for animal research. The effectiveness of new eye treatments is often



Kathleen Chirco, Ph.D., grows retinal organoids as part of the Retinal Stem Cell Center.

demonstrated in non-human primates with normal retinas, but using retinal organoids can replace much of this testing. In addition, the mini-retinas are created using human cells from patients with IRDs, so the results of the research are more reliable and potentially better at predicting how well treatments will work in clinical trials.

Phase One: A dream becomes reality

The researchers' determination and the generosity of Casey's donors have made Phase One of the Retinal Stem Cell Center's work a reality. A research team is in place and successfully growing 3D retinas for study. Ten stem cell lines are now available for basic research.

With additional funding from the National Eye Institute and the Foundation Fighting Blindness, the Casey team is studying Usher syndrome type 1B and Bardet-Biedl syndrome, two inherited diseases that cause vision loss.

Phase Two: Expanding to do more

Now, Phase Two of the Retinal Stem Cell Center development is ready to launch with philanthropic support. When it is fully funded, the center will be able to expand by:

- Developing retinal organoids for even more inherited diseases
- Studying dozens of diseases a year, instead of two or three
- Testing multiple types of treatments
- Creating a collection of tissues with specific genetic mutations that can be shared with other labs around the world for study and research collaboration.

The future of IRD treatment

Will the "mini-retinas" developed at Casey's Retinal Stem Cell Center ever be used to replace a diseased retina entirely? Theoretically, this is possible, Pennesi says. Using a patient's own cells to create the new retina would reduce the chance of rejection for the transplanted tissue.

For now, this remains a goal, not a reality. But researchers at the Retinal Stem Cell Center will continue to innovate with the goal of changing as many lives as possible through sight-saving treatments. Their hard work, and the compassion of those who support it, are creating new possibilities for people with inherited retinal disease.

Marking 20 years of providing vision screenings to Oregon kids

On a mission to identify problems early

Kosmo Quiroz Acero was 3 years old when the Elks Preschool Vision Screening Program visited his Head Start classroom in 2011. Vision screeners found a potential problem and referred him to the Elks Children's Eye Clinic at OHSU Casey Eye Institute, where a pediatric ophthalmologist diagnosed him with amblyopia in both eyes.

The physician showed his mother, **Delfina Acero Chuma**, how to help her son place an eye patch over one eye at a time, which sharpens vision in the other eye by forcing it to develop further. Routine use of prescription eye drops and prescription eyeglasses also helped improve his vision.

"When I first found out about the lazy eye, I felt sad," Acero Chuma recalled through a Spanish interpreter. "But later the doctor said other boys also have lazy eye and that glasses can make it better. I didn't realize Kosmo had a vision problem until that screening. We never would have known about it without this program coming to his school."

With just the push of a button, a photo-screening device can take measurements of a child's eye and detect if they should be referred to an ophthalmologist or optometrist for further evaluation. Every fall, the OHSU Casey Eye Institute's Elks Preschool Vision Screening Program visits 200 Head Starts and other preschool classrooms across Oregon to screen for common eye diseases. Since 2003, the program has provided free vision checks to more than 65,000 toddlers, about 15% of whom need glasses. The program is generously supported by the Oregon State Elks Association.

"A screening that lasts just a few seconds can save a child's sight," said program director **Joannah Vaughan, M.B.A.,** who is also an assistant professor of ophthalmology in the OHSU School of Medicine.

Now 15, Kosmo can see well with the help of eyeglasses. He's currently a junior at Cleveland High School in Southeast Portland and is considering becoming an engineer. To date, the Elks Preschool Vision Screening Program has correctly identified



OHSU Casey Eye Institute's Elks Preschool Vision Screening Program provides free vision checks to more than 65,000 toddlers, about 15% of whom need glasses. (OHSU/Christine Torres Hicks)

amblyopia in at least 1,000 kids like Kosmo. Preventing permanent vision loss

Preventing permanent vision loss

About 80% of early learning is done visually, and children can fall behind in school without clear vision.

Vaughan founded the program twenty years ago after realizing that few providers were screening children between 3-5 years old, when many children attend preschool. When caught early enough, treatments like patches, special drops and glasses can help stop or reverse visionthreatening conditions.

The program screens for many common eye conditions, including amblyopia, farsightedness, nearsightedness and astigmatism. When a potential vision issue is flagged, the program follows up with the family to recommend they make an appointment with an eye doctor and provides resources and support to remove barriers to accessing care.

Marking 20 years (cont.)

Advancing vision screening research

Preventing vision loss doesn't stop with screenings, and the program continues to look for new ways to advance kids' vision through innovation and research.

To learn how to improve the program and help more Oregon children, the program team follows up with the children they screen to evaluate the accuracy of their screenings and understand the outcomes of referrals and follow-up care.

Using this data, the team has published 19 peerreviewed papers, presented at numerous medical and health conferences, and advised the state and others on vision screening best practices.

As a leader in vision screenings, research and advocacy over the last 20 years, the program continues to advance eye health for kids in Oregon.



"A screening that lasts just a few seconds can save a child's sight."

– Joannah Vaughan, MBA

Honors and Awards



Dr. David Huang receives a National Medal of Technology and Innovation from President Joe Biden at a White House ceremony. Photo credit: Ryan K. Morris and National Science and Technology Medals Foundation.

Congratulations to David Huang, M.D., Ph.D., who recently received two of the nation's highest scientific awards for co-inventing the imaging technology called optical coherence tomography. or OCT. In September, Huang and his OCT co-inventors were awarded the 2023 Lasker-DeBakey Clinical Medical Research Award, which is the United States' most distinguished biomedical research award. In October, Huang and his colleagues were awarded the National Medal of Technology and Innovation by President Biden, which is the nation's highest honor for technological achievement. OCT is now also used to diagnose and treat conditions of the eye, heart, brain, skin and more.



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To make a gift, please contact Liz Arrington, Senior Director of Development at the OHSU Foundation: arringtl@ohsu.edu or 503-552-0716

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