

SHINE the LIGHT

Spring 2019

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RESEARCH UPDATE

Retinitis pigmentosa stem-cell trial enters new phase

The promising use of progenitor cells to treat retinitis pigmentosa, which was developed at UCI Health Gavin Herbert Eye Institute (GHEI), has entered a new phase to evaluate the effectiveness of the treatment and its safety.

More than 80 volunteers who have retinitis pigmentosa, a vision-robbing condition for which there is no effective treatment, have received injections in one eye for the Phase 2b trial, said GHEI researcher Dr. Henry Klassen, principal investigator and director of the stem cell and retinal regeneration program.

Only one eye was given an injection of progenitor cells, which are partly developed stem cells, so that researchers can evaluate how much the person's vision improved in the treated eye. They'll start unmasking the results in June, 2019.



Dr. Henry Klassen

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The Phase 2b trial also differs in terms of whether and how patients were treated. During the initial trial, which emphasized safety, all patients received one of four different doses of progenitor cells, which were injected into the vitreous of the eye. For the newest trial, there were two different dose levels and one of the three patient groups did not get any cells, in order to serve as a control group. The patients and most staff do not know which group they are in, but certain doctors can figure it out, Klassen said, because they can see the cells in the eye. However, even these doctors don't know who received the higher or lower dose.

The earliest trial, even though it was focused on safety, resulted in many happy retinitis pigmentosa patients. Some reported seeing colors for the first time in years or even decades. Those who started with somewhat better vision were, in some cases, able to see eye charts again. The treatment also was well tolerated.

Rosie Barrero, one of the patients in the first trial, was thrilled with the results. She noticed that her vision clearly improved and continued to do so over time. She could see the time on her cell phone, something that hadn't been possible in many years.

"I feel so blessed to be born at this time," Barrero said.

While Klassen and his team wait to get the results from the Phase 2b trial, they have plenty to keep them busy.

Patients in the initial trial were offered, and most eagerly accepted, a chance to receive the treatment in their other eye. The results from that second injection will also be ready for evaluation soon, Klassen said.

The progenitor cells appear to be a potentially effective and long-lasting treatment, Klassen said, but they are not a cure. Early indications are that patients will need to continue receiving injections every year or two, he said. Volunteers in the initial trial will also be the first potential candidates for follow-up treatments.

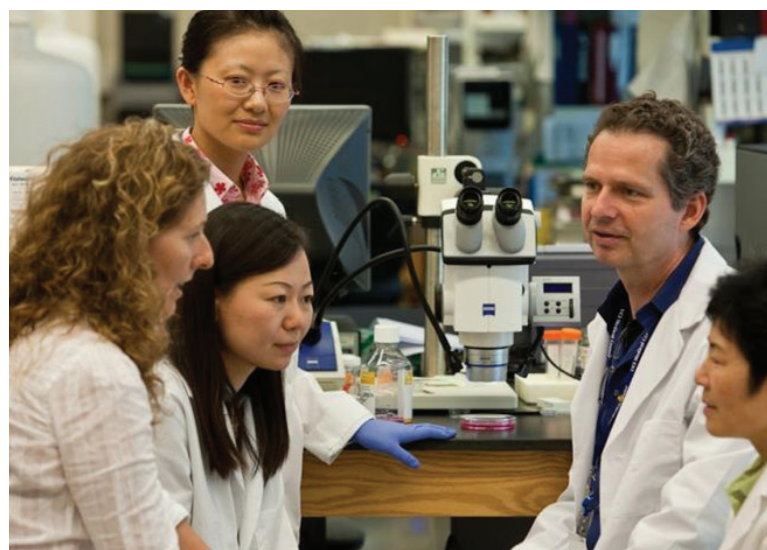
The team is preparing for a Phase 3 trial on the treatment's effectiveness, which they anticipate will be approved and funded after the results of the current trial are published.

Klassen said the next trial will involve a changed scenario. The U.S. Food and Drug Administration wants researchers to use a commercially prepared product, just as the general population would receive.

"It means that you go from a small batch of cells made at a smaller facility to large batches generated by a third party at a commercial manufacturing organization," Klassen said.

This involves a transfer of expertise to that outside organization in order to manufacture the progenitor cells, which is a relatively complicated procedure. The company that Klassen co-founded to commercialize the treatment, jCyte, is playing a lead role in the technology transfer and oversight.

Klassen and his group also are looking even further ahead. The progenitor cells might be used to treat people with age-related macular degeneration, another progressive eye disease. Macular degeneration is a leading cause of vision loss, affecting an estimated 11 million Americans. Effective treatments are badly needed, he said.



MESSAGE FROM THE CHAIR

When it comes to a world-class medical and academic staff, the Gavin Herbert Eye Institute has been showing that we're hard to beat. The past few months have been filled with extraordinary hires of highly esteemed clinicians and researchers.

I'm thrilled to welcome Dr. Mohammad Riaz, an internationally esteemed pediatric retina ophthalmologist. He spent two years with us doing retinal research and seeing patients during a sabbatical from the University of Tehran. His work is so impressive that we asked him to stay.

Riaz brings us to a new level of expertise in pediatric retinal care, specializing in treatment and surgery of retinopathy in premature infants. He performs highly specialized repair on the eyes of children whose retinopathy either wasn't treated soon after their premature birth or was treated inadequately. He has performed extensive research in this area.

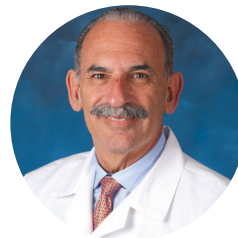
In previous messages, I have written about our plan to hire clusters of specialized researchers and clinicians who will enhance our efforts to cover all aspects of vision medicine from leading-edge research to patients' bedsides. You've read about the institute's good fortune in hiring a group of high-impact scientists led by world-respected ocular pharmacologist Professor Krzysztof Palczewski.

We have added two important members to this cluster with Professor Timothy Kern at the Department of

Ophthalmology and Assistant Professor Philip D. Kiser at the Department of Physiology and Biophysics. Kern conducts research in diabetic retinopathy while Kiser studies possible treatments for age-related macular degeneration. We are one major hire away from rounding out the cluster of top experts working with Palczewski, and I look forward to sharing more news on that soon.

Meanwhile, our outstanding faculty and clinicians continue to shine every day. Dr. Andrew Browne, who conducts research in diabetic retinopathy and macular degeneration, was recently awarded a KL2 award, a prestigious national award from the National Institutes of Health for early career scientists. And Professor Palczewski, holder of our Irving H. Leopold Chair, received double honors as the Retina Research Foundation's Paul Kayser International Award in Retina Research, and as an honoree of the Future Vision Foundation.

Great people, supported with the right resources, are what make GHEI a world-class eye institute with an ever-growing reputation. It is an honor to work with them.



Baruch D. Kuppermann, MD, PhD

Chair, Department of Ophthalmology
Director, Gavin Herbert Eye Institute

Faculty members

Cataracts, Cornea, External Disease and Refractive Surgery

Marjan Farid, MD
Vice Chair, Ophthalmology Faculty
Sumit (Sam) Garg, MD
Vice Chair, Clinical Ophthalmology
Sanjay Kedhar, MD
Matthew Wade, MD

Cataracts and Glaucoma

Anand Bhatt, MD
Richard Hill, MD
Ken Y. Lin, MD, PhD
Sameh Mosaed, MD

Comprehensive Ophthalmology

Kavita K. Rao, MD

Low Vision

Rebecca L. Kammer, OD

Neuro-ophthalmology

Chantal J. Boisvert, MD
Robert Wade Crow, MD

Oculoplastics

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Ophthalmic Pathology

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Optometry

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Linda Shi, OD

Poonam Varsani, OD

Pediatric Ophthalmology

Chantal J. Boisvert, MD
Charlotte Gore, MD

Robert W. Lingua, MD

Mohammad Riaz, MD

Jennifer L. Simpson, MD

Retina and Vitreous

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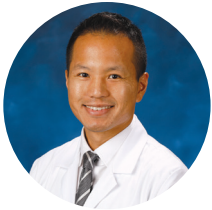
Uveitis

Sanjay Kedhar, MD

Research

Andrew Browne, MD, PhD
Lbachir BenMohamed, PhD
James V. Jester, PhD
Tibor Juhasz, PhD
M. Cristina Kenney, MD, PhD
Timothy Kern, PhD
Henry J. Klassen, MD, PhD
Baruch D. Kuppermann, MD, PhD
Anthony B. Nesburn, MD
Vice Chair, Research
Eric Pearlman, PhD
Jing Yang, PhD

Changing lives one cataract at a time



Dr. Ken Y. Lin

UCI Health Gavin Herbert Eye Institute ophthalmologist Dr. Ken Y. Lin has restored and maintained the vision of many patients, but none was more memorable than the 78-year-old man on whom he operated during his

residency at UC Irvine Medical Center. The patient was depressed, had diabetes, was estranged from his grown daughter and lived in a nursing facility because, without eyesight or a support system, his extensive health problems required institutional assistance.

Lin removed a diabetic cataract in one eye; the next day, with good vision restored to that eye, the patient said he “felt like I just woke up from a long sleep.” A follow-up appointment was set to remove a cataract in the other eye.

But the patient never showed up. Instead, he sent a postcard to the young ophthalmologist several months later, saying that his new vision had inspired him to “escape” from the facility and reunite with his daughter in Costa Rica, with whom he was living. He had no plans to have his other eye treated because, he wrote, “one good eye is enough for one happy soul.”

With cataract surgery, Lin can generally bring about transformative improvements in vision, as he did for this “happy soul.”

But glaucoma, Lin’s other area of specialization, is tougher. There is no cure for glaucoma. Medication can slow or even halt its progression. Surgery can also alleviate pressure in the eye, but scar tissue often renders these results temporary. That’s why Lin has centered his research on developing more successful surgical techniques for glaucoma.

His glaucoma patients, who tend to be elderly, often have trouble accepting that their eye problem must be managed rather than simply fixed.

That’s when Lin uses another clinical technique: spending quality time talking with and listening to his patients, explaining why their new medication will probably need to be taken for the rest of their life.

“This is not a curable disease. You cannot defeat glaucoma,” he said. But instead of just telling his patients, “ ‘Here are the eye drops, take the eye drops,’ I spend a lot of time during their initial visit helping them to understand exactly what they’re dealing with. Otherwise, more than half of the patients are going to slack off their medication and their condition will get worse.”

“My goal is to make sure the patients and I are on the same page.”

Welcome new faculty



Mohammad Riazi, MD
Health Sciences
Clinical Professor,
Retina / Vitreous

Dr. Mohammad Riazi is a UCI Health ophthalmologist who specializes in vitreoretinal surgery and the management of vitreoretinal diseases, including retinopathy of prematurity (ROP) in infants.

Riazi earned his medical degree at Tehran University of Medical Sciences (TUMS) in Iran. He completed a residency in ophthalmology and a fellowship in retinal

diseases at TUMS Farabi Eye Hospital, where he later served as director of the residency training program and co-founder of the ROP clinic. He also completed fellowships in vitreoretinal research at Tulane University School of Medicine in New Orleans and vitreoretinal surgery at UCI School of Medicine.

Riazi’s clinical interests include premature retinopathy and retinal problems in children, an area in which he has conducted clinical trials and published extensively. Other clinical interests include diabetic retinopathy, diabetic macular edema, age-related macular degeneration, management of retinal detachment and macular surgery.

Better vision for special-needs patients

Sarah Hoel badly needed new eyeglasses. She had for years. But her mother had been advised to wait until Sarah, who has severe autism, stopped scratching her eyes. The scratches were changing her vision, which would quickly render any new glasses useless.

Asking Sarah to stop wouldn't work because she is unable to follow directions. Because the 19-year-old is non-verbal, she cannot explain why she scratches her eyes. Yet her poor vision was one of many factors reducing her quality of life. She couldn't even see well enough to go to the movies, something she'd always enjoyed, said her mother, Jill Marie Hoel. With that and a chronic jaw problem so painful that she cannot eat solid food, Sarah suffered through a protracted rough time.

"She's been through a lot in her life," Jill Marie Hoel said. "As my husband always says, we're doing our jobs as parents if we can make her smile." But there wasn't much smiling going on.

With a new liquid diet and some medication changes, Sarah eventually stopped the damaging self-harm and this past December, it was finally time to have her examined for new glasses. But that brought new challenges: Sarah can't tell a doctor whether she sees better with one lens or another. It's hard enough for her to sit still for an examination.

That's why her mother brings Sarah to UCI Health Gavin Herbert Eye Institute when she needs vision care. Dr. Robert Lingua used an autorefractor to determine Sarah's new eyeglass prescription so that she didn't need to communicate. Just as important, Hoel said, the ophthalmologist and his team are gentle and extremely patient with Sarah, allowing her mother to help keep her still, and providing toys and taking their time so that she doesn't feel overwhelmed.

Lingua wishes he could do far more for Sarah and his other special-needs patients. "There's such an enormous need to create a specialized environment for kids with



Sarah Hoel

special needs when it comes to an eye exam," he said. "What I conceived about 10 years ago was something that we nicknamed VISN (pronounced 'vision'), which stands for Vision Institute for Special Needs."

Lingua noted that technological advances have introduced machines that can provide a prescription without patients having to fit their faces within a machine, so they're not as frightened. There also are miniaturized instruments that are less intimidating, ones he could hide away in a desk and take out one by one. They don't require drops and they can measure eye pressure without a burst of air against the eye. But the equipment costs a total of about \$200,000, he said, and donations have been slow.

Lingua feels certain that if he could provide a less overwhelming experience for special-needs patients, he could give more of them better vision that would in turn improve their lives. Not all of them can sit still as Sarah did for her eye exam.

In fact, as soon as Sarah got her new glasses, her mother decided it was time for them to go to the movies again. She picked "Dr. Seuss' The Grinch" because the story was familiar to Sarah. The simple outing proved transformative.

"She was literally beaming," Jill Marie Hoel said. "She was extremely happy. After so many years of Sarah being in such pain, it was very emotional for me to see her experience such joy."

Johnson & Johnson: Supporting a brighter world for children



The Eye Mobile for Children has been traversing Orange County for three years now, bringing vision care to children whose parents lack insurance and cannot otherwise afford this kind of care. And because the Eye Mobile, a program of the UCI Health Gavin Herbert Eye Institute, rolls right up to preschools and schools, there's no worrying about finding transportation to an optometrist's office.

This vision van has now screened thousands of children, provided free eyeglasses to those who need them, and made referrals for those with issues requiring an ophthalmologist's care. Very little of this would have been possible without the support of funders like Johnson & Johnson Vision.

The van now is rolling into its fourth year, thanks to a generous \$100,000 donation from the company, which has a headquarters in Santa Ana.

Through its charitable initiatives, the company provides support for vision programs in the communities "where we work and live," Johnson & Johnson communications specialist Kelly Novak said.

"Our vision as a company is to help people see better, connect better and live better," Novak said. "The Eye Mobile is great because it hits all three of those tenets."

Studies have repeatedly shown that improving poor vision in children also results in better reading and writing achievements and enhanced cognitive ability. In other words, Johnson & Johnson Vision's donation helps create a brighter, clearer world for the children who visit the Eye Mobile.

Thank you to our donors

Gifts of \$25,000 and above received since July 1, 2018, to the general fund of the Gavin Herbert Eye Institute:

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Anne Brownstein

Gifts of \$50,000 and above received since July 2018 to the Irving H. Leopold, MD Endowed Professorship:

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To make a donation toward the Roger F. Steinert Endowed Chair, contact Janice Briggs, senior executive director of development, at 949-824-0091 or jbriggs@uci.edu.



David and Molly Pyott with Tom Sullivan at 20/20 Society event

Dedicated to helping others see

Tom Sullivan has been a singer, a scriptwriter, composer and television correspondent. He had a recurring role on the TV show Highway to Heaven, sang the national anthem at the Super Bowl and traveled the world speaking to doctors about the need to run more patient-friendly practices. He's written more than a dozen books, is a triathlete who also golfs, skis and skydives, and at age 72, he likes running on the beach with his dog.

His guide dog, that is. Sullivan has been blind nearly from birth, a result of retinal damage from too much oxygen in the incubator when he was a premature infant. As extraordinary as his life is, Sullivan wishes he could see those waves along the beach, a rainbow, a mountain, the faces of his wife and children and so much more of the world. Helping others see by supporting medical research on sight-robbing illnesses has become a passion of Sullivan's as he works to raise money for the UCI Health Gavin Herbert Eye Institute (GHEI).

Sullivan came to know GHEI's namesake, eye industry leader Gavin Herbert, and admire his work during a 15-year stint working for the company Herbert founded, Allergan, to promote more patient-centered medical care.

Sullivan also understood early on the role research has played in preventing blindness among premature babies.

"At the time I was born, blindness like this was an epidemic," he said. "It doesn't happen anymore. That

could only occur because of research."

Sullivan talked about this, as well as the importance of vision and his strong beliefs about the importance of supporting research at a meeting last fall of the 20/20 Society, made up of people whose vision has been helped by GHEI doctors. He explained that he considers the word 'pride' an acronym that stands for "personal responsibility for individual daily effort."

"I said they had to develop a sense of pride and purpose, through the 20/20 group, and be passionate about the preservation of vision."

Sullivan is thrilled by the stories of GHEI patients whose vision has been stabilized despite a diagnosis of keratoconus, or those for whom the progression of macular degeneration has been slowed. These days, he's particularly excited about the work of GHEI researcher Dr. Henry Klassen, who is using progenitor cells in an effort to restore the vision of people with retinitis pigmentosa. A friend of Sullivan's who was largely blind participated in an early-stage trial of the treatment.

"She called me about two months ago, crying on the phone," Sullivan said. "She said, 'Tom, I was in my bathroom this morning and I saw my mascara.'"

"Can you imagine? She was able to see her mascara."

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EVENTS

Location for all events:

850 Health Sciences Road, 3rd Floor, Irvine, CA 92697

RSVP info for all events:

RSVP.ghei@health.uci.edu | 949-824-7243

LASIK Surgery Seminars

Free, informational seminar to learn about LASIK surgery and whether you might be a candidate

Thursdays at 6:30 p.m.

March 14, 2019

April 11, 2019

May 9, 2019

June 6, 2019

Community Lecture

Eye and Brain Interaction

How can your eyes tell you if there are neurological problems?

Chantal Boisvert, MD

Monday, March 25, 2019 | 7 p.m.

Annual Eye Exams

Eyevolution: The future in vision care

Patrisha Elbeck, RDO

Monday, April 8, 2019 | 7 p.m.

Make an appointment

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Optical Shop: 949-824-7690

UCI Health Medical Center

101 The City Drive South, Pavilion 2, Orange, CA 92868

Appointments: 714-456-7183

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