

RETINA RESEARCH FOUNDATION NEWSLETTER

Foresight for Sight

December 2024

Issue Number 3 / Volume 46

Retina Research Foundation Newsletter is published three times per year: Spring, Summer and Fall.



With Gratitude this Holiday Season

The rewards of investment in retina research
can be many years in the making.

So, as we reflect on the year's remarkable successes
made possible by our community of
vision scientists and supporters, we thank you
for your generous, patient and
steadfast belief in RRF's mission to
fund research and educational programs and
End Blindness due to Retinal Disease.

Wishing you and yours
all the joys of a happy and healthy holiday!

*The Board of Directors
of Retina Research Foundation*





2024 RRF Chairs and Professorships



David Gamm, MD, PhD
Emmett A. Humble
Distinguished Director
McPherson Eye Research Institute
University of Wisconsin
*Modeling and Treating Retinal
Disease with Human Induced
Pluripotent Stem Cells (hiPSCs)*



Bikash Pattnaik, PhD
Daniel M. Albert Chair
McPherson Eye Research Institute
University of Wisconsin
*Treatment for Rare Diseases
that Challenge Healthcare*



Nader Sheibani, PhD
RRF Research Chair
Department of Ophthalmology
and Visual Sciences
University of Wisconsin
*Pathophysiology of Eye Diseases
with Neovascularization*



Timothy M. Gomez, PhD
MD Matthews Professor
McPherson Eye Research Institute
University of Wisconsin
*Understanding Mechanisms
of Human Photoreceptor
Development and Regeneration*



Kevin W. Eliceiri, PhD
Walter H. Helmerich Chair
McPherson Eye Research Institute
University of Wisconsin
*Open Source Computational Imaging
of Cellular Microenvironments*



Shaoqin "Sarah" Gong, PhD
Edwin and Dorothy Gamewell
Professor
McPherson Eye Research Institute
University of Wisconsin
Ocular Gene and Cell Therapy



Krishanu Saha, PhD
Kathryn and Latimer Murfee Chair
McPherson Eye Research Institute
University of Wisconsin
*Bioengineering of Novel Cell
and Gene Therapies for the
Retinal Disorders*



Mrinalini Hoon, PhD
Rebecca Meyer Brown Professor
McPherson Eye Research Institute
University of Wisconsin
*Impact of Photoreceptor Input
Loss on Retinal Connections*



Dear Friends,

With the close of 2024, Retina Research Foundation will realize 55 years of funding basic retina research projects and providing educational opportunities to retina specialists and vision researchers across the globe. Our endeavors have been guided by a profound commitment to helping the millions of people whose vision is or will be impacted by retinal disease and to the physicians and researchers dedicated to providing them with sight saving care. Steered by the vision held by our founder, Dr. Alice R. McPherson, RRF has been at the forefront of discovery for nearing six decades. As we reflect on all that has been accomplished, we are filled with immense appreciation for your support in these endeavors. We are equally awed by the future therapeutic possibilities that today's vision research discoveries hold for treatment improvements. We see how the discovery of the genes that cause inherited retinal diseases leads to the development of therapies to treat these diseases. Understanding the mechanisms of retinal diseases results in the development of therapeutic options that halt the progression of eye diseases thought previously to be untreatable. We partner with other organizations and individuals who share our mission and vision, and together, we are making a meaningful impact. Our work is far from over – retinal disease has not been cured and exists in ever increasing levels as the worldwide population ages.

As we look toward new research challenges and the therapeutic opportunities on the horizon, we see research related to retina cell regeneration is nearing clinical phases. The science of discovery is changing rapidly, and capabilities enabled by genetic understanding, bio-engineering, imaging technology and AI are forever expanding our abilities to accurately diagnose and treat retinal diseases. RRF stands firm in funding the ever-changing needs of our researchers as they search for answers. Their work exploring the causes of retinal dysfunction gives us hope about the prospects of new therapies to cure the retina diseases that threaten our sight.

On behalf of all the Directors of the Retina Research Foundation, I want to express our heartfelt gratitude to our collaboration partners, organizational supporters and all of our dedicated individual supporters who are a part of this incredible journey. We are witnessing the transformative power of philanthropy in action through investing in basic research, and your unwavering commitment has been instrumental to our success. If you have not yet given to RRF this year, we hope you will consider doing so. Every donation makes a difference. We thank you for your interest and send warmest wishes for the upcoming holiday season.

Sincerely,

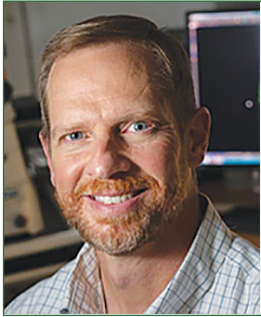
Katharine W. Orton

RRF Managing Director, 2024 Fund Drive Chair

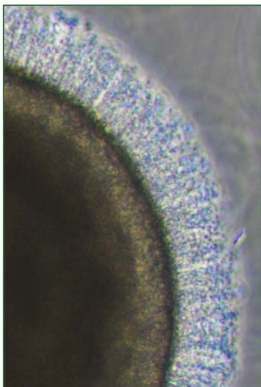
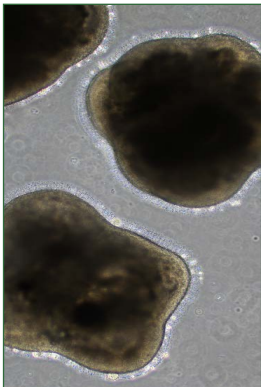


Retina Research Foundation is dedicated to the eradication of retina disease through programs in research and education.
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Much Anticipated Clinical Trials Announced To Determine Therapeutic Potential To Restore Vision To People Living With Primary Photoreceptor Diseases



David Gamm, MD, PhD
McPherson Eye Research
Institute
University of Wisconsin-
Madison



Images of hiPS cell-derived
retinal organoids, bottom
image with further
magnification.



In early September 2024, BlueRock Therapeutics, a clinical stage cell therapy company and wholly owned subsidiary of Bayer AG, announced it received clearance from the U.S. FDA to launch a **Phase 1/2 clinical trial for OpCT-001. OpCT-001 is the first induced pluripotent stem cell (iPSC)-derived cell therapy for the treatment of primary photoreceptor diseases.** These diseases represent a subgroup of inherited retinal disorders that includes retinitis pigmentosa, Usher Syndrome, and cone-rod dystrophy that affect the structure and function of the photoreceptor cells in the retina, leading to irreversible vision loss in children and adults. The study is expected to enroll participants in multiple sites in the U.S., beginning in at least one location in 2025. OpCT-001 was exclusively licensed from FujiFilm Cellular Dynamics and Opsi Therapeutics, the latter of which was co-founded by **David Gamm, MD, PhD.** RRF and others have funded research conducted by Dr. Gamm for development of the induced pluripotent stem cell approach to be used in the trial.

The study will utilize photoreceptors derived from induced pluripotent stem cells (iPSCs). Researchers produce iPSCs by taking a small sample of blood or skin from an adult, human donor. The cells are then genetically modified to return them to a stem-cell-like state. As stem cells, they can be coaxed to mature into virtually any cell type in the body, including photoreceptors of the retina. Billions of cells representing many therapy doses can be produced from the initial cell sample.



Dr. McPherson and Dr. Gamm

Stem-cell-derived photoreceptor replacement therapy has been one of Dr. Gamm's long-term research objectives, one which began with the development of a process to create the human photoreceptor cells from iPSCs. Then, he and his collaborators had to determine whether the resulting cells could detect light, produce energy, are wired correctly, and are able to make neural connections – all necessary functions of photoreceptor cells that enable human vision. This progressive set of research breakthroughs, many years in the making, together represent the immense scientific progress leading up to the announced OpCT-001 clinical trials. At present, however, OpCT-001 is an investigational cell therapy that has not been approved by any regulatory authority, and its efficacy and safety have not been established or fully evaluated.

Dr. Gamm is considered a world-renowned retinal cell therapy pioneer, and RRF is proud to have continuously funded his research since 2006. He is the RRF Emmett A. Humble Distinguished Director of the McPherson Eye Research Institute (McPherson ERI), at the University of Wisconsin-Madison, a position he has held since 2012. Dr. Alice McPherson, RRF's founder and a significant benefactor of the McPherson ERI, was from the outset, an enormous supporter of Dr. Gamm's research ambitions, speaking with him, often monthly, about his progress.

In that first year of RRF funding, Dr. Gamm's project was entitled "*Deriving Photoreceptors from Human Pluripotent Stem Cells,*" and early goals for the research

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were to “successfully derive photoreceptors from human pluripotent stem cells to advance not only the knowledge of basic photoreceptor development, but also offer a source of human cells for drug testing and transplantation.” As the research progressed, it became clear that this cell type has great promise as a treatment for retinal and neurodegenerative diseases. Fortunately, for those with inherited retinal diseases, the technology has now evolved such that it has received FDA clearance to launch clinical human trials for the treatment of primary photoreceptor diseases, and as Dr. Gamm says, “Now we are on the cusp of seeing whether they are a safe and effective means to help patients.”



From left: Dr. Art Willis, RRF President, Dr. David Gamm, Mr. John Dawson, RRF Chairman, at the 2024 RRF Luncheon where Dr. Gamm shared updates on his research with the RRF community, May 2024.

Sources: BlueRock Therapeutics News Release, McPherson Eye Research Institute Insights Fall 2024 Magazine, RRF Annual Reports

Retina Research Foundation has provided significant funding to Dr. Gamm’s research to develop retinal and photoreceptor cell therapies derived from iPSCs that will be used in clinical trials for treatment of primary photoreceptor diseases.



Eminent Inventor and Retina Surgeon Receives RRF’s 2024 Charles L. Schepens MD/AAO Award



Dr. Steven T. Charles

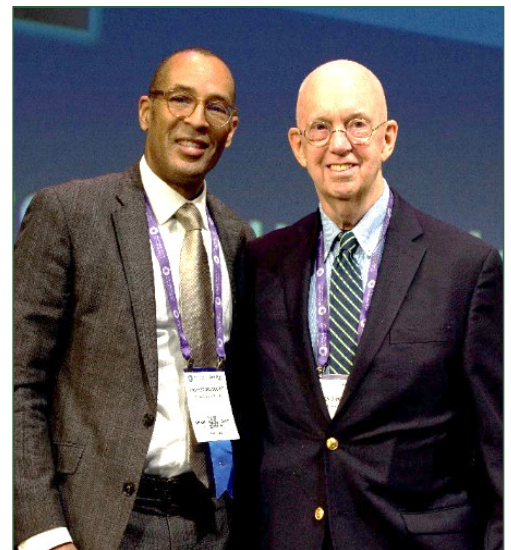
Dr. Steven T. Charles is a legendary innovator and retinal surgeon. His lifetime achievements were most recently recognized with his selection as the 17th recipient of RRF’s Charles L. Schepens MD/AAO Award. Dr. Charles delivered the Schepens lecture on the morning of the Retina Subspecialty Day at the American Academy of Ophthalmology’s (AAO) annual meeting in Chicago, IL, on October 18, 2024. He was introduced by Dr. Steven McLeod, CEO of the Academy.

Dr. Charles’ lecture, *Systems Engineering at the Intersection of Technology and Technique*, recounted the pioneering work and technical innovations that enable today’s retina surgery, including noteworthy surgical inventions he has contributed from the over 200 issued or pending patents he holds.

In his lecture and drawing from his own experience as an engineer and a retina surgeon, Dr. Charles focused on the collaborative process and need for multidisciplinary teamwork between engineering and surgery, and the problem-solving mindset that drives innovation. He stressed the importance of ethical and transparent partnerships between surgeons and medical device companies that provide substantial capital for research and development, funding that is increasingly crucial as the complexity and cost of developing new technology increases.

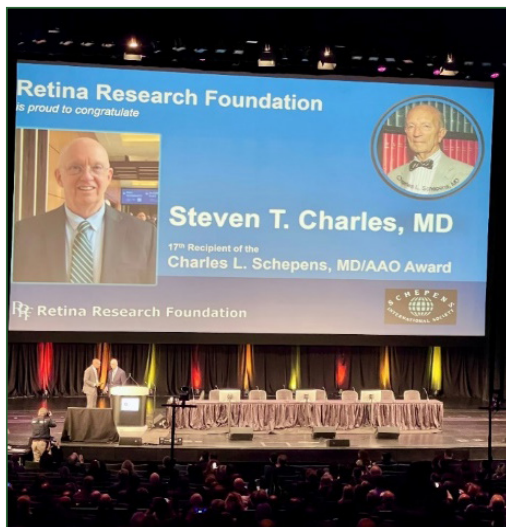
Dr. Charles reviewed milestones in retina surgery, discussing the evolution of vitrectomy surgical tools away from older methods to safer, more precise

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Dr. Steven McLeod and Dr. Steven T. Charles

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Dr. Charles Schepens Award Ceremony, AAO 2024

endophotocoagulation techniques, along with the reduction in cutter weight, the shrinking gauge sizes and the development of a disposable, self-sharpening cutter. Dr. Charles highlighted the innovations in the use of fluids in surgery (fluidics), from early manual syringe aspiration by an assistant to the development of foot pedal-controlled systems, which enable more precise and responsive aspiration, controlled by the surgeon, in air and gas injection, drainage of subretinal fluid, and vitrectomy port technology.

Dr. Charles Schepens is recognized as establishing the retina specialty within ophthalmology, and he is considered the father of modern retina surgery. His devices and surgical techniques have been credited with raising the success rate of retinal reattachment surgery from 40% to 90%. Dr. Steve Charles also is a significant contributor to the technical evolution responsible for the even higher success rate that today's retina surgeons' patients benefit from – further narrowing that difficult, final 10% gap to approaching 100% success. It is most fitting that he is this year's recipient of the Charles L. Schepens MD/AAO Award.

Sources: Retinalphysician.com; Retinaroundup.com

It's the Holidays, Go Ahead and Enjoy Some Nuts!



Nuts are good for your eyes and your health in general. Packed with antioxidants, vitamins, minerals, and healthy fats, nuts play a vital role in slowing the progression of sight loss conditions such as macular degeneration, diabetic retinopathy and even cataracts, making them an integral part of a diet aimed at preventing blindness. Remember, a balanced diet and a healthy lifestyle are crucial for maintaining overall eye health.

Specifically, nuts provide a healthy serving of vitamin E, an antioxidant that helps your eyes manage and repel “free radicals.” At high concentrations, free radicals can generate oxidative stress, a deleterious process that can damage all cell structures and plays a major part in the development of chronic and degenerative ailments, including age-related macular degeneration or cataracts, eye diseases that left untreated can cause blindness. Some reports show that vitamin E can actually slow down the development and ease symptoms for these eye diseases. Vitamin E is also fundamental to your immune system and the prevention of cancer and systemic diseases. Your body needs vitamin E, but it doesn't produce the proper amounts by itself. Your diet can give you all the nutrients essential for eye health, the key is to choose the right foods. There are many nuts that are rich in antioxidants. Grab them as a snack alone, or mixed together with dried fruits and seeds as trail mix, or use them in other nutrient-rich ways:



- Almonds – try almond-crusting baked salmon or almond butter;
- Cashews – add to a stir fry with veggies and protein for extra crunch;
- Hazelnuts – toasted for desserts, breads and pesto;
- Peanuts – try peanut butter on toast, or fruit!
- Pecans – add pecans and apples to leafy greens for salads;
- Pine nuts – sprinkle over salads or make pesto with parsley or kale;
- Pistachios – see recipes below;
- Walnuts – add to overnight oats with fruit for breakfast.

If you're allergic to nuts, you can get vitamin E from pumpkin seeds, sunflower seeds, sweet potatoes, and vitamin-enriched cereals, among other foods.

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Particularly fond of Pistachios? This beautiful green nut can be a holiday centerpiece. Here are two holiday season recipes, great to take for celebrations with family and friends.

Pistachio Bread – makes one (1) loaf

Ingredients

1 cup Pistachio kernels, ground
½ cup Butter
⅔ cup Sugar
2 Eggs
½ teaspoon Vanilla extract
½ teaspoon Salt
1 Tablespoon Baking powder
1¾ cups All-purpose flour



Instructions

Preheat oven to 350°F. Grind pistachios in a blender or food processor leaving them a little chunky to add texture. Cream together butter, sugar, and pistachios in a stand mixer for 2–3 minutes. Add in eggs and vanilla, and mix on low until incorporated. Measure out and mix up your dry ingredients in a bowl. Turn mixer on low and pour in dry ingredients a little bit at a time. Mix on high for about 30 seconds. Butter a large loaf pan. Pour batter into pan and bake for 40-50 minutes until golden and baked through.

Courtesy of Sweet Paul Magazine.

Pistachiomolé – makes one (1) pound

Ingredients

2 cups Pistachio nuts
2 Ripe avocados
¼ White onion: finely diced
1 Serrano chile: stemmed, seeded & minced
½ cup Cilantro sprigs
2 tablespoons Extra-virgin olive oil
1 tablespoon Fresh lime juice
½ teaspoon Coarse salt
¼ teaspoon Black pepper

Garnishes

¼ cup Pistachio nuts: crushed or finely chopped
Cilantro sprigs
Lime wedges
Tortilla chips



Instructions

Lightly toast the pistachio nuts. Finely chop or process in a food processor. Seed and peel the avocado. Dice the avocado. Combine all of the ingredients in a mixing bowl. With a spoon, stir the mixture while lightly mashing the avocado. Do not over process – the mixture should have a slightly coarse texture. Transfer the Pistachiomolé to a serving dish. Sprinkle the surface with the crushed Pistachios. Garnish with the cilantro sprigs and lime wedges. Serve with tortilla chips.

More recipes using pistachios in preparation of appetizers, soups, salads, entrees and desserts can be found at: americanpistachios.org/holiday-recipes.

Sources: AOA.org, Americanpistachios.org, SightresearchUK.org/nutrition

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FOR THE
BLIND OR
HANDICAPPED

CHANGE SERVICE REQUESTED

Please indicate requested service change and make any corrections needed next to your name and address, then return entire address label to RRF.

- Change name or address as shown on address
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JoClaire and Henry Gissel
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Dr. Alice R. McPherson
Rhett Butler Charitable Foundation
Joyce S. Rubenstein

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IN HONOR OF

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Joyce S. Rubenstein

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Jerome and Isaac Goodman

CONSIDER MAKING A DONATION TODAY!

RRF is a public charity and our efforts to cure retinal disease are enhanced by support from our community. RRF accepts secure donations at retinaresearchfnd.org. To go directly to the RRF online giving page, focus your phone's camera (Android or iPhone with iOS 11 or later) or your iPad, on the QR code displayed here and open the link. Of course, you can always mail your donation to the RRF office.

For more information on ways to give, call: 713-797-1925. Thank you for helping us fund innovative research to discover cures for the retinal diseases that damage and destroy vision!



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