

Optogenetics: Hope for Vision Restoration for Advanced Retinal Diseases



A person's eye having an exam with a light shining in their eye.

Early, encouraging results from two human studies – trials launched by Bionic Sight and GenSight – are putting optogenetic therapies in the spotlight for patients with advanced vision loss (e.g., only light perception) from retinal conditions

such as retinitis pigmentosa (RP) and age-related macular degeneration (AMD).

In the Bionic Sight trial, investigators reported that the first four RP patients dosed can now see light and motion. Two of the patients can detect the direction of motion; that is, they can determine if objects are moving to the right or left. One patient said that one of the first new things he saw was Hanukkah candles on the eighth day of the holiday when they were all lit. Also, two patients who practice martial arts saw the robes of their opponents against the dark blue mat.

GenSight reported results for a 58-year-old man who entered the trial with only light perception due to advanced RP (Usher syndrome type 2A). After receiving the optogenetic therapy, the patient was able to locate and reach for objects on a table while

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HELP FIGHT BLINDING DISEASES

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IN THE COMMUNITY

Listen to the New Eye on the Cure Podcast

The new Eye on the Cure Podcast from the Foundation Fighting Blindness provides science information, news, and insights from the world of vision and retinal diseases. The podcasts are hosted by Ben Shaberman, senior director scientific outreach, and often include conversations with guests from the research and vision communities. Stream the Foundation Podcasts on SoundCloud, Spotify, Audible, Pandora, and more. Learn about streaming by visiting: FightingBlindness.org/podcasts



Shop the Foundation Merchandise Store

The Foundation Fighting Blindness has an official merchandise store – designed to raise awareness and support the Foundation’s mission! Show off your support of the Foundation with various branded merchandise and apparel, like t-shirts, hoodies, hats, YETI® tumblers, and more. Check out the website by visiting: ShopFightingBlindness.org



✓ Foundation and VisionWalk branded dog bandannas

✓ Navy Foundation branded can koozie



^ Navy Foundation branded YETI® Rambler 20 oz. Tumbler



^ Grey Vision Warriors graphic t-shirt

COVER STORY
CONTINUED FROM FRONT COVER

wearing the image-capturing eyewear. Results from the GenSight trial were reported in the journal *Nature Medicine*. The Foundation funded lab research that led to the initiation of the GenSight study.

In simple terms, optogenetic treatments bestow light sensitivity to cells that normally don't respond to light or cells that have lost their light-sensing ability. And, they're gene-agnostic, designed to work independently of the mutated gene causing the patient's retinal disease.

The emerging therapies from Bionic Sight and GenSight are designed to enable retinal ganglion cells to respond to light, so they can work like a backup system for photoreceptors, the cells that normally make vision possible. Ganglion cells often survive after photoreceptors are lost to advanced retinal disease. In both treatments, copies of an algae-derived gene that express a light-sensing protein are delivered to the ganglion cells. Both approaches use viral gene delivery systems and include eyewear to enhance the visual information sent to the retinas.

Nanoscope Therapeutics recently launched a clinical trial in the U.S. for its optogenetic therapy, which involves viral delivery of a light-sensitive gene (a multi-characteristic opsin)

to bipolar cells in the retina. The company will enroll 27 people with advanced RP in its trial.

An emerging protein-based optogenetic approach being developed by Vedere Bio and funded by the Foundation's RD Fund was recently acquired by Novartis. After the acquisition, Vedere Bio II was launched to develop another optogenetic therapy.

Two groups are working on optogenetic therapies designed to resurrect dormant cone photoreceptors in people with advanced retinal disease. One of the groups, SparingVision, is funded through the RD Fund. The other group is led by Hendrik Scholl, MD, Institute of Molecular and Clinical Ophthalmology Basel, and funded by the Foundation's Translational Research Acceleration Program (TRAP).

Keep in mind that while there is much promising research activity for optogenetic therapies, the approach is still at an early stage of clinical development; there is much that the research community is learning about the potential for meaningful, natural vision restoration.

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Physicians differ in their approach to incorporating research results into their clinical practices. You should always consult with and be guided by your physician's advice when considering treatment based on research results.

BEACON STORY

Blind YouTuber Finding His Spot in Life

by Lauren Reeves

Sam Seavey is a husband and dad of two children living in Kentucky. Sam's also a well-known YouTuber in the blind and visually impaired community. Sam began his YouTube channel to share his visually impaired perspective on life, but he wasn't always so open and candid about his vision loss.

Sam was diagnosed with Stargardt disease when he was just 11 years old, shortly after his older sister was also diagnosed with Stargardt. Having someone close that could understand how it felt to be losing their sight was helpful for Sam to cope. And his parents were very accepting and wanted to help him and his sister as much as they possibly could.

Sam felt like a typical kid outside of school, but in school, Sam often felt like he struggled to fit in.

"You don't want to be the different kid, you want to be like everyone else," says Sam. "But it's hard to do that when you're the one sitting in the front row of the classroom with the large print books and a monocular trying to see what the teacher is doing."

Sam attended public school through most of high school and recalled pretending he could see so his peers wouldn't know he was blind. But between his junior and senior years, he attended a life-changing summer program at Governor Morehead School for the Blind in Raleigh, North Carolina, and graduated from the school.

"I finally felt normal in school my senior year," says Sam. "I wasn't the weird kid anymore. I was finally around others that were like me, and I loved it."



Sam holding his tripod and camera in one hand and his white cane in the other

Around Sam's late 20s to early 30s, he finally started to feel self-assured and accepting of his visual impairments. He credits a lot of that confidence to his wife, Rachel.

"I finally realized there's nothing I can do about it, and it's not going to change, right now, at least," recalls Sam. "I was wasting too much time and energy pretending to be sighted when I wasn't, and I wasn't fooling anyone."

Everyone in his family, including himself, feels more empathetic and more willing to help others because of his experiences with vision loss.

"Losing your vision makes you gain a deeper appreciation of your life," says Sam. "Especially if you have a degenerative eye disease because you don't know how many more sunsets you'll see, so I better enjoy this one."

As Sam's vision loss progressed over the years, employment grew more difficult to find. So, when his daughter was born, he stayed home with her while his wife worked. But once his daughter grew old enough to go off to school, he figured he needed to start doing something with his time.

In 2012, Sam was hired to create videos for a mobile technology YouTube channel. He added a few videos specifically about accessibility apps and features for people with low vision

and received a lot of positive feedback. He did some searches and realized there weren't any resources on YouTube for those with Stargardt disease. This encouraged Sam to start his own YouTube channel in December 2013, which was dedicated to sharing his honest experiences living with vision loss.

In the beginning, the channel's content was just whatever came to Sam's mind. Sam started the channel with the name The Blind Spot, as an homage to the vision impairments he experiences and because it's a literal spot on the internet that people can come to learn about vision loss. But as the channel has progressed, he's narrowed down the content, focusing

"The Blind Life is a representation of what is going on in my life and what I enjoy, all from a visually impaired perspective," says Sam. "I'm a big fan of mobile technology and low vision accessibility, but I enjoy also highlighting the humorous side of living with low vision while still sharing tips and tricks I've learned from the last 30+ years."

The Blind Life is currently the largest resource for assistive technology on the internet, with over 35,800 subscribers and over 645 videos. Sam produces everything on the YouTube channel himself, including the editing of each video. He uploads a new video to the channel every Saturday morning.



Sam leaning against a brick wall holding his white cane.

“

WHEN YOU'RE FIRST DIAGNOSED, IT'S VERY SCARY. THERE ARE MANY STAGES OF GRIEVING THAT YOU GO THROUGH, BUT IN THE END, IT'S GOING TO BE OK. YOU'RE GOING TO BE ABLE TO DO EVERYTHING YOU WANT AND NEED TO, YOU JUST MAY NEED TO LEARN A NEW WAY OF DOING IT.”

mostly on assistive technology and community-driven requests. As a member of many low vision Facebook groups, Sam will often read about other's interests in a specific topic or product. He'll then reach out to the company to share his platform and collaborate with them for a video. So, a couple of years ago, he renamed the channel The Blind Life, which better represents the well-rounded video content about living, adapting, and thriving with vision loss.

Now 44 years old, Sam can reflect on his life so far and realize his vision loss is "not the end of the world." He wants to share this with others in the blind and visually impaired community that may not have had their "aha" moment yet.

"When you're first diagnosed, it's very scary," says Sam. "There are many stages of grieving that you go through, but in the end, it's going to be OK. You're going to be able to do everything you want and need to, you just may need to learn a new way of doing it. I hope I can help you navigate through that and live your life to the fullest."

Retinal-Disease Therapy

Inherited Retinal Diseases and Dry AMD: 45 Trials (Select) | Updated July 2021

GENE THERAPIES

Progress

Achromatopsia (CNGB3) – AGTC	Phase 1/2
Achromatopsia (CNGB3) – MeiraGTx/Janssen	Phase 1/2
Achromatopsia (CNGA3) – AGTC	Phase 1/2
Achromatopsia (CNGA3) – Tübingen Hosp	Phase 1/2
AMD (Dry) – Gyroscope	Phase 2
Choroideremia (REP1) – 4DMT	Phase 1/2
Choroideremia (REP1) – Biogen	Phase 3
Choroideremia (REP1) – Spark	Phase 1/2
Choroideremia (REP1) – Tübingen Hosp	Phase 2
LCA (GUCY2D) – Atsena	Phase 1/2
LCA and RP (RPE65) – MeiraGTx/Janssen	Phase 1/2
LCA and RP (RPE65) – Spark	FDA Approved
RP (PDE6B) – Horama	Phase 1/2
RP, Usher, others (optogenetic) – Allergan	Phase 1/2
RP, Usher, others (optogenetic) – Bionic Sight	Phase 1/2
RP, Usher, others (optogenetic) – GenSight	Phase 1/2
RP, Usher, others (optogenetic) – Nanoscope	Phase 2
RP (RLBP1) – Novartis	Phase 1/2
RP (PDE6A) – Tübingen Hosp	Phase 1/2
Retinoschisis (RS1) – NEI	Phase 1/2
X-linked RP (RPGR) – AGTC	Phase 1/2
X-linked RP (RPGR) – MeiraGTx/Janssen	Phase 1/2
X-linked RP (RPGR) – 4DMT	Phase 1/2
X-linked RP (RPGR) – Biogen	Phase 2/3

CELL-BASED THERAPIES

Progress

AMD-dry (RPE) – Astellas	Phase 1/2
AMD-dry (RPE) – Cell Cure	Phase 1/2
AMD-dry (RPE from iPSC) – NEI	Phase 1/2
AMD-dry (RPE on scaffold) – Regen Patch	Phase 1/2
RP, Usher (retinal progenitors) – jCyte	Phase 2b
RP, Usher (retinal progenitors) – ReNeuron	Phase 2
Stargardt (RPE) – Astellas	Phase 1/2

MOLECULES, PROTEINS, AONS

Progress

AMD-dry (C3 inhibitor) – Apellis	Phase 3
AMD-dry (CB inhibitor) – Ionis	Phase 2
AMD-dry (C5 inhibitor) – Iveric bio	Phase 2
LCA (CEP290, AON) – ProQR	Phase 2/3
LCA (CEP290, CRISPR) – Editas	Phase 1/2
RP (RHO, AON) – ProQR	Phase 1/2
RP (NAC-anti-oxidant) – Johns Hopkins	Phase 2
Stargardt disease (emixustat) – Acucela	Phase 3
Stargardt disease (deuterated vit A) – Alkeus	Phase 2
Stargardt disease (C5 inhibitor) – Iveric bio	Phase 2
Stargardt disease (anti-RBP4) – Belite Bio	Phase 1
Stargardt disease (anti-RBP4) – Stargazer	Phase 2
Usher syndrome (NACA-anti-oxidant) – Nacuity	Phase 1/2
Usher syndrome 2A (AON) – ProQR	Phase 1/2

Visit [FightingBlindness.org/Clinical-Trial-Pipeline](https://www.fightingblindness.org/Clinical-Trial-Pipeline) for more details and trial contact information. This document is for informational purposes only. Information is subject to change, and its accuracy cannot be guaranteed.

EVENT HIGHLIGHTS

Hope from Home: A Night to Save Sight

On Sunday, February 28, 2021, the Foundation Fighting Blindness kicked off the 50th anniversary celebrations with Hope from Home, which included celebrity emcee Saturday Night Live alum Kevin Nealon, musical performances by Lachi and Charlie Kramer, and the presentation of our highest research honor – the Llura Liggett Gund Award – to Jean Bennett, MD, PhD. More than 600 households logged in to experience the Foundation's first-ever virtual gala, which was chaired by Foundation board members and leaders Jonathan Steinberg, MD and Alice Cohen, MD. Thanks to the generosity and support of the event committee, sponsors, attendees, and donors, over \$600,000 was raised for the Foundation's mission.

Night for Sight: Celebrating 50 Years of the Foundation

On Thursday, June 24, 2021, the Foundation Fighting Blindness celebrated its 50th anniversary and its new Beacon Society through Night for Sight, a virtual gala presented by Two Blind Brothers. The Foundation cannot say thank you enough to everyone who made this event a success! Thanks to the generosity and support of so many, Night for Sight raised over \$570,000 for critical research finding treatments and cures for blinding diseases. This event was led by co-chairs and Foundation board members Jason Ferreira and Evan Mittman and featured phenomenal entertainment from music icon and nine-time Grammy winner Sheryl Crow. Through Night for Sight, six spectacular leaders were recognized as part of our inaugural Beacon Society: Allegiance Retail Services, Peter & Gretchen Crowley, Porky Products, Glenn Sblendorio, Scott Sennett, and John Sharko.

Join a Fall VisionWalk!

The Foundation is excited to announce the return of this fall's VisionWalks in person! Please join us for a day of family fun that you won't want to miss.

Fall VisionWalks are returning in the following regions:

- Columbus
- Indianapolis
- Cincinnati/Northern Kentucky
- Twin Cities
- St. Louis
- Philadelphia
- Colorado
- Pittsburgh
- Seattle
- Charlotte
- San Diego
- Boston
- Los Angeles
- Westchester-Fairfield
- Triad

To register for your local VisionWalk, visit: [VisionWalk.org](https://www.visionwalk.org)



Ten VisionWalk participants wearing past VisionWalk t-shirts with two guide dogs laying down in front of the group.

CHAPTER SPOTLIGHT

The Strength of the Foundation is Through Our Communities

The Foundation Fighting Blindness Chapter network brings people together across the country to join in fighting blinding retinal diseases. Our family of volunteers and leaders stand up for those impacted by blinding diseases. From day one, they share the latest research and advancements, and provide access to local resources needed to guide individuals through their personal journey. And together, they celebrate the Foundation's mission.

We are winning thanks to the dedication of our Chapters focusing on three key areas: education, resources, and revenue. Through informative sessions, Foundation staff, community, and national experts provide research information, low vision resources,

and outreach to our local retinal specialists and eye care professionals. To continue accelerating our mission, each community is invited to be a stakeholder by participating in our fundraising events.

Our Chapter leadership team is growing and is looking for dedicated supporters like you! Join your local Chapter leadership team and help move forward the mission of the Foundation as we work to find treatments and cures for blinding diseases.

Not interested in leadership but still want to be involved? Reach out to your local Chapter to learn more about the upcoming events in your area.

For questions and more on getting involved, contact: Chapters@FightingBlindness.org

RESEARCH HIGHLIGHTS

Update on Clinical Trial for jCyte's Cellular Therapy for RP

At the May 2021 virtual meeting of the Association for Research in Vision and Ophthalmology (ARVO), David Liao, MD, PhD, a partner at Retina Vitreous Associates Medical Group in Los Angeles, presented an encouraging update on jCyte's 84-participant, Phase 2b clinical trial of its cellular treatment for people with retinitis pigmentosa (RP) and related conditions. Thanks to the positive results, jCyte is planning a Phase 3 trial for the emerging treatment.

The goal of jCyte's treatment is to preserve photoreceptors and restore function to photoreceptors that are no longer providing vision but haven't fully degenerated. The approach, known as neuroprotection, is designed to work regardless of the mutated gene causing the patient's vision loss.

Dr. Liao reported that the greatest effect from the treatment was observed for patients receiving 6 million cells, the highest dose. The average change in visual acuity for all patients receiving the highest dose was 4.5 letters (about 1 line on an eye chart) greater than for those receiving a sham procedure (placebo).

However, the effect of the treatment was more significant for a subpopulation of patients with better vision and more remaining photoreceptors at the start of the trial. The average change in visual acuity for this subpopulation receiving the highest dose was 14.5 letters (about three lines on an eye chart) greater than for those receiving the sham procedure. jCyte believes this subpopulation will be better responders to the treatment in the planned Phase 3 clinical trial.

Fighting RP on the Foundation's Frontline

Michelle Glaze, the Foundation's associate director of professional outreach, shared her personal story with retinitis pigmentosa (RP) in the film 'Decoding disease,' as part of the International Council of Biotechnology Associations and Biotechnology Innovation Organization's Nature's Building Blocks series, produced by BBC StoryWorks, and presented by Blueprint Genetics. Watch the 'Decoding disease' film by visiting our website (FightingBlindness.org/michelle) and read her story in her own words below.

by Michelle Glaze

My vision loss journey started long before I was aware that I had a retinal disease. I grew up in a small town in California. My days were full of exploration and excitement. A tomboy at heart, I was always outside, active, and engaged in sports. My first glimpse of any visual challenges surfaced in my early 20s after a long day of co-ed softball. My eyes took a long time to adjust to the change in light from outside to indoors. Shortly thereafter, I went to the optometrist for a checkup and learned that something was atypical with my retinas. The physician recommended that I see a retina specialist for further testing.

About a year later, in 2004, I was clinically diagnosed with an inherited retinal disease. As one can imagine, this is a devastating moment, one that I will never forget. I would lose my vision over time, and there was not a treatment.

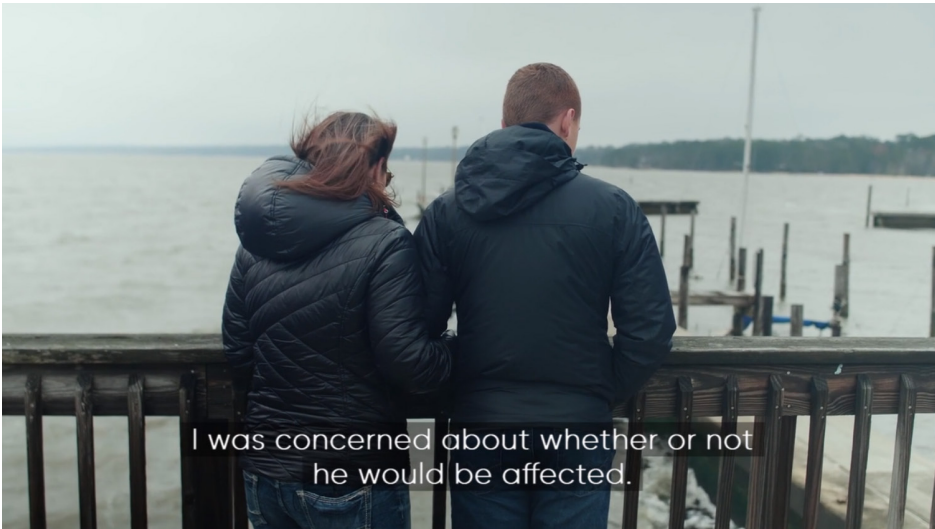


Over the next several years, I did my best to ignore the diagnosis. I was in complete denial, determined that my vision would not worsen. As I noticed changes in my sight, I did everything possible to hide the signs. I was embarrassed, angry, and afraid.

Four years after my diagnosis, my beautiful son was brought into this world, and that changed everything. He inspired me to push forward, adjust, and take control of my visual challenges. I wanted to DO SOMETHING

– fight to save my vision. This deep desire led me to the Foundation Fighting Blindness. In 2011, I shared my story for the first time and formed a VisionWalk team. It was empowering to be part of the mission to drive research in hope of finding treatments and cures for blinding retinal diseases. My son was by my side during that VisionWalk and has been with me every step of the way ever since.

After being encouraged by my retina specialist to do so, I sought genetic testing in



After many tears of joy and gratitude, I embraced that reality and gift when I was offered the position of associate director of professional outreach. For over two years, I have been helping eye care professionals around the U.S. learn about all the wonderful resources available for individuals with an inherited retinal disease.

My work with the Foundation has helped me accept my vision loss while providing a tremendous amount of optimism for the future. The research and clinical trials underway give me hope. My dream is to see my son's face clearly one day. However, if my remaining vision is simply preserved, that would have a huge impact, and I would be extremely grateful. Thus, I will continue to move forward towards a future where there is a treatment, a cure, with my son by my side every step of the way.

Still shot from the 'Decoding disease' film of Michelle Glaze and her son on a pier with subtitles that say, "I was concerned about whether or not he would be affected"

2013. I was very concerned that my son may have the same genetic disorder as mine. Additionally, I wanted to know my genetic mutation so that I could find and follow any research that may be underway. Genetic testing results indicated mutations in RP1 to be the likely cause of my retinal disease, retinitis pigmentosa. Through genetic testing and genetic counseling, I learned that my disease is recessive. This was

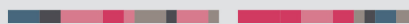
a huge relief, as it meant that my son has little to no risk of having retinitis pigmentosa. I felt like a weight was lifted from my shoulders the moment that I heard this wonderful news.

In 2019, an opportunity to join the Foundation as an employee surfaced, and I was excited to be considered. I would have never believed that one day, I would join the team at the Foundation.

FOUNDATION FIGHTING BLINDNESS



Blueprint Genetics



InformedDNA[®]
Genetics, Decoded.

The Foundation, in partnership with Blueprint Genetics and Informed DNA, offers an open access, no-cost genetic testing program that may be used by any retinal healthcare provider capable of diagnosing an inherited retinal disease. If you're interested in learning more, visit: FightingBlindness.org/genetics

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Are you an eye care professional?

The Foundation Fighting Blindness can help eye care professionals provide support and information to enable patients to better understand and manage their retinal conditions. The Professional Outreach team educates eye care professionals about patient resources, the My Retina Tracker® Genetic Testing Program and Registry, clinical research and emerging therapies, as well as Foundation-hosted educational meetings. To find out more, visit: FightingBlindness.org/for-eye-care-professionals

IN FOCUS

This and previous issues of **In Focus** are available online, where you can get the latest retinal-research information, as well as updates on the Foundation's activities, on your PC and mobile devices.

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