IN FOCUS

SPRING/SUMMER 2023

A Publication for Members of the FOUNDATION FIGHTING BLINDNESS

FDA Approves Apellis' SYFOVRE™, for the Treatment of Geographic Atrophy Secondary to Age-Related Macular Degeneration (AMD)

Apellis

Apellis Pharmaceuticals, a global biopharmaceutical company, announced the U.S. Food & Drug Administration (FDA) has approved SYFOVRE™ (pegcetacoplan injection) for people with geographic atrophy (GA) secondary to age-related macular degeneration (AMD), a leading cause of devastating central vision loss in people over 55 in developed countries.

The newly approved therapy is the first ever approved by the FDA for GA. The company expects an approval decision for SYFOVRE from the European Medicines Agency (EMA) in early 2024. A marketing application has also been submitted to Health Canada.

SYFOVRE slowed the growth of lesions (areas of retinal cell loss) in two Phase 3 clinical trials. In the trials, known as DERBY and OAKS, the treatment was safe and its beneficial effect increased over time. More than 12,000 injections were delivered to trial participants over 24 months.

At 24 months in the OAKS trial (637 participants), monthly injections slowed GA lesion growth by 22 percent. Injections administered every-other-month slowed GA lesion growth by 18 percent.

At 24 months in the DERBY trial (621 participants), monthly injections slowed lesion growth by 18 percent. Injections administered every-other-month slowed lesion growth by 17 percent.

SYFOVRE is delivered through an injection into the vitreous, the soft gel in the middle of the eye, once every 25 to 60 days in an eye doctor's office. Frequency of the injections is at the doctor's discretion.

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WHAT'S INSIDE

HELP FIGHT BLINDING DISEASES

Use the enclosed envelope to make a life-changing gift.

In Focus is published by:
Foundation Fighting Blindness, Inc.
6925 Oakland Mills Road, #701
Columbia, MD 21045

COVER STORY
CONTINUED FROM FRONT COVER

"We are delighted by the FDA's approval of SYFOVRE for addressing this critical unmet medical need. This is a historic moment in the fight against blindness associated with dry AMD," says Claire Gelfman, PhD, chief scientific officer, Foundation Fighting Blindness.

SYFOVRE is designed to slow the progression of GA by inhibiting C3, a protein in the complement system. While complement is part of the human immune system that wards off harmful bacteria and viruses, it can cause damage if not controlled properly. Researchers have strong evidence that an overactive complement system is involved in the death of retinal cells in AMD.

GA lesions are the regions in the retina where degeneration from dry AMD has occurred. The fovea is a small pit in the center of the retina, rich in cone photoreceptors, that provides the sharpest vision, including the ability to read and recognize faces. GA lesions often first develop outside of the fovea — these are known as extrafoveal lesions. Over time, the lesions encroach on the foveal region leading to more significant vision loss. Experts believe that targeting extrafoveal lesions with treatments before they affect the fovea may be the optimal strategy for preserving vision in people with GA.

"The approval of SYFOVRE is truly a special moment for the Foundation Fighting Blindness family and a victory for all people with advanced dry AMD who otherwise face relentless vision loss," says Jason Menzo, chief executive officer at the Foundation Fighting Blindness. "With our unwavering commitment to driving the advancement of treatments and cures for dry AMD and inherited retinal diseases, we celebrate this historic milestone."

If you have geographic atrophy (GA) associated with dry AMD, consult your retinal specialist to determine if SYFOVRE is appropriate for you.

Apellis Pharmaceuticals is a corporate partner of the Foundation Fighting Blindness.

CHAPTER HIGHLIGHTS

We Are Growing!

The Foundation is actively expanding our Chapters across the country, growing from 41 to 58 in the next year. The first three new Chapters are in Portland, Maine; Miami, Florida; and Oklahoma City, Oklahoma. The local volunteer leadership team in each community is already planning Chapter activities and events.

LULIE'S NEXT CHAPTER for LIGHT & VISION
FOUNDATION FIGHTING BLINDNESS

Get engaged with your local Chapter by visiting, **www.FightingBlindness.org/find-your-local-chapter**.

Interested in helping to launch a Chapter in your area? Reach out at: **Chapters@FightingBlindness.org**

Vision Seminars Are Back

The Foundation's Vision Seminars are live and in-person again! These free half-day seminars provide the latest information on blinding retinal diseases and age-related macular degeneration (AMD). Featured speakers will share recent scientific advancements, current and upcoming clinical trials, the latest on genetic testing, and more.

We kicked off this year by hosting our first Vision Seminar in Phoenix, Arizona, on Saturday, January 21. Constituents from all over Arizona came to hear all the latest information on wet/dry age-related macular degeneration (AMD) from Dr. Benjamin Bakall, Dr. Mark Barakat, Dr. Claire Gelfman, and Dr. Jordan Graff.

Our second Vision Seminar was in Tampa, Florida, on Saturday, February 4, where the speakers shared the recent advancements and treatments on AMD. This event brought together three leaders in the AMD space, including Dr. David Eichenbaum, Dr. Claire Gelfman, Dr. Sandeep Grover, and Dr. Martin Pera.

On Saturday, February 18, our Houston Chapter hosted a Vision Seminar in Texas. This Vision Seminar was the first to focus on the entire realm of blinding retinal diseases. Dr. Steve Daiger, Ben Shaberman, Dr. Tim Stout, and Dr. Jenny Wood all focused on the latest and greatest research and clinical trials.

Want to learn about the latest science news from the world of vision?

Check out our Eye on the Cure Podcast, hosted by Ben Shaberman, VP, Science Communications. Stream the Podcast on SoundCloud, Spotify, Audible, Pandora, and more:

www.FightingBlindness.org/Podcasts

Scan the QR code to go directly to the web page on your device.

BEACON STORY

Birding Blind: Identifying Birds by Song

When springtime arrives in late March, Martha Steele is filled with excitement to hear that first bird song of the season. Born and raised in Vermont, Martha's second home in the Northeast Kingdom has well over 100 bird species within the property line alone. Martha's been birding for over 30 years, but the way in which she birds has changed over the years.

Martha was diagnosed with severe hearing loss at the age of five. Subsequently, she received hearing aids and attended for her first-grade year a school for children with hearing impairments, which was about 70 miles away from where her family lived, so she stayed with a foster family while attending school. Throughout her childhood, Martha also experienced night blindness and issues with her peripheral vision. But it wasn't until she was 25 years old that she was also diagnosed with retinitis pigmentosa. After attending a Foundation Fighting Blindness VISIONS conference almost 13 years later, Martha discovered that she had Usher syndrome. Later genetic testing revealed that she had mutations in her USH2A gene.

As she gained more information about her disease, Martha became more involved with the Foundation Fighting Blindness. Martha is now the Boston Chapter President, chaired the Boston VisionWalk for several years, and participates in the VisionWalk every year, raising around $25,000 annually and a total of about $350,000 over 14 years. She's also been a National Trustee since 2013 and Board Director as of July 2020.

"I'm very passionate about the Foundation's mission," says Martha. "As a scientist, I'm

Martha Steele outside birding with her husband, Bob Stymeist, and her guide dog, Alvin.

pretty realistic, so I realize finding treatments and cures will take a long time and persistent support. I often encourage others in the vision loss community that if we don't support the research, then we can't expect others to do it instead."

While remaining involved with the Foundation, Martha also had a successful career working for the Massachusetts Department of Public Health (MDPH) for many years. In 2015, she retired from her position as Deputy Director of the Bureau of Environmental Health at MDPH.

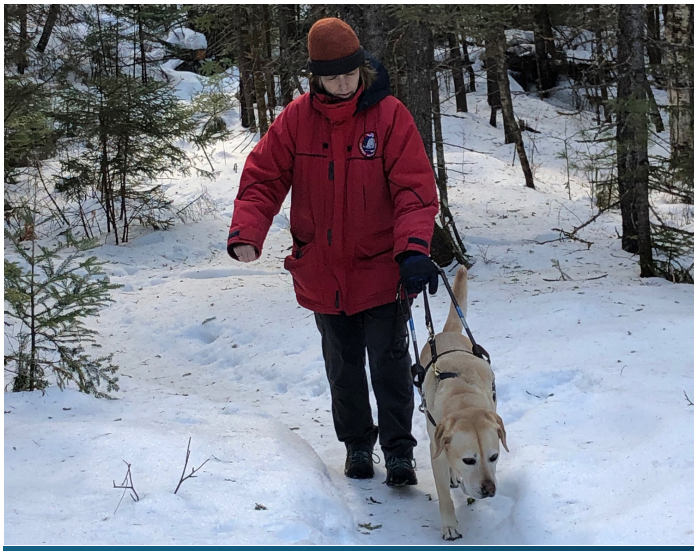
Over the years, Martha's vision has changed drastically. In the early 2000s, Martha's central vision began to decline, and it became difficult to see people's faces or lips, which

can be challenging for someone with a hearing impairment. In 2010, because of increasing difficulty reading lips to help her understand speech, Martha received her first cochlear implant and then got her second one in 2015.

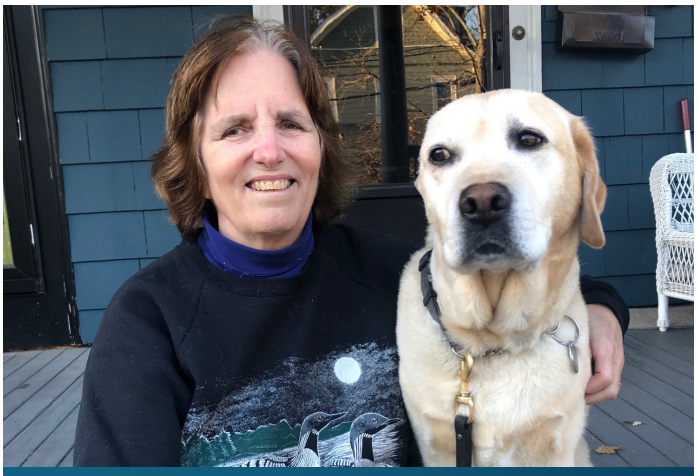
Martha is also on the board of directors for the Carroll Center for the Blind in Newton, Massachusetts, where she received her vocational training to gain general independent living skills.

“There are many stages to vision loss, and those stages are different for everyone,” says Martha. “Many people don’t understand legal blindness or that blindness isn’t just black and white. I just want to be a part of that education to teach people vision loss is a spectrum.”

Now 70 years old, Martha has given up some activities, such as scuba diving, because they are dangerous. But the one activity that’s been steady in her life for the past 30-plus years is birding. Martha and her husband, Bob



Martha outside walking in the snow with her guide dog, Alvin, leading her.



Martha and her guide dog, Alvin.

Stymeist, met while birding. Bob is one of the top birders in New England, so he’s been a great teacher for Martha.

When Martha first started birding, she could see the birds but not hear them. But since she received her cochlear implants and her vision declined to total blindness, Martha now birds entirely by ear and has learned the songs of about 150 bird species.

“Sighted birders actually bird a lot by ear and use what they hear to better locate a bird visually,” says Martha. “So, birding is one of those avocations where a blind birder can actually be better than a sighted birder who is not very familiar with identifying birds by song.”

During the height of bird migration in the spring, Martha and Bob are outside almost all day birding. Birding is calming and grounding for Martha, and she loves being focused on just that moment.

“Very often, you can hear a bird, but you can’t see it, especially in a dense forest,” says Martha. “So I’m able to identify them by ear without ever seeing them. It really levels the playing field; birding is a hobby that someone blind or visually impaired can do as well, if not better than others. It always feels great when I’m out in the field with a bunch of sighted birders, and I can tell them what bird just sang.”

CLINICAL-TRIAL PIPELINE

Retinal-Disease Therapy

Inherited Retinal Diseases and Dry AMD: 41 Trials (select) | Updated March 2023

GENE THERAPIES (GENE TARGET)	PROGRESS
Achromatopsia (CNGB3) – AGTC	Phase 1/2
Achromatopsia (CNGB3) – MeiraGTx/Janssen	Phase 1/2
Achromatopsia (CNGA3) – MeiraGTx/Janssen	Phase 1/2
Achromatopsia (CNGA3) – Tubingen Hosp	Phase 1/2
AMD-dry (CFI) – Novartis (Gyroscope)	Phase 2
AMD-dry (CFH) – Perceive Bio	Phase 1/2
Batten disease (CLN5) – Neurogene	Phase 1/2
Choroideremia (REP1) – 4DMT	Phase 1/2
LCA (GUCY2D) – Atsena	Phase 1/2
LCA and RP (RPE65) – MeiraGTx/Janssen	Phase 1/2
RP (PDE6B) – Coave	Phase 1/2
RP (RLBP1) – Novartis	Phase 1/2
RP (NR2E3, RHO) – Ocugen	Phase 1/2
RP (PDE6A) – Tubingen Hosp	Phase 1/2
Retinoschisis (RS1) – NEI	Phase 1/2
X-linked RP (RPGR) – AGTC	Phase 2
X-linked RP (RPGR) – MeiraGTx/Janssen	Phase 3
X-linked RP (RPGR) – 4DMT	Phase 1/2

RNA/OTHER THERAPIES (MECHANISM)	PROGRESS
AMD-dry (CB inhibitor) – Ionis	Phase 2
LCA (CEP290, AON) – ProQR	Phase 2/3
RP, Usher, others (optogenetic) – Bionic Sight	Phase 1/2
RP, Usher, others (optogenetic) – GenSight	Phase 1/2
RP, Usher, others (optogenetic) – Nanoscope	Phase 2
Stargardt disease (optogenetic) – Nanoscope	Phase 2
Usher syndrome 2A (AON) – ProQR	Phase 2/3

CELL-BASED THERAPIES (CELL TYPE)	PROGRESS
AMD-dry (RPE) – Lineage	Phase 1/2
AMD-dry (RPE) – Luxa	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
Stargardt disease (RPE) – Astellas	Phase 1/2

SMALL MOLECULES (MECHANISM)	PROGRESS
AMD-dry (C5 inhibitor) – Iveric bio	Phase 3
RP (NAC-anti-oxidant) – Johns Hopkins	Phase 3
RP (methotrexate) – Aldeyra	Phase 2
RP (small molecule) – Endogena	Phase 1/2
RP (small molecule, photoswitch) – Kiora	Phase 1/2
Stargardt disease (deuterated vit A) – Alkeus	Phase 2
Stargardt disease (C5 inhibitor) – Iveric bio	Phase 2
Stargardt disease (anti-RBP4) – Belite Bio	Phase 3
Stargardt disease (metformin) – NEI	Phase 1/2
Usher syndrome (NACA-anti-oxidant) – Nacuity	Phase 1/2

Note: Some trials listed may have been paused and/or the sponsors are seeking partners to continue their trials.

For more details and trial contact information, visit **www.FightingBlindness.org/Clinical-Trial-Pipeline**
This document is for informational purposes only. Information is subject to change, and its accuracy cannot be guaranteed.

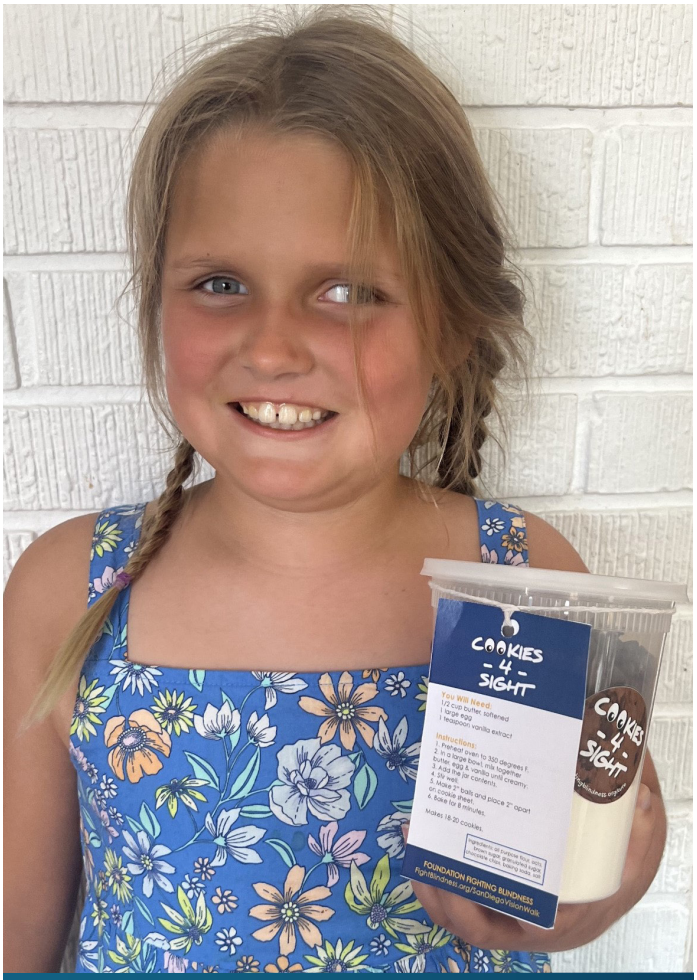
Our VisionWalkers are getting creative! Check out how participants across the country are raising awareness and funds for their local VisionWalk!

Cookies-4-Sight Fundraiser

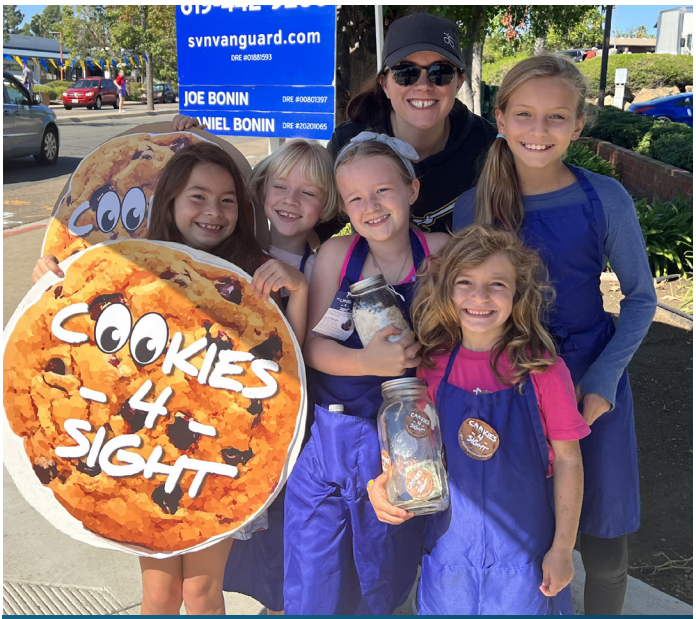
Denise and Peter Keller and big sisters Mackenzie and Kylie welcomed baby Paisley in 2013. Having a third child completed the family, but it wasn't long before life became more complicated. After many tests, Paisley was diagnosed with Leber congenital amaurosis (LCA). When Paisley turned four, the Keller family became involved with Foundation Fighting Blindness and started participating in the San Diego VisionWalk.

In 2017, Denise and friends were brainstorming on how they could raise more money and awareness for those with vision loss, like her daughter Paisley. Out of those discussions, Denise decided to create a fundraiser called Cookies-4-Sight.

In the first year, volunteers known as the "Cookie Tribe" assembled 100 jars of the best chocolate chip cookie mix early in the morning. A hang tag provided the recipe and also shared Paisley's story along with information about the Foundation Fighting Blindness. The "Cookie Tribe" went to busy street corners to sell the jars, raising over $1,000 in the first year. Last year, in 2022, Cookies-4-Sight celebrated its five-year anniversary, selling 400 jars and raising over $6,000 for their VisionWalk team.



Paisley holding one of the chocolate chip cookie mix jars with a Cookies-4-Sight tag.



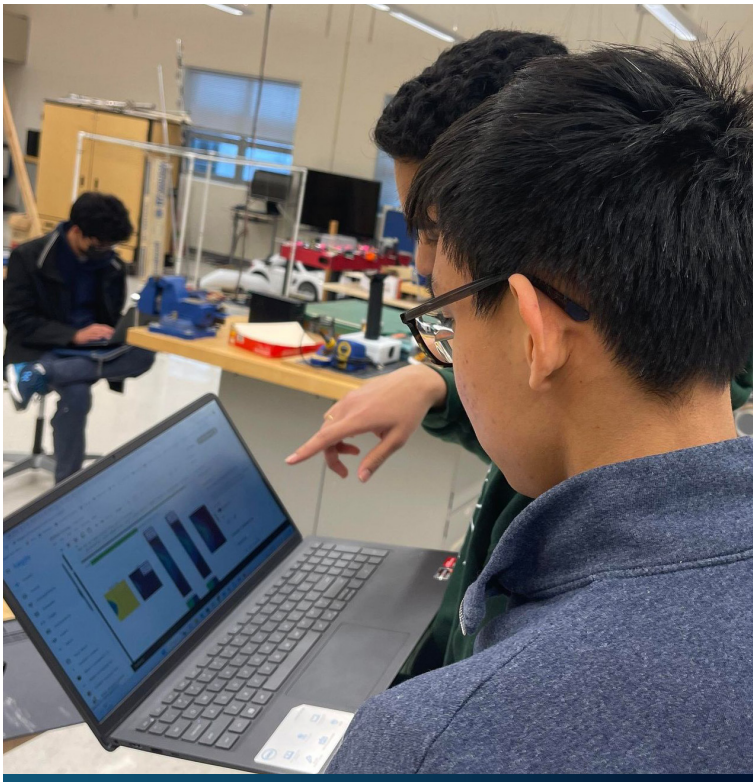
Group of girls wearing blue aprons holding Cookies-4-Sight jars and signs, shaped like a chocolate chip cookie.

VISIONWALK SPOTLIGHTS

CodeOn 2023 Fundraiser

Edward Zhang was diagnosed with X-linked retinoschisis as a young child. While ongoing complications put him in the operating room frequently, he has always continued to find new ways to learn, play, and function as his vision has changed over time.

He recalled adapting his methods to solve Rubik's cubes as his changing vision made it difficult for him to see and track the colors. Throughout his journey, his sense of confidence and belonging was bolstered by meeting optometrists, researchers, and others with visual impairments.



Edward and a friend looking and pointing at a laptop.

Edward is now a high school senior in Northern Virginia. His experience inspired him to give back to the visually impaired community, so he organized a hackathon called CodeOn 2023 in support of his DC Metro VisionWalk team this spring.

"I had previously established an artificial intelligence research club at my school to teach kids how to apply AI algorithms to real world problems, and the hackathon is an extension of this," explained Edward. "Teams of students will tackle real world problems as we spread awareness. A team could, for example, create a usable low vision navigation aid as part of the hackathon."

To learn more about Edward's journey and the hackathon event, visit: **Give.FightingBlindness.org/CodeOn2023**

Join Us for a Spring VisionWalk!

The Foundation is excited to be hosting our VisionWalk events across the country this spring/summer to raise awareness and funds for the Foundation's mission. Join the tens of thousands of people taking important steps toward treatments and cures by participating in a VisionWalk near you. Register for your local Walk today by visiting, **www.VisionWalk.org**.

SparingVision Receives Authorization to Launch US Clinical Trial for its Cone-Preserving Treatment

SparingVision, a French company developing therapies for ocular conditions including inherited retinal diseases, has received authorization from the U.S. Food & Drug Administration (FDA) to launch a Phase 1/2 clinical trial known as PRODYGY for SPVN06, its gene-independent, cone-preserving therapy for people with retinitis pigmentosa (RP). The company is also seeking authorization to conduct the trial in France. The U.S. trial will take place at the University of Pittsburgh Medical Center. SparingVision plans to enroll a total of 33 RP patients who have disease-causing mutations in PDE6A, PDE6B, or RHO.

SparingVision is funded through the Foundation's RD Fund, a venture philanthropy fund for emerging therapies that are in or nearing early-stage clinical trials. The Foundation also provided several years of research grant funding for the preclinical development of SPVN06.

Opus Genetics to Launch Gene Therapy Clinical Trial for LCA5 Patients

Opus Genetics, a company developing gene therapies for people with inherited retinal diseases, has received authorization from the U.S. Food & Drug Administration (FDA) to launch a clinical trial for its emerging gene therapy for Leber congenital amaurosis 5 (LCA5), which causes significant vision loss in children with mutations in the gene that expresses the protein lebercillin. The Phase 1/2 clinical trial, enrolling nine adult patients, will take place at the University of Pennsylvania. The company plans to begin patient enrollment in the first quarter of 2023.

The LCA5 gene-therapy clinical trial will be the first launched by Opus, a company originally conceived and formed by the Foundation Fighting Blindness. Founded in 2021, Opus received $19 million in seed funding from the Foundation's RD Fund. The company is led by Ben Yerxa, PhD, former chief executive officer of the Foundation.

Atsena's LCA-GUCY2D Gene Therapy Improves Vision in Phase 1/2 Clinical Trial

Atsena Therapeutics, a gene therapy development company focused on preventing and reversing blindness, announced positive results from its Phase 1/2 gene therapy clinical trial for people with Leber congenital amaurosis type 1 (LCA1), which is caused by mutations in the gene GUCY2D. Data from the trial was presented on October 1, 2022, at the American Academy of Ophthalmology Annual Meeting in Chicago.

The company reported results for 15 trial participants. Overall, the gene therapy, ATSN-101, was well tolerated. The nine patients receiving the highest dose of ATSN-101 had clinically meaningful vision improvements as measured by a full-field stimulus test (FST), which measures the patient's ability to respond to different levels of light, and by their ability to navigate a multi-luminance mobility course.

RAISING OUR SIGHTS DIY STORY

SeeShore Fest Fundraiser Success

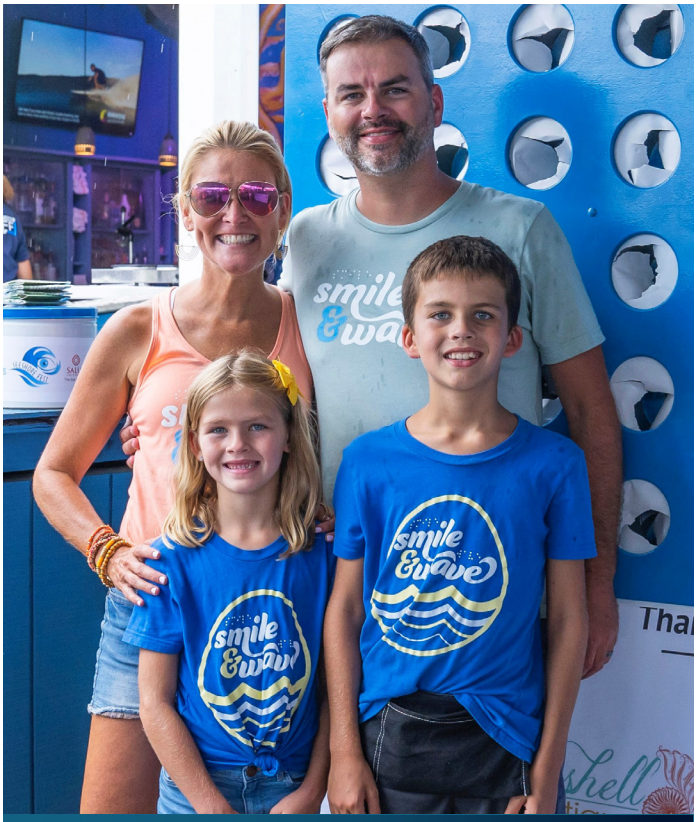
Heather Napolitano distinctly remembers the first time she noticed the vision in her left eye was beginning to diminish. It was 2013, and her regular eye doctor in Baltimore, Maryland, couldn't figure out what was going on with her eyes, so she was referred to another eye specialist. This turned into a prolonged search for Heather's diagnosis.

"At that time, I thought you were blind, or you weren't," recalls Heather. "I didn't realize blindness was a spectrum then, and I didn't know who to turn to for help."

A year into seeking the cause of Heather's vision loss, a friend suggested she reach out to the Foundation Fighting Blindness. Heather got connected with her local Foundation Chapter at the time, learned more about blinding diseases, and met others like her by participating in the Philadelphia VisionWalk and other local events.

Amid waiting for answers, Heather continued working at her sales and marketing job at Red Bull until 2014, when her vision began to deteriorate further. In 2015, Heather had to give up driving, so she and her family moved to Rehoboth Beach, Delaware, where her parents live for additional support.

After the Napolitano's move, Heather wanted to stay busy and felt inspired to help raise awareness for those with vision loss like herself. So, Heather decided to create a Raising Our Sights do-it-yourself (DIY) fundraiser to benefit the Foundation Fighting Blindness. Heather knew the manager at The Starboard in Dewey Beach, Delaware, so she started by asking if she could host a fundraiser there. The manager quickly gave her a date, and she immediately began brainstorming how to fundraise.



Heather Napolitano and her husband Dom and two children, Daxton and Laityn, at the 2022 SeeShore Fest.

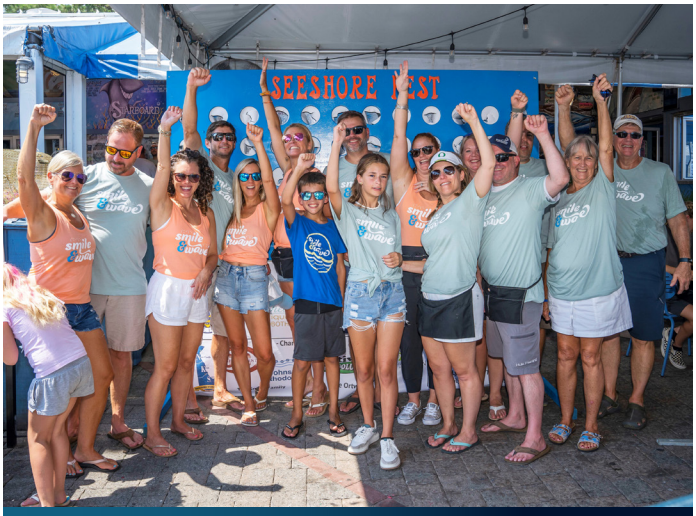
One of those days, Heather was watching The Price Is Right, and a game came on called "Punch-A-Bunch." Heather thought this could be a great opportunity to raise funds, so her husband, Dom, built a board for their event with 60 punches. Heather decided that every "punch" would cost $20, and the prize behind it was valued at $25 or more. And all the prizes were donations Heather received from asking local businesses.

The first year, Heather's DIY fundraiser, called SeeShore Fest, was a huge hit, with the punch board being the highlight of the day. So, in the following years, Heather filled several punch boards with prizes throughout the SeeShore Fest. In 2021, Heather decided to create an even bigger punch board, with punches costing $40 a piece and the prizes valued even higher. Heather also raises funds at the SeeShore Fest by selling custom t-shirts, raffle tickets, and sponsorships.

In the spring of 2022, Heather was finally given a diagnosis of autoimmune retinopathy after connecting with doctors at Scheie Eye Institute and the Wills Eye Hospital in Philadelphia, Pennsylvania, who worked tirelessly to test and get a definitive answer for her.

Additionally, Heather recently connected with Salus University's Erin Kenny, OD, FAAO, Chief of the Feinbloom Low Vision Center, whose assistance and recommendation of low vision resources have been life changing. Dr. Kenny also works with the Foundation's Professional Outreach Team to provide resources for her inherited retinal disease (IRD) patients. So, the 2022 SeeShore Fest also benefited Salus University's Feinbloom Low Vision Center.

The 2022 SeeShore Fest, which was its seventh annual, took place on August 27, 2022, and with over 1,000 attendees throughout the day, they raised over $26,000, putting the fundraiser total since inception over $109,000.



SeeShore Fest participants punching their arms in the air wearing their 'Smile & Wave' t-shirts.

"I've met so many people in my community over the years from the SeeShore Fest," says Heather. "Vision loss is such a difficult disability because no one else can see it, which is why I think raising awareness through events like this is so important."

Join Us for the Philadelphia Tough Mudder

Earlier this year, the Foundation launched the brand-new **Vision Warriors** Endurance program (as part of the Raising Our Sights do-it-yourself program). The Vision Warriors program is committed to taking on the challenge of ending blinding diseases and pushing beyond our limits through fun, active and challenging programs to support the mission of the Foundation Fighting Blindness.

To kick off this new initiative, the Foundation is partnering with **Tough Mudder**. Built on teamwork and overcoming obstacles, Tough Mudder is the chance to unplug from the daily grind, experience the unexpected, and accomplish something bigger than yourself. Our Vision Warriors receive special race day perks and an invitation to join as a team or take on the challenge as an individual. All fundraising goes to support the Foundation's mission.

Join us to get down and dirty at our upcoming Spring Vision Warriors Tough Mudder events in **Philadelphia** on **May 20 and 21**! For more information on these upcoming opportunities, please visit, **www.FightingBlindness.org/VisionWarriors** or email **RaisingOurSights@FightingBlindness.org**.

IN FOCUS
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FOUNDATION FIGHTING BLINDNESS
Together, we're winning.

Foundation Fighting Blindness, Inc.
6925 Oakland Mills Road, #701
Columbia, MD 21045

Foundation Fighting Blindness meets all of the standards of the BBB Wise Giving Alliance.

Leave Your Legacy

The Foundation is fighting every day to find treatments and cures for blinding diseases, but we can't do it without your help. Most people can make a far more significant gift by including the Foundation in their will or trust or making a gift by beneficiary designation. Often called "legacy gifts," these types of deferred gifts allow you to continue to be part of the fight to end blinding diseases for many decades to come. For more information, visit:
www.FightingBlindness.org/legacy-giving

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.

For an online and accessible version of **In Focus**, visit **www.FightingBlindness.org/In-Focus-Newsletter**.

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