IN FOCUS

FALL/WINTER 2020

A Publication for Members of the FOUNDATION FIGHTING BLINDNESS

Foundation Fighting Blindness Commits $6.5 Million for New Retinal Disease Research Grants

New grants include development of CRISPR/Cas9 gene-editing treatments, new disease models, and a retinal regeneration therapy

If you or a loved one is losing vision to an inherited retinal disease, imagine having a drug that could enable your retinas to grow new photoreceptors and restore vision.

Tom Reh, PhD, retinal regeneration expert at the University of Washington.

Tom Reh, PhD, a retinal regeneration expert at the University of Washington, is receiving a new Foundation grant to do just that. He is continuing his innovative research in developing a treatment that empowers the retina for self-regeneration. While most regenerative retinal therapies involve transplantation of new retinal cells derived from stem cells, Dr. Reh's approach would enable a diseased retina to grow its own new photoreceptors, the cells that make vision possible.

"The immediate goal is to find out whether we can stimulate regeneration of new neurons in human retina from the Muller glia using the same factors that work in mice," says Dr. Reh.

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HELP FIGHT BLINDNESS

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

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Columbia, MD 21045

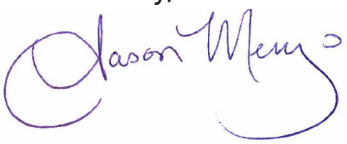
A MESSAGE FROM OUR COO

During times like these, I'm so proud to be a part of the Fighting Blindness family. Our entire community, including our staff, board members, trustees, committees, volunteers, chapters, and team captains from across the country, have stepped up to keep the Foundation's mission moving forward – so thank you for all your hard work and dedication.

Although our day-to-day lives are very unpredictable right now, our commitment to finding treatments and cures for blinding retinal diseases is unwavering. While there are clearly economic and healthcare challenges in today's COVID-impacted environment, the Foundation Fighting Blindness continues to have tremendous optimism for the breadth and potential of research happening across academia and the industry.

The COVID-19 pandemic has undoubtedly impacted the Foundation's fundraising and is forcing us to be creative as we move forward. Building off our virtual experiences in the spring, we are excited to continue hosting new and innovative events virtually through the rest of this year. With your continued support, we are committed to keeping the momentum going with robust and expanded research funding and meaningful community engagement. We will get through these tough times stronger than ever. We are stronger together – as a community.

Sincerely,



Jason Menzo, *Chief Operating Officer*

SCIENCE UPDATE

Continued from front cover

"If we are successful, I imagine a day when an ophthalmologist will give injections of a gene or two into a late-stage patient over a period of a few weeks and this will set in motion a process of remaking the cone photoreceptor cells in the fovea and restoring some vision to that person."

Dr. Reh's project is being supported by one of 15 new research grants, providing more than $6.5 million in funding, recently awarded by the Foundation. The projects were selected from 134 proposal submissions made by investigators in fall 2019. The submissions were rigorously evaluated and scored by the Foundation's Scientific Advisory Board, which is comprised of the world's top retinal disease experts. The new grants bring the Foundation's research portfolio to 84 projects.

"First and foremost, we are committed to projects that will lead to vision-saving treatments and cures," says Benjamin Yerxa, PhD, chief executive officer at the Foundation. "Our funding strategy also focuses on critical research gaps, that when addressed, will move the whole field forward in a significant way. For example, the grants for new models for RP, Usher syndrome type 1B, and Stargardt disease will have a major impact on therapy development. Proof-of-concept for a therapy in a model that closely replicates human disease can be the springboard for clinical trials."

For the complete list of new grants, visit: **FightingBlindness.org/funded-grants-2020**

FOUNDATION UPDATE

My Retina Tracker Registry: Recent Update

My Retina Tracker Registry is the Foundation's international registry for people with an inherited retinal disease (IRD). The goal of the Registry is to accelerate the development of treatments and cures for IRDs. It achieves this by providing a single comprehensive source of de-identified information for researchers, biotechnology and pharmaceutical companies, about people with an IRD. Using this data, these partners can understand the prevalence of each IRD by clinical diagnosis, gene, and mutation; and the variability of each disease. When there are opportunities in research studies, clinical studies, or clinical trials, partners can contact eligible patients, through the Registry staff.

As of May 2020, the Registry acquired several new updates, including a rebranded logo, a more user-friendly registry interface, and a significantly improved website layout that is easier to navigate, featuring the ability to integrate new technologies such as wearable data devices. The new Registry has also added enhanced security features, including two-factor authentication, to ensure data privacy.

FOUNDATION FIGHTING BLINDNESS

My Retina Tracker® Registry

To update your current profile or join if you are not already a member, visit: **MyRetinaTracker.org**

BEACON STORIES

Jumping into an Uncharted Arena

by Lauren Reeves

Thirty-one-year-old Wren Blae Zimmerman always loved horses. But it wasn't until a few years ago that Wren finally learned to ride. Now her life revolves around this equestrian dream, and she's given herself the title of the "Blind Show Jumper."

Wren has worn glasses since second grade, but it wasn't until high school that she started noticing problems seeing the board in class. When her regular eye doctor was unable to correct her vision, Wren was sent to several ophthalmologists and retina specialists who, after multiple tests, diagnosed her with Stargardt disease at the age of 17.

Over the next few years, Wren lost more of her central vision. She was pronounced legally blind halfway through college. The reality of Wren's situation began to set in, and she began to appreciate the importance of her happiness.

"Focusing on my quality of life has been really important in coping with my diagnosis," says Wren. "I've learned that it's OK to put yourself and your wants first. Just because you have a diagnosis like Stargardt doesn't mean you have to stop doing what you love."

At twenty-four Wren started taking horseback riding lessons at a therapeutic riding center in exchange for helping with the program. She then put everything on hold to pursue her lifelong dream of learning to jump horses.

Wren's instructor told her she would never be able to jump or control a horse due to her vision impairment. Undeterred, Wren continued to search for a trainer, and after being turned down by several, she finally found a trainer who took her from never having jumped an entire course to successfully competing against riders with 100% of vision. Within four years, Wren was on the same level as lifelong riders in shows at the 1.00-meter jumper level.

"Many people have told me that riding horses and competing like I am is literally impossible," says Wren. "But I'm doing it and well, so anything is possible."

Wren recently relocated to Lexington, Kentucky, where she can focus full-time on riding. Private donors and grants cover her training and competition fees, and she recently bought her own horse, Cassicasca (also known by his barn name, Valentine).

The strategy Wren has to take to prepare for a horse jumping show is much different and more intense than her competitors.

A typical show lasts four or five days and the position of jumps change every day. Each morning of competition, Wren goes into the arena with an aide, walks the perimeter, and stands by each jump, asking questions so

Wren with her horse Cassicasca, also known by his barn name, Valentine.

Wren jumping her horse, Cassicasca, over a hurdle.

she can get an idea of the space and the surroundings to make what she calls a "mental map."

Next, Wren goes to the course posting, which is a simple piece of paper, where her aide will transcribe the course to a large white board so that Wren can see it and memorize. Wren then goes back to the arena to walk out the course she has memorized and counts out the number of strides between each jump. This whole process usually takes Wren about two hours before the show even begins, while other riders can just go look at the course and then compete.

Wren also wears a Bluetooth earpiece for her trainer to instruct her on turns and how she's looking throughout the show.

"People will see me ride and it's only after my show that they say to me, "Wait, you're blind?" Wren recalls. "I love that I can be a part of this movement to make people realize, yes, I'm visually impaired, and I can ride horses, too."

Wren is the only blind and visually impaired person in United States who is competing at this jump level, with the goals of making the United States Equestrian Federation (USEF) Show Jumping Ranking List, becoming the first blind show jumper to compete at the top level, and eventually competing in the Paralympic Games.

Wren is advocating for the sport of jumping to become a Paralympic discipline and hopes that her endeavors will inspire and encourage interest, acceptance, and development of the sport of para-show jumping.

"Riding has given me back my confidence and a sense of independence that I thought I'd never have or feel again," says Wren. "This sport has allowed me to look forward, to dream, and to hope again."

Wren is an inspiration to children with vision impairments who reach out to her on social media. Many of them have also been told they couldn't do something because of their disability. Wren tells them they should stop at nothing.

"I've come to realize that my diagnosis has opened up doors for me to help others," says Wren. "My hope is that I can change society's perception about people who are blind and visually impaired and show that we can do anything."

If you'd like to learn more about Wren and help support her journey, visit: **wrenblae.com**

CLINICAL-TRIAL PIPELINE

Retinal-Disease Therapy

Inherited Retinal Diseases and Dry AMD: 41 Trials (Select) | Updated August 2020

GENE THERAPIES	Progress
Achromatopsia (CNGB3) – AGTC	Phase 1/2
Achromatopsia (CNGB3) – MeiraGTx	Phase 1/2
Achromatopsia (CNGA3) – AGTC	Phase 1/2
Achromatopsia (CNGA3) – Tubingen Hosp	Phase 1/2
AMD (Dry) – Gyroscope	Phase 1/2
Choroideremia (REP1) – 4DMT	Phase 1/2
Choroideremia (REP1) – Nightstar	Phase 3
Choroideremia (REP1) – Spark	Phase 1/2
Choroideremia (REP1) – Tubingen Hosp	Phase 2
LCA (GUCY2D) – Atsena	Phase 1/2
LCA and RP (RPE65) – MeiraGTx	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 (RLBP1) – Novartis	Phase 1/2
RP (PDE6A) – Tubingen Hosp	
Retinoschisis (RS1) – NEI	Phase 1/2
X-linked RP (RPGR) – AGTC	Phase 1/2
X-linked RP (RPGR) – MeiraGTx	Phase 1/2
X-linked RP (RPGR) – Nightstar	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
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
Usher syndrome (NACA-anti-oxidant) – Nacuity	Phase 1/2
Usher syndrome 2A (AON) – ProQR	Phase 1/2

Visit **ClinicalTrials.gov** 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.

VIRTUAL VISIONWALK

VisionWalk Going Virtual for 2020

The Foundation's first-ever National Virtual VisionWalk on Saturday, June 6th was a celebration of the Fighting Blindness community! Participants from across the nation joined together virtually to show their united, unwavering commitment to the Foundation's mission. Throughout the day, VisionWalk supporters tuned into the virtual opening ceremony and then celebrated VisionWalk by walking around neighborhoods, running on treadmills, dancing in driveways and more.

Thanks to everyone's support, this spring, the National Virtual VisionWalk raised a total of $1.2 million for sight-saving research!

If you didn't join us this spring, you can participate in our **Fall Virtual VisionWalk** on **Saturday, October 24th, 2020**. While we may not be in the same places physically, we can still all join together to show the world what it means to fight blindness and that together, we're VisionWalk Strong!

Register to participate by visiting: **VisionWalk.org**

Left: Five VisionWalkers wearing their favorite VisionWalk t-shirts from past years.
Middle: Young girl wearing a past VisionWalk t-shirt holding her completed VisionWalk bingo board.
Right: Couple walking outside on a trail for the Virtual VisionWalk.

VIRTUAL VISIONS

VISIONS 2020: Our Mission Is Your Vision

The Foundation made the tough decision to pivot on our approach to VISIONS 2020, the national conference of the Foundation Fighting Blindness, that was planned for Minneapolis, MN, to be an all virtual event. The new virtual approach was a great success and we hope everyone that participated gained insight to the most recent advances in retinal research and experienced a sense of community with the Fighting Blindness family. Our mission is your vision!

All Virtual VISIONS 2020 presentations were recorded and are now available to watch at:
FightingBlindness.org/recorded-sessions

Thank You to Our VISIONS 2020 Sponsors:

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VISIONWALK SPOTLIGHT

Steve and Dot's Team Lancelot of Baltimore

Westminster, Maryland residents Steve and Dot Dutterer both have kind and approachable personalities that captivate everyone they meet. With their expansive network of family and friends, and a lot of hard work and dedication over the past 19 years, Steve and Dot have raised more than $743,000 for the Foundation Fighting Blindness.

Steve, who is now 76 years old, was diagnosed with retinitis pigmentosa (RP) when he was just 21 years old. Steve had just finished business school and wanted to join the National Guard. But during his routine eye exam in basic training, the doctors diagnosed him with RP. Due to his diagnosis, Steve chose to take honorable medical discharge, even though at the time Steve felt like his eyesight wasn't a problem. But a few years later, Steve finally went to the Johns Hopkins Wilmer Eye Institute for testing where they told him he was going to go blind.

"They didn't know if I would go blind in 6 months, 2 years, or later in life," says Steve. "It was hard to hear, but soon after, Dot and I still got married and have been married for over 52 years."

Now, Steve describes himself as not having any sight, and Dot found out she has congenital cataracts, which their two sons have as well, so visual impairments are very personal for the entire Dutterer family.

In 2000, Steve and Dot wanted to get involved in the vision loss/blindness community and found the Foundation Fighting Blindness when it was just a Maryland support group. They then started fundraising through a local Foundation regatta, called Sail for Sight.

In 2008, the Foundation had their first Baltimore VisionWalk and since then, Steve and Dot have participated every year.

"The VisionWalk is a fun time to get together with your family and friends, but also to catch up with others affected in the area that we've gotten to know over the years," says Dot. "I love that people can come together for a common cause; the comradery is great."

Steve and Dot Dutterer wearing their 'Team Lancelot' team t-shirt at the Baltimore VisionWalk.

Each year, they participate as 'Team Lancelot,' named after the French Briard sheepdog that was used for research by Dr. Gustavo D. Aguirre, professor of medical genetics and ophthalmology at Penn Vet. Lancelot was the first dog to have his vision restored from a gene-therapy.

With that and much more as their inspiration, Steve and Dot started out their first year of fundraising for VisionWalk by only asking their close friends and family they knew well to contribute. But as time went on, their fundraising has grown into a letter writing campaign, sending out over 1,400 letters to family and friends, with the list continuing to grow the more people they meet.

Steve was the Director of Finance for the city of Westminster and Dot was the Assistant Principal for Westminster Elementary School for many years, so they both had jobs where they networked quite often. Now that they are both retired, they're avid cruisers, meeting people from all over the world. Steve and Dot will ask anyone and everyone that they encounter. They even told their mailman why they were putting so many letters in the mail one year and now he's one of their biggest contributors.

"Never assume someone doesn't want to hear about your passions," says Dot. "Think about who you come in contact with every day, even just when you're at the doctors in the waiting room, you just never know who you'll meet."

As Steve and Dot meet someone new, they hand them a business card, which explains that they are volunteers for the Foundation Fighting Blindness. Dot also keeps a log with the name, address and notes on the people they meet.

"You just don't know when you're going to meet someone that is very generous, supportive, or even affected by a blinding disease," says Steve. "The business card plants the seed and reminds them of us for when we send the letter."

Steve and Dot start working on their letter writing campaign around January each year, which is several months before the June VisionWalk. A similar printed letter is used each year, but Dot always makes sure to write a handwritten, personal note, to every single person. Dot also handwrites all the addresses for another personal touch, which they think makes people more willing to open the mail, especially a new person they've just met.

"It takes quite a while to add the handwritten personal note to each letter, but I know so many people really appreciate it," says Dot. "Adding a personal touch really makes a difference in helping us stand out. Each year I get notes back from people thanking us; it really makes you feel good to know that people are appreciating your effort."

Steve and Dot are full of fundraising "tips and tricks." They have included a challenge in their letter, offering to personally match donations up to a certain amount. They also send a second follow-up letter, as soon as the walk has passed, to those who haven't contributed yet but have in the past years. They have found that this reminder really helps for those who want to give and have simply forgotten.

"Our letter writing campaign is unlike any other fundraiser because of the way we do it," says Steve. "Most other VisionWalk participants that raise a lot have a large team and it's a group effort, but we just have a large network of contributors and support."

After 19 years of fundraising, Steve and Dot both agree they've learned a lot about themselves and the many people they may not have met if it weren't for the letter campaign that they can now call their dear friends.

"Steve has taught me to just be open about sharing our story and the generosity of others always amazes me," says Dot. "We couldn't raise so much every year if it weren't for the wonderful contributors we meet. They're the important ones."

As Steve reflects on his journey with RP and meeting so many new people over the years, his biggest fundraising advice is, "Don't be afraid to share your story. Ask anyone and let them be the ones to say no, don't say no for them."

It's undeniable that Steve and Dot together as a team, are unstoppable fundraisers. But their tremendous passion for the Foundation's mission year after year shows truly how selfless they are.

"I don't do all this for myself," says Steve. "I'm 76 years old, so I do this to help others down the road, so they can see in their lifetime."

The Dutterer's 'Team Lancelot' members wearing their team t-shirts in front of the VisionWalk balloon arch.

Foundation Invests $3 million in Atsena Therapeutics, New Company Developing GUCY2D-LCA1 and MYO7A-USH1B Gene Therapies

The Foundation Fighting Blindness is investing $3 million in Atsena Therapeutics, a newly formed company with three retinal disease gene therapies in development. The first is for Leber congenital amaurosis (LCA) caused by mutations in GUCY2D, which is in a Phase 1/2 clinical trial at the University of Pennsylvania. The second, in preclinical studies, is a dual-vector gene therapy for Usher syndrome type 1B (USH1B), which is caused by mutations in MYO7A. The third program, also in preclinical studies, will be disclosed in the coming months. Atsena Founder and Chief Scientific Officer Shannon Boye, PhD, University of Florida, is the preclinical developer for the company's emerging gene therapies.

Nacuity's Emerging Anti-Oxidative Therapy Moves into Clinical Trial

Fort Worth-based Nacuity Pharmaceuticals is launching a Phase 1/2 clinical trial in Australia for NPI-001, an oral treatment designed to slow vision loss in people with retinitis pigmentosa (RP) and RP associated with Usher syndrome. The trial, known as SLO-RP (Safety and Efficacy of NPI-001 Tablets versus PLacebO for Treatment of Retinitis Pigmentosa associated with Usher Syndrome) will enroll at least 48 patients with Usher syndrome and follow them for two years. If results for SLO-RP are favorable, Nacuity plans to launch clinical trials of NPI-001 for people with RP in the US and Australia in 2021.The Foundation is investing $7.5 million in NPI-001 development through its RD Fund, a venture philanthropy fund for emerging therapies that are approaching, or in, early-stage clinical trials.

AGTC Planning Phase 2/3 Clinical Trial for XLRP Gene Therapy

Applied Genetics Technology Corporation (AGTC), a developer of gene therapies for rare diseases, is planning to launch a Phase 2/3 clinical trial in the first quarter of 2021 for its emerging X-linked retinitis pigmentosa (XLRP) gene therapy for people with mutations in the gene RPGR. AGTC is also expanding its Phase 1/2 XLRP gene therapy trial. In the fourth quarter of 2020, the company plans to dose approximately 20 additional patients in the Phase 1/2 trial to collect more functional data. A mobility test is being added as a supplemental endpoint. In January 2020, AGTC reported a favorable safety profile and evidence of efficacy for the XLRP gene therapy in the Phase 1/2 trial.

MeiraGTx and Janssen Pharmaceuticals Report Promising Interim Results from its Phase 1/2 Clinical Trial for XLRP Gene Therapy

MeiraGTx and Janssen Pharmaceuticals report stable or improved retinal sensitivity for five of seven participants — those in the low and intermediate dose groups — in a Phase 1/2 clinical trial for its X-linked retinitis pigmentosa (XLRP) gene therapy. The therapy is for XLRP caused by mutations in the gene RPGR. The Phase 1/2 trial is underway at five sites in the US and the UK. Males as young as five are being enrolled. Thanks to the encouraging interim results, the company is planning a Phase 3 trial for the treatment.

jCyte Reports Promising Results for Phase 2b Clinical Trial of its Cellular Therapy for RP

jCyte, a biotech company based in Newport Beach, California, announced that its emerging cellular therapy for people with retinitis pigmentosa (RP) and related diseases has performed encouragingly in a Phase 2b clinical trial. The company plans to launch a Phase 3 clinical trial for the treatment in 2021. The emerging therapy is designed to work independent of the mutated gene causing vision loss.

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

Ways to Leave a Lasting Impact

Many people who don't have the resources to make a significant gift during their lifetime choose to leave a meaningful gift in their will. By leaving a gift to the Foundation, you will provide vital support for the research that will soon find an end to vision loss. For more information on how you can leave a legacy of sight, visit **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 **FightingBlindness.org/In-Focus-Newsletter**.

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