

DONOR PROFILE

Meet Sue Sanger. Sue was diagnosed with retinitis pigmentosa (RP) at the age of 29 but has continued to travel, advance in her legal and political career, and more. In the following story, Sue explains, in her own words, all she has accomplished since learning she has RP and why she's a supporter and member of the Foundation Fighting Blindness Legacy Society.



ABOVE: Sue Sanger, Legacy Society Member.

Sue Lives Life to the Fullest and Wants to Help Others Do the Same

By Sue Sanger, Legacy Society Member

In my early 20s, I realized that I couldn't see very well in the dark. I consulted with several ophthalmologists – and all told me not to worry as “everyone has more trouble seeing in the dark.” Finally, when I was 29 years old, another ophthalmologist checked my eyes, and quickly excused himself from the exam room. I heard him talking with another doctor and one said, “I get her for a research subject!” Both doctors then told me I had retinitis pigmentosa (RP) and explained that there was no treatment or cure available. This was scary, but it was also a relief to finally know what was wrong. I decided the only rational response was to go to France for a vacation!

Since then, I have continued to travel all over the world. I went to law school and practiced employment law for 23 years. I also served as an elected member of the St. Louis Park (Minnesota) City Council for 22 years. I have been involved in many other political campaigns and have volunteered with several non-profit community organizations. I play bridge frequently, became a Life Master, and play in tournaments as well. Throughout these

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JOIN OUR MISSION

Help Us Build Our Legacy Society

Have you already included the Foundation Fighting Blindness in your will, trust, by beneficiary designation, charitable gift annuity, or some other aspect of your estate plan? If so and even if you told me in the past, please contact me so I can be sure you are properly included as a member of our Legacy Society. The Legacy Society is an elite group of very special, committed people who have informed us that they have included the Foundation as part of their lasting legacy.

Email me at JCorneille@FightingBlindness.org or call at (800) 683-5555 ext. 4578.

John R. Corneille, J.D.

Director of Legacy Giving

LATEST NEWS

Retinal Research Updates

First Gene Therapy for Dry AMD Moves into Clinical Trial in UK

Gyroscope Therapeutics, a gene-therapy development company in the UK, has launched the first gene therapy clinical trial for the dry form of age-related macular degeneration (AMD). The 10-participant, Phase 1/2 study is being led by Dr. Robert MacLaren, professor of ophthalmology at the University of Oxford, and will be taking place at three locations in the UK.

“There are currently limited treatments for dry AMD, which in many cases will progress to the wet form several years later,” says Dr. MacLaren, who is also the lead investigator for X-linked retinitis pigmentosa and choroideremia gene therapy clinical trials. “We now know a lot about the role of the complement system in AMD and also how to deliver gene therapies safely to the fovea in patients with good visual acuity. Hence it is logical now to explore early preventative interventions that might have an impact in stopping progression of this disease .”

The emerging Gyroscope treatment was developed to slow the progress of dry AMD, thereby preventing or slowing debilitating central vision loss. Therapy developers believe a single subretinal injection of the gene therapy may work for several years or the lifetime of the patient.

ProQR Receives Authorization to Begin Clinical Trial for Autosomal Dominant Retinitis Pigmentosa Treatment

ProQR, a Netherlands-based developer of RNA therapies, has received authorization from the US Food and Drug Administration (FDA) to launch a clinical trial in the US for QR-1123, its treatment for people with autosomal dominant retinitis pigmentosa (adRP) caused by the p23h mutation in the gene RHO. The company plans to begin enrolling patients for the Phase 1/2 trial in 2019 at sites in North America. The treatment will be delivered through intravitreal injections. More information on the trial will be posted to www.clinicaltrials.gov.

QR-1123 becomes ProQR's third clinical program for an inherited retinal disease (IRD). The company's other clinical programs are QR-110 for LCA 10 caused by the p.Cys998X mutation in the CEP290 gene and QR-421a for Usher syndrome type 2A caused by mutations in exon 13 of the USH2A gene. The Foundation Fighting Blindness is investing up to \$7.5 million in the development of QR-421a through its RD Fund, a venture philanthropy fund for emerging therapies in early or planned clinical trials.

Allergan and Editas Begin Recruiting for CRISPR/Cas9 Clinical Trial for LCA10

Allergan, a global pharmaceutical company, and Editas Medicine, a developer of gene-editing therapies, have begun patient recruitment for a Phase 1/2 clinical trial for a CRISPR/Cas9 treatment for people with Leber congenital amaurosis 10 (LCA10). The treatment targets a specific mutation (c.2991+1655A>G in Intron 26) of the gene CEP290.

Known as the Brilliance clinical trial, the study is the first for a CRISPR/Cas9 treatment for an inherited retinal disease. It is also the first clinical trial for a CRISPR/Cas9 therapy administered inside the human body.

Brilliance is a dose escalation trial that will enroll adult and pediatric (3 – 17 years of age) patients in the US. Massachusetts Eye and Ear (Boston) is currently enrolling patients.

“The launch of the Brilliance clinical trial is an exciting milestone for people with inherited retinal diseases. CRISPR/Cas9 is potentially powerful technology for correcting a mutated gene so that it expresses a functional protein and provides better vision to the patient,” says Benjamin Yerxa, PhD, chief executive officer at the Foundation Fighting Blindness.



ABOVE: Doctor shining a light into a woman's eye during an exam.

Known as EDIT-101, the CRISPR/Cas9 gene-editing technology developed by Allergan and Editas is designed to locate and remove the mutation in LCA10. The treatment works like a pair of molecular scissors to cut out the mutation. The treatment is delivered to photoreceptors by a subretinal injection.

Save the Date for VISIONS 2020

The National Conference of the Foundation Fighting Blindness!

June 18-20, 2020
Hyatt Regency Minneapolis
Minneapolis, Minnesota

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experiences, I have rarely let my RP prevent me from doing whatever I want – though sometimes I have to do things in a different way, such as switching from biking solo to riding on the back of a tandem bicycle. I have learned to ask for support. My partner Henry and my many friends provide transportation (I can't drive), an arm to hold on to, and advance warnings of stairs, curbs and other tripping hazards. Most importantly, they provide love and encouragement to continue to lead an active and independent life – and they don't laugh too hard when I bump into things or knock over that wine glass I didn't see. And as my vision has declined and has become more obvious to the general public, I've been amazed that strangers have been very helpful and accommodating. In short, RP is a nuisance and a challenge, but not an insurmountable obstacle.

I support the Foundation Fighting Blindness because it raises critical funds for basic research and clinical trials to find treatments and cures that can benefit all of us with inherited retinal diseases. By making annual contributions, as well as including a bequest to the Foundation in my will, I believe that I am helping to provide hope for all of us with visual challenges. It is both a privilege and a responsibility to share my funds so that others may see. 

Need More Information?

We are here to help, and we welcome your calls and emails if you have questions.



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The information in this document does not constitute legal or financial advice. You should discuss all of your estate planning questions with your own advisors before taking any actions.