

SOFIA
SEES **HOPE**

LOOKING
FORWARD,
LOOKING
BACK

2014-2021
and beyond

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Sofia Sees Hope is a 501(c)3 patient advocacy organization dedicated to generating awareness, raising funds for research, and providing support, education and outreach to the LCA and rare inherited retinal disease community.

From the Founder

Looking back over the last eight years since we incorporated, I am struck by the incredible generosity of so many people who don't have any direct connection to our rare disease. They are strangers, businesses, friends, coworkers and neighbors, and they have built the foundation of our little organization with their financial support, their time, and their spirit. If you are reading this letter, then you are probably one of these people, and I thank you for joining us on this journey.



Looking back, I am also stuck by how much we've learned and changed at Sofia Sees Hope. We started out just fundraising for research, and quickly learned that wasn't enough to advance the science. We needed to support the community of people living with Leber congenital amaurosis and other rare retinal diseases because we can't just sit back and wait for treatments to appear. We need to inform the science by getting genetically tested, by participating in registries and natural history studies, and by supporting one another so we have a voice in advancing treatments for our disease.

Looking back, I am overwhelmed with gratitude for our small and mighty team at Sofia Sees Hope that has met our community's needs. They have provided outreach programs, shared stories, hosted conferences, and created opportunities for those living with or caring for someone who is losing their sight to connect with one another, with industry, and with research.

Looking back on these last eight years, there was a glimmer of hope of a treatment for just one of the nearly 30 genetic variants that cause blindness. Today there are more than 40 treatments in trial, and one approved treatment to reverse blindness is available to patients. Wow. Really, wow. With treatment for fewer than 15 percent of all rare diseases, what are the chances that one of those treatments would happen in our retinal disease community?

In just a few years, what was a ripple of hope has become a wave of reality, and Sofia Sees Hope has been there. Our donors, volunteers, staff, founders, sponsors, all of you — you've not just kept us afloat, you've made it possible for us to ride this incredible, exciting wave of scientific advancement that is transforming the lives of those living with vision loss.

Looking forward, I can't say for sure just how quickly more treatments will be available, or when our next in-person conference may be, or if my own daughter's fading vision might some day be restored, at least in part. What I do know is that as we generate awareness, our community is growing. More families are getting a diagnosis, and more parents and individuals are reaching out for a connection to others, to research, to hope.

Looking forward, our organization will continue to support our community by funding research, by funding genetic testing, and by finding new ways to connect and support families and individuals living with retinal disease. When a new mom and dad receive the news that their newborn baby is blind, or when a teenager realizes that she will never be able to drive a car, we'll be here to let them know that they are not alone, and that there is so much hope for treatment in the coming years.

Looking forward, I also know a few REALLY EXCITING things that I can't share just yet...but I can't wait to tell you soon!

With sincere gratitude and optimism for what lies ahead,

A handwritten signature in blue ink, appearing to read 'Laura Manfre', written over a light blue circular graphic element.

Laura Manfre
President & Co-Founder

A Small Group Helps Create An Influential and Global Nonprofit Ready for the Future

By Rosanne Smyle

From hatching ideas at the kitchen table to testifying before the FDA for a breakthrough gene therapy, Sofia Sees Hope has come a long way since its humble beginnings in 2014.

Laura Manfre and her husband, Chuck Priebe, and their friend, Elisse Rosen, started the nonprofit after doctors confirmed a diagnosis of Leber congenital amaurosis in Sofia Priebe, the daughter of Laura and Chuck. That diagnosis took seven years to obtain, a struggle in pursuing a genetic diagnosis no family should have to endure.

For Sofia's father, founding Sofia Sees Hope was all about trying to bring about change.

"When we first received the diagnosis, the doctor stated there was nothing that we could do, and that when Sofia was an adult, she could move to a city with public transportation to get around," Chuck said.

"Sofia Sees Hope is our way to connect others that have a shared or similar experience, to raise awareness about LCA and inherited retinal diseases, and to help fund research for treatments."

Among its contributions to outreach, support, advocacy and research, Sofia Sees Hope has given more than \$450,000 to support research into treatments and provide families free access to genetic testing.

A unique blend of personalities with talents in leadership, strategy, medicine, creativity and business acuity, and their countless volunteer hours and

enormous emotional and financial investment, has brought Sofia Sees Hope to where it is today: A leading global advocacy group for people and families living with LCA and other rare inherited retinal diseases and a key supporter of cutting-edge research that is populating the pipeline to treatments.

HASHING OUT A MISSION

Elisse Rosen met Laura Manfre in a Pilates class.

The two became friends and Laura asked Elisse if she'd like to help her start a nonprofit. From Laura's kitchen table in Ledyard, Connecticut, to Elisse's tiny loft in nearby Groton, and lots of coffee-shop visits along the way, Sofia Sees Hope came to fruition.

"When we first started talking, Sofia had been diagnosed, and Laura, as a parent, was very distraught and felt things and vented. Nobody was talking about rare diseases," Rosen recalled.

Laura knows how to pick people to help get things done. For more than 30 years, Elisse worked at pharmaceutical-giant Pfizer as head of global regulatory compliance before retiring. Now, she is certified in leadership development as an organizational coach.

Laura, Chuck and Elisse hashed out a mission and a vision, and Elisse wrote strategies to achieve that vision. Rare disease required a different strategy than that of big companies looking for blockbuster drugs.

A stickler for details, she wrote policy and bylaws and networked



to expand the organization's reach throughout the community and beyond with donations and sponsorships.

"The fact that Sofia Sees can tap into these families and tell them what options are out there for them is wonderful. Some people do their own research, some people don't know where to begin. What they offer is something unique."

"We really took off," she said. "We became an organization. The advocacy part is what I think carries Sofia Sees Hope."

"The one thing I think that provides the drive and engages people is the fact that it's about children and families. Here's an organization that will at least help you find the avenue to go down; it's not the be-all and end-all; it's the catalyst for getting started."

"So, what we see today with Sofia Sees is a lot different than when we sat down at the table. They've found their place and they did it without changing their mission. They are what they said they were going to do, but they've refined it."

FAMILY DOCTOR TURNED BOARD MEMBER

Dr. George Adrian, an optometrist in Groton, Connecticut, and part of the Founders' Circle, played an integral role in the Sofia Sees Hope story. He met Sofia after her LCA genetic diagnosis, one of the more

than 25 forms of LCA known as IQCB1/NPHP5. Sofia is now 18.

"She was referred by the Board of Education and (CT) Services for the Blind to determine if there were any ophthalmic devices or treatments that could restore her vision in any way," Dr. Adrian said.

"There were no treatments or cures for LCA, very little knowledge of the genetic details, and little hope, as this is a congenital and progressive blinding eye disease.

"I had the heartbreaking news to deliver that there was little that could be done for Sofia's vision other than basic Low Vision aids, such as Tele-Microscopes that could only help her use what remaining vision she had.

"I will never forget the look of determination and fortitude on Laura and Charles' faces, the look that must be energized by the parental love of a child, and that for me was the beginning of Sofia Sees Hope.

"Now, after several years and countless hours of volunteer work, board meetings and numerous fund-raisers that have raised literally hundreds of thousands of dollars for the research and advocacy of inheritable retinal diseases, Laura and Charles have made a difference."



Sofia, Age 2

Dr. Jeanette Jezick, an optometrist in Gales Ferry, Connecticut, met Laura through a mutual friend.

"I was immediately interested in Sofia's story and admired Laura's drive and tenacity to find a cure for her daughter. When I learned what she was doing, I wanted to be involved," Jezick said.

"The first Dinner in the Dark event will be a memory I will have forever. Everyone should experience what it is like to not have the precious gift of sight. The short time of eating blindfolded, trying to find your fork, glass, food, as well as simply having a conversation with someone that you cannot see, was challenging and opened my heart to the struggle. My husband, Peter, and I have been involved since near the beginning and love being a part of this special 'family.'"

CONNECTING THE COMMUNITY

As Sofia Sees Hope has evolved, its focus on connecting those within the LCA and IRD communities has sharpened. The organization launched a quarterly newsletter to share patient and family stories, provide research updates, and maintain a calendar of relevant events.

A program called Family Connections was created in which SSH literally brings together people whose diagnosis is similar to provide each other support. One of the first to join the program was Tami Morehouse, 55, of Ohio.



Tami Morehouse

Tami made research history in 2009, when at age 44 during trials for gene therapy medication for LCA2 (RPE65), doctors injected under her retinas a human-engineered virus that gave instructions on how to produce an essential protein for vision. Spark Therapeutics developed the drug that was marketed as LUXTURN[®] following Federal Drug Administration approval in December 2017.

Since successfully completing the trial, Tami often talks to families and patients who are considering LUXTURN[®] through the Family Connections program,

including a young boy whose mother was considering having him undergo the surgery:

"I talked to him about things like, the fact that my surgery wasn't painful for me but did feel a little funny afterward; that I did have to have lots of eye drops; that my surgery didn't even take very long; that the doctors and nurses were really nice; that my family could be with me after the surgery when I needed them; and that I was pretty comfortable through the whole thing," Tami recalled. "I told him that the best part is that I can see a little better than I could before I had the surgery.

"At the beginning of the conversation, the little guy seemed pretty quiet and uncomfortable. As the conversation went on, he appeared more relaxed and seemed to be listening pretty intently."

Even better than setting the boy's mind at ease was the relationship Tami — who also speaks at SSH LCA Family Conferences and serves as an organization Ambassador — formed with his mother, Sarah St. Pierre Schroeder of Florida. Sarah was considering LUXTURNA® treatment for her then 9-year-old son, Creed Pettit. Creed became the youngest patient to undergo the retinal surgery for LCA2 (RPE65) in 2018.

"I will never forget the emotions I felt when Tami reached out to me. Every sentence in her email brought me more comfort about what I was doing for Creed," Sarah said. "She was so open about her journey, I felt like we had known each other forever. I felt like she was with us" when Creed had his surgery.

"Suddenly I was able to ask someone all the questions I had and get answers. Not just 'Maybe this will happen.'"

SPREADING THE WORD

In 2020, Sofia Sees Hope expanded a program it had been running since 2018, called Ambassadors. Made up of people with direct connections to LCA, either their own or a family member's diagnosis, Ambassadors are a network of passionate supporters who enhance our mission in their communities across the globe.

"Our Ambassadors are caretakers, patients, clinicians and friends from across the United States, in Canada, Mexico and The Netherlands," said Ashley Luppold, Ambassador Program director. "Each team member brings their own unique experience and talents to the team. This program simply allows us to tap into all that energy, and keep everyone informed and aligned around new research, resources and initiatives," she said.

Among the Ambassadors are Ashlyn Lincoln of Nashville, Tenn., who well remembers what life was like in the immediate wake of her 6-year-old son Gunner's LCA diagnosis: "I was overwhelmed, exhausted, emotional, determined and lost. All at once."

At the first event she ever attended for families living with vision loss, she received love, support and a helping hand. They helped entertain Ace while she tended to an overwhelmed and overstimulated Gunner. They passed on books and games and toys and gave her information for more resources.

Ashlyn has taken her experience of creating and sending care packages to her husband while he was deployed with the Marines to another level by fashioning personalized care packages for families of young children and newborns living with LCA or IRDs.

"Since becoming an Ambassador, I have prepared a number of boxes families will receive," Ashlyn said. "After seeing how many visually impaired-friendly books, toys and games that Gunner has outgrown, I felt it was time to pass these things on to other families, just as they were passed down to me."

She includes books, games, tactile paper for fine motor skills, along with photos of Gunner as he has grown, and an encouraging note: "Let me be that support for you today," she writes. "Please, take it all! I am passing it on with love, just as it was passed onto me."



The image depicted contains models and is being used for illustrative purposes only.
Reference: 1. Blindness and vision impairment. World Health Organization. Published October 9, 2020. Accessed November 30, 2020. <https://www.who.int/news-room/fact-sheets/blindness-and-vision-impairment>
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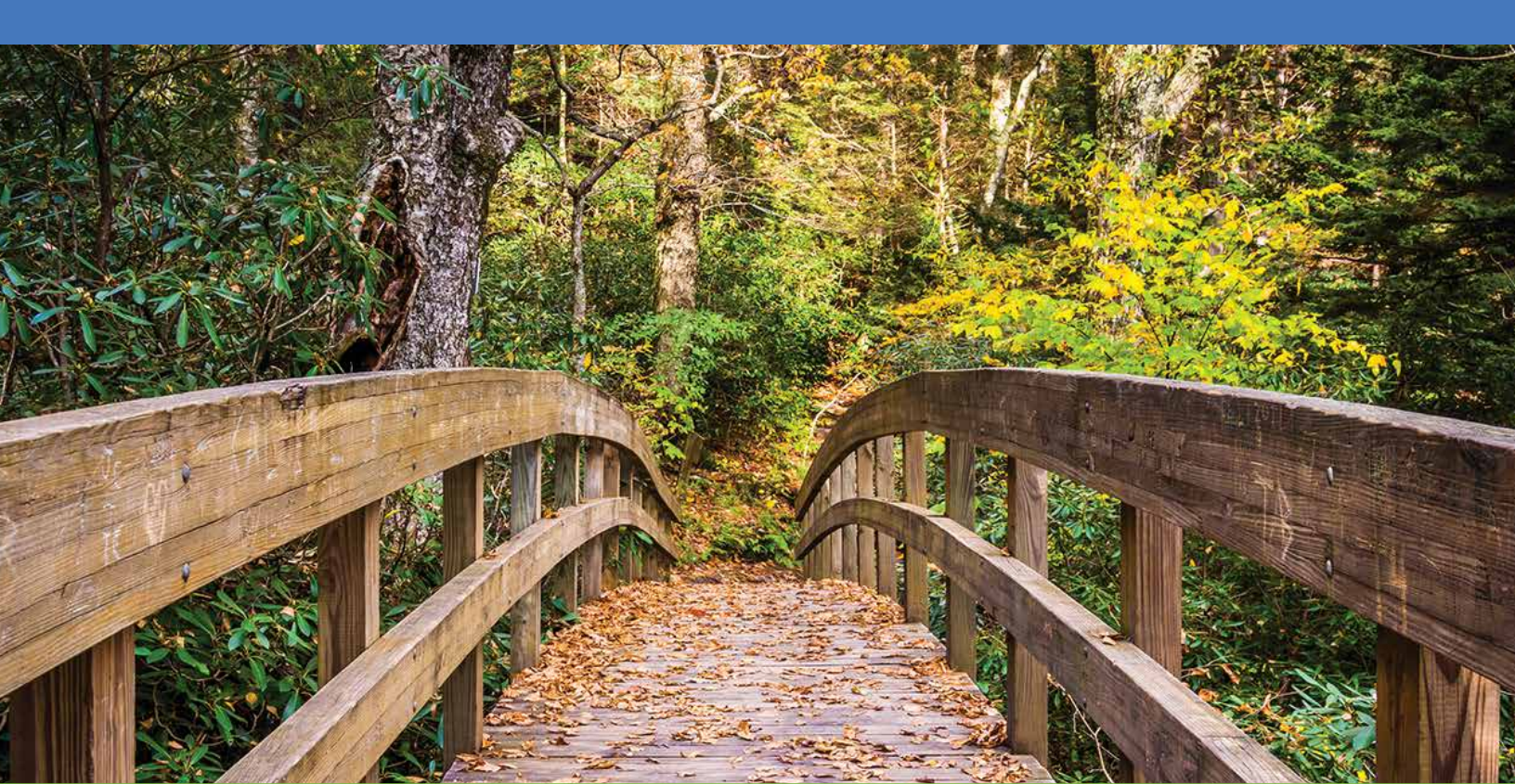
Committed to preserving vision and hope.

Our commitment to retinal diseases comes from a legacy of pioneering science and patient care. Working together with the brightest minds in ophthalmology, we're innovating breakthrough therapies designed to transform the lives of millions worldwide.¹

See our commitment at janssen.com/retinal-disease

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Making Connections in the LCA Community Provides a Path Forward

By Rosanne Smyle

Danielle Senick of Norwich, Connecticut, reached out to Sofia Sees Hope more than three years ago when she needed answers about her deteriorating vision.

Doctors diagnosed Danielle at age 6 months with Leber congenital amaurosis (LCA), and her parents learned that this rare inherited retinal disease (IRD) would cause retinal degeneration to the point of having little or no vision.

She could see light and shadows, but she noticed a pronounced deterioration in her vision in her early 20s. We asked Danielle to talk about her experiences since she connected with us. Here is her story.

“Though the (vision) changes were subtle, they caused me to realize how little I knew about

my condition and I wanted to learn more. I did a little research and stumbled across Sofia Sees Hope. I was intrigued to hear about Sofia’s story, and I wanted to learn more, so I contacted the organization. I heard back, almost immediately, from Danielle Chiaraluce, who worked for the organization at the time.

“We had a lengthy conversation and she told me that she and Laura (Manfre, Co-Founder and Board Chair of Sofia Sees Hope) were interested to learn more about me, and they invited myself and a guest to Dinner in the Dark. I was honored to be invited, and it was a very fun, yet emotional, experience. It was wonderful to have my aunt learn a little bit about what my life is like to have no vision.



Danielle with her mom, Patty at 2018 LCA Family Conference in Mystic, CT

“At this event I met Laura, Sofia, David Brint and his wife, Betsy, (their son has LCA), and several other influential individuals. I was so honored during the auction to see how many people were willing to contribute their hard-earned money to the cause (including my aunt who bid on a trip to Bali).

UNDERGOING GENETIC TESTING

"Later I spoke with David and he helped me to get in touch with Spark Therapeutics, where I underwent free genetic testing. I had not seen a specialist or done anything about trying to learn about my diagnosis in several years because I often stumbled across roadblocks and it seems that the doctors that I previously had seen were not as educated about LCA. However, this experience was much different.

"I gave a blood sample and sent it to the lab and once my results came in, I spoke with Dr. Jill Harris in great detail about my results. She made me aware that my gene mutation is LCA-CRB1 (LCA8). Unlike LCA-RPE65 (LCA2), (for which there is a federally approved treatment), information about this mutation is still in the works. There is no cure, so I knew all along not to expect a cure and that with genetic testing you must be patient. I joined My Retina Tracker® (a Foundation Fighting Blindness free, secure online registry that helps connect families and enables researchers and doctors to track progress, prevalence and other variables of IRDs to move research forward) and receive emails about any new information on the condition and still remain hopeful.

"About a year later, through social media, I became connected with Kristen Steele of Iowa. (Kristen has LCA10 caused by a mutation in her CEP290 gene.) She is a remarkable young woman who is extremely confident, well-spoken, and independent, and like me, a very passionate licensed massage therapist.

"When I spoke with her, I was in the process of attaining my license and still in school. I was having a little trouble getting the results from my license test in braille and she told me that was unacceptable and filled me in about her journey and how hard she had to fight to

pave the way to get her testing accommodations. When I set up my accommodations with the testing center all I had to do was mention her name and request my accommodations and the center provided me with everything I needed.

"For that I am very thankful. I am now a licensed massage therapist myself and although we are both busy and haven't talked in a while, Kristen is always available if I ever have any questions about my journey. I was fortunate enough to meet Kristen by attending the LCA Family Conference in 2019 (in Philadelphia). I also attended the family conference with my aunt and mother in 2018 in Mystic (Connecticut) and was quite intrigued by all the speakers.

LCA COMMUNITY WELCOMES DANIELLE

"At this conference I also met two individuals that are about my age who both have children with LCA. It was wonderful to meet with them and answer some of the questions that they had about what it was like growing up with LCA and any suggestions they had about being a parent of a child with LCA. As I have received so much guidance and help along the way, it's wonderful to give back by helping others and giving them advice.

"I have stayed in contact through social media and text messaging with these two individuals, Deanna Carroll and Ashlyn Lincoln, and have potential plans to go down to North Carolina and meet their children! I was able to meet up with them again in 2019 for the Philadelphia family conference. I brought my brother with me as a guest and he was also intrigued to learn so much about my condition.

"There was a plethora of informative speakers from all over the country, and it was amazing to see how far research had come even since 2018 at the



Danielle with her aunt, Nancy, (L), and mom, Patty, at 2018 LCA Family Conference in Mystic, CT

last conference. I was especially intrigued to hear Tami's story (Tami Morehouse, from Ohio, underwent RPE65 gene therapy as part of the LUXTURN® treatment clinical trials) and how much it changed her life.

"I became quite emotional when she described all the little things that had come from it and changed her life, such as being able to go to her children's soccer games and cheer them on, actually being able to see them score goals, where before she had to rely on others to describe what was going on.

"The main takeaway from this process has been to continue to remain hopeful and stay involved. It is quite a process and the research is quite extensive and costly, so we would not have been able to come as far as we have without the generosity of others involved with Sofia Sees Hope.

"It's amazing to see what a little bit of poking around on the Internet has led to and how much it has changed my life. My curiosity and doing a little bit of research to learn about LCA led to my becoming involved with Sofia Sees Hope, receiving genetic testing, attending wonderful events and conferences, and meeting truly amazing individuals, both sight-impaired and sighted."

Editor's note: Danielle Senick passed away at the age of 32 on Dec. 24, 2020. Danielle was a warm, vibrant and engaged young woman who was looking forward to forging her own path. We will miss her.

MEET OUR RARE DISEASE *Community*

By Rosanne Smyle

Since its founding, Sofia Sees Hope has created a network of families within the LCA community and has shared their stories on our website and in our quarterly newsletters. This work speaks directly to our vision and mission: To transform the lives of those affected by blindness caused by rare inherited retinal diseases and provide outreach and support to those affected by Leber congenital amaurosis and other rare retinal diseases.

We have met some incredible people along the way, and we are honored that they shared their stories with us, so we can share them with the world. Through them, we hope to broaden the understanding of rare disease.

Please visit our website, sofiasees.org/resources/blog/ to read the full stories.

JORDYNN ERVIN, 4, NEW YORK

A force to be reckoned with when it comes to music and movement, blink and you just might miss Jordynn as she rockets past you. The 4-year-old received her genetic diagnosis of LCA2 (RPE65) when she was 1½, just a few months before the U.S. Food and Drug Administration in 2017 approved LUXTURN[®] to treat her form of LCA.



Her mom, Joy Goodwine, grandmother, Gwen Goodwine, and aunt Jackie traveled to Philadelphia to SSH's 2019 LCA Family Conference, where they talked with LUXTURN[®] researchers and developers. They also connected with other families.

"Learning about the treatment and getting the education about all of it really gave me something to think about as my daughter's journey continues as she lives with this visual impairment," Joy said. "Knowing that my daughter can thrive and live a happy life with some occasional bumps in the road was a wonderful feeling."

Jordynn was on track to be part of an RPE65 study early last year, when she tragically lost her doctor and then COVID struck. The family is excited about meeting their new doctor.

Jordynn sees her Grandma Gwen almost every day as they live near each other and she takes care of her while mom cares for the elderly and those with dementia. Gwen set up rugs like oversized dominoes throughout her house, blue-and-white rugs, from the family room, to the living room, to the kitchen, to the bedroom.

"That's how she learned how to navigate," Gwen said. "She runs through here like she's got 20/20 vision."

She attends school two days a week and her therapies have resumed at school, mom said.

"They love her and she loves school."

ANGÉLICA BRETÓN MORÁN, 24, MEXICO

Angélica Bretón Morán is a concert pianist and opera singer from Mexico. The 24-year-old was diagnosed at age 2 with LCA. At age 20, she received a confirmed genetic diagnosis of LCA6 characterized by a mutation in the RPGRIP1 gene.



When Angélica realized no Facebook group existed to support LCA6 patients and families, she created one.

"It feels good to be able to talk to people who have the same genetic mutation. I am very happy when concerned parents write in the group and the other members respond with encouraging messages," she said. "That is something that my parents didn't have so I thank God that the parents of these children can do it."

Angélica graduated in the summer of 2020 with a degree in music from Autonomous University of Nuevo Leon. At the university's Faculty of Music, she helps young people with visual impairment in the Musical Research Area for the Blind and Visually Impaired, a project she began when she was 10.

She attended Sofia Sees Hope's LCA Family Conferences and she is a Sofia Sees Hope Ambassador. In Mexico, with little or no support from government sources for people living with LCA, she founded "Amaurosis Congénita de Leber A.C.", or its Spanish acronym, ACL, a civil association supported by donations to help ACL patients.

"You have to be aware of the limitations," Angélica said of any challenge, including living with LCA. "But you also have to find a way around the obstacles, or pass through them, or use them as a catapult, or see them as a feature that makes us unique and special as people, as we all are."

HELENA DAMAS, 15, CONNECTICUT

Helena is a young artist experiencing gradual vision loss.

Her art brings her comfort and calm, but her vision over the years has become more blurry and difficult to see colors. She was born with optic nerve hypoplasia that caused an unidentified retinal condition.



"It's scary because when your vision's decreasing, all you can think about is how one day you might go completely blind," Helena said when we met her several years ago. "I'm already like halfway blind; what's going to happen next?"

Helena, now 15 and in the art program at high school, draws and paints whatever comes to her, along with lots of Disney characters, including princesses and Aladdin. Her acrylics and oils and drawings hang all over her house, along with murals on her bedroom walls.

Her vision has been fairly stable over the past three years, and she receives regular exams locally and at Boston Children's Hospital.

Helena's mom, Shanda Easley, a former member of the SSH Board of Directors, has received few answers in her quest for a clinical genetic diagnosis for her daughter's deteriorating vision.

"You do have a helpless feeling because you can't control it, you can't change it, you can't fix it," she said in the past. But, she recently said she understands she can't force science.

Helena said she tries not to think about not being able to draw again because she doesn't know what is going to happen and something might be able to change.

"So, I try to just think about what's going to go on tomorrow or what's going on right now."

GUNNER LINCOLN, 6, TENNESSEE

Gunner's parents, Ashlyn and Alex Lincoln, learned when their little boy was 6 months old in February 2015 he had LCA10, caused by a mutation in his CEP290 gene. Doctors determined Gunner came into this world with no usable vision cells and no light perception. His older brother, Ace, is sighted.

"Both Ace and Gunner teach us many life lessons, regardless of who is sighted and who is blind," said their mom, Ashlyn. In 2020, Ashlyn joined our Ambassadors program, and creates personalized care packages for families living with LCA. She includes visually impaired-friendly books, games, toys, tactile paper for fine motor skills and photos of Gunner as he has grown.



Gunner loves prekindergarten, especially gym time and swimming lessons. He listens, to try to understand musical instruments, and he loves making art and writing on the Braille.

The boys have a typical sibling relationship, blaming each other, kicking each other, playing their own world, Ashlyn said. "Ace pretty much treats him like he is sighted. He adjusts (when he remembers Gunner cannot see) and goes right back to thinking it. It's the best of both worlds.

"Their positive outlook and attitudes on life really help us be better ourselves. I just feel so lucky to be able to always have different outlooks on everything that others may not realize."

ENZO MEENS, 6, THE NETHERLANDS

Enzo's parents, Laura Steinbusch and Merlijn Meens of The Netherlands, wondered how to raise their son as normally as possible and help him discover the world. Doctors diagnosed Enzo with LCA10 (CEP290) when he was 10 months old.

"The solution turned out to be simple," said Laura, who also serves as an SSH Ambassador.

"We will not despair and will come up with creative solutions so he can participate in everything that this world has to offer."

Building on her son's love of music, singing, moving and reading books, Laura just finished producing a multilingual children's songbook with songs in Dutch, English, French and German. She got the idea because Enzo, now 5, easily learned new words in French or English through songs, especially when he already knew the song in Dutch.

The songbook's characters, Lux the lizard and Louise the mouse, help children learn music with braille.

It also exposes blind or visually impaired children to other languages from an early age and allows them to independently explore the world. The music scores for the five songs in the book are in print and in braille, so children can learn to read braille music as soon as they have learned to read braille text.





JULIET SOADY, 5, CALIFORNIA

Scott Soady needed more answers after he and his wife Heather's younger daughter, Juliet, was diagnosed with LCA at 6 months old. He attended a Foundation Fighting Blindness conference where he met Sofia Sees Hope co-founder Laura Manfre.

"That was truly amazing. You feel so alone, the condition is so rare, there's not going to be someone you just run into," he said.

He was touched that for the first time, people asked how *he* was doing. Meeting parents dealing with LCA and seeing young people with LCA function as normal teen-agers brought Scott comfort and hope that everything's going to be OK.

SSH connected Scott and Heather to free genetic testing programs for Juliet's genetic testing. The test revealed she has what is called a *de novo* mutation of the CRX gene. The rare condition, LCA7 (CRX), is a result of a gene mutation, rather than inheriting the gene from her parents.

Today Scott is a Sofia Sees Hope Ambassador, attending meetings and conferences to articulate our mission of generating awareness, raising funds for research and providing support, education and outreach to the LCA and rare inherited retinal disease community.



JACK MCCORMICK, 24, CANADA

Acceptance leads to respect and opens pathways to social accessibility and, in turn, helps fuel research, Jack McCormick explained to an international audience at a Foundation Fighting Blindness conference. Doctors diagnosed Jack in high school with LCA2 (RPE65).

"I accepted it in myself and that is the most important thing you can do," he said. "To accept it in yourself and be so confident about it that you walk around with your blindness as a badge of triumph rather than a badge of sorrow or something you're trying to hide. And in doing that, people are going to be very committed to you, they're going to respect you and respect is a very powerful thing."

Jack is a Sofia Sees Hope Ambassador and writes the column "Navigating Adulthood" (formerly called "College Connection") for *Seeing Hope*, our quarterly newsletter.



SILVIA, MOTHER OF VICKY, 7, UNITED KINGDOM

Silvia worked to find people around the globe — the U.K., Italy, Germany, South Africa, China, Australia, Canada and the U.S. — to partner with families and to raise money to fund research to correct the mutated RDH12 gene that caused LCA13 in her daughter, Vicky.

"Maybe the message is cliché, but it's really about hope and doing everything we can, said Silvia. "I don't want to give up. I feel a sense of urgency because I feel it might come a little too late.

"I want to say 50 years from now, I've done everything I can. In the worst-case scenario, it's going to help future kids, and still it is going to show Vicky the importance of fighting for your hopes and beliefs.

"In the best-case scenario, imagine a cure is coming in time and we can help kids like Vicky see for longer! If I had even a little role in making this happen, imagine how fulfilling that would feel."



CLAUDIA ZAGHI-BIGGS, WIFE OF BRANDON BIGGS, 29, CALIFORNIA

Brandon has LCA8 (CRB1). Sofia Sees Hope profiled him, his parents, Atom and Sonja Biggs, as well as his wife, Claudia.

"I consider living with a blind partner a precious exercise of mindfulness," Claudia said. "If I'm alone, I walk in the street careful enough to be safe, but I am immersed in my thoughts, listening to music with my isolating headphones. When I'm with Brandon, I acknowledge everything around me: people, buildings, colors, behaviors, my own emotions, and I feel that I am present in the moment."



MONROE LE, 8, CALIFORNIA

Monroe sees her most favorite place in the world, Disneyland, and her most favorite attraction — Pirates of the Caribbean with Capt. Jack Sparrow — in a whole new light since her treatment for LCA2 (RPE65).

“There’s been numerous times in a trip where she asks me ‘Mama is that new, or has it always been there and I’m just seeing it for the first time?’ ” her mother Heather said.

She and her husband, Bruce, see a much more confident little girl who no longer needs to hold hands in dim places.

“I can’t believe it’s the same girl who was so timid and afraid to let go of my hand,” she said.



MISTY LOVELACE, 21, KENTUCKY

Doctors told Misty at age 12 that she’d be blind by 18 because of her LCA caused by a mutation in the RPE65 gene. She left school in the middle of seventh grade to undergo retinal surgery as a clinical trial patient during studies for what would become LUXTRNA® in 2017. She returned at the beginning of eighth grade with greatly improved vision.

“When I came back, everybody thought I was so different. They didn’t know what happened. It was a complete turnaround. Everyone wanted to be my friend.”

She is now 20 and trains horses.

“Now I love looking up in the sky and seeing stars. I love thinking that at one point in time, they were imaginary for me.”

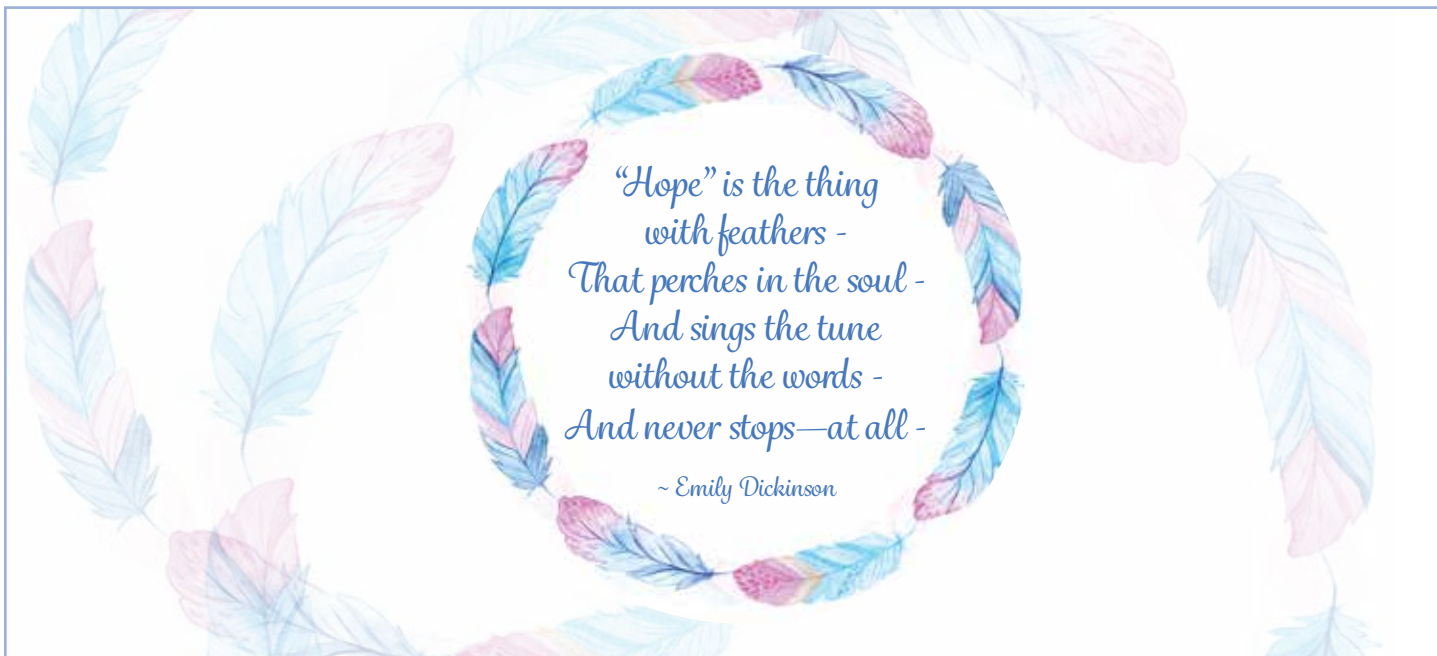


MIKAYLA LARSON, 32, TEXAS

“I feel there are a lot of parents out there that are terrified for their kids to live this life,” said Mikayla, who has LCA6 (RPGRIP1) and is the mother of four children. “And, while it’s not ideal, it doesn’t mean that they aren’t capable of living a fulfilled life and love it like they should.

She has some light perception and equates her field of vision to about the length of a drinking straw. With lenses, her visual acuity is 20/200 and 20/400. “We are still smart and capable. We have different challenges but that doesn’t mean we aren’t able,” she said.

“It does no good to have pity, or on the other side, to put people on a pedestal for doing things everyone can do. It’s demeaning and degrading as a human to get praise for doing something everyone else can do, just because we can’t see well.”



**At Spark Therapeutics, we are
committed to challenging the
inevitability of genetic disease
by discovering, developing and
delivering treatments in ways
unimaginable – until now.**



LEARN MORE:

Visit www.sparktx.com, or contact
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We're helping the communities we
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There are many things we can do to help hold our communities together. And at Dominion Energy, that means we do more than write checks. So while we're very proud of contributing nearly \$48 million in 2019 to community causes throughout our footprint and beyond, we're even prouder of Dominion Energy's

employees for volunteering more than 130,000 hours of their time. From refurbishing homeless shelters to replenishing local food banks to cleaning up parks to helping soldiers and their families, we're donating the most precious resource of all: our energy. Learn more by visiting [DominionEnergy.com/Foundation](https://www.dominionenergy.com/foundation).



Actions Speak Louder

FROM A RIPPLE TO A WAVE: 150 YEARS OF THE LCA STORY

148 years passed from the time Theodor Karl Gustav von Leber first gave name to a severe form of early-infancy vision loss to the time the first treatment was approved in the U.S. for one genetic form of the LCA. The good news is that since that first treatment was approved in 2017, advances have been made by leaps and bounds.

Each year, more advances are made, more clinical trials are launched, and more treatments come closer to approval. The promise of helping those with LCA to see leaves on trees and stars in the night sky remains just around the corner for so many of us.

We were founded on hope. It is this tantalizing promise, these advancements, these patient stories that have always kept our hope afloat. As the science moves faster, so do we, ramping up our fundraising and programs so we can not only support the research but support its acceleration, and ensure that our LCA community is able and ready when the time comes to support the science.

This year, more than 30 retinal disease therapies are now in the clinical trial pipeline! THIRTY.
And it's only four years since LUXTRNA® was approved for RPE65 treatment.

OUR HOPE...IS BECOMING REALITY.



1869

Theodor Karl Gustav von Leber describes a severe form of early-infancy congenital vision loss as “amaurotic,” related to amaurosis, meaning dimming, darkening, dark or obscure.

1959

Dr. Carl-Henry Alström confirms LCA is genetic in nature; he is credited with recognizing syndromic forms of LCA and other early-onset retinopathies.

1971

Foundation Fighting Blindness is established.

1990

The Human Genome Project is launched in the United States. Three gene therapy products are granted FDA applications to proceed with clinical development.

1996

GUCY2D (LCA1) is identified as the first gene associated with LCA.

1997

RPE65 gene mutation that causes LCA2 is identified.

2001

RPE65 gene therapy preclinical large animal studies are conducted in Briard dogs.

2006

Eleven genes associated with LCA are identified.

2007

RPE65 gene therapy clinical trials begin.

2013

Spark Therapeutics is launched and licenses SPK-RPE65 from Children's Hospital of Philadelphia.

2014

SPK-RPE65 receives orphan drug status in the US and European Union, and breakthrough-therapy designation from the FDA.

SOFIA SEES HOPE

Sofia Sees Hope is co-founded by Laura Manfre, Chuck Priebe and Elisse Rosen, as a 501(c)3 NPO to support research into treatments, genetic diagnosis, and to provide outreach and support to those living with blindness from LCA and other rare IRDs.

SOFIA SEES HOPE

Sofia Sees Hope holds its first Dinner in the Dark fundraiser, an event that becomes an annual favorite in southeastern Connecticut.

2015

Spark Therapeutics reports positive Phase III RPE65 clinical trial results.

2016

SOFIA SEES HOPE

SSH launches Family Connections program designed to help end the isolation that many feel when they receive a rare inherited retinal disease diagnosis.

SOFIA SEES HOPE

SSH expands its advocacy reach with the launch of its Ambassador Program, comprised of supporters who enhance our mission in their communities across the globe.



Indicates timeline for the development of the first and currently the only approved gene therapy available for LCA today, for patients with inherited retinal disease due to mutations in both copies of the RPE65 gene.

SOFIA SEES HOPE Indicates items that are specific to Sofia Sees Hope.

2017

SOFIA SEES HOPE

SSH publishes the first edition of Seeing Hope in print and online, delivering news, patient stories, and research updates to our LCA community and donors.

MeiraGTX receives compassionate use approval from EMA for AIPL1 (LCA4) gene therapy.

25 genes associated with LCA are identified.

More than 100 gene therapy products have been granted FDA applications to proceed with clinical development.

Representing the patient community, Laura Manfre testifies before the FDA Advisory Committee in October, supporting the recommendation for approval of the gene therapy to treat LCA2 (RPE65) (to be marketed as LUXTURN[®]).



In December, the FDA approves LUXTURN[®], the first gene therapy for any inherited genetic disease in the U.S. and the first genetic therapy targeting a retinal disease worldwide.

2018

SOFIA SEES HOPE

Vision improvements reported in Phase 1/2 clinical trial for ProQR's RNA therapy for CEP290 (LCA10).

More than 200 gene therapy products have been granted FDA applications to proceed with clinical development.

Sofia Sees Hope in July sponsors its first LCA Family Conference in Groton, Connecticut.

Iveric bio partners with University of Massachusetts Medical School to develop CEP290 (LCA10) gene therapy.

2019

SOFIA SEES HOPE

PTC Therapeutics announces licensing agreement with Odylia Therapeutics for RPGRIP1 (LCA6) gene therapy.

Phase 2/3 clinical trials begin for Sepofarsen (QR-110), a disease-modifying therapy for LCA10 due to a mutation in the CEP290 gene.

More than 27 genes associated with LCA are identified.

Sofia Sees Hope in July sponsors its second LCA Family Conference in Philadelphia.

The first clinical trial begins at Editas Medicine for a CRISPR-based genome editing drug candidate under development, called EDIT-101, for the treatment of LCA10 (CEP290).



2020

● Horama signs licensing agreement with Leiden University Medical Center for CRB1 (LCA8) gene therapy.

● Atsena Therapeutics founded, launches Phase 1/2 clinical trial for GUCY2D (LCA1) gene therapy).

● Allergan and Editas Medicine announce dosing of first patient in landmark phase 1/2 Clinical Trial of CRISPR medicine AGN-151587 (EDIT-101) for the treatment of LCA10.

2021

● More than 30 retinal disease therapies are now in the clinical trial pipeline.

SOFIA SEES HOPE

SSH launches new programs to advance education and treatments: the free Let's Chat About monthly web series brings experts to the LCA community for informational conversations. Scientific Advancement Workshops (in partnership with the Foundation Fighting Blindness) bring together researchers and stakeholders in the hopes of advancing work.

Looking ahead...

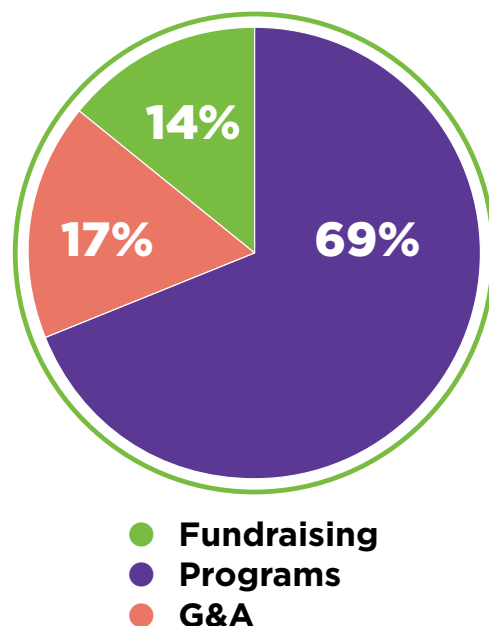
With your help, we know the future holds continued advances in research, faster progress towards treatment for retinal blindness, and more opportunities for advocacy for the rare disease community. We couldn't have come this far without you, and we can't wait to share the next milestones together.

Financially Speaking...

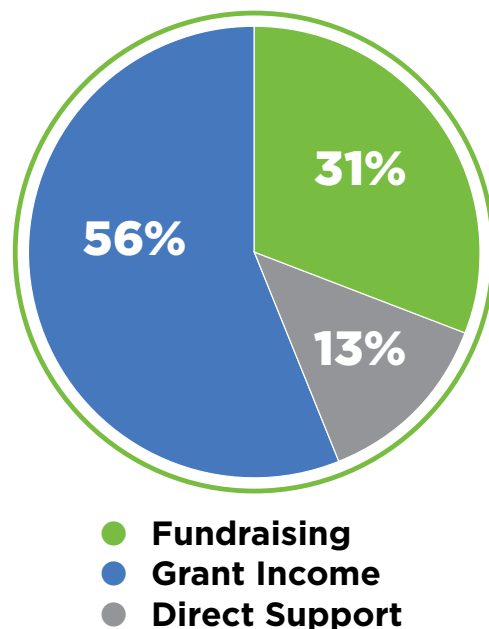
We take great care to use every dollar that we raise to advance our mission. 2020 was not a typical year for Sofia Sees Hope as we, like many other organizations, had to cancel our primary fundraiser Dinner in the Dark. Thanks in large part to continued grant funding from our corporate sponsors we were able to expand our outreach, education and community support programs. As a result of sensible planning and the continued support from our donors throughout the pandemic, we utilized a portion of our savings to make a generous year-end contribution to research and genetic testing access for our community. Even with uncertainty ahead, the Board of Directors felt secure in voting unanimously in December of 2020 to make this contribution and respond to the urgency and need to fund retinal disease research.

2020

HOW WE SPENT THE MONEY

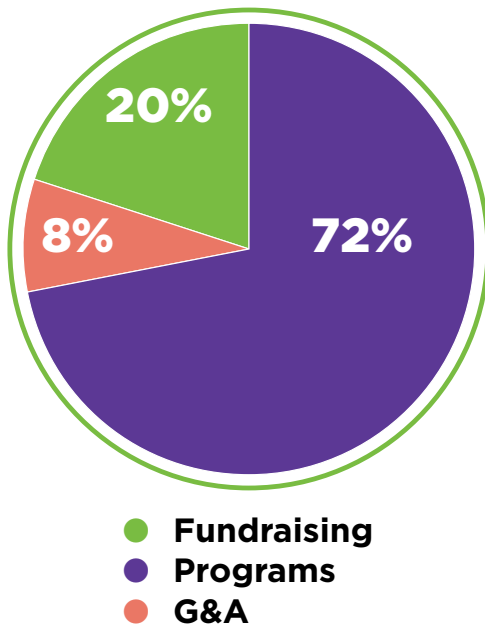


WHERE THE MONEY CAME FROM



2019

HOW WE SPENT THE MONEY



LAST YEAR WAS ANYTHING BUT NORMAL...

So we went back to 2019 to see what a more “typical” year looks like for Sofia Sees Hope, and to understand how COVID impacted our financials. Overall, income was down 40% in 2020 when compared to 2019, in large part due to the cancelation of multiple in-person events, represented under Fundraising, and as Program income (income related to our LCA Family Conference). Program income does not appear in 2020 as we canceled our in-person LCA conference activities for both 2020 and 2021.

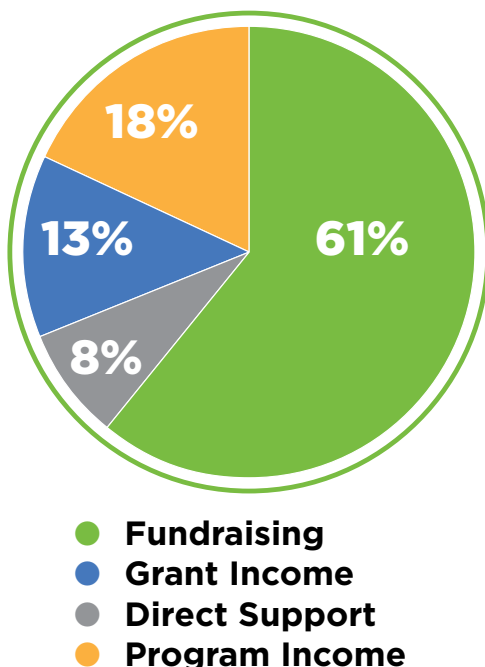
LOOKING AHEAD...

We are playing it safe in 2021, hoping to host some small in-person events later this year. As research and clinical work advances, we continue to see an increased need for our outreach and support programs, and are making a concerted effort to double down on organizational capacity and infrastructure. This year, we expect our G&A expenses to increase as a result of these efforts. We are at a turning point where we are just large enough to keep up with the needs of our community, but know that we must make some changes in order to grow our organization and support an ever-expanding patient community.

In 2020, thanks to grant support and fundraising efforts, we are already underway with three major initiatives to better position ourselves for the future:

- 1 A NEW DATABASE MIGRATION** that will allow us to better track, report, and build relationships with our donors and with the patient community that we support.
- 2 EXPANDING OUR TEAM** so we can better manage our current outreach activities and continue to add new programs to support our ever-growing and increasingly global patient community.
- 3 POSITION OURSELVES** for where we will be five and 10 years from now, with a major initiative that...well, that's still under wraps but, we look forward to sharing with you before the end of the year!

WHERE THE MONEY CAME FROM





BIDDING ~~DINNER~~ IN THE DARK

**Benefiting treatments for blindness
and the rare retinal disease community**

In keeping with the theme of our annual
Dinner in the Dark (returning 2022),
we invite you to this fun, playful experience
that will raise awareness and needed funds
for research for treatments.

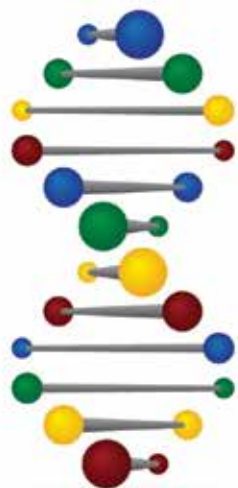
COMING OCTOBER 2021.
Stay tuned for more information.

Sponsorship opportunities are available at sofiaseeshope.org.
For more info, please email info@sofiaseeshope.org or call 860-556-3119.



Developing RNA therapies to
**CHANGE THE LIVES OF
PEOPLE WITH INHERITED
RETINAL DISEASES**

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agtc

Visionary science for
life changing cures.

Sofia Sees Hope Has Significant Impact on Inherited Retinal Disease Research

By Rosanne Smyle

Since its inception in 2014, Sofia Sees Hope has donated \$250,000 to support early-stage proof of concept research programs. To date, Sofia Sees Hope has provided financial support for research related to the genes CEP290, RPGRIP1 and IQCB1/NPHP5.

Our funding has also provided financial support for various treatment approaches. These include injecting a modified virus containing the corrective gene into the retina; using molecular biology to create a genomic medicine that precisely edits, by locating and removing, the targeted mutation; and using an antisense oligonucleotide (AON) product as a disease-modifying therapy that works like genetic tape to block the mutation.

These efforts have been led by renowned researchers, including Dr. Rob Collin, Radboud University Medical Center in Nijmegen, The Netherlands; Dr. Wolfgang Baehr, The University of Utah; and Dr. Eric Pierce, Mass Eye & Ear, among others.

Sofia Sees Hope's advisors and partners include Dr. Jean Bennett from the University of Philadelphia, senior geneticist Emily Place from Mass Eye and Ear, and partner organizations including Foundation Fighting Blindness, Global Genes, and National Organization for Rare Disorders.

Some of the researchers we have worked with spoke about the impact Sofia Sees Hope has had on the rare retinal disease community.

Jean Bennett, MD, PhD, Professor of Ophthalmology at the Center for Advanced Retinal and Ocular Therapeutics and the F.M. Kirby Center for Molecular Ophthalmology at the Perelman School of Medicine, University of Pennsylvania:



"Sofia Sees Hope is just magnificently instrumental in developing funding for LCA research. By bringing together families, scientists, representatives from pharmaceutical companies and regulatory experts, Sofia Sees Hope cross-fertilizes the research space, thereby identifying areas that require further monetary support and energy. It is through education and shared experiences that plans are made to combat blinding disease, and this foundation does an amazing job bringing all of the appropriate 'players' together.

"It's raising awareness about the disease, which is the first step in bringing the patient and their advocates together. I can't tell you how many times I have heard patients and their family members describe the challenge of identifying what is wrong with their loved ones vision, only to then be told 'there is nothing we can do.'

"There is now so much that we can do. By raising awareness of the disease and the fact that a new age has begun in which it is possible to develop treatments for many of these diseases, we share the urgency for moving forward quickly. It is like the saying 'Out of sight, out of mind.' Here, we want to place the idea of treating blinding disease directly in people's minds so that we don't lose focus.

Bennett and her colleagues at Children's Hospital of Philadelphia were involved in the first human clinical trials for what would become LUXTURN[®], Spark Therapeutics' gene therapy approved by the FDA in 2017 for use on patients with LCA2 (RPE65 genetic mutation). It is the first and only approved gene therapy for inherited disease in the United States and Europe. The drug is an engineered virus that delivers the human RPE65 gene by subretinal injections.



Benjamin Yerxa, PhD, Chief Executive Officer of Foundation Fighting Blindness and CEO of FFB's Retinal Degeneration Fund:

"We are very fortunate to have organizations like Sofia Sees Hope because their hard-earned dollars are channeled to us to provide free genetic testing. And the more people we can get tested, the more people can have an idea about what's in store for them."

**Ben Shaberman,
Director of
Scientific
Outreach and
Community
Engagement
at Foundation
Fighting
Blindness:**



"Genetic testing is critical to diagnosing patients and it's critical to getting these families identified and included in the clinical trials. We need clinical trials if we're going to get treatments across the finish line and out to the general community.

"There are more than two dozen genes that, when mutated, can cause LCA. It's critical that each patient be genetically screened so that their disease-causing mutation can be identified. Knowing the gene mutations confirms the diagnosis and puts the patient on the path toward clinical trials for emerging therapies. Furthermore, therapy developers need patients for clinical trials to get FDA approval for the treatments and out to all who can benefit from the treatment.

"The Foundation is very fortunate to have Sofia Sees Hope as a key partner for financially supporting resources such as the My Retina Tracker® registry, no-cost genetic testing for patients, and sight-saving research. These resources are costly, but essential to the Foundation's sight-saving mission."

**Michael Schwartz,
MS, MBA, ProQR
Therapeutics'
Vice President
and Global
Project Leader
Sepofarsen
(QR-110), a
disease-modifying
therapy for LCA due
to a mutation in the
CEP290 gene:**



"Sofia Sees Hope gives us that opportunity to meet with patients so that we can find real medicines, develop real clinical trials, so that we really have the best chance to be successful in the market with these types of drugs.

"Sponsors conduct clinical trials all of the time, but not always with success. Trials often fail because the drug doesn't work, but also because the sponsor did not take the time to understand the patient and what is important for the patient. This leads to bad trial designs, wrong endpoints and a delay in finding real medicines. I believe it is important to understand the patient.

"Sofia Sees Hope gives us the opportunity to see through the lives of a patient and, therefore, allows us to create better trial designs that better fit with the patient need. This ultimately will lead to higher success rates in clinical trials, which ultimately will lead to more medicines available to patients. Many diseases, such as inherited retinal diseases, have no treatments available. Sofia See Hopes increases the chances in doing this!"

Research & Genetic Testing Contributions

2014-2020: \$455,200

Sofia Sees Hope supports innovative early-stage proof of concept research programs and IRD genetic testing through fundraising events and individual donations. We partner with the Foundation Fighting Blindness (FFB) to designate our research contributions, and to ensure free genetic testing and counseling continues to be available to individuals through FFB's My Retina Tracker® program.

The My Retina Tracker® program is an open access, no-cost genetic testing program for individuals living in the USA with a clinical diagnosis of an inherited retinal disease (IRD). For the most current information on research advances, please visit our partner's website at www.fightingblindness.org.

YEAR	CONTRIBUTION DETAIL AS OF DECEMBER 2020	
2020 (\$80,200)	\$20,000	W. Clay Smith (University of Florida) — <i>Enhancing Metabolism in Photoreceptors with a Modified Arrestin to Treat Retinal Degeneration</i>
	\$20,000	Rob Collin, PhD (Radboud University, The Netherlands) — <i>Development and optimization of AON-based therapies for selected splice defects</i>
	\$20,000	Sandro Banfi, MD (Fondazione Telethon, Italy) — <i>AAV-Sponge-mediated modulation of microRNA-181a/b: a potential therapeutic approach for Inherited Retinal Disease</i>
	\$20,200	My Retina Tracker® program
2019 (\$100,000)	\$15,000	My Retina Tracker® program
	\$15,000	Sandro Banfi, MD (Fondazione Telethon, Italy) — <i>AAV-Sponge-Mediated Modulation MicroRNA-181a/b: A Potential Therapeutic Approach for Inherited Retinal Disease</i>
	\$20,000	Clay Smith, PhD (University of Florida) — <i>Enhancing Metabolism in Photoreceptors with a Modified Arrestin to Treat Retinal Degeneration</i>
	\$25,000	Krishanu Saha, PhD (University of Wisconsin-Madison) — <i>Gene Editing Nanomedicines to Correct Pathogenic Mutations in the Retina</i>
	\$25,000	Rob Collin, PhD (Radboud University, The Netherlands) — <i>Development and Optimization of AON-based Therapies for Selected Splice Defects</i>

CONTRIBUTION DETAIL AS OF DECEMBER 2020		
YEAR		
2018 (\$100,000)	\$25,000	My Retina Tracker® program
	\$20,000	Rob Collin, PhD (Radboud University, The Netherlands) — <i>Development and Optimization of AON-based Therapies for Selected Splice Defects</i>
	\$15,000	Eric Pierce, MD, PhD (Massachusetts Eye and Ear Institute) — <i>Efficacy, Safety, and Toxicity of AAV-Mediated Human RPGRIP1</i>
	\$40,000	Krishanu Saha, PhD (University of Wisconsin) — <i>Gene Editing Nanomedicines to Correct Pathogenic Mutations in the Retina</i>
2017 (\$65,000)	\$65,000	My Retina Tracker® program — <i>support in its pilot year</i>
2016 (\$65,000)	\$25,000	Anand Swaroop, PhD (National Eye Institute) and Wolfgang Baehr, PhD (John A. Moran Eye Center, University of Utah) — <i>seeking therapies for LCA caused by mutations in NPHP5 (IQCB1), CEP290 (LCA10), and additional genes, and seeking to understand the underlying causes of LCA by observing similarities and differences between gene defects. (First funded in 2015.)</i>
	\$25,000	Rob Collin, Assistant Professor, Radboud University Nijmegen Medical Center (The Netherlands) — <i>for antisense oligonucleotide therapy for a type of gene splicing for the CEP290 (NPHP6) gene variation. The study has led to the development of Sepofarsen by ProQR. (First funded in 2015.)</i>
	\$15,000	My Retina Tracker® program
2015 (\$45,000)	\$15,000	Anand Swaroop, PhD (National Eye Institute) and Wolfgang Baehr, PhD (John A. Moran Eye Center, University of Utah) — <i>seeking therapies for LCA caused by mutations in NPHP5 (IQCB1), CEP290 (LCA10), and additional genes, and seeking to understand the underlying causes of LCA by observing similarities and differences between gene defects.</i>
	\$30,000	Rob Collin, Assistant Professor, Radboud Univeristy Nijmegen Medical Center (The Netherlands) — <i>for antisense oligonucleotide therapy for a type of gene splicing for the CEP290 (LCA10) gene variation. The study has led to the development of Sepofarsen by ProQR.</i>

Thank you for your support in 2020!

INDIVIDUALS

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ENGINEERING HOPE

Discover how we're using gene therapies to help build
a better future for patients with serious diseases.

Please visit our website to see our ongoing
Inherited Retinal Disease clinical programs:

MeiraGTx.com



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What's next?

WE CAN'T WAIT TO TELL YOU!

Stay tuned as we continue
our journey towards treatments
for rare retinal disease
and support for the
LCA and rare IRD community.



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