HOPE in FOCUS
Seeing a cure for blindness
Supporting the LCA and rare retinal disease community
SPECIAL EDITION
SEEING HOPE | Newsletter
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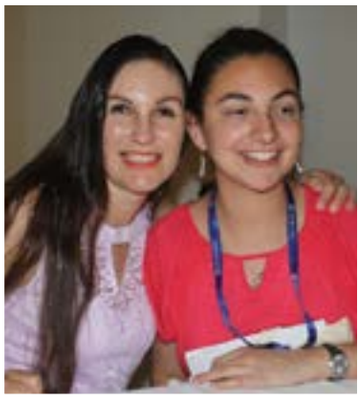


HOPE IN ACTION:
A Conversation with Laura Manfre

How did Hope in Focus begin?

In 2013, after our daughter, Sofia, was diagnosed with Leber congenital amaurosis (LCA) *IQCB1/NPHP5*, the Foundation for Retinal Research (FRR) asked if my husband and I would be willing to raise funds for treatments for her genetic mutation. The FRR was started in 1998 by David and Betsy Brint, whose son had LCA. Little was known then about the disease, and there wasn't an LCA community when they began. The Brints faithfully stewarded the research and a handful of LCA families to grow that organization into a real community.

So, we said yes to the FRR's request and quickly raised about fifty thousand dollars, largely from our local community in southeastern Connecticut. This success blew us away, but there wasn't an active research project for Sofia's gene. Realizing that so many people were willing to help us, we thought, why not raise funds for all LCA conditions so we can put the funds we raise into use sooner? A broader funding goal would allow us to designate monies for the most promising research and advance projects already in the pipeline. All boats float in a high tide, right? It was the right thing to do. Honestly, I also thought pushing the projects that are further along faster might be the most effective way to help advance the research related to Sofia's gene.

In 2014, we incorporated our 501(c)(3). Setting up the nonprofit made it easier to steward and thank donors. In Connecticut, three people are needed to create a nonprofit. So, my husband, Chuck, myself, and our good friend, Elisse Rosen, founded Sofia Sees Hope, later called Hope in Focus when we changed our name in 2021.

I think it was in early 2017 when David Brint became chair of the board of directors for the Foundation Fighting Blindness (FFB) that the FRR was rolled into the FFB. This change was significant for LCA research because of the FFB's incredible expertise in research and influence. At the same time, because the FFB serves all forms of blindness, this change opened a gap for focused support for our LCA community. As a single, rare disease-focused organization, Hope in Focus was able to fit right in and fill that gap.

How did your mission/vision evolve?

LCA is a rare disease, which means we are a small community,

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Hope in Action: A Conversation with Laura Manfre

Continued from page 1

but we have shared experiences that create a strong sense of unity. As I interacted increasingly with industry and researchers, I learned just how vital it was to have an empowered community equipped to engage with research and biotech to support the advancement of treatments. At the time, it was also apparent that we needed to focus on genetic testing. Genetic diagnosis is still an issue for our community, although we've come a long way, and I'm pleased that our work at Hope in Focus has made a real impact in this arena with our fundraising, outreach, and awareness campaigns. When individuals in our community have a confirmed genetic diagnosis and understand how treatments are developed and brought to market, they will also know why we must speak up, be involved in fundraising, and consider participating in patient registries, natural studies, and, hopefully, clinical trials.

When I think about how we've evolved, although our mission has stayed the same and is still very relevant, the biggest "a-ha" for me has been realizing that it's not enough to raise money for research. Understanding the importance of advocacy and our voice in developing our treatments is critical. As a community, we can't wait for these treatments to fall like manna from heaven. That might be how it works for a lucky few in the future, but that is not how this process works. We must actively participate in registries, natural history studies, patient advisory boards, and legislation. And the community with a voice and a level of representation will garner interest in its rare disease. The more aware and connected we are with the entire ecosystem involved in developing treatments, the greater the opportunity to influence these things.

What have you learned about the research community?

I was surprised by how meaningful it was to meet the people behind the science. As I spoke at events and met the community working on treatments, I stopped thinking about them as a monolith. These are dedicated, caring individuals working daily to benefit people they don't know. When they have a chance to hear from folks living with LCA, these interactions help inform their work and motivate them to keep at it. We need them to keep working hard to find those treatments, and I think hearing our stories helps.

As I experienced these interactions, I realized that to achieve our mission, we needed to facilitate engagement across the entire LCA ecosystem, including everyone impacted by the disease, biotech, regulatory agencies, investors, other nonprofits, and advocacy groups. It takes a lot of people to bring a treatment to market, and we all have a part to play.

Another thing I learned was that an informed LCA community needs to make sure that positive policy decisions are made regarding research and access to treatments. Unfortunately, many legislators don't understand rare conditions, patient protections, and the actual cost of living with a life-long disease. We must educate our legislators and ensure they make sound, sensible policies.

What helps you see the progress made by Hope in Focus?

I was very fortunate to testify at the FDA AdComm (Advisory Committee) meeting in 2017 for the approval of LUXTURNA®. That opportunity was very emotional and a significant milestone not just for those living with LCA but for all rare diseases and gene therapy. For me, it also underscored the importance of our organization.

Two years later, at the LCA Family Conference we hosted in 2019 in Philadelphia, a woman from Washington State shared the story of her son. Before he turned three years old, he was genetically diagnosed with *RPE65* and treated with LUXTURNA, still the only genetic treatment for LCA available on the market. Wow! The room went silent. We were talking about getting to this point, but I was surprised it was already happening to someone in the room.

Meanwhile, there were families in that room who were still unable to get a genetic diagnosis. Hearing about her son's early diagnosis and treatment was incredible and gave us all hope. I want to hear more of these stories for all genetic variants of LCA.

Another exciting development is the growth of our international community. At our LCA Family Conference in 2023, we had folks attend from all over the world, including a family living in China who told us about their database of 130 families with the *CRB1* genetic mutation. Everyone was shocked that such a large group was identified for a single gene. LCA is a rare disease, making it hard to find and connect people, and here were 130 families with the same gene. Again, wow!

2023 LCA Family Conference in Indianapolis

What do you hope for the future?

When we started Hope in Focus, it didn't seem like anything was happening with research for LCA from my perspective as a parent. But under the surface, research was moving forward, and many dedicated folks in the LCA ecosystem were hard at work. Ten years later, we have a treatment on the market, LUXTURNA®, with four potential treatments currently in the clinic, several preparing for trials, and multiple gene-agnostic therapies in development. There is so much hope as the science is advancing. On the other hand, it has been SEVEN YEARS since I testified before the FDA, and LUXTURNA was approved for just one of our LCA genes, and there are still no new treatments on the market for other LCA variants. We need to keep the pressure on.

What do I hope for the future? This year, Hope in Focus is engaging in strategic planning to ensure that, as an organization, we are prepared and able to meet the changing needs of our LCA families and individuals as science, regulation, and industry evolve over the next 10+ years.

When I started ten years ago, LCA research was a ripple, and now it feels more like a big wave. Hope in Focus needs more help to row the boat as that wave grows, including program funding, volunteer support, and dedicated staff. As we plan for the next ten years, we must define what this looks like and what it will take to get there.

I also hope that Hope in Focus will participate in more Advisory Committee meetings for drug approvals. I'm disappointed I only got to testify at one of these events! How cool would it be if we could shift from a focus on supporting the advancement of treatments to market to helping members of our community as they evaluate whether or not they want to choose a treatment and then supporting them through that process? (Make sure to read Jack's story.)

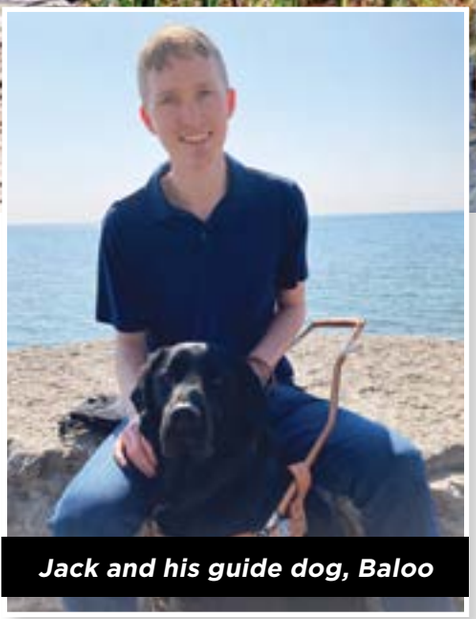
After creating Hope in Focus, have you asked yourself, what have I done?

Yes, I ask myself that question all of the time. At the very beginning, I was one person doing almost everything. Managing a startup nonprofit is exhausting. Today, we have a small but mighty team that includes one full-time employee, but I'm still volunteering at almost the same level as in those early years. Why? Because Hope in Focus is like tapioca; it just keeps growing. I'm very grateful for all of the volunteers and our incredible staff. We've connected with and helped so many people from whom I've learned so much. While growing Hope in Focus has been very demanding, it also fills my cup, and I feel so lucky to be a part of it.

What other thoughts would you like to share?

Hope in Focus has given me a sense of control over something I had no control over and felt utterly overwhelming. I think our work does the same for other parents and individuals living with this rare disease. I see how we are helping folks today, and I know that we're helping to advance treatments that will help someone in the future. Hope drives action, and as we keep pushing forward, I'm excited to see what the next ten years bring for our community and Hope in Focus.

A TREATMENT JOURNEY:
Jumping Hurdles, Taking Risks



Jack and his guide dog, Baloo

"If someone told me that having access to perfect vision tomorrow meant not having impaired vision for my entire life, I would not change the past. While my visual impairment has created many challenges, it has shaped who I am and influenced me in many positive ways. I've become more resourceful, compassionate, creative, and resilient." **—Jack McCormick**

By Katherine L. Kraines, MS

Jack McCormick lives a story of hope, tenacity, and measured success while embracing an unknown visual future. When Jack was two, a diagnosis of cone-rod dystrophy confirmed that he had impaired vision. As a teenager, genetic testing revealed that he had Leber congenital amaurosis type 2 (LCA2), due to a mutation in the *RPE65* gene and that he was losing his vision. This form of LCA interferes with the production of the *RPE65* protein affecting the eyes' ability to process Vitamin A, leading to progressive vision loss. "It was tough to accept. I was only 15 and thought my life would be a certain way. Now, it was going to be different," Jack recalled.

Fast-forward to 2022, when Jack, now 25, was treated with LUXTURNA®, a gene therapy for *RPE65*, becoming the first Canadian to receive a gene therapy. But his journey to treatment involved overcoming substantial hurdles with the Canadian healthcare system, even as his sight declined.

To have a chance for treatment, Jack realized he had to advocate for LUXTURNA to be available in Canada. "I wrote letters to Parliament and was interviewed on national television to create awareness and to help expedite the process," he explained. "My vision was getting worse every time I visited the eye doctor, and my window as a viable patient for this treatment was quickly narrowing."

Even after clearing the bureaucratic obstacles and identifying a hospital and qualified surgeons, there was still no clear path for funding the expensive treatment. "There was a year lag between Health Canada approving LUXTURNA and my lining up private funding," he said.

While Jack wanted the treatment, the immediate or long-term outcomes were not guaranteed. His doctors set his expectations sufficiently low, saying he might see a little better in dim lighting. "My goal was to have stable vision for longer," he said.

Jack finally received the treatment in March 2022. But the surgery was not easy. It took a long time to recover, and the effects were not immediate. His eye with the poorest vision was treated first, and the second eye was treated two weeks later.

"It is important to be realistic about the surgery. They're injecting an air bubble and a drug into your

eye. There are incisions with microscopic stitches that rub against the eye, causing an an intense headache," Jack said. "I was on my back for the first 24 hours. Later, I had to prevent putting pressure on the eye and couldn't bend over."

Another huge challenge was having to quickly decide after the first surgery whether to pursue the treatment for the right eye. "With my left eye, it took a week to see what I saw before the surgery," he explained. "A few days later, my doctors began talking about the next surgery, and I still wasn't seeing better. It was very stressful and resulted in multiple conversations with my ophthalmologists about whether to proceed."

After weighing the risks, Jack chose to have his right eye treated. "At that point, my goal was to see as well as I did before the surgery and, ideally, a little better," he said. "I'm very happy I did it because the treatment made a much bigger difference for my right eye." His doctors were also pleased when testing showed vision improvement in dim lighting and an increase in color detection.

"Before the surgery, I didn't see much walking around the city at night. Now, I might notice someone passing me. I can also see the lines of a crosswalk," he said. "For me, these improvements are huge!" It's unknown how long these changes will last, but it's a future Jack is willing to live with.

His advice for people seeking treatment is to be persistent. "My obstacles were fighting the government and finding a way to pay for the treatment privately," Jack said. "It's also vital to find a community. It's one thing to be a visually impaired person saying that I need this treatment. But it is much more impactful when a group says the same thing and gets others to care about it. Allying with organizations like Hope in Focus helps unite our voices while providing much-needed support and education."

Jack McCormick graduated in 2018 from Canada's Wilfrid Laurier University in Waterloo, Ontario. He was diagnosed in high school with LCA2 *RPE65*. Jack is a Hope in Focus ambassador, helping people living with LCA and IRDs. You can read his blog at **jackdamccormick.wordpress.com**

Jack and his family

From left to right; Alex, Jack and Jacob standing in the center of a hanging bridge during their trip to Costa Rica.

Jack (L) with guide Charlie, runner John, and guide Christian at a half marathon in Ottawa

HOPE

10 YEARS

You know us as an organizatio
advocacy and science, keepin
step of the way. We are brid
between the people that infor
that has the power to

For ten years, Hope in Foc
keeping our LCA communit
treatments at the cer

RESEARCH in FOCUS

We raised more than a half-million dollars designated for LCA therapies.

We support access to free genetic testing through My Retina Tracker®.

We co-hosted gene-specific Scientific Advancement Workshops to drive LCA research.

"Hope in Focus is just magnificently instrumental in developing funding for LCA research. By bringing together families, scientists, representatives from pharmaceutical companies, and regulatory experts, Hope in Focus cross-fertilizes the research space, thereby identifying areas that require further monetary support and energy."

—Jean Bennett, MD, PhD, Co-Founder of Opus Genetics and the F.M. Kirby Emeritus Professor of Ophthalmology at the Perelman School of Medicine, University of Pennsylvania

ADVOCAC

We support BioBonds, a proposed Congressional Act to help researchers launch clinical trials for emerging treatments.

We partner with Foundation Fighting Blindness, National Organization for Rare Diseases, Global Genes, and other IRD and LCA organizations.

"Let me be that support for you today, please, take it all! I am passing it on with love, just as it was passed on to me."

—Ashlyn Lincoln, Hope in Focus Ambassador, mother of Gunner diagnosed with LCA10 *CEP290*

in FOCUS

IN ACTION

**n that adeptly moves between
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m the science, and the science
transform the people.**

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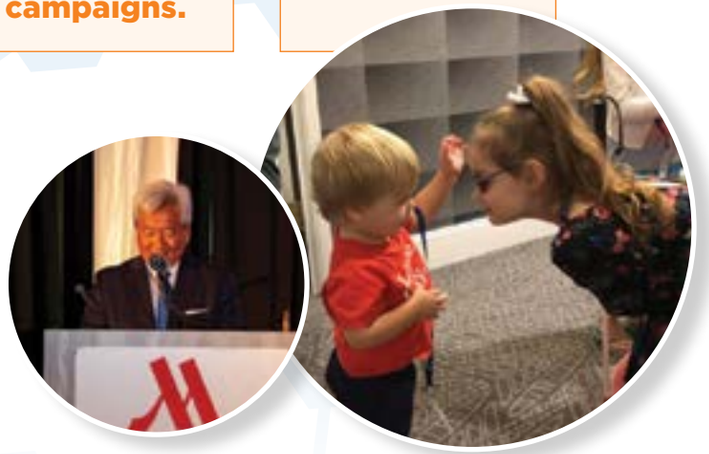
CY in FOCUS

We drive awareness and raise funds through social and marketing events, including Dinner in the Dark, A Rare Opportunity, and rare disease awareness campaigns.

Identifying a need for the FDA to hear first-hand what is meaningful to our community, we hosted the first FDA Listening Session for LCA in 2023.



"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. Knowing that my daughter can thrive and live a happy life with some occasional bumps in the road was a wonderful feeling."

—Joy Goodwine, mother of Jordynn "Jojo" diagnosed with LCA1 *GUCY2D*

COMMUNITY in FOCUS

We make it easy for those with LCA or IRDs to connect with people through our Family Connections program.

We publish a print and online newsletter called *Seeing Hope* with family stories and research updates.

We organize LCA Family Conferences connecting people living with LCA and their families with researchers, industry experts, regulatory agencies, and resources.

We host "Let's Chat About…" a webinar series featuring experts in research, industry, regulation, and our LCA and IRD community.

Théa Acquires LCA10 and USH2A RNA Treatments, Plans Further Clinical Development

However, the harrowing journey for these therapies is a cautionary tale

Ben Shaberman
Vice President,
Science Communications
Foundation Fighting Blindness

The development path for emerging therapies is often described as linear. When I present a general overview of the process for constituents, I typically say that a therapy moves step-by-step through lab studies in Petri dishes and animal models into a Phase 1, safety-focused human trial. If results are favorable, it continues to progress through Phase 2 and Phase 3 trials for efficacy evaluation. If the therapy meets its primary endpoint for efficacy in the Phase 3 trial, the therapy developer will seek regulatory approval. It is an expensive process that takes at least a decade but seems straightforward on the surface.

But the reality is that the development journey for many therapies is anything but smooth and linear. Whether ultimately approved by regulators or not, a therapy's development path may be fraught with hiccups and delays, including funding shortfalls, manufacturing problems, patient recruitment challenges, missed clinical trial endpoints, and unanticipated regulatory issues.

A Dramatic Journey for Two Promising Treatments

The story for two RNA therapies, initially developed by ProQR Therapeutics, is a prime example of how the treatment development road can be pretty bumpy. By April 2022, ProQR had advanced two RNA therapies into Phase 2/3 clinical trials: sepofarsen for Leber congenital amaurosis 10 (LCA10) caused by the IVS26 mutation in *CEP290* and ultevursen for retinitis pigmentosa or Usher syndrome 2A caused by mutations in exon 13. Excitingly, both treatments showed impressive efficacy in humans.

However, sepofarsen, which was furthest along in development, did not meet its primary endpoint at the conclusion of the pivotal Phase 2/3 trial. The US Food and Drug Administration (FDA) said ProQR would need to conduct another Phase 2/3 trial if it had any hope of getting approval. The enormous expense of another Phase 2/3 for sepofarsen and the uncertainty of outcomes for the ultevursen trial, which had recently moved into Phase 2/3, led ProQR in August 2022 to announce it was moving out of the ophthalmology business entirely to focus on other assets. That news was tough for the retinal disease community. We were so close to the finish line for two sight-restoring therapies!

But hope was reignited a year later when Laboratoires Théa, a leading European developer of eye care products, acquired the licenses for both sepofarsen and ultevursen and announced plans to continue clinical development of the two treatments. The promising RNA therapies appeared to have been saved.

However, the story took another distressing turn in October 2023 when Théa announced it was terminating its agreement with ProQR to acquire the RNA therapies because the agreement stipulated that certain employees from ProQR would transition to Théa but hadn't.

Fortunately, about two months later, the employment issue was resolved, and Théa announced its acquisition of sepofarsen and ultevursen was complete. Phew!

An Evolving Story

The sepofarsen and ultevursen story continues. Théa still needs to launch late-stage clinical trials for the two treatments, and hopefully, those will yield results worthy of the company applying for regulatory approval.

We are undoubtedly at an exciting and hopeful juncture with several dozen retinal disease therapies in clinical trials. But as this story indicates, we must understand and appreciate the inevitable challenges along the development journey.

To learn more about sepofarsen and ultevursen: bit.ly/4byRHnP

To read an inspiring story of a young patient in the sepofarsen Phase 2/3 clinical trial: bit.ly/3wcZNT4

Visit **FightBlindness.org** to stay informed about the latest research advances for LCA and other IRDs.

We take great care to use every dollar that we raise to advance our mission.

Although we returned to in-person events in 2023, the year presented new challenges for our organization as biotech **grant income** decreased and biotech companies were impacted by financial events. Despite positive scientific and clinical trial advances, some key supporters exited the retinal arena. At the same time, newer entrants to our space had not yet ramped up their advocacy funding.

Fortunately, we had our most significant success to date with Dinner in the Dark, thanks to our local community and faithful donors and sponsors. Although we hosted four **fundraising events** to engage the community, Dinner in the Dark continues to be our most significant fundraising initiative.

Notably, our **LCA Conference** was a substantial **program expense** in 2023. This biennial event is critical in supporting our LCA community and connecting all stakeholders involved in advancing treatments. In addition to the expense of the conference, we also created a scholarship fund, making it possible for more individuals and families to travel to this important event. Income for this fund is reflected under **direct support**, and we are grateful to our dedicated donors for their contributions.

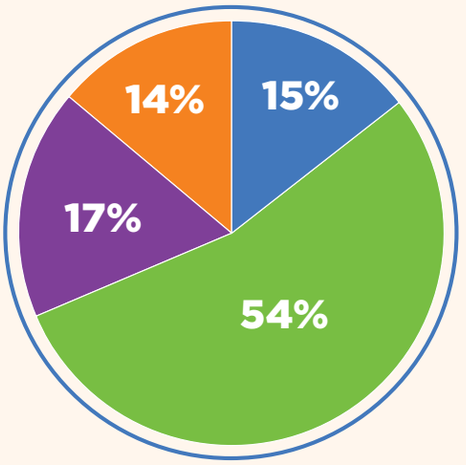
In 2023, our **research contribution** of $35,000 was considerably lower than in previous years. This contribution included 100 percent of the dollars specifically designated for research plus an additional amount from our undesignated assets. The amount reflects the Board of Directors' recognition of the importance of funding research while acknowledging that we are still building back our cash reserves following the pandemic and spending more on **outreach and advocacy programs** in an environment where grant funding has decreased.

Our most significant **program expense** last year was the LCA Conference. We decided to focus on the conference as a key initiative. Arguably, it is even more important for our organization to focus our funding on outreach and educational initiatives because we are the only global advocacy organization 100 percent dedicated to supporting those living with LCA.

Looking ahead...
2023 was a different kind of year for us as we doubled down on outreach, education, and advocacy work. Moving forward, we hope to see a significant investment in our advocacy work and programs as clinical trials advance, new companies enter the IRD space, and our global reach continues to grow. We also recognize the critical importance of diversifying our funding sources—an essential discussion item for our strategic planning initiative.

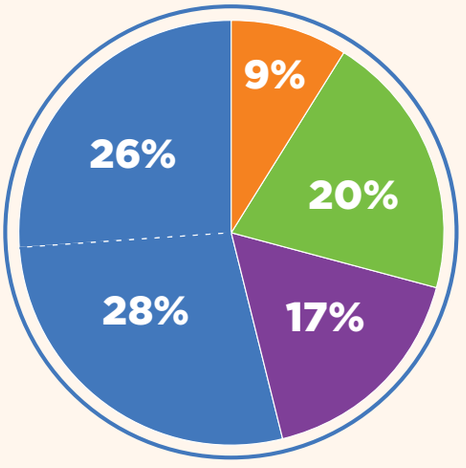
2023

INCOME



- **Fundraising Events**
- **Grant Income**
- **Direct Support**
- **Programs** (LCA Conference)

FUNDRAISING EXPENSES



- **Fundraising Events**
- **General Administration**
- **Programs** (Outreach & Education)
- **Program** (LCA Conference)
- **Research Contribution**

For more detailed information and to view our past Form 990s, visit our profile on GuideStar.

Thank You

FOR YOUR SUPPORT IN 2023!

INDIVIDUALS

Michele Ahlborn
Jeanne Allyn
Jacquelyn & Nathan Andersen
Rena Antoniou
Anabel Averso
Patience Banister
Cari & William Barnes
Andrea & Paul Barnhill
Steven Bentley
Rebecca & Daryl Beyus
Iva Boas & Jeffrey Finman
Beth & Andrew Borysewicz
Paul Bourguignon
Tracy Bourguignon
Todd Brady
Jane & Dan Brannegan
Marcie & Howard Brensilver
DJ & Brendan Broadbin
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Martha Delaney
Connie Desillier
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Amy & Kenneth DiRico
Cole DiRico
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Jean & Duncan Erickson
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Ornet Hines
Laura & Adam Hoffacker
Denise Hoffman
Anita Hwang & Robin Lee
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Jeanette Jezick
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Liz Kelly
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Nancy Ku & Thomas Wei
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Cristina Manfre
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Joan McCue
Catherine McFate
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James Mitchell
Margaret Moffitt
Gina & Kevin Morin
Pamela Nardella
Carole Nosseck
Kristen Nunes
Emily & Harley Nussman
Alice & Craig Nussman
Debbie O'Brien
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Jeffrey P'an
Delena & Gordon Painter
Cynthia Palmer
Adrienne & Andrew Parad
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Mark Rogers
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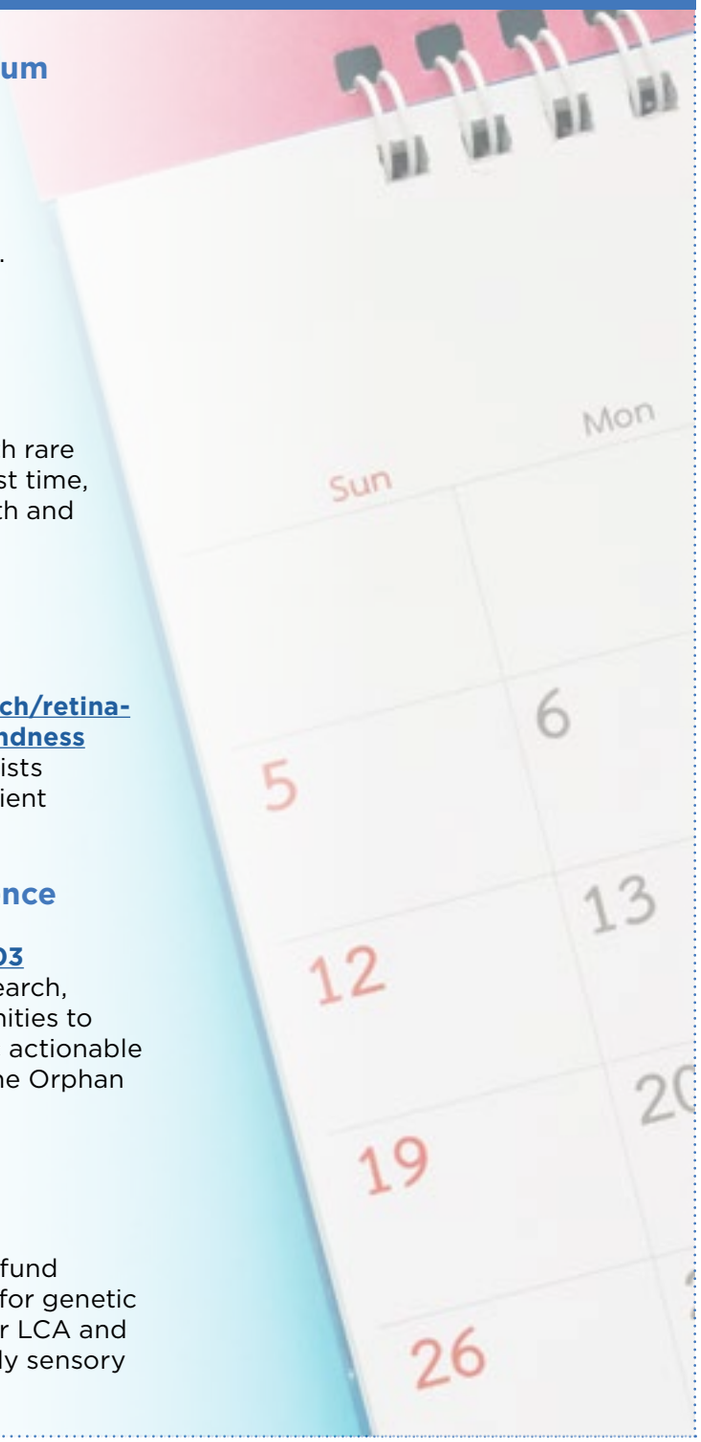
Marcela Staudenmaier
Rebecca Stracuzzi
Jennifer Dahlgren & Steven Sweeney
Gay Tevlin
Jonathan Travelyn
Barbara & Lon Truax
Rita Volkmann
Madeleine Wedvik
Maggie Wei & Thomas Robarge
Paula & Dennis Widstrom
Gerald Williams
Amelia Willson
Sheri Winter
Darlene Winter
Nancy & Ed Yarrish
Emma & Peter Zelken
Pat & Fred Ziegler

CORPORATE & ORGANIZATIONAL SUPPORT

Danielle Marie Senick Memorial Fund
Duncklee Cooling & Heating
Entact, LLC
F.W. Webb Company
Hoyt, Filippetti & Malaghan
Janssen
MeiraGTx
Melissa Allen Pilates & Movement
Mystic Financial Group
Mystic Lions
Niantic Lions
Noesis Capital Management
Simply Majestic
Spark Therapeutics
Town Fair Tire Foundation

Event Calendar

Do you have an event you want to share? *Let us know!*
Email info@hopeinfocus.org with the information and a link.



Global Genes • RARE Drug Development Symposium

April 29–May 1, 2024 • Philadelphia
globalgenes.org/event/rare-disease-drug-development
The Symposium, hosted by Global Genes and the Orphan Disease Center of the University of Pennsylvania, equips advocates with the knowledge, skills, and connections they need to advance therapy development for their communities.

NORD® • Living Rare, Living Stronger® Patient and Family Forum

June 7–8, 2024 • Los Angeles
livingrare.org
The Living Rare Forum is an opportunity for people living with rare diseases and their families to come together, often for the first time, to gain practical knowledge about how to manage their health and live their best rare lives.

Retina International & Fighting Blindness Ireland Retina International World Congress

June 5–8, 2024 • Dublin, Ireland
https://www.fightingblindness.ie/how-we-can-help/research/retina-international-world-congress-2024-hosted-by-fighting-blindness
This event brings together the world's foremost retinal scientists and clinicians alongside leaders in the fields of advocacy, patient representatives, health research and support organizations.

Foundation Fighting Blindness • VISIONS Conference

June 21–22, 2024 • Chicago
https://www.fightingblindness.org/events/visions-2024-503
The Foundation's global conference features sessions on research, advancements, practical adapting and thriving, and opportunities to connect with the blind and low-vision community. A dynamic actionable program and agenda were developed in collaboration with the Orphan Disease Center of the University of Pennsylvania.

Hope in Focus • Dinner in the Dark

October 19, 2024 • Foxwoods Resort Casino
hopeinfocus.org/get-involved/dinner-in-the-dark
Dinner in the Dark, our primary fundraiser for the year, helps fund research to cure blindness caused by LCA, provides support for genetic testing, and drives awareness, education, and connections for LCA and IRD families. Get ready for an incredible evening that is a lively sensory adventure with a stellar menu, fine wines, and more!

HOPE in FOCUS

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The *Seeing Hope* Newsletter is published quarterly by Hope in Focus, a 501(c)3 patient advocacy organization dedicated to generating awareness, raising funds for research, and providing education and outreach to the LCA and rare inherited retinal disease community.

To learn more about Hope in Focus, visit www.hopeinfocus.org.

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