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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2023 LCA Family Conference: Living with LCA

Panelists with varied vision share life successes and challenges

By Rosanne Smyle

Mohamed Farid and Mirielle St. Arnaud at the conference

Tami Morehouse is grateful for improved vision after getting groundbreaking gene therapy treatment at age 44 for LCA2 *RPE65*, but she's sometimes still sad and disappointed at the difficulties presented to her as a person with some functional vision, but who cannot see well.

Mohamed Farid found life living with LCA5 more challenging as a child than as an adult, especially before screen readers and other assistive technology. The young professional also remembers growing up and his grandmother spending a lot of time convincing him he couldn't be a pilot.

Mirielle St. Arnaud, a junior in high school living with LCA caused by a mutation in her *IQCB1/NPHP5* gene, tries to get involved with clubs and activities to ward off struggles with socializing. She learned to adapt to change and said her experiences with vision challenges have been good.

Tami, Mohamed, and Mirielle talked about their day-to-day lives, challenges, and feelings at our LCA Family Conference in Indianapolis in late June. They took part in a panel discussion called **Living with LCA**, moderated by Beth Borysewicz, an educational consultant with the Connecticut Department of Aging and Disability Services with Bureau of Education Services for the Blind.

The three panelists — an LCA research pioneer, a young professional, and a high school student — helped illustrate the manifestation of research advances and assistive technology in the last decade or so. ***Continued on page 2***

From the Founder:

Laura Manfre

On the heels of our third LCA Family Conference, our Hope in Focus team is psyched to be back in the swing of things.

Hosting the conference for families living with LCA represented another big step toward normalcy after the pandemic.

More than a hundred people from across the country and the globe converged in Indianapolis in late June for our 2023 LCA Family Conference filled with exciting LCA news and offering the perfect conditions for people living with LCA to get to know each other.

We witnessed the beginning of many friendships that often become deep and long-lasting relationships, as people have so much to share while navigating life with LCA.

The conference reached more people around the country and around the world — from Canada, Mexico, Turkey, and China — where they are also working to bring LCA advocacy to their countries. (At Hope in

Continued on page 3

2023 LCA Family Conference: Living with LCA

Continued from page 1

Here are some of what they shared with more than 100 people convened for the third Hope in Focus LCA Family Conference.

TAMI MOREHOUSE

Tami, 59 and from the Cleveland area, is thankful to see with more brightness following her participation in a pioneering clinical trial for what now is known as LUXTURNA®, the only federally approved treatment for one of the 27 identified forms of LCA — LCA2 *RPE65*.

She gained some vision in 2009 and 2010, when she received the treatment under development by Spark Therapeutics.

“My whole world was a lot brighter,” she said.

She could see a cup on the table, gorgeous sunsets, the lush green of spring, and details in the faces of her three children. It also improved her parenting skills.

“Mom can see things now,” she joked. “It’s bittersweet in our world.”

She also feels she lives in a kind of limbo between seeing and not seeing. She wished and still wishes the public would be more receptive to her being between having sight and not having sight, rather than being completely blind.

Tami’s world has widened, and she enjoys much happiness since receiving the gene therapy. She is a Hope in Focus Ambassador, working with our Family Connections program, reaching out to people living with LCA, offering them comfort and kindness. The research pioneer works as an information and referral specialist for 211 in Ohio.

People in the LCA community, like others dealing with a rare disease, experience anxiety, depression, and social isolation.

Tami opened up at the conference, sharing struggles to maintain mental wellness.

She experienced a lot of awkward moments and has worked at becoming comfortable with who she is as a blind person.

“There are so many times when I’m very sad and disappointed about my limitations. There are a lot of barriers in the way and that’s hard to take.”

She misses experiences she could have had with her children and husband and parents.

“Those are just facts of life. Those are my facts, but I think many others go through the same.

“Sometimes we just feel the burn.”

MOHAMED FARID

Mohamed Farid navigates life with LCA deftly, now that he is an adult.

“It’s harder growing up. It’s easier when you’re older.”

Life became easier with innovative technology, such as screen readers and other optical character-recognition technology that extracts and converts data into a machine-readable form.

“It’s all about assistive technology,” he said.

The 28-year-old founded MKF Continuity, a middle-market investment firm in Chicago. He earned an MBA from Harvard Business School.

There was a time when he was running away from his blindness.

“Now, I’m just ‘whatever’. I need to be independent. It’s important for my dignity. I don’t want to be relying on people.

“Every once in a while, I think the world is unfair,” he said, but over time he’s developed self-acceptance and is at peace with his blindness.

He compared moving through life now not even thinking about his blindness to his perception of his vision as a child.

“My grandma had to spend a lot of time convincing me I could not be a pilot.”

In contrast, as an adult at his workplace, people think he can create a slide presentation.

Mohamed said his blindness has made him strong, describing himself as “alert, scrappy, and resilient.”

MIRIELLE ST. ARNAUD

Mirielle St. Arnaud, a 16-year-old from the Chicago area, said she’s dealt with people who assume that she’s not a capable person.

“We’re probably more resourceful than you think,” she said.

Mirielle is a junior in high school, where she runs on the cross-country team with a guide and is a captain of the Congressional Debate team.

She learned to advocate for herself and set boundaries for others.

Mirielle worked on difficulities with socialization at school by getting involved in the blind and sighted communities, whether through summer camps or extra-curricular activities, and by meeting as many people as she can.

“At some point, you’ll find your people who will understand you.”

She characterizes her ability to see as “Swiss-cheese vision.”

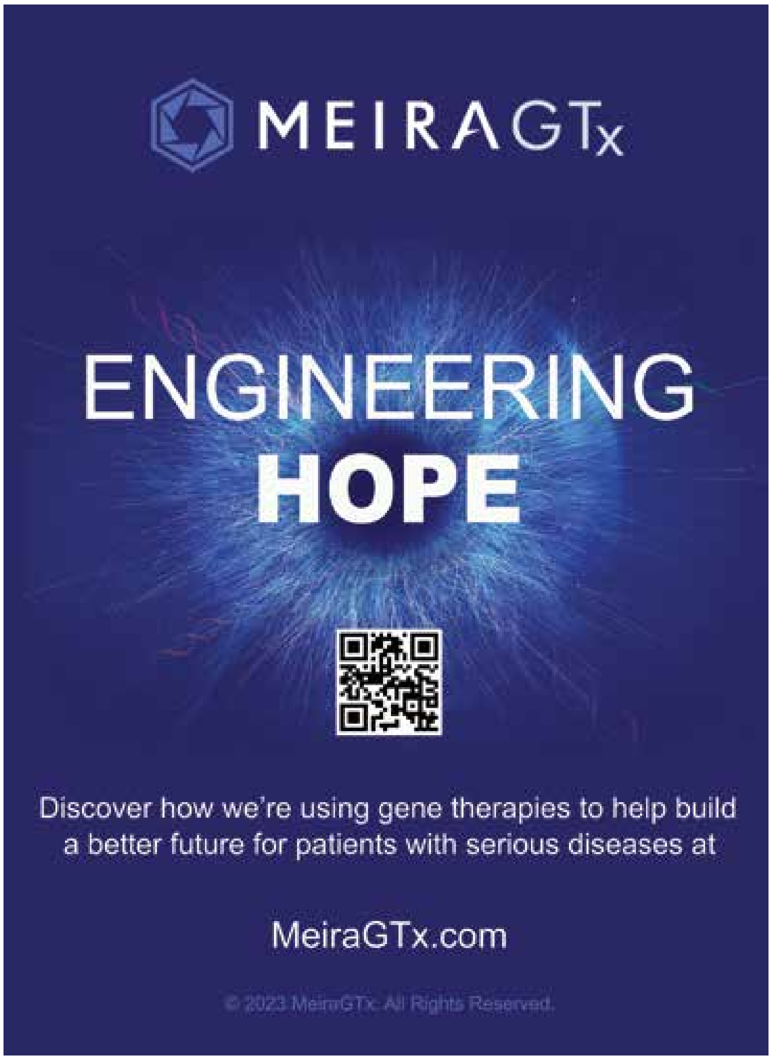
At school, she works with an advocate throughout the year, using an Independent Education Plan as a guide.

Mirielle said vision specialists collaborating with her schools have been especially helpful along the way. She’s worked with Teachers of students with Visual Impairments (TVIs), who are educators with expertise with the visually impaired, and Orientation and Mobility (O&M) specialists, who teach safe and effective navigation through environments.

“I’ve had pretty good experiences,” she said.

As the session wrapped up, one of the panelists shared a message with the audience.

“Our journey can be bumpy,” Mohamed said. “Don’t ever give up hope.”



From the Founder:

Continued from page 1

Focus, we’re already thinking about how we can better support these important grassroots efforts in other countries.)

We’ve just finished unpacking from the conference, and we’re already deep into our next grand event — our 8th Annual Dinner in the Dark, taking place October 14.

Dinner in the Dark is an occasion not to be missed. You’ll want to be part of this extraordinary sensory experience of culinary delights — while wearing a blindfold — to experience just a bit of what it’s like to be without sight. You’ll find more details about our gala fundraising event in this newsletter.

We’ll also share in this edition LCA stories from our family conference and news about funding for LCA research.

And, as we look forward to all that is to come, we also are grateful for your support. All of our activities, including our conference and our ability to keep it affordable for many participating families, would not have been possible without your generosity and participation.

Thank you so much!

With gratitude and focus,

Laura

Hope Rules at 2023 Hope in Focus LCA Family Conference in Indianapolis

By Rosanne Smyle

Our Hope in Focus LCA Family Conference in Indianapolis offered many reasons to feel hopeful about more retinal disease treatments coming to fruition, despite the deep complexities and tremendous expense of researching and developing LCA therapies.

More than 100 people gathered from around the globe in Indianapolis for the 2023 Hope in Focus LCA Family Conference — making friends, meeting researchers, and establishing connections to help better navigate life with Leber congenital amaurosis, a rare inherited degenerative retinal disease.

The conference, from June 23–24, created an ideal environment for thoughtful and interactive exchanges of knowledge, ideas, and viewpoints in sessions focused on research, future treatments, advocacy, and sharing stories.

The Hotel Omni Severin's mezzanine buzzed with sounds of families socializing with conference speakers and other families, kids running around, playing games, and grownups meeting other adults living with the rare disease.

As one caregiver said after a conference session: "This is fantastic! I'm getting answers to questions I didn't even know how to ask."

People from the Midwest, the rest of the country, and around the world from Canada, Mexico, Turkey, and China, converged in the racing capital of the world to hear from people who make up part of the LCA ecosystem: Families, doctors, researchers, advocates, regulators, and representatives of the biotechnology, pharmaceutical, and healthcare industries.

Navigating the LCA Ecosystem

Laura Manfre, Board Chair and Co-Founder of Hope in Focus, welcomed the group to the third LCA Family Conference, our first in four years because of limitations caused by the COVID-19 pandemic.

Advancing treatments for LCA is part of an ecosystem that many may not have thought about before being thrown into it after getting a rare disease diagnosis.

"No one asked to be a part of this disease or came into this knowing how to navigate this journey for their child, their family, or themselves," Laura said. "We are all figuring it out as we go, and the more we can connect with one another, the better we are able to learn and share and support one another."

Even having the kids go to the Children's Museum of Indianapolis, while parents attended panel discussions, marked a milestone of sorts for children with and without sight enjoying a day together.

Laura, her husband, Charles Priebe, and their friend Elisse Rosen founded Hope in Focus nearly 10 years ago to raise funds for research, after doctors told Laura and Chuck their 11-year-old daughter Sofia had LCA caused by a mutation in her *IQCB1/NPHP5* gene.

Sofia could not attend the

conference and her mother explained:

"She is now 20, a rising junior in college, and her vision is getting worse," Sofia's mom said. "She was supposed to be here and be on a panel, but she finally, after years of waiting, got a guide dog and is just wrapping up two weeks of training with her new dog." (Sofia officially received her new pal, Winsome, a beautiful black lab.)

Sofia's mom learned raising funds was important, but money alone doesn't solve the issue.

"Personally, and for Hope in Focus, the last decade has been a learning experience of all the things that need to come together to pave the way for treatments for ourselves and our children.

"That's everything from making sure our community has access to genetic testing and is getting diagnosed and not misdiagnosed, which is still an issue in some regions. It includes participating in patient registries and in natural history studies. It means advocating for policy changes with our legislators, and making sure we are well-informed and educated, and we are all heard."

Zero to more than 30 Clinical Trials

Ben Shaberman, Vice President of Scientific Communications for the Foundation Fighting Blindness, said when he started at the Foundation in 2004, research into blindness consisted of working with mice and sometimes dogs, not humans.

Four years later, researchers began clinical trials for a drug to treat a form of LCA. In 2017, after 12 years and $500 million in research and development, the gene therapy LUXTURNA® received regulatory approval to treat LCA2 *RPE65*.

Today, about three dozen clinical trials involving retinal disease are underway, with 40 biotechnology companies investing in a range of treatment therapies.

"It really is an ecosystem, and you are so much a part of that," said Ben, who also is an Advisor to Hope in Focus. "You are moving mountains. Please, please, please, do not underestimate the power that you have to move this forward."

Hope is Imperative

Hope is a very powerful thing.

"It's the bedrock of why we founded this organization, and why we still exist," Laura said. "Ten years ago, we didn't have a single treatment in sight, and we only had hope — hope that someday there just might be a treatment for our child.

"Everyone is gathered here today because we have some kind of hope in focus — hope to learn something new about this rare disease community, hope to make a meaningful connection with someone else on this journey, hope to bring new information back to organizations in their country, or hope to be the one that brings the next LCA treatment to market."

2023 Dinner in the Dark: A sensory event not to be missed!

By Rosanne Smyle

It's time to celebrate! Our Hope in Focus team is happy to invite you to our 8th Annual Dinner in the Dark, a one-of-a-kind fundraiser to advance treatments and cures for Leber congenital amaurosis and other rare inherited retinal diseases.

Join us Saturday, Oct. 14, 2023, at the Mystic Marriott Hotel & Spa in Groton, Connecticut.

Now is the time to make plans to be in beautiful Connecticut in the fall and make sure you have a ticket to this extraordinary culinary delight of a night. For details, please see: **hopeinfocus.org**.

You will be treated to a gourmet, multi-course dinner, with paired libations. The menu is secret; you'll get to figure that out — blindfolded. Guests with visual challenges and who can read Braille will know what's on their plates because of a Braille-printed menu. The rest of us will get a small taste of the challenges that can accompany vision loss.

This event serves to connect others with our mission and has helped raise more than a half-million dollars throughout the years to fund research to treat blindness. With heartfelt gratitude, we thank you and hope to see you in October!

HOPE

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COMMUNITY SURVEY

Hope in Focus undertook a major effort during 2022 in the design and implementation of a community survey to better understand the journey and needs of those living with LCA. We collected and analyzed information about diagnosis, genetic testing, feelings on clinical trials, and resources needed by the community. That data has helped guide our programming forward.

VISIONS 2022

The Hope in Focus team attended the Foundation Fighting Blindness' VISIONS conference in June 2022. Six of us represented the LCA community and led the mix and mingle for our disease. We met three individuals, all with similar diagnostic journeys — finding out only in recent years they actually had LCA. It was a great return to an in-person conference for the inherited retinal disease community.

DINNER
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Issues of *Seeing Hope* published to 650+ households in 12 countries

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HLIGHTS

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RESEARCH CONTRIBUTIONS

The return of our major fundraiser, Dinner in the Dark, and other in-person fundraising events helped move us closer to our pre-pandemic levels of research funding. Our board of directors authorized a $75,000 donation to research for the calendar year 2022. This marked great progress toward our $300,000 pledge to the Foundation Fighting Blindness for LCA therapies.

LEGISLATIVE ACTION

Hope in Focus advocated for years on behalf of our rare retinal disease community for its home state of Connecticut to create a Rare Disease Advisory Council, which came to fruition in 2022 with Connecticut Gov. Ned Lamont's signature. We gave a statement to the Public Health Committee, explaining LCA and IRDs, and the effects of living with a degenerative retinal disease.

%

e in our
community

18

Stories written that share important information across the LCA experience

$75K

Donated to research

Your Tax Dollars at Work: Two Federal Programs Target Therapies for LCA and Other IRDs

Ben Shaberman
Vice President,
Science Communications
Foundation Fighting Blindness

I am excited to report that significant funding from two federal programs — the National Institutes of Health (NIH) Common Fund and the Bespoke Gene Therapy Consortium (BGTC) — is advancing development of therapies for Leber congenital amaurosis (LCA) and other inherited retinal diseases (IRDs).

Gene-editing treatment development for LCA16 *KCNJ13* and Best disease

A multi-disciplinary retinal research team from the University of Wisconsin-Madison (UWM) has been awarded a five-year, $29 million U19 grant from the NIH Common Fund to develop CRISPR/Cas9 gene-editing treatments for two inherited retinal conditions: Best disease caused by the *R218C* mutation in the BEST1 gene and Leber congenital amaurosis caused by the *W53X* mutation in the LCA16 *KCNJ13* gene.

Known as The CRISPR Vision Program: Nonviral Genome Editing Platforms to Treat Inherited Retinal Channelopathies, the grant will advance the LCA16 *KCNJ13* and Best disease treatments toward evaluation in clinical trials.

The Common Fund is a line item in the NIH budget supporting programs that are goal-driven strategic investments intended to catalyze research across multiple biomedical research disciplines. Common Fund programs must be transformative and advance research that no other entity is able or likely to do.

CRISPR/Cas9 gene editing is an emerging therapeutic approach that works like a pair of molecular scissors to cut out or modify the mutated region of the gene. Gene editing is different from gene (replacement) therapy. In gene therapy, copies of an entirely new gene are delivered to the retina to replace the defective copies. In CRISPR/Cas9 gene editing, only the mutated region of the gene is corrected.

For the LCA16 *KCNJ13* and Best disease projects, investigators will use induced pluripotent stem cells, which provide a powerful starting material to model human diseases to create human models of the conditions to evaluate the CRISPR/Cas9 treatments. The team will extract a small sample of skin or blood cells from patients and tweak the cells, so they revert to a stem-cell-like state. Then, the cells will be coaxed forward to differentiate into retinal pigment epithelial cells, the supportive cells affected in people with LCA-*KCNJ12* and Best disease. The team will also evaluate the LCA16 CRISPR/Cas9 treatment in a mouse model.

Investigators for the UMW project include: Bikash Pattnaik, PhD, associate professor of pediatrics, ophthalmology and visual sciences at UWM; Krishanu Saha, PhD, associate professor of biomedical engineering and faculty member of the Wisconsin Institute for Discovery (WID); David Gamm, MD, PhD, professor of ophthalmology and visual sciences and director of the McPherson Eye Research Institute; and Shaoqin "Sarah" Gong, PhD, professor of ophthalmology and visual sciences and biomedical engineering and WID faculty.

Investigators at Spotlight Therapeutics, the Morgridge Institute for Research, and the UMass Chan Medical School will work closely with the UWM team on the project.

AAV gene therapy development for LCA-*NPHP5/IQCB1* and RP-*CNGB1*

The NIH and U.S. Food and Drug Administration, along with 15 commercial and nonprofit organizations, launched the Bespoke Gene Therapy Consortium in 2021 to boost gene therapy development and manufacturing for several rare genetic diseases, including LCA caused by *NPHP5/IQCB1* mutations and retinitis pigmentosa caused by *CNGB1* mutations.

More than $100 million in funding is being provided for eight BGTC projects over five years. The Foundation Fighting Blindness is a BGTC private partner.

A major goal of the BGTC is to generate resources the gene therapy community can use to streamline development of adeno-associated virus (AAV)-based gene therapies for rare diseases. Human-engineered AAVs are commonly used as delivery systems for gene therapies because they are safe and can be designed to effectively penetrate many different cell types, including retinal cells, with therapeutic genetic cargo. LUXTURNA®, the FDA-approved gene therapy for LCA2 (*RPE65* mutations) uses an AAV.

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

Jack McCormick column

Learning Along the Way in Setting and Attaining Goals


Jack and his guide dog, Baloo

In my last column, I wrote about getting into running and my plans to run my first half marathon. I thought I'd dive deeper into the lessons I've learned from running.

My first goal was to run 10 kilometers in under an hour. When I started, I was literally miles away from this goal. I was only able to run two miles and had to take several walking breaks. As I improved, I was reminded that with consistent practice, I can get good at just about anything — emphasis on "consistent."

I felt a bit foolish the first time I trained in the middle of a snowstorm, and, even more so, the first time I woke up early for a 5 a.m. run, but those instances helped me get to where I am now.

I initially planned on running one half marathon in May. One soon turned into two. My first was in Toronto. I did not do as well as I wanted but learned several valuable lessons, including having a backup guide and starting closer to the front so I don't have to waste as much time and energy moving around slower runners.

I am glad I had the opportunity to run the second race in Ottawa. Thanks to my learnings from Toronto, I finished in 1 hour and 47 minutes, 10 minutes faster than Toronto and 3 minutes faster than my goal. I learned that no matter how well prepared you think you are, you will always learn how to do things better.

And, in Ottawa, I was reminded of one of the benefits of being visually impaired — the opportunity to meet some of the kindest people. A friend connected me with some locals to guide me during the race. I ran a half marathon with guides I met the day before. Talk about trust. It turned out to be a good decision — they were some of the most welcoming people!

I now have my sights set on running a full marathon. However, I am recovering from a minor knee injury, which leads me to the last lesson I'd like to share from my experience running.

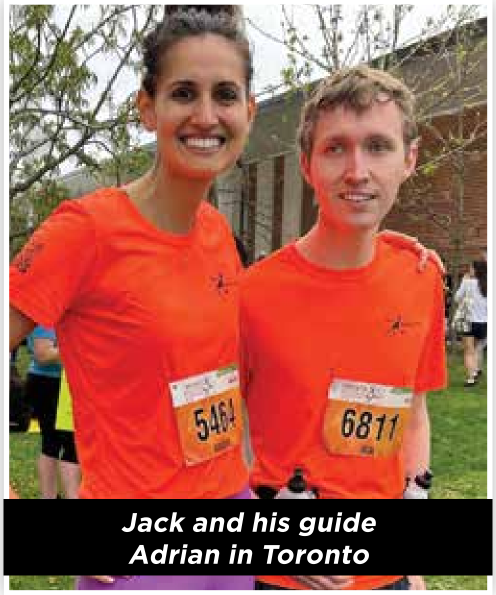
Things don't always go as planned. I wish I didn't have an injury, but, through my recovery, I've learned a lot about preventing it from happening again. I've been reminded to look for positives.

The same is true of my visual impairment; I sometimes wish I didn't have all the challenges that come with it, but, if I choose to look at it more positively, I realize it has helped me become the strong person I am today.

There must be truth in that adage: "Whatever doesn't kill you makes you stronger."

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 (L) with guide Charlie, runner John, and guide Christian in Ottawa



Jack and his guide Adrian in Toronto

HOPE in FOCUS

DINNER IN THE DARK

Saturday, October 14, 2023

Join us for a unique culinary experience.

Mystic Marriott Hotel & Spa • Groton, CT
5:30 PM – 7:00 PM Cocktails
7:00 PM Dinner, live auction & dancing

Made possible thanks to these sponsors

Sponsor opportunities available at hopeinfocus.org.

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Event Calendar

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

Hope in Focus • Dinner in the Dark

October 14, 2023 • Groton, CT
hopeinfocus.org/get-involved/dinner-in-the-dark-2023

Dinner in the Dark, our primary fundraiser for the year, helps fund research to cure blindness caused by LCA and to drive awareness, education, and connections for LCA and IRD families. Get ready for a stellar menu, paired libations, and a lively sensory adventure.

Hope in Focus • FDA Listening Session

October 30, 2023
hopeinfocus.org

Hope in Focus has been preparing since last year to meet with the U.S. Food and Drug Administration for an FDA Patient Listening Session on LCA. Patient Listening Sessions are small meetings that help the agency better understand a disease or disorder by hearing directly from patients and caregivers. The session is not open to the public, as attendance is limited to help patients and caregivers feel comfortable sharing their personal experiences. Please visit our website for a comprehensive summary following the session.

SAVE THE DATE
Foundation Fighting Blindness VISIONS Conference

June 21–22, 2024 • Chicago, IL
www.fightingblindness.org/events/visions-2024-503

The Foundation's national 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 have been developed in collaboration with the Orphan Disease Center of the University of Pennsylvania.

Welcome, Marie!

Hope in Focus is pleased to announce the addition of Marie Thomas, our new Marketing Manager.

Marie joins in our mission of advocating for people living with LCA and driving research to find treatments and cures for blindness.

She brings knowledge, compassion, and experience to our team after a decade of diverse corporate roles, including those in client services, project management, and marketing in finance, legal, and technology.

We saw Marie in action in June at our LCA Family Conference in Indianapolis, where she dynamically connected with families living with LCA, retinal researchers, and biotech and pharma representatives.

“I was honored when Hope in Focus asked me to lead the creative strategy for the LCA Family Conference,” she said. “It gave me the opportunity to put my skills to the test by helping convey Hope in Focus' message and highlighting its brand story authentically and accurately.

“My goal was to raise as much awareness as possible so that people in our community truly understand the challenges and will join in supporting our global advocacy efforts to advance LCA and IRD research.”

Marie earned a Bachelor of Arts degree from the University of Connecticut and studied at the New York Institute of Art & Design.

Welcome, Marie. We're happy you're here.

HOPE in FOCUS

P.O. Box 705 | Ledyard, CT 06339

The *Seeing Hope* Newsletter
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To learn more about Hope in Focus, visit www.hopeinfocus.org.

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