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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Teen Living with LCA5 Hopes to be a Filmmaker

Family Excited About New LCA5-Lebercilin Research Program


Naomi Mills

By Rosanne Smyle

John Mills says he would crawl over broken glass if it led to a cure for his daughter's visual impairment.

Fourteen-year-old Naomi Mills of Virginia lives with the rarest form of Leber congenital amaurosis — LCA5, which encodes the protein lebercilin. Lebercilin is responsible for moving proteins up and down, between the inner and outer segments of the photoreceptor cell so it can operate properly and stay healthy.

While most parents of children diagnosed with a rare inherited retinal disease (IRD) can identify with John's feelings toward finding a cure, a

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From the Founder


Laura Manfre

Here we are in the summer of 2022, and the world is making progress toward getting back to a new normal after more than two years of COVID-19 and life as we knew it.

Our Hope in Focus Special Edition newsletter presents a manifestation of our progress, as we highlight last year's accomplishments, despite pandemic-related challenges, and share updates on promising research advances into Leber congenital amaurosis and other rare inherited retinal diseases.

This edition of *Seeing Hope* gives an in-depth look at the many clinical trials and studies advancing research into various forms of LCA — a lot more since 2017, when the FDA approved a gene therapy for one of the 27 forms of this rare disease.

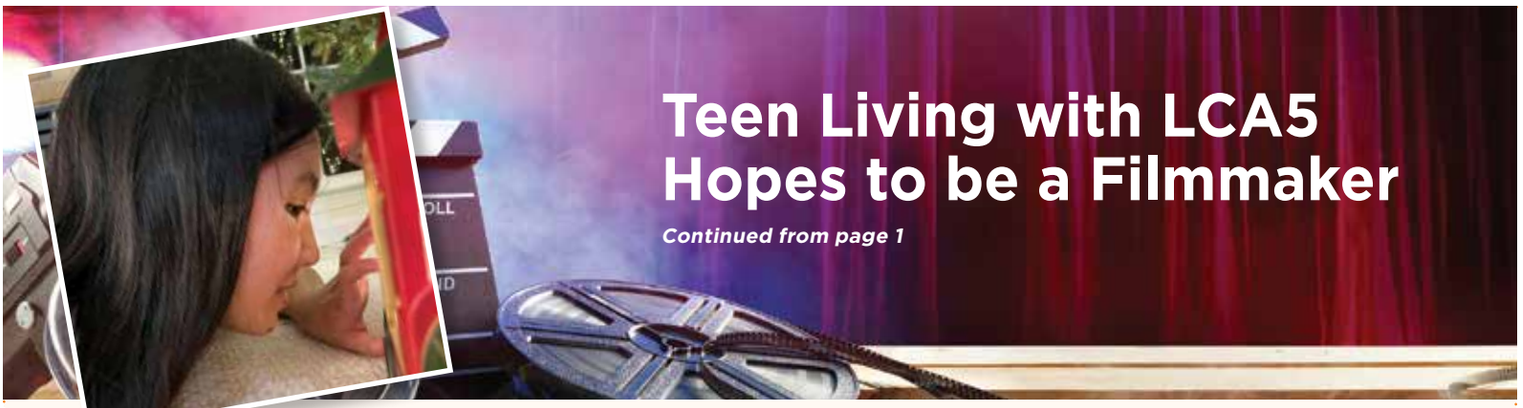
You'll also learn about a teen living with LCA5, who sees herself as a future filmmaker. She and her family are excited about an upcoming study on LCA5, an extremely rare form of LCA, affecting about one in 1.7 million people.

We'll introduce you to an incredible teacher who makes a living helping children with visual disabilities realize their potential as self-determined adults. Hopefully, you'll also catch a bit of her infectious joy and exuberance that enhances success in her many charges.

You'll also hear from our columnist, who'll tell you about his experiences in moving to Canada's biggest city.

And, looking ahead to the fall, we're excited to bring back our gala annual event, Dinner in the Dark — live and in-person!

With gratitude and focus,



Teen Living with LCA5 Hopes to be a Filmmaker

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recently launched genetics company just might save his hands and knees from harm. Opus Genetics based in Raleigh, N.C., plans to prioritize research into this severe form of LCA that affects about one in 1.7 million people.

"Kathie (John's wife) and I pray every day that there'll be a pathway to improve her vision, that she'll be able to drive someday.

"We're very excited. We know there's a lot of moving parts here — doctors, advocacy groups, medical research, the FDA. Trying to get all of those aligned for the moonshot is quite an orchestration."

HIGH ASPIRATIONS FOR TEEN WITH LCA5

Low vision aside, her father said Naomi wants the freedom of mobility in owning a car.

"Naomi was given a suggestion by her wonderful teacher that a best practice is to buy a car, even if you're blind, so you can ask someone to 'Please drive me in my own car,' " John said. "She also hopes that she can drive her own car someday."

The couple's daughter also loves music, plays piano, and wants to be a filmmaker.

How can a person with visual difficulty make a film? Naomi did on a recent Sunday afternoon, her father said, documenting the finishing of the family basement, recording clips of action, inserting music, and editing everything into a film.

"It's beautiful," he said. "She's very good at whipping films together."

He also said Naomi is her own person.

"She wants to grow up and move out as fast as she can and get her own place and live on her own. That's a good thing," John said. "She just loves the thought of being independent. We encourage that."

With 20/500-600 vision, Naomi needs bright light, and, while colorblind, she can see contrasts and large black letters on white paper.

"My understanding and interpretation is that Naomi has tunnels of goodness that she can see out of. It's like looking out of a Wiffle ball, tunnels she can see through but not consistently," he said. "She can read large print and she can write fairly well. She's also proficient at reading and writing braille."

CONFIRMED LCA5-LEBERCILIN DIAGNOSIS

The couple adopted Naomi in 2010 at age 2 from China, knowing doctors diagnosed her with Retinitis Pigmentosa, and John said, "We're taking her on faith. It doesn't matter."

Their son, Michael, now 32, accompanied his parents to China when they adopted their first daughter, Sarah, from an orphanage in 2001. Sarah, now 21, went to China with John and Kathie when they brought Naomi home from foster parents nine years later.

Back in the United States, the couple followed up any leads to help Naomi with her vision.

"It was very murky and confusing, trying to connect with resources and groups," he said.

The family received Naomi's confirmed genetic diagnosis after a visit to a doctor at the National Institutes of Health.

Kathie became highly proficient in braille and initially homeschooled Naomi, who now attends the Virginia School for the Deaf and the Blind.

In the past several years, Naomi made the national finals in the Braille Challenge, the only academic competition of its kind in North America for students who are blind or visually impaired.

"She is so sharp, so smart, and such a blessing," Naomi's mom said.

Navigating a New Move — Jack McCormick column

LIVING IN A BIG CITY:

Finding a balance between more convenience and better resources versus deafening sirens and hordes of people

I recently moved to Toronto — Canada's largest city. Having now experienced living in a small town, a medium city, and now a large city, I thought I would reflect on my experience and share some of the benefits of each for people who are blind or partially sighted.

I'm coming to really appreciate living in the big city. The subway comes every two minutes during rush hour and the nearest station is only five minutes from my place, which is a huge improvement from the every 30-minutes' bus schedule I am used to.

Better yet, just about everything I need is within a 10-minute walk from me. I love how easy it is to get groceries, go to the bank or eat out. I have also learned that there are many more resources here for blind and partially sighted people. For example, there is an organization down the street where I can borrow a free tandem bike.

Living in the city does come with its trade-offs though. The streets can be very busy and overwhelming, making it hard to navigate especially with vision loss. Add on to that all the sound and at times it can be a nightmare.

Imagine trying to walk in the opposite direction of hundreds of people while a fire truck blasts its sirens, and you can begin to understand what I mean.

Also, with more people comes more problems. Most recently I had a man on the subway think it would be a good idea to continually make noises to try to get my guide dog's attention and when that didn't work, he threw a bunch of food on the ground to try to get my guide dog to eat it.

The jury is still out on whether I prefer living in a smaller community. However, these are some of the things I've noticed so far.

No matter where you choose to live, I think it is important to reflect on your needs and determine how you will do the basics like getting around and getting food. If there is anything I've learned as someone with a visual impairment, it is the more prepared I am, the better things tend to work out.

Jack and his guide dog, Baloo

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***

Let's Chat About... Self-Advocacy and Supporting Your Child's Education with Beth Borysewicz

Beth Borysewicz

By Rosanne Smyle

Children living with visual impairment become more independent and empowered when parents set high expectations for their kids and challenge them every day.

Just ask Beth Borysewicz. In her role with Connecticut's Bureau of Education Services for the Blind, she makes a living helping children with visual disabilities realize their potential as strong, self-determined adults. And she's the first one to say, often with tears in her eyes, that her job is to work herself out of a job.

She described her work in helping children from birth to 22 years old with visual impairment or blindness become more independent as adults as part of the Hope in Focus "**L**et's **C**hat **A**bout..." webinar series. Our March episode, moderated by Courtney Coates, Director of Outreach and Development, featured Borysewicz, an Education Consultant for the Department of Aging and Disabilities, Bureau of Education Services for the Blind. You can view the episode at https://hopeinfocus.org/for-families/lets-chat-about/.

Borysewicz found her passion working with the blind and low-vision community unexpectedly 16 years ago, when she had a 3-year-old student named Sofia, who was diagnosed with LCA. Yes, that would be the same Sofia as in Sofia Sees Hope, our organization's original name until a recent rebranding to Hope in Focus. Borysewicz also is Vice Chair of our Board of Directors.

She said parents need to be the biggest advocates for their children.

"If you think your child is not getting what they need, you can ask for it."

She also encouraged connections with people who have been on this journey before, bringing to mind the Hope in Focus Family Connections program that helps ease feelings of isolation that can arise when a family member is diagnosed with a rare disease.

"It's the people that have already gone through it who will help you the most, including Hope in Focus. That's why I'm on the Board. What Hope in Focus does for families is immeasurable."

ALL THE LITTLE THINGS WE DO EVERY DAY

As a Teacher of Students with Visual Impairments (TVI), Borysewicz focuses on teaching students self-advocacy and exploring the Expanded Core Curriculum (E.C.C.), distinguished from a school's standard core curriculum consisting of courses in math, science, reading, and the like.

E.C.C. comes from the perspective of teaching students with blindness or low vision and encompasses nine areas: Compensatory Skills, Orientation and Mobility, Social Interaction, Independent Living, Recreation and Leisure, Sensory Efficiency, Assistive Technology, Career Education, and Self-Determination.

The curriculum is more than a checklist or lesson plans for learners with a visual impairment, according to the E.C.C.'s website, www.theeccandme.com. It's all the little things we do every day, done with intention so children with visual impairments can learn skills they need for a fulfilling life.

"I wish everyone would do what I do," Borysewicz told her webinar audience. "There is a shortage worldwide of TVIs."

She advised parents to set expectations high for their children, challenge them every day, and give them a safe place to learn.

"Children can do anything they set their minds to. Do anything you can do to empower them," she said. "Celebrate everything. Celebrate every little thing."

She talked about self-determination, saying it's her favorite part of the curriculum and the most important.

"It's teaching a child to believe in themselves and just take that leap," she said. "It's taking that step off the curb to cross the street with a cane or initiating a conversation at a lunch table that builds self-confidence."

MAKING LEARNING FUN AND EXCITING

Her work with people from birth to age 22 encompasses figuring out resources for newly diagnosed children, for school-aged students, and for young adults transitioning to the workforce or college.

"I switch hats from appointment to appointment every day," she said. "As you can tell, I love my job."

Working with individualized education plans (IEP), she and her team help students become the best they can be in all the E.C.C. areas.

"It's so important for the student to say, 'This is what I need and why I need it,' and just building those skills will make them successful as adults."

And a lot of it is fun, especially with Borysewicz who excels in the Recreation and Leisure department. It goes back to when she was growing up and her dad always told her she was good at playing with people and should get a degree in play.

She uses that play degree often by creating board games to make math more fun or putting together programs to help students from prekindergarten through third grade explore the nine E.C.C. areas in their daily lives.

In an Expedition to Explore, students in the Young Passport Program work on accumulating life skills at home over the summer. Each student has a passport consisting of pages designated for each of the nine E.C.C. areas, with a slant toward adventure.

For example, "Career Education Caves" focuses on conversational skills, encouraging children to stay connected with their friends over the summer, known in the business world as networking, and holding mock interviews with their siblings or stuffed animals.

In "Self-Determination Safari," a goal is to get the child to ask for help. A parent asks a child to do an unfamiliar chore, such as taking out the trash or putting toys away but doesn't give guidance on how to do it or where to put the trash or toys, prompting or encouraging the child to ask for assistance or directions.

"Social Skills Glaciers" encourages children to spread kindness to neighbors and the community and recommends an online guide called "100 Acts of Kindness for Kids." Activities include listening, following directions, taking turns, ignoring distractions, cooperating, and showing empathy. (Sounds great for adults, too!)

RESOURCES FOR PEOPLE WITH BLINDNESS OR LOW VISION

Borysewicz talked about her work from the perspective of Connecticut and said services may differ from state to state.

She authors a blog dedicated to professionals, families, and students called I Love Brl (Braille) (https://ilovebrl.com), and she provided webinar viewers a list of online resources that can be found at: https://hopeinfocus.org/lets-chat-about-self-advocacy-and-supporting-your-childs-education-with-beth-borysewicz/

*To learn more about our "**L**et's **C**hat **A**bout..." series, visit **hopeinfocus.org/for-families/lets-chat-about/***

HOPE in FOCUS
LET'S CHAT ABOUT...

LET'S CHAT ABOUT...

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LET'S CHAT ABOUT...

We introduced a free webinar series that brings in experts for informational conversations on current topics important to those living with an IRD, along with those in research, industry, and regulatory communities involved in the LCA space.

RARE SCAVENGER HUNT

We hosted our second annual virtual scavenger hunt where teams signed up to participate in a series of challenges designed to drive awareness of rare disease and our organization, and have some goofy fun. The event raised more than $5,000 for research.

SCIENTIFIC ADVANCEMENT WORKSHOPS

In partnership with the Foundation Fighting Blindness, we hosted two workshops attended by leading research and industry representatives to share research, hear patient perspectives, and identify next steps to advance treatments for the patient population. The workshops focused on *CRB1* and *IQCB1/NPHP5* variants.

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NEW TEAM MEMBER

We hired a full-time Director of Outreach & Development, Courtney Coates, to help support our many initiatives. Courtney's focus is to engage and support the Leber congenital amaurosis and inherited rare disease community, advance our development activities, and provide general office management and administration.

RE-BRAND

We undertook one of the most exciting efforts in 2021 — rebranding our organization to Hope in Focus. As Sofia Sees Hope grew from its beginnings, so did its reach. We are an organization that moves adeptly between advocacy and science, keeping the people we serve — the LCA and broader inherited retinal disease community — in focus each step of the way. We are bridging an unnecessary chasm between the people who inform the science and the science that transforms the people. The new name has since been embraced by the community and is powering the next chapter for our organization.

HOPE in FOCUS

Seeing a cure for blindness

BIDDING IN THE DARK

We continued our fundraising efforts in 2021, despite Covid-19 in-person limitations, and raised more than $50,000 in our second annual Bidding in the Dark fundraiser.

NEW BOARD MEMBER

Our Board of Directors appointed David Schulz as a new member. Schulz began his career at Pfizer Central Research in 1989, becoming Executive Director of Central Nervous System Discovery Biology. He also led the Smoking Cessation project, resulting in Chantix. His service to organizations includes the Maina Foundation and the Community Foundation of Eastern Connecticut.

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RESEARCH CONTRIBUTION

As COVID-19 continued into last year, fundraising events still represented a challenge. In 2020 the organization dipped into its reserves for our annual research contribution. In 2021 the board decided to make a smaller contribution with the hopes that a larger one could be made the following year as we return to a level of normality. Despite these trying times, Hope in Focus announced a commitment to raise $300,000 for LCA therapies by 2025.

THE NUMBERS

9
Webinars hosted

3
Publications
(1 magazine & 2 newsletters)

15,394
Impressions on Facebook

1,643
Impressions on Instagram

40K
Impressions on Twitter

21
Stories shared

$35K
Donated to research

A Review of Therapy Development for Leber Congenital Amaurosis

By Ben Shaberman
Senior Director, Scientific Outreach & Community Engagement

FOUNDATION FIGHTING BLINDNESS

Since the Food & Drug Administration's approval of LUXTURNA® for people with Leber congenital amaurosis (LCA) and Retinitis Pigmentosa (RP) caused by *RPE65* mutations, therapy development has expanded for many other forms of LCA.

In addition to the gene- and mutation-specific approaches reviewed here, emerging gene-agnostic therapies (e.g., optogenetics, stem cells) may also benefit people with LCA.

LCA1 (*GUCY2D*): Atsena clinical trial

Atsena Therapeutics is conducting a Phase 1/2 gene-therapy clinical trial for people with LCA1 caused by mutations in the gene *GUCY2D*. Vision improvements were reported for the first three patients treated with a low dose of the emerging therapy.

LCA10 (*CEP290*): Editas Phase 1/2 clinical trial

Editas Medicine reported that two of three participants in the mid-dose group of its BRILLIANCE Phase 1/2 clinical trial for its LCA10 (*CEP290*) CRISPR/Cas9 treatment showed improvements in vision. Known as EDIT-101, the emerging gene-editing therapy was the first of its kind to be administered directly to the human body.

LCA10 (*CEP290*): ProQR Phase 2/3 clinical trial

ProQR Therapeutics, a Dutch RNA therapy development company, reported that its Phase 2/3 ILLUMINATE clinical trial of sepofarsen did not meet its primary endpoint of best corrected visual acuity (BCVA), nor did it meet secondary endpoints, which included mobility. Previously, the company reported vision improvements in the Phase 1/2 trial for sepofarsen. Also, vision improvements were reported for some participants in the Phase 2/3 trial. The emerging RNA therapy was designed for people with LCA10 caused by the mutation p.Cys998X in the *CEP290* gene. The company is further analyzing study results.

LCA10 (*CEP290*): Iveric Bio gene-therapy research

Iveric Bio is developing a gene therapy for people with LCA10 (*CEP290*). Preclinical research for the emerging approach has come out of a lab at the University of Massachusetts. The gene is too large for the viral containers (adeno-associated viruses or AAVs) frequently used in retinal gene therapies, so UMASS has been developing a *CEP290* minigene to fit in the AAV delivery system and express functional protein.

Inherited Retinal Diseases: Opus Genetics' gene-therapy programs

The Retinal Degeneration Fund (RD Fund), the venture philanthropy arm of the Foundation Fighting Blindness, has launched Opus Genetics, a patient-focused, gene-therapy company targeting IRDs. Two of the company's programs are licensed from the University of Pennsylvania and will focus on treatments for gene mutations causing different forms of LCA. One addresses mutations in the LCA5 gene, which encodes the lebercilin protein. The other will focus on restoring protein expression and halting functional deterioration in patients with retinal dystrophy caused by LCA13 (*RDH12*). Opus' third program, licensed from Mass Eye and Ear, is for LCA9 (*NMNAT1*).

LCA6 (*RPGRIP1*): Odylia Therapeutics' emerging gene therapy

A research team from Odylia Therapeutics is developing a gene therapy based on an adeno-associated virus for LCA6 (*RPGRIP1*). Plans include generating a clinical-grade gene-therapy vector for toxicology studies, and ultimately, launching a clinical trial. The Odylia team also identified seven families with *RPGRIP1* mutations. An earlier study showed that gene therapy rescued degenerating rods and cones in a mouse model of the condition.

LCA8 (*CRB1*): Duke University gene-therapy research

Jeremy Kay, PhD, at Duke University, has identified a *CRB1* protein isoform that will potentially work well in a gene therapy for people with LCA8. With Foundation funding, he is evaluating the rescue efficacy and expression pattern of this isoform. His efforts will help *CRB1* gene-therapy developers design the optimal gene therapy for people with *CRB1* mutations.

LCA4 (*AIPL1*): MeiraGTx gene therapy for compassionate use

Mutations in *AIPL1* cause a particularly severe form of LCA. The gene-therapy development company MeiraGTx is providing compassionate use of a gene therapy for this form of the disease known as LCA4. The compassionate use designation can be given to treatments for serious conditions that haven't been approved by regulators. Children with *AIPL1* mutations who are 5 years of age or younger may be eligible for the treatment.

LCA17 *IQCB1/NPHP5*: Penn vet researchers advancing studies

Foundation-funded researchers from the University of Pennsylvania School of Veterinary Medicine improved retinal structure and restored vision in a canine model of LCA caused by mutations in *NPHP5/IQCB1*. The advancement is providing a path forward for translation of the therapy into a clinical trial.

LCA16 (*KCNJ13*): University of Wisconsin–Madison editing therapy

With Foundation funding, Dr. Krishanu Saha is developing a CRISPR/Cas9 gene-editing therapy for LCA16 caused by the W53X mutation in the gene *KCNJ13*. Dr. Saha and his team designed gene-correction nanoparticles proven to be active in correcting the mutation associated with LCA16. Functional rescue of protein activity was demonstrated in retinal pigment epithelial cells. Evaluations are ongoing in mice.

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

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We're Back!

SAVE THE DATE!

DINNER IN THE DARK

Saturday, October 22, 2022

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

Tickets on sale now at hopeinfocus.org

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Mary Ann Kaczmarek
The parents of Betty & Chris Manfre
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Events

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Email rosanne@hopeinfocus.org with the information and a link.

Hope in Focus "Let's Chat About..." webinars

hopeinfocus.org/for-families/lets-chat-about

"**L**et's **C**hat **A**bout..." is a free webinar series hosted by Hope in Focus. We've developed the series with those living with LCA and IRDs in mind, but it is open to anyone interested in what's happening in our communities. Check our website for information on upcoming episodes and recordings of previous sessions. Topics this fall include treatment pricing with Spark Therapeutics, and a look at international support for LCA.

Foundation Fighting Blindness & Microsoft Scramble for Sight Golf Tournament

July 18, 2022
Sedalia, CO
www.fightingblindness.org/events/microsoft-scramble-for-sight-golf-tournament-287

Please join Microsoft and the Foundation Fighting Blindness to help fund valuable research to find a cure for blinding retinal diseases. The urgent mission of the Foundation is to drive the research that will provide preventions, treatments, and cures for people affected by retinitis pigmentosa, age-related macular degeneration, Usher syndrome, and the entire spectrum of retinal degenerative diseases.

Global Genes RARE Patient Advocacy Summit

September 12–15, 2022
San Diego, CA
globalgenes.org/event/rare-patient-advocacy-summit/

Global Genes annually convenes one of the world's largest gatherings of rare disease patients, caregivers, advocates, healthcare professionals, researchers, partners, and allies.

Rare Diseases and Orphan Products Summit 2022 • National Organization for Rare Diseases

October 17–22, 2022
Washington, DC
nordsummit.org

NORD's summit brings together top leaders from the FDA, NIH, patient organizations, industry, payers, and research institutions to address issues of critical importance to the rare disease community. Last year NORD welcomed about 1,000 registrants from 33 countries at its summit.

Dinner in the Dark • Hope in Focus

October 22, 2022
Groton, CT
hopeinfocus.org

Our primary fundraiser for the year, Dinner in the Dark, helps fund research to cure blindness caused by LCA, provide support for genetic testing, and drive awareness, education, and connections for LCA and IRD families. Be prepared for a unique menu, fine wines, and a lively sensory adventure. In person!

Join the LCA Tele-Support Group

lighthouseguild.org

For the last 13 years, social worker Judith Millman, LCSW, has facilitated an LCA tele-support group connecting caregivers and individuals of all ages who are affected by LCA. To join, you can enroll online at **lighthouseguild.org**, or for more information, including upcoming dates for tele-support sessions, please email Judith Millman at judithmillman@aol.com.

Judith Millman

HOPE in FOCUS

P.O. Box 705 | Ledyard, CT 06339

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.

***Seeing Hope* Newsletter Team**
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- Rosanne Smyle, Writer
- Gina Morin, Graphic Designer

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