HOPE in FOCUS
Seeing a cure for blindness
Supporting the LCA and rare retinal disease community

SEEING HOPE | Newsletter

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July 2026 | Issue 30

From the Director

Courtney Coates

Dear Friends,

It is an exciting time for the LCA community. Over the past several months, we've seen remarkable momentum in research, with encouraging updates from clinical trials and new data presented at scientific conferences worldwide. These advancements are a testament to years of dedication from researchers, clinicians, industry partners, and, most importantly, the individuals and families who have participated in research to help advance the field.

As the treatment landscape evolves, Hope in Focus remains committed to providing families with the information, resources, and support needed to navigate these opportunities. Every step forward brings us closer to a future with more treatment options for LCA.

This summer, we invite you to join our Party for Sight fundraiser. Whether you host a gathering, attend an event, or make a donation, your support fuels the programs, partnerships, and research efforts that are driving progress.

We are also looking ahead to our flagship fundraiser, Dinner in the Dark, on October 24. This year, we are offering an inherited retinal disease seminar before the event, in partnership with the Foundation Fighting Blindness, to bring our community together for education, connection, and celebration.

Thank you for being part of this journey. The progress we see today is possible because of you.

With gratitude,

Courtney Coates

Courtney Coates, *Executive Director*

Ashley Hunter and her dog

Gaming Through Adversity: Ashley Hunter's Story

By Marty Dubecky

Growing up means encountering everything for the first time. Adolescents and young adults move through life much like advancing through different levels in a video game; each new setting and challenge offers opportunities to learn and master additional skills. In life, as in video games, abilities are not infinite, but progress is possible. Ashley Hunter, a young adult living with Leber congenital amaurosis (LCA), has always found refuge in video games and knows firsthand the connection between playing them and facing life's challenges.

LIFE LEVEL ONE: DIAGNOSIS

Early in life, Ashley's symptoms were misdiagnosed as neuroblastoma. When her doctors observed nystagmus (roving eye movements), they then thought she might have a cancerous brain tumor. Finding out what was wrong was her first major life challenge. After three years of extensive testing, doctors determined that she didn't have a tumor but instead had LCA.

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Gaming Through Adversity: Ashley Hunter's Story

Continued from page 1

Years later, when she was about 27, genetic testing revealed that Ashley had LCA caused by a mutation in the *CRX* gene, also known as LCA7. This form of LCA is often severe and has an early onset, resulting in significant visual impairment from infancy. It is usually inherited as an autosomal dominant trait, rather than the other LCA subtypes, which are recessive.

Looking back, Ashley and her mother can recall only certain emotions and the general outcome of the early years of testing. "At the time, it was just my mom and me…It was very stressful for her. I don't really [remember] too much about it. When I ask my mom, she doesn't really remember much either," Ashley said. But they do recall that weathering this process was Ashley's first step toward building resilience.

LIFE LEVEL TWO: CHILDHOOD

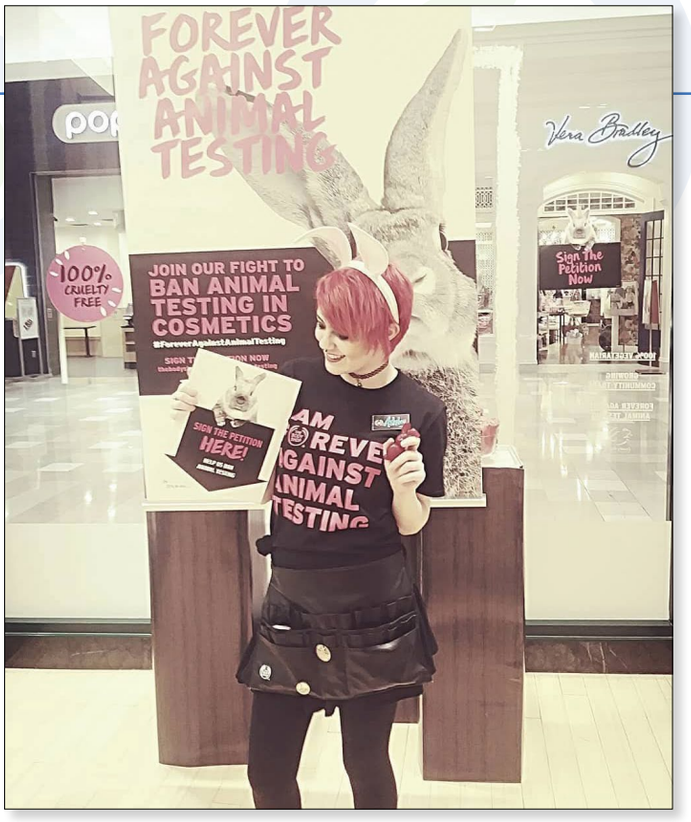
As a young child, Ashley quickly acquired skills. Living near Scottsdale, Arizona, her parents (mother and stepfather) discovered the Foundation for Blind Children (FBC). Through the FBC, as a preschooler, she began learning braille, started mobility and orientation training, and became comfortable with assistive technologies. Ashley's parents knew her vision limitations would be challenging, so they became proactive and supportive of her future endeavors.

In kindergarten, Ashley's vision was limited, but she was still given a certain amount of independence. She wanted to play soccer with the rest of her classmates, and her parents encouraged her to try anything she was drawn to. "All I remember about playing soccer was being hit with the soccer ball many different times," she recalled, laughing.

She quit soccer, but took on new challenges, later trying gymnastics and dance, among other childhood activities. Although none of them worked out in the long term, Ashley credits these early activities with teaching her what she was truly capable of.

LIFE LEVEL THREE: MIDDLE AND HIGH SCHOOL

Most video games feature levels that get harder as the player progresses. Life can follow a similar trajectory. Ashley recalled middle and high school as



Ashley advocating at her retail job

the first time she experienced significant hardships associated with her LCA. The unfortunate reality she had to face was that her vision would only continue to get worse, and seemingly in concert, kids around her got meaner. Though she was surrounded by good friends who understood her abilities and limitations, Ashley also encountered others who took advantage of them. For example, she used large-text textbooks at school, but some classmates hid them and laughed as she struggled to find them.

Ashley attended a few middle school dances but missed out on those in high school. She was unable to participate in gym classes and had to find another option. Even mobility training outside of school became loathsome because of its close proximity to her high school.

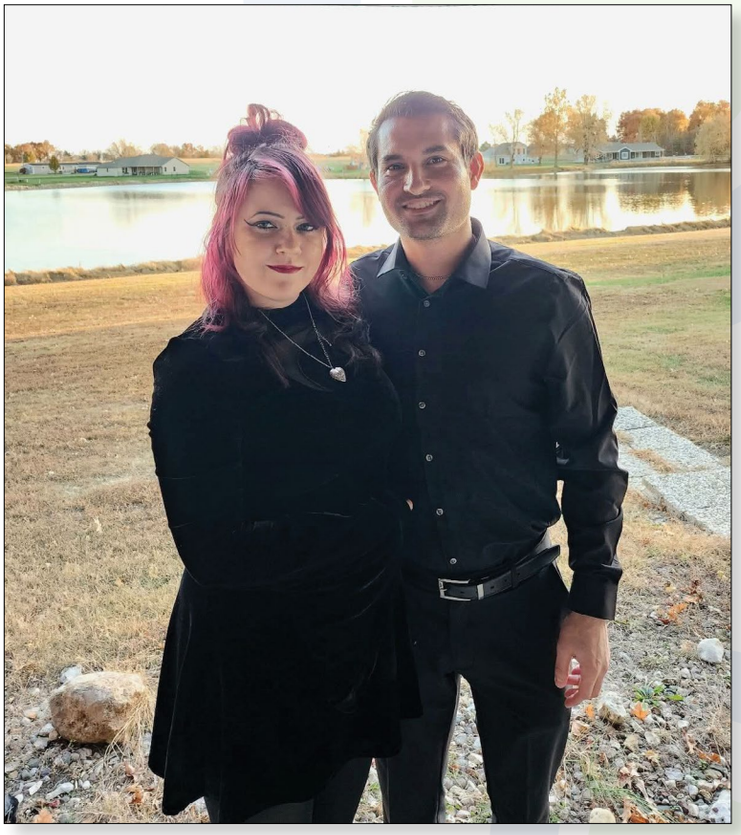
Each of these problems was compounded by constant anxiety. There was the general anxiety that afflicts many teenagers, but also the dread of becoming blind. Despite all of the hurdles, Ashley persevered. She had friends to drive her to places she needed to go. She found weightlifting, a practice that strengthened her body and deepened her relationship with accessible exercise, and fell in love with music. She began attending concerts by her favorite bands and even crowd-surfed, once

making her way onto the stage. When she turned 16, she began working various retail jobs.

Through all of life's hurdles and stresses, Ashley played video games, which became a lifeline for her. They were a bastion of comfort, yet they tested her abilities.

LIFE LEVEL FOUR: ADULTHOOD

Today, Ashley, now age 29, lives in a small town in Missouri with her partner, Mykal, where she is facing new life challenges. She reflected on shifting from big-city life to small-town living. Near Scottsdale, she found that people were more accustomed to seeing or interacting with people with visual impairments. However, her small-town experience was more challenging. For instance, at work, she has learned to deal with customers and coworkers who do not fully understand the difference between legal and total blindness.

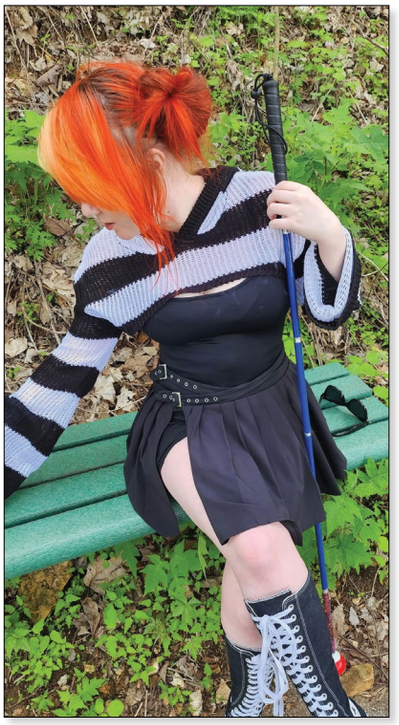


Ashley and partner Mykal Sandoval

"People hear the word blind or legally blind and assume it is just a black or white kind of thing," Ashley said. "It can be frustrating. Especially when I've worked so hard my whole life to be where I am [with] my experience in retail and management, and it is often overlooked because of my visual impairment."

LIFE LEVEL FIVE: VIDEO GAMER

The challenges Ashley faces have been made more difficult by the gradual decrease in her vision. She can no longer navigate independently outside, as bright light has made it more difficult to see, and her nystagmus and peripheral vision are worsening. While circumstances can stand in the way, Ashley won't let them stop her. After all, she still is, and always has been, an avid video gamer, which provides a platform for her to rise to new challenges and perfect new skills.



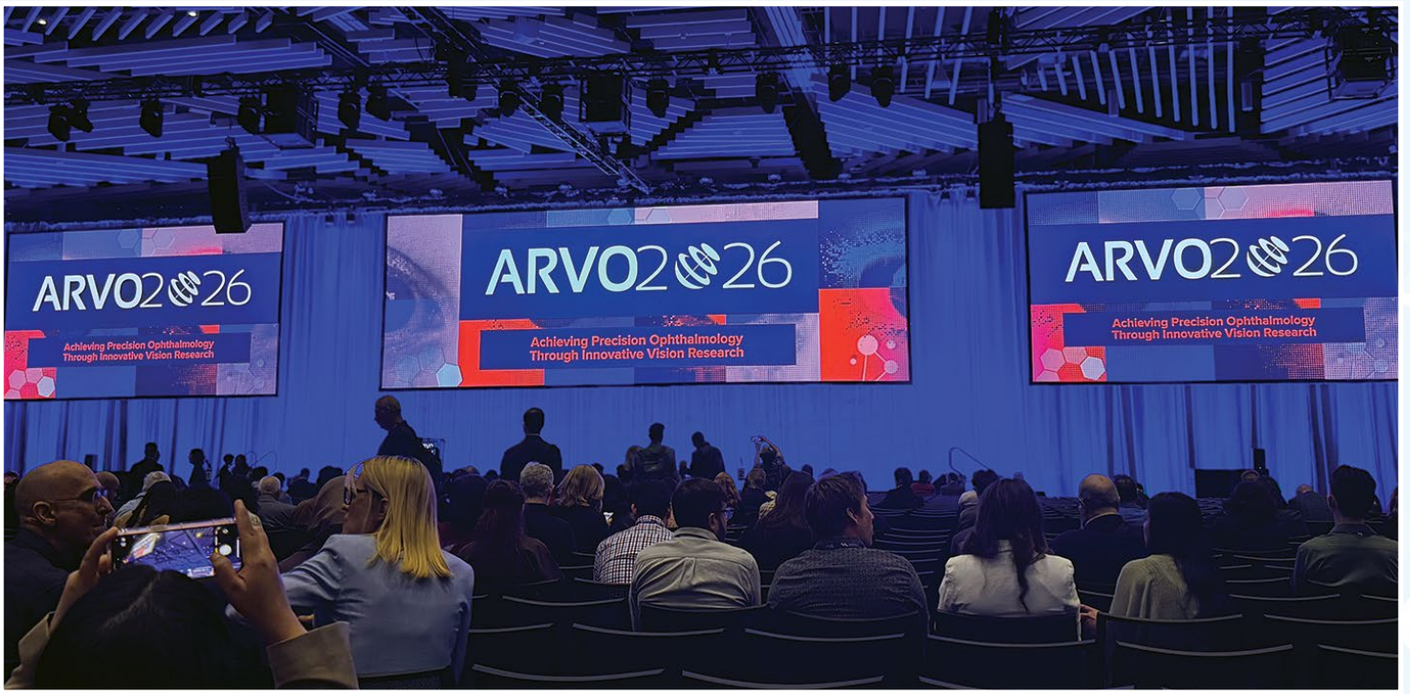
Ashley posed with her cane

Ashley spends her time playing retro PlayStation 2 video games like Jak and Daxter and Crash Bandicoot, as well as more modern games like Fortnite, and has streamed her gaming on the popular streaming site Twitch. She's found a sense of accomplishment in being a talented gamer despite her visual impairment. In fact, she's found a community of visually impaired online gamers.

Video games are a huge part of her life. While Ashley knows it might not be something she can do forever, she takes full advantage of what she can do now. The games are more than an activity. They are, instead, demonstrations of her resilience and persistence. While living with LCA can certainly present challenges, gaming has shown Ashley that it will never stop her from trying anything she sets her mind to. "I feel like my visual impairment and struggles have formed me into the person I am today," Ashley said. "And I like that person! All of this has made me more resilient and adaptive."

If you, or someone you know, has an inherited retinal disease due to mutations in the *CRX* gene, email **info@hopeinfocus.org** to join our contact database so we can give you up-to-date happenings with your gene.

REPORT FROM 2026 ARVO:
Several Emerging LCA Gene Therapies are Restoring Vision



2026 Association for Research in Vision and Ophthalmology opening keynote stage

Ben Shaberman,
Science Communications Advisor
Hope in Focus

In reporting on emerging therapies for LCA and other retinal diseases, my mantra has always been "just the facts." If you're old enough, you might remember those iconic words from the stoic detective Joe Friday on the TV show "Dragnet." Bottom line, I prefer not to embellish my reporting with hyperbole or emotion. (But I don't wear a trench coat.)

With that said, I can't help but be genuinely excited by the news on LCA research that came from the 2026 Association for Research in Vision and Ophthalmology (ARVO) meeting in Denver, May 3–7. Nearly 11,000 research-focused professionals from 72 countries attended this year's meeting. Not all the LCA reports were breaking news, but collectively, the presentations and posters by researchers from all over the globe were inspiring.

Here are some clinical development highlights from the meeting:

LCA13 (*RDH12*): InnoVec's emerging *RDH12* gene therapy performed encouragingly at 12 months in a Phase 1/2 clinical trial in China. Ten patients (three with LCA) received one of two doses. The vision of two patients with LCA went from only hand-motion perception to modest visual acuity. InnoVec is working to launch a trial for the gene therapy in the U.S. Opus Genetics also plans to launch an *RDH12* gene therapy clinical trial in the US by the end of 2026.

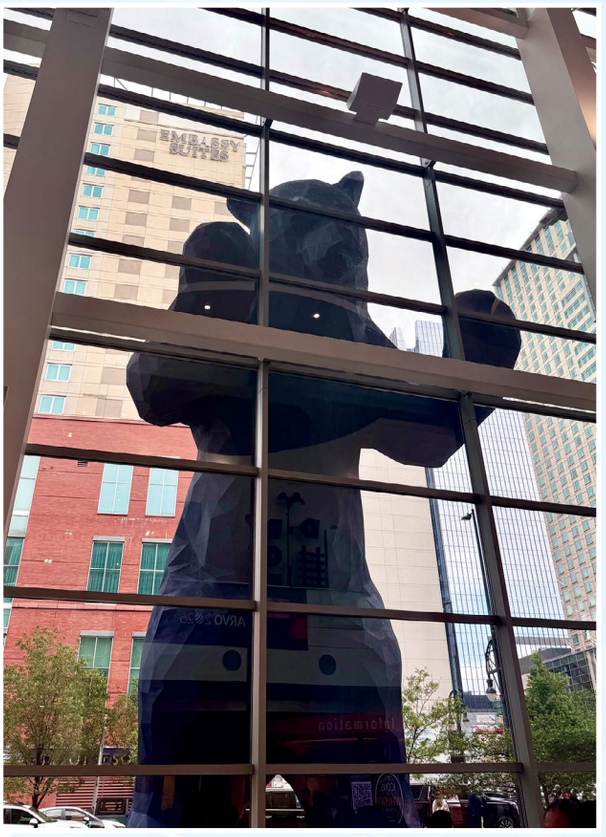
LCA5: Opus Genetics reported meaningful vision improvements in three adult and three pediatric patients (aged 16–17) in its Phase 1/2 LCA5 gene therapy clinical trial at the University of Pennsylvania. LCA5 is one of the rarest and most severe forms of LCA. Some patients saw objects for the first time after treatment. Others had meaningful improvements in visual acuity. Opus is now recruiting for its Phase 3 cohort and plans to dose younger pediatric patients.

LCA4 (*AIPL1*): LCA4 is extremely rare and severe. At an early age, many children have only light perception or hand-motion vision. Incredibly, researchers at Great Ormond Eye Hospital and Moorfields Eye Hospital restored meaningful vision in 11 patients (aged 4 or younger) with an LCA4 gene therapy developed by MeiraGTx in the U.K. under a Specials License. MeiraGTx has licensed the LCA4 gene therapy to Eli Lilly, which is pursuing regulatory approval for it.

LCA2 (*GUCY2D*): Atsena Therapeutics reported 36-month results for its 15-patient Phase 1/2 LCA2 gene therapy clinical trial. Patients receiving the highest dose maintained significant improvements in retinal sensitivity and other functional measures. Furthermore, there were no serious adverse events related to the therapy. The company plans to launch its global Phase 3 cohort in the second half of 2026.

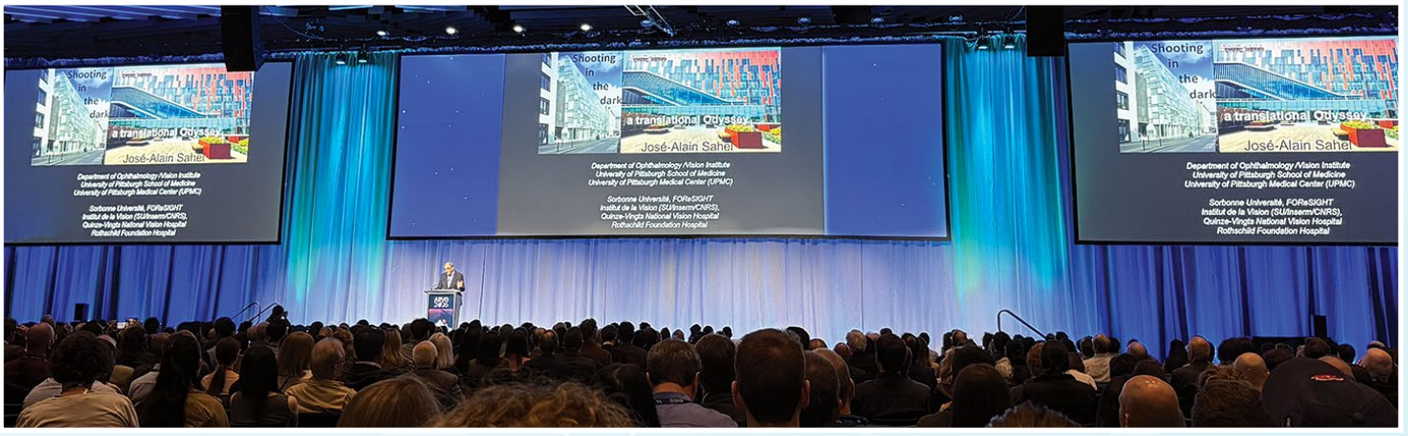
LCA10 (*CEP290*, IVS26 mutation): Sepul Bio continues to recruit for its global Phase 3 HYPERION clinical trial for its RNA therapy, sepofarsen. At ARVO, a post-hoc, paired-eye analysis of the previous Phase 3 ILLUMINATE clinical trial for sepofarsen was presented. It showed that treated eyes performed better than untreated eyes in the same patient. Why is this important? In the original ILLUMINATE clinical trial, the treated eyes of patients were compared with untreated eyes in a different patient cohort (i.e., a control group). Ultimately, ILLUMINATE, completed in 2022, didn't meet its primary endpoint. The good news: HYPERION uses a paired-eye design, which researchers believe can lead to a stronger efficacy signal and a better opportunity to meet the primary endpoint.

This is by no means an exhaustive list of ARVO research presentations on LCA. Please feel free to reach out to me at **ben@hopeinfocus.org** if you have any questions about these or other projects.



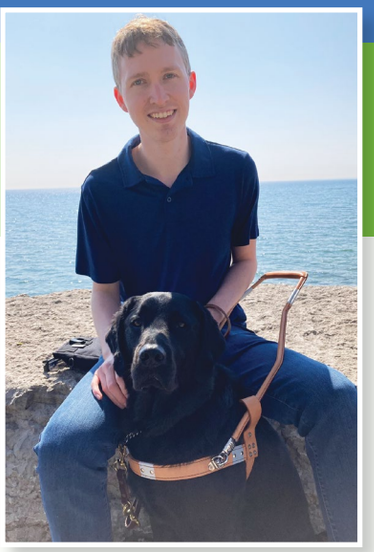
Denver Colorado Convention Center bear that greets attendees

Hope in Focus attendees Courtney Coates, Executive Director and Ben Shaberman, Science Communications Advisor



Dr. José Alain-Sahel receives the 2026 Proctor Medal Award

NAVIGATING LIFE: *Thriving in a Visually Designed World*



Jack and his guide dog, Baloo

As someone with a visual impairment, getting around once felt overwhelming. I was plagued by the "what ifs." What if I got lost? What if I missed a bus or got off at the wrong stop? What if I couldn't find the right platform at the train station? If you or a loved one is dealing with these concerns, this article is for you.

The good news is that over time, navigating has become easy for me. Because of my experiences, I'd like to share some tips for moving through the world with a visual impairment.

Step 1: Orientation & Mobility
It is important to learn how to use a cane and practice using it every day. Many people with vision loss live in denial of their visual impairment. If you're not currently using a cane or a guide dog and have recently bumped into something you didn't see, start using one.

Once you've mastered the basics of the cane, learn to navigate independently in your immediate neighborhood. (Parents, this is when you need to step back.) Have a friend or family member walk beside you the first time, pointing out obstacles and naming streets as you cross them. When starting out, it can be helpful to count how many streets you cross before you need to turn. Consider making notes on your phone to help you remember. For example, "To get to the doctor's office, turn right out of my house, turn left at the first intersection, turn left after walking under the train bridge, and cross two streets before turning right. The doctor's office is the first building on the left."

If you are nervous the first time you try the route alone, have someone walk behind you and ask them not to talk to you unless you get completely lost. It is okay to get a bit lost! Part of becoming a better navigator is making mistakes. You can also video call a friend or family member if you need help.

Step 2: Generalization
Eventually, you will start to notice patterns such as how buildings are spaced, that street addresses with odd numbers are usually on the east or south sides of the street, and the typical layout of train stations, etc.

At this point, you won't need someone's help traveling a route for the first time. Instead, you will use the tools available to you—reviewing routes before you leave your home, leveraging GPS along the way, and not being afraid to ask someone to confirm your location or that you are at the correct building.

Step 3: Challenge Yourself
When I was in my second year of university, I was invited to attend an event to receive a scholarship I'd won. But it was two hours away. I called my mom to ask for a ride. She lived an hour from me in the opposite direction from the event. She said that if I wanted to go, I'd need to figure out how to use the bus on my own. It was one of her best parenting decisions.

I was terrified and had to ask a lot of people for help along the way, but I did it. It was an eye-opening experience and set the wheels in motion for me to feel comfortable traveling. In previous articles, I've talked about hiking in the mountains and trekking up Mount Kilimanjaro. These amazing experiences wouldn't have been possible without a lot of experience negotiating daily life with my visual impairment. With enough practice, I am confident that you can reach a point where you can travel as easily as I do.

Jack McCormick is a human resources professional working in the tech sector. He was diagnosed in high school with LCA2 (RPE65). Jack is a Hope in Focus ambassador, helping people living with LCA and IRDs. Learn more about him on his LinkedIn profile by scanning the QR code.

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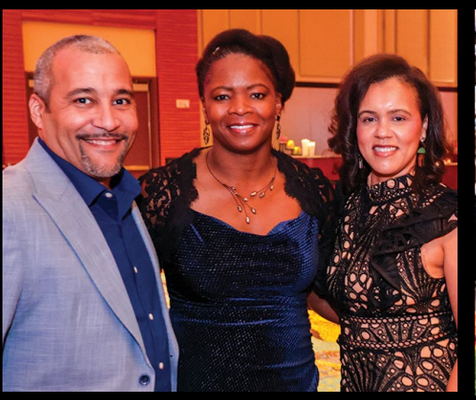

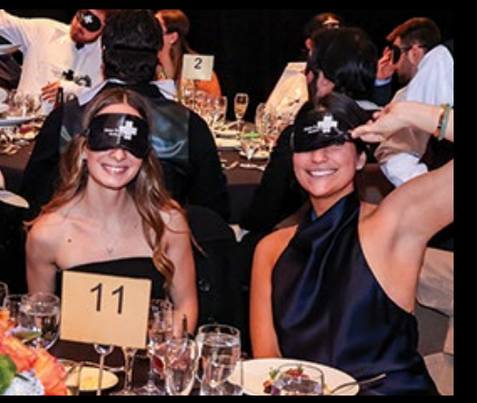

DINNER IN THE DARK

Saturday, October 24, 2026

Join us for a unique culinary experience

Foxwoods Resort Casino
Mashantucket, CT

5:30 PM – 7:00 PM Cocktails
7:00 PM Dinner, Auction & Dancing

IN ADDITION...

Register for the **Free Vision Connection** beforehand
from 1:00 PM – 3:00 PM at Foxwoods Resort Casino
Includes light snacks & networking after the Seminar

Photos by: PubliCreatives

Scan code or visit hopeinfocus.org for more info, tickets and sponsorships

Events

DO YOU HAVE AN EVENT YOU WANT TO SHARE? LET US KNOW!
Email info@hopeinfocus.org with the information and a link.

Dinner in the Dark

October 24, 2026 • Foxwoods Resort Casino, Mashantucket, CT
hopeinfocus.org/events/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!

NORD Breakthrough Summit

October 25–27, 2026 • Washington, DC
nordsummit.org

Shape the future of rare disease treatments, research, and policy at the NORD® Rare Diseases and Orphan Products Breakthrough Summit®. Together, we can advance innovation for the more than 30 million Americans—and more than 400 million people worldwide—with rare diseases!

VisionWalk — Foundation Fighting Blindness

www.fightingblindness.org/visionwalk

Since its inception in the Spring of 2006, VisionWalk has raised over $71 million to fund sight-saving research. Join a VisionWalk in your community! Together, we step closer to fighting blinding diseases.

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.

Learn more at hopeinfocus.org

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