

THE CYSTINOSIS Advocate

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CRN at the American Society of Nephrology Conference, in San Diego in November 2006.

CRN CELEBRATES 30 YEARS! 1996-2026

**30 years of community,
30 years of connection.**
(See more on [Page 3](#))



Join us in Charlotte, NC, for the 2027 CRN Family Conference, July 22-24. More details soon at cystinosis.org.

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The President's Letter

Dear Cystinosis Community,

There are seasons in life that have a way of bringing everything into sharper focus.

For my family, this past year has certainly been one of them. Our daughter, Elle, received a new kidney last summer after reaching kidney failure, and a few months later, I had the opportunity to become a living kidney donor myself. I share that not because our story is unique within this community—in many ways, it isn't—but because living through these experiences has reinforced something I have believed throughout my time with CRN: the work we do matters because of the lives behind it.

Every research grant, every conversation with a policymaker, every family connection, every educational resource, and every dollar raised ultimately comes back to people. To children growing up with cystinosis. To adults navigating a disease they have managed for decades. To siblings, parents, caregivers, researchers, physicians, and advocates who have all become part of this community.

And there is a lot happening.

Over the past year, CRN has continued expanding the ways we serve and connect our community. We have strengthened relationships with researchers and clinicians, continued our advocacy efforts, supported families through established programs and regional gatherings, and worked to ensure that the patient and family voice remains present wherever decisions about cystinosis are being made.

Our work is also becoming increasingly global.



Jonathan Dicks, at the 5th CNE conference held in Dublin, Ireland in July.

This summer, I had the opportunity to represent CRN at the CNE International Conference in Dublin. Spending time with cystinosis organizations, researchers, clinicians, patients, and families from around the world reinforced just how important collaboration will be to the future of this disease. With a patient population as small as ours, progress cannot happen in silos. We have to learn from one another, share ideas, challenge one another, and find opportunities to work together.

That same thinking is guiding our approach to research.

As we look toward the next chapter of CRN's research program, we are thinking carefully about how we can support meaningful basic, translational, and clinical research while also creating stronger connections between investigators and the community they serve. Funding research remains central to who we are, but so does making that science understandable and

The President's Letter, continued

accessible to families. Patients should not simply be the beneficiaries of research—we should be partners in helping shape its future.

At the same time, we cannot lose sight of the everyday realities of living with cystinosis.

Sometimes progress looks like a laboratory discovery or a clinical trial. Other times, it looks like helping a family attend a conference, connecting a newly diagnosed parent with someone who understands, supporting a student through a scholarship, helping an adult patient find resources, or bringing families together who previously thought they were navigating this disease alone.

Both matter.

One of the great privileges of serving as President of CRN has been watching this organization continue to evolve while remaining rooted in the same purpose: improving the lives of people affected by cystinosis and relentlessly pursuing a better future.

I am incredibly grateful to our

families, volunteers, Board members, researchers, clinicians, industry partners, donors, and advocates who continue to make that possible. CRN has never been the work of one person or even one Board. It is the collective effort of a community that refuses to accept that rare should mean forgotten.

My family's experiences over the past year have only strengthened my resolve.

A kidney transplant is extraordinary. A living donation is extraordinary. But neither is a cure for cystinosis.

That distinction matters.

We can be profoundly grateful for how far medicine has brought us while remaining completely unwilling to settle for where we are today. We can celebrate longer lives, better treatments, successful transplants, and scientific progress while continuing to ask what comes next.

And we will.

There is tremendous reason for hope in this community—not passive hope,

but the kind built through research, advocacy, collaboration, generosity, and people willing to keep showing up for one another.

Thank you for continuing to show up.

Together, we keep moving forward.

Jonathan Dicks
President | VP Development
Cystinosis Research Network



Jonathan Dicks with Sanjay Shewakaramani, co-owner at Revive Strength and Wellness, at a national Multiple Sclerosis walk in Cincinnati.

CRN Celebrates 30 Years

By Terri Schleuder, Director

2026 marks a significant milestone for the cystinosis community, 30 years since the founding of the Cystinosis Research Network. CRN was started in 1996 by a small group of dedicated parents whose goal was better treatments and a better quality of life for their children affected by this rare disease.

Establishing a vision for the acceleration of the discovery of

a cure, development of improved treatments, and a mission of providing resources for family support, while educating the public and medical profession about this rare disease became the goal.

Since then, CRN has fulfilled its purpose by hosting 12 biennial family conferences to bring families, doctors, researchers and industry partners together to learn and connect. Our

13th will be in Charlotte, NC in July 2027.

Over the last 30 years CRN has funded numerous research studies to improve the lives of those affected by cystinosis and continues to do so. They have attended many medical meetings such as the American Society of Nephrology, (ASN), and the Pediatric Academic Society, (PAS) to educate those doctors in

CRN Celebrates 30 Years, continued

attendance about this disease. They have also brought the needs of the rare disease community to our policy makers on Capitol Hill each February by attending Rare Disease Week in Washington, D.C.

CRN has provided numerous programs and resources to support the cystinosis community. This

includes a biannual newsletter, dedicated social media sites, and a website filled with information and resources. Over the years CRN has offered academic scholarships, the Cystinosis Care Package Program, the Cystinosis Warrior Impact Program, the Cystinosis Memorial Fund, the Live Like Laura Fun Fund and so many more. Please check our website

at cystinosis.org for details on each of these.

We look forward to many more years of advocacy, education and support for our community. It takes a village with a dedicated purpose, and heart to serve. Thank you CRN for continuing to be a beacon of light in the darkness that has helped to improve so many lives.

What's Your 57?

Our 9th Cystinosis Awareness Day campaign on May 7th provided an opportunity for 57 different individuals or families impacted by cystinosis from around the world to come together to share their stories while educating others about this rare disease. Thank you to everyone who offered their "57" and to all who shared posts, amplifying our rare perspectives across the GLOBE! It was an awareness opportunity and fundraising campaign that surpassed our \$5,700 goal. Check out our Facebook and Instagram pages for more stories.



WHAT'S YOUR 57?

"Our motivation is to spread awareness and support funding for Carson and those living with Cystinosis." - Emily



 **Cystinosis Awareness Day**
May 7 

WHAT'S YOUR 57?

"My dad...he was so inspired by us and our experiences he donated his kidney in March 2020." - Ava



 **Cystinosis Awareness Day**
May 7 

Your Story Matters: Finding Your Voice to Advocate for Cystinosis

Sisters Lucy and Sam, both living with cystinosis, were invited by Cystinosis Network Europe to take part in media interviews leading up to their international summer event in Dublin. What started out promoting the conference became an opportunity to raise awareness, educate others, and turn lived experience into understanding for those outside the rare disease community.

Lucy's advocacy work evolved through a social media management position at Cystinosis Ireland. When the chance came to share her story, she decided to step outside her comfort zone. *"I was nervous at first, but after doing a couple of interviews, I felt it was going smoothly and I was sharing my story well,"* she said.

For both sisters, the interviews opened the door to conversations that people outside of cystinosis rarely get to hear. For Sam, that meant talking about organ donation - a topic she feels doesn't get enough attention. Most diagnosed with nephropathic cystinosis will need one, if not multiple, kidney transplants in their lifetime. *"I find it important to talk about organ/kidney donation, as not everyone realizes you can donate a kidney and still live with one! So I made sure to mention that part of our cystinosis journey,"* she said.

For Lucy, it was about discussing a harder-to-see reality: that a rare disease isn't always visible. *"I definitely think it helps to educate people and show them how rare diseases can affect people on a day-to-day basis; especially when on the outside I might look healthy,"* she said.

The impact of their interviews reached far beyond the studio with one TikTok video gaining over 20,000 views. *"It's really important to raise your voice on awareness and the struggles and triumphs we go through with this rare disease, to help others too,"* Sam said.

But for her, the value of speaking out isn't measured in views, it's what someone takes away from hearing a real person's experience. *"I hope people learn something, whether it's helping a friend who has a chronic condition or helping a new parent with a newly diagnosed baby,"* Sam said.

Both sisters are clear about one thing: advocacy doesn't require sharing everything, and it doesn't have to happen before someone feels ready.

"It [sharing your story] can be difficult, but it's important to have our voices heard," Sam said. *"Take it at your own pace, as it can be triggering sometimes to talk about the negative side of things."*

Lucy's advice for anyone who has thought about sharing their story but isn't sure they're ready: *"I would say you deserve to speak about your story and raise awareness."*

Lucy and Sam's experiences show that advocacy creates connections far beyond the storyteller. A conversation can educate a healthcare professional. A social media post can reach thousands. And sharing what life with cystinosis actually looks like may help someone recognize the condition in a way a medical description never could.



Lucy and Sam interviewed by Midwest Radio ahead of the Dublin, Ireland Cystinosis conference.

“No matter how small, your story matters.”

- Lucy



Beckwith sisters, Lucy and Sam.

Across Borders, One Cystinosis Community

By Jonathan Dicks, CRN President, and Vice President of Development

This summer, I had the privilege of representing the Cystinosis Research Network at the 2026 CNE International Conference in Dublin, Ireland. Gatherings like this are always a reminder that while cystinosis is incredibly rare, the community surrounding it is anything but small.

The conference brought together patients, families, researchers, clinicians, and advocacy organizations from across Europe and around the world. It was an opportunity to hear about advances in cystinosis research and care, but equally important, to spend time together exchanging ideas, comparing experiences, and strengthening relationships across borders.

I was proud to share the work of CRN and the incredible community behind it. Our message in Dublin centered around a simple idea: Advancing Science. Connecting People. For CRN, those two things have always gone hand in hand. Research creates hope for better treatments and, ultimately, a cure—but relationships are often what allow that research, education, and advocacy to move forward.

One of my biggest takeaways from Dublin was how much we can learn from one another. Different countries approach patient education, transition of care, research, family support, and advocacy in different ways. Some of the most valuable conversations happened outside of the formal presentations—hearing what another organization is trying, discovering a program that could be adapted for

families in the United States, or simply realizing that another community halfway around the world is wrestling with many of the same challenges we are.

For a disease as rare as cystinosis, international collaboration isn't a luxury. It's a necessity. No single organization, researcher, clinic, or country has enough patients, resources, or answers to tackle this disease alone. Progress happens faster when we share what we know.

I returned home from Dublin with new ideas, stronger relationships, and an even greater appreciation for the global cystinosis community.

CRN has an important role to play in that community—not only supporting families here in the United States, but helping connect people, organizations, clinicians, and researchers around a shared purpose.

Our languages, healthcare systems, and approaches may differ, but the goal doesn't.

One global community. One shared mission. One future without cystinosis.

Go raibh míle maith agaibh — a thousand thanks — to Cystinosis Ireland, Cystinosis Network Europe, and everyone who made Dublin such a meaningful gathering.



Jonathan Dicks with Leslie King, Emily Mello, Heidi Hughes, Irati Vilariño, and Carl Schleuder at the 2026 CNE Conference in Dublin.

The CNE conference meant something different to everyone who attended. A few attendees shared what stood out to them. Continue reading on the next page to hear their reflections.

CNE International Conference 2026: Reflections from the Cystinosis Community



Some of the attendees of the 5th CNE Conference held in Dublin, Ireland, July 2-4.

“ I think many people already know this, but it’s worth remembering that developing new treatments takes time, and patient safety must always come first. While progress can sometimes feel slow, there is real momentum in cystinosis research, and it’s encouraging to see promising discoveries steadily moving closer to the clinic. ”

- Heidi Hughes

“ After my second kidney transplant and recently turning 40, I made a promise to myself: to experience life fully and prioritize my health so I can thrive. When the idea came to attend the European Cystinosis Conference in Ireland, I immediately thought, ‘Sign me up!’ It meant a new stamp in my passport, the chance to meet new friends and families, reunite with lifelong friends from overseas, and celebrate the wedding of Freek and Jolien. More than anything, the promise of abundant memories and meaningful connections motivated me to make the trip a reality. This fortunately included a mini road trip through the beautiful Irish countryside - driving in the left lane! - with my best friend and cysta, Emily Mello. ”

- Heidi Hughes

“ What gave me the most hope is the progression of new treatments. In particular, the CF10 prodrug and the contact lens. These have both been discussed at cystinosis meetings for many years, and while I know patients and families wish these advances could happen faster, as a scientist to see these ideas develop from early concepts to now clinical trials is amazing. ”

- Dr Jennifer Hollywood



Best friends and “cystas” Emily and Heidi.

Reflections from the Cystinosis Community, *continued*

“ My biggest takeaway was how incredible this community really is. Having attended these conferences over the past few years, I’ve gotten to know many of the patients and families, and it’s always wonderful to see them again. There aren’t many areas of research like cystinosis where scientists have the opportunity to present their findings directly to the people their work is intended to help. It can be both confronting and incredibly rewarding, and it reminds me why this research matters so much. ”

- Dr Jennifer Hollywood

“ I love all the educational information and the updates...but, the really meaningful part of cystinosis conferences, for me, is connecting to people with shared life experiences and other patients, like me, who have cystinosis. ”

- Steve Schleuder

“ One of the things about having a rare disease...is that when you find people who ‘get’ what you’re going through, you don’t care where they come from. You just care that they exist. ”

- Steve Schleuder



Koji and Kento Maeda, from the Japan Cystinosis Patients and Families Association, visit with Steve and Carl Schleuder from Michigan, USA.

“ If you have the opportunity to attend a cystinosis gathering, go. Listen, share, connect, and allow yourself to belong. Immerse yourself in others’ stories and share your own when you are ready. Seeing that there is a cystinosis family around the world brings a deep sense of comfort and belonging. We may be rare, but together we are strong and unstoppable. ”

- Heidi Hughes

Community Update: First Patient Treated in Phase I/II CYStem Clinical Trial in Nephropathic Cystinosis

July 24, 2026

Dear Cystinosis Community,

We are pleased to share an important update on CYStem, the Phase I/II clinical trial evaluating the safety and efficacy of the investigational therapy DFT383 in pediatric participants with cystinosis. The first participant has now been treated. As specified in the study protocol, the Data Monitoring Committee (an independent group of experts that reviews study data to protect participants' safety) has reviewed available study information and recommended that the study may proceed as planned. We would like to thank everyone involved for all their efforts and dedication in reaching this important milestone.

About the CYStem Study

CYStem is an open-label, multi-center, Phase I/II study to assess safety, tolerability and efficacy of DFT383 in pediatric participants with nephropathic cystinosis [Study Details | DFT383 in Pediatric Participants With Nephropathic Cystinosis | ClinicalTrials.gov](#)

The purpose of the study is to investigate if DFT383 is safe and effective in children aged 2-5 years who have nephropathic cystinosis. DFT383 is a hematopoietic stem cell-based gene therapy. This study includes an active treatment group (Cohort 1), where participants will receive DFT383, and a concurrent reference group (Cohort 0). Participants in Cohort 0 will not receive study treatment and will continue receiving standard of care (i.e. cysteamine). The study is not randomized. Data from the reference group will help better understand the disease and the results observed in the children receiving DFT383. Investigational sites may enroll participants in one or both cohorts.

We understand the significant impact that cystinosis has on individuals and families. Research studies like CYStem are only possible because of the commitment of the cystinosis community. We are deeply grateful to everyone who contributes to advancing research, whether through participation, advocacy, or support.

Novartis remains committed to transparency, collaboration with the cystinosis community, and sharing updates as development continues. We encourage anyone who is interested to review the study information posted on [ClinicalTrials.gov](#) and to speak to their healthcare provider.

Kind regards,

The Novartis CYStem Cystinosis Study Team

Novartis Pharmaceuticals
1-888-NOW-NOVA (1-888-669-6682)
novartis.email@novartis.com

FA-11737894

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Midwest Cystinosis Family Gathering, 2026

By Doretta Hoffmann



Attendees of the 2026 midwest gathering of cystinosis families.

The Midwest Cystinosis families came together in Mankato, Minnesota, from July 31st through August 2nd for a meaningful and memorable gathering hosted by Brent & Holly Erdman and their family.

The weekend opened with a birthday celebration for Nick Wagner, complete with pizza, delicious pie, and a round of happy birthday singing that set the tone for the days ahead.

A highlight of the gathering was a thoughtful and informative recap from Rebekah Palmer about the Next Generation of Cystinosis support group. Families shared openly about where they are in their cystinosis journeys, creating space for connection, understanding, and encouragement.

One of the most valuable conversations centered on muscle wasting in adults with cystinosis, a topic explored in depth. The shared experiences and insights were deeply appreciated and beneficial for

everyone present.

The Midwest families expressed sincere gratitude for the financial support that made attendance possible. This assistance ensured that families could come together, learn

from one another, and strengthen their community bonds.

With connections strengthened, the group looks forward to gathering again in 2027.



The adults living with cystinosis in attendance – left to right – Briana Dundon, Rebekah Palmer, Jana Healy, Sara Healy, Shawn Reuter, and Nick Wagner.

Education & Awareness Update

By Marybeth Krummenacker, Vice President of Education & Awareness

As I begin writing this CRN Newsletter update, I find myself reflecting on the last 37 years... I would not be here today without the cystinosis community. CRN has been referred to as the “gold standard” of rare disease non-profits. I am so proud of all we have done and continue to do.

I have been a part of this organization since day one. As I think of finishing my term as Vice President of Education and Awareness this coming year, I am extraordinarily proud of what we have accomplished and continue to accomplish.

Cystinosis Research Network began with a small group of parents who knew we had a voice, but needed to figure out how to use that voice to make people listen. We networked with the umbrella organization,



Laura with her goddaughters, niece, Parker, on the left, and cousin Maeve on the right.



Laura, Marybeth with 95 year old Aunt Claire.

National Organization for Rare Disorders (NORD). As we did, so we learned and we listened.

My most vivid memory is listening to NORD founder, Abbey Meyers, tell her story by saying “I’m just a grandmother from Connecticut.” Well, that grandmother was the driving force in getting the Orphan Drug Act of 1983 passed. Without that piece of legislation there would have been no approved cystinosis medication... Cystagon. Abbey’s words continue to inspire me personally. We showed up at NORD’s Annual Meetings and took our time to tell our stories. We always represented the cystinosis community with respect for everyone. We needed a VOICE. We were the stories that needed to be told, and we told them. We continue to tell those stories.

We are looking forward to representing CRN at the upcoming NORD Summit in Washington D.C. In 2027 we plan to attend The World

Lysosomal Symposium in California. In addition, Rare Disease Week in early March of 2027 will provide another opportunity to tell our stories. These meetings lead up to Cystinosis Awareness Day on May 7th and will be followed by our CRN Family Conference in July 2027, in Charlotte, North Carolina!

With our hard work through the years, I wanted to share some photos of the reason why I do what I do... my daughter Laura. I was told when she was diagnosed 37 years ago that she may not live a long life or that her life would be filled with medications and doctors. While that has been true, we have been extraordinarily blessed with brilliant doctors. I thank God she was able to get such wonderful care, and the medications she needed. She has defied everyone’s expectations and will be 41 years old in February. She is a 27 year kidney transplant survivor and 9 year breast cancer



Laura speaking at a breast cancer fundraiser for Team Komen, supported by her friends Jacklyn, Carmen, and Kristen.

Education and Awareness Update, *continued*

survivor. I tried to teach her not to “hide” her illnesses or her medications, and that cystinosis does not control her life; it fits into her life. Laura has learned she can do whatever she wants, as long as she takes her medicine, and she has! Maybe not always perfectly, but she did the best she could on any given day. She is blessed in many

ways and in an odd sort of way, cystinosis is one of those blessings. She has met incredible people along the way and learned how to advocate for herself. She has learned from her community how to overcome anything. Hopefully she has been an example to the community of strength, optimism and above all love!



Laura with her nephew, Finley.

CYSTINOSIS WARRIOR IMPACT PROGRAM



The Cystinosis Warrior Impact Program (CWIP) was designed with an ambitious goal: to positively impact each person living with cystinosis through mental health support, financial assistance (scholarships), medical resources (support programs), education, or connection to critical networks. We encourage you to reach out today to learn how CWIP can support you or someone you know.

How to get help or get involved:



Contact us. Email info@cystinosis.org and we can confidentially discuss how to support your needs.



Referrals. Do you know a cystinosis warrior in need? Provide them with our email to get started.



Resources. Know of a great program or resource the cystinosis community qualifies for? Send it our way and we can expand the cystinosis resource library.



Share. Help us spread the word to reach even more cystinosis warriors.

“ Thank you for being a source of comfort. ”

“ THANK YOU for introducing us to this doctor, he has been such a dream to our family and [our daughter's] health. ”

“ It literally doesn't matter what I need, I know I can call on you at any time. I still remember our first conference spent together in Philadelphia. The CRN has been with us every step of the way. ”

“ Thank you to the Cystinosis Research Network for making the connection. These moments matter. And to anyone in the community who has a story to tell - share it, however you can. Your voice could change someone's path - or even save a life. ”

Introducing our 2026 Academic Scholarship Winners

By Gail Potts, Director

CRN is proud to announce the winners of the 2026 Academic Scholarships. This year we had two excellent candidates for the Sierra Woodward Sibling Scholarship. We awarded Tessa Tisdale and Jordan Sextone \$2,000 each. Our award for the CRN Academic Scholarship for an Individual with Cystinosis went to Yassely Suazo Solis. The Deanna Lynn Potts Scholarship award went to Josie Sexstone. These two young ladies also received \$2,000 each.



Tessa Tisdale, one of two recipients of the Sierra Woodward Sibling Academic Scholarship.

Tessa Tisdale will be attending Bradley University, in Peoria, IL. She is the sister of Rylee, a 16-year-old, who was recently diagnosed with cystinosis. This has made a big impact on Tessa's life. Cystinosis shifted her perspective on priorities of faith, family, academics and balancing multiple jobs. Tessa is pursuing a civil engineering career. She attended Illinois Central College before transferring to Bradley University. Her professors there complimented her on her ability to communicate with teachers and peers alike. Tessa is

noted as a student with motivation, time management and leadership skills. She has shown high academic achievement. She has been involved in the American Society of Civil Engineers, student chapter. Tessa did a summer internship as a structural engineering intern, which solidified her career direction. Her professors highly recommend her for this scholarship.



Jordan Sextone is our second Sierra Woodward Sibling Academic Scholarship winner.

Jordan Sextone completed her Bachelor of Science in Forensic Chemistry and Pre-Med AOE in Global Health in Biology at West Virginia University in May 2026. She will now continue her graduate studies at the same university. She comes highly recommended by her professors. Her primary research in her undergraduate program involved the response of a protozoan to fungi that differed in toxic ergot alkaloid content. Her results were presented at two national conferences and published in a peer-reviewed scientific journal. This is a rare accomplishment for an undergraduate student. She is strongly committed to her education.



Yassely Suazo Solis, this year's winner of the CRN Academic Scholarship for an Individual with Cystinosis.

The CRN Academic Scholarship for an Individual with Cystinosis was awarded to Yassely Suazo Solis. She is attending the University of St. Thomas in Houston, Texas. Yassely is studying Criminology and Criminal Justice with a focus on becoming a detective. Yassely's desire to be a detective is a career she feels will allow her to help people who are ignored or unheard in society. She wants to help the victims of injustice. She was diagnosed with cystinosis at 7 years of age, the first known case in Honduras. Her Mom was her rock in many ways, that helped to shape her attitude and fortitude. The recommendations from her former professors described her as an individual with drive and determination. She carries herself with honor, dedication, and integrity as expressed by one of her professors. The professors considered her as a strong candidate for this award.

The Deanna Lynn Potts Scholarship was awarded to Josie Sexstone, who will be attending the University of Cincinnati College of Design, Architecture, Art and Planning in Cleveland, Ohio. She plans to study

2026 Academic Scholarship Winners, *continued*

Communication Design. Josie wants to use design to create spaces like ones that comforted her at Cincinnati Children's Hospital. "Cystinosis has taught me a valuable lesson that I cannot control my circumstances, only how I react to them". Her letters of recommendation from her teachers were glowing. Josie is described by her teachers as quiet, motivated, dependable, and exhibits a strong work ethic in the classroom. She was an active member of the National Honor Society, Key Club, and Student



Josie Sextone is this year's Deanna Lynn Potts Academic Scholarship winner.

Council. She received the strongest recommendations for this award.

Living with cystinosis and still being able to achieve academic, social, and behavior successes is truly commendable as stated by one teacher. That comment sums up the resilience of our cystinosis warriors, which is exemplified by our yearly scholarship recipients. It is with great pleasure that we award these scholarships this year and wish our recipients much success in meeting their goals.



By Frankie McGinnis, Director

The Live Like Laura Fun Fund was established in memory of Laura McGinnis, to allow those who live with cystinosis to experience life's adventures and fun as Laura loved to do. Learn more about the fund and how to apply at cystinosis.org/llff.

March 23, 2026, would have been Laura's 30th birthday had she lived. Even with her gone it felt like a moment that needed to be acknowledged. So that is what we did. To recognize the significance of the month we decided to give a birthday award from the Live Like Laura Fun Fund to an applicant. Usually, we don't get bombarded with applications, but this brought in several requests. I have explained before, this award is two-fold, yes, it gives someone funds to go for an adventure, but it also gives my pea brain something positive to focus on. We had 6 very different requests come in, and it was such a hard call on who should get the special award. I will not spoil their story that they will share in a later newsletter, just suffice to say it ended up with someone who meant a lot to my family in our journey with cystinosis. Awarding them the funds for their adventure was such an honor. I say often how lucky I am to have found this wonderful group of people 29 years ago, and even with my person gone my love for this community is unending—this is family.

A Night to Remember: Jaxon's Live Like Laura Fun Fund Experience

By Chelsea Meschke, Vice President of Family Support

Our family was honored to be chosen as recipients of one of the Live Like Laura Fun Fund scholarships, and we couldn't be more grateful for the opportunity it gave our son, Jaxon.

Since he was little, Jaxon has had a love for music. From playing drums on pots and pans and grabbing every microphone within reach, to strumming both toy and real guitars, music has always been a natural part of who he is. Singing and dancing came just as naturally as trying to play the music himself.

So, when we had the opportunity to see one of Jaxon's favorite singers, Morgan Wallen, in concert, we knew this would be an experience he would never forget. But this wasn't just any concert—it was at one of the most iconic venues in our home state, The Big House, home of the University of Michigan football team.

Although Jaxon was a little nervous at first, the moment the music started, everything changed. Once he realized that the feeling of the bass vibrating through his chest was perfectly normal—and would be part of the experience for the next two hours—he was on his feet! He sang his heart out, jumped up and down, and knew the words to every song he could.

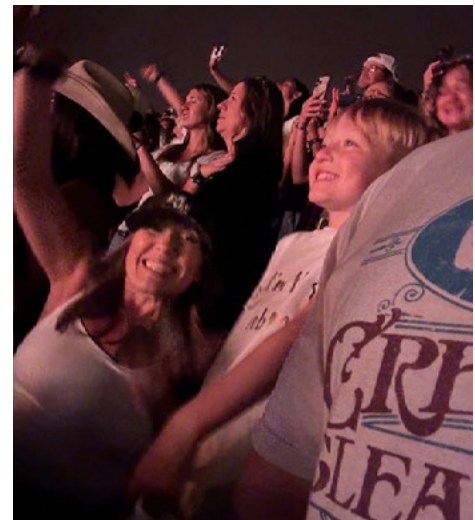
The smiles on our faces throughout the night were a direct reflection of the huge smile on Jaxon's face. Watching him experience something he loves so much was an absolute joy. There is something incredibly special about seeing a memorable moment through your child's eyes, and this was a night we will cherish as a family for years to come.



Jaxon, getting ready to enjoy his favorite musical artist, Morgan Wallen.



Chelsea, Jaxon and Brian Meschke, enjoy the Morgan Wallen concert at the "Big House".



The Meschke family, making memories with Morgan Wallen.

We are SO incredibly thankful to the Live Like Laura Fun Fund for making this unforgettable experience possible for Jaxon and our family. Your generosity gave him more than just tickets to a concert—you gave him a

memory, an experience, and a night filled with pure joy.

Thank you for helping Jaxon live, laugh, sing, dance, and make memories! We will forever be grateful.

cystaran[®]
(cysteamine ophthalmic
solution) 0.44%

Your eyes are part of your cystinosis care too.

Cystinosis can affect many parts of the body, including the eyes.

Staying on top of your eye health is an important part of living with cystinosis. Talk with your healthcare team about your eye care routine with Cystaran.



Build a Routine

Find a medication routine that helps you follow your prescribed dosing schedule consistently.



Keep up with Eye Exams

See your ophthalmologist every 6-12 months to monitor corneal cystine crystals and your overall eye health.



Know Your Baseline

Knowing what your ophthalmologist is monitoring and how your eye health will be tracked over time can help you take a more active role in your cystinosis eye care.

CYSTARAN (cysteamine ophthalmic solution) 0.44% is an eyedrop medication used to treat cystine crystal accumulation in the corneas of patients who have cystinosis.

IMPORTANT SAFETY INFORMATION

What is the most important safety information I should know about CYSTARAN?

- To help prevent contamination of the dropper tip and eyedrop medication, try to make sure that CYSTARAN is dropped directly onto the eye without touching it. Try not to touch the eyelids or surrounding areas with the dropper tip of the bottle when you are using CYSTARAN. Keep the bottle tightly closed when not in use.
- CYSTARAN contains an ingredient called benzalkonium chloride which can be absorbed by soft contact lenses. Remove contact lenses before using CYSTARAN eyedrops and wait at least 15 minutes before reinserting them.
- CYSTARAN should only be used as an eyedrop medication.

What are the side effects of CYSTARAN?

- The most common side effects of CYSTARAN, which have occurred in at least 10% of people using the medication, were sensitivity to light, eye redness, eye pain and irritation, and headache.

The risk information provided here is not comprehensive. To learn more, talk to your healthcare provider or pharmacist about CYSTARAN. The full FDA-approved product labeling can be found at www.cystaran.com.

You are encouraged to report negative side effect of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call FDA at 1-800-FDA-1088.



Development Update

By Jonathan Dicks, Vice President of Development

When we talk about development at CRN, it can be easy to reduce the conversation to dollars raised, grants secured, or fundraising goals met.

Those numbers matter. But they aren't really the story.

The story is what those resources allow us to do.

Over the past several years, CRN has continued to grow—not simply in the amount of support we receive, but in what we are able to provide in return. Research funding, family programs, scholarships, educational resources, advocacy, conferences, patient support, professional engagement, and opportunities to connect our community all require resources. More importantly, they require people and organizations who believe those things are worth investing in.

We have been fortunate to have exactly that.

Our industry partners continue to recognize the importance of investing directly in the cystinosis community. Our individual donors give generously because they believe in our mission. Families create fundraisers, share our story, attend events, and introduce CRN to new supporters. Researchers and clinicians give their time and expertise. Volunteers take ideas and turn them into programs.

Development is the engine that helps bring all of those pieces together.

And as CRN grows, our responsibility grows with it.

Securing funding is only half of the job. The other half is making sure those resources are used thoughtfully, responsibly, and in ways that create meaningful impact for people living



Teddy, Shirley, Elle and Finn Dicks.

with cystinosis. That means continuing programs that work, improving the ones that can be better, and being willing to invest in new ideas when we see an opportunity to serve our community differently.

It also means thinking bigger.

Rare disease organizations are often forced to become remarkably resourceful. Our patient populations are small, our challenges are complex, and the needs of families can span an entire lifetime. We don't have the luxury of solving one piece of the puzzle.

We have to support the family receiving a diagnosis today while investing in the research that could change what that diagnosis means tomorrow.

We have to support children while

recognizing the growing needs of adults with cystinosis.

We have to advocate for access to today's treatments while pushing for better ones.

We have to bring families together while also creating opportunities for researchers, clinicians, advocates, and industry to come together around them.

That is why development at CRN is about much more than fundraising. It's about building capacity.

Every new relationship creates another possibility. Every successful partnership gives us an opportunity to expand our reach. Every donor who chooses CRN is placing trust in us to turn their generosity into something meaningful.

Development Update, *continued*

We take that trust seriously.

As we look toward 2027, I am excited about where CRN is headed. We have opportunities to strengthen existing programs, deepen our investment in research, expand collaborations in the United States and internationally, and continue finding new ways to support people with cystinosis throughout every stage of life.

None of that happens because of one funding source, one corporate partner,

one fundraiser, or one person.

It happens because a community decides that progress is worth investing in.

To our industry partners, foundations, individual donors, fundraising families, volunteers, and everyone who has opened a door for CRN: thank you. Your support isn't sitting on a balance sheet. It is being transformed into research, education, advocacy, connection, and opportunity.

There is still much more we want to accomplish...and that's a very good thing.

Because development isn't simply about funding the organization CRN is today. It's about building the organization our community will need tomorrow.

Jonathan Dicks
President | VP, Development
Cystinosis Research Network



Care Package Program



When life with cystinosis feels overwhelming, even the smallest gestures can make a difference. The Care Package Program was created to remind families and individuals that they are not alone on this journey. Each package is thoughtfully assembled with supportive materials. On the next page, you'll find details on how to receive a package for yourself or nominate someone you care about.

[CYSTINOSIS.ORG/CARE-PACKAGE](https://cystinosis.org/care-package)

As a mom of 2 non-transplanted adult children with cystinosis, our time arrived this June when our daughter Alexis required her 1st kidney transplant. I was her donor. We were as “prepared” as any family could be, as I was a former Executive Board member with CRN for many years and we traveled all over the US going to and planning the biennial conferences learning as much as possible about this rare disease.

When major complications post-transplant occurred, one of CRN's board members, Jen Wyman, put together a care package, on behalf of CRN and the Care Package Program, for both myself and Alexis. What an unexpected gift of generosity that helped ease the immense burden of her lengthy hospitalizations, and softened the isolation we all felt during this time. CRN is a non-profit that fulfills its mission dedicated to making a difference in the lives of those affected by this disease.

*With my deepest gratitude,
Brittney LeBeau*



Brett, Alexis and Brittney LeBeau

Patients in England to Gain Access to Procysbi

By Alex Hutchison

Patients and families living in England with the ultra-rare condition cystinosis will have access to a life-changing treatment, following a recent funding decision by NHS England.

The treatment – delayed-release mercaptamine bitartrate (brand name Procysbi) – means that parents will be able to administer life-saving medication to their children every 12 hours, instead of every six hours. Similarly, adult patients will have more control over their medicine regimen to fit with their work and lifestyle needs. The decision by the Clinical Priorities Advisory Group (CPAG) – which assesses medicines, medical devices and treatments according to their clinical effectiveness, benefit for patients and value for money – brings England into line with Scotland, Wales and Northern Ireland where Procysbi is already available.

The news comes after more than three years of campaigning by Cystinosis Foundation UK, Metabolic Support and Kidney Research UK.

Caused by a rare genetic mutation, cystinosis results in the build up of crystals of the amino acid cystine in the body's cells. These initially form in the kidneys and eyes and later in muscles, pancreas, thyroid gland and white blood cells. The only treatment available until now has to be taken four times daily – including a dose in the middle of the night – and has a huge impact on patients and their

families, including lack of sleep, as well as many other life-impacting side effects.

“We are simply delighted,” said Will Newman, Chair of Trustees for Cystinosis Foundation UK. “This comes as a huge relief as now our patients and families will be able to have a full night's sleep. It feels that common sense has prevailed and equity has been restored by giving choice to all cystinosis patients in the UK.”

“It has been a tortuous process requiring lots of people to work together to bring about this momentous change. Some of our patients living with cystinosis have not had a full night's sleep for over 40 years.”

He added: “The approval means patients and caregivers can now explore whether this formulation better suits their individual circumstances, lifestyles, and tolerability needs.”

“We are delighted that NHS England has agreed to fund the use of Procysbi for patients with cystinosis,” added David Game, Consultant Nephrologist and Clinical Lead for the Delayed-Release Mercaptamine Policy Proposal.

He added: “We are acutely aware that this has been a frustratingly long time to wait. Thanks to all involved in the policy submission: clinicians, charities and other stakeholders together with those lobbying independently for this treatment.”

“We hope this will improve quality of life for patients and their families: truly a team effort.”

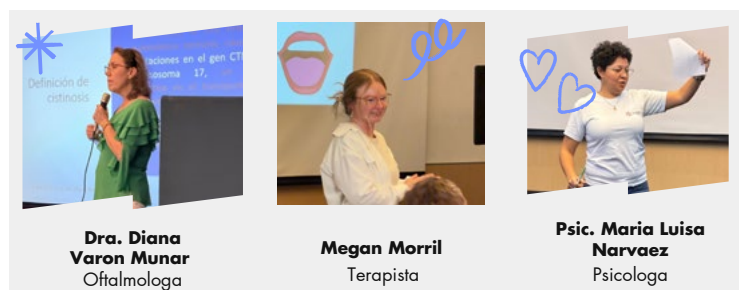
For people living with cystinosis, this means the possibility of keeping more closely to their treatment regimen, experiencing fewer side effects, and following a treatment schedule that better fits with daily life. This is particularly important for children, young people, and working adults whose routines can be disrupted by frequent dosing.

Cystinosis Foundation UK would like to acknowledge the huge support from partner organisations Metabolic Support and Kidney Research UK who have given valuable guidance and practical help throughout the process of making this treatment accessible to the people who matter.

Important information for UK patients: If you are prescribed a medicine for cystinosis, please speak to your healthcare professional about how best to manage your treatment. This article is intended to provide general information and does not replace medical advice. If you experience any side effects, speak to your doctor, pharmacist or nurse. You can also report side effects directly via the <https://yellowcard.mhra.gov.uk>. By reporting side effects, you help improve the safety of medicines.

Cystinosis Family Day 2026 in Mexico City

By Víctor Gómez, President of the Mexican Cystinosis Association (Asociación Mexicana de Cistinosis A.C.)



Our Cistinosis Family Day 2026 conference was successfully held in Mexico City on May 15 and 16, 2026, organized by the Mexican Cistinosis Association, A.C. This event brought together doctors, patients, and their families in a setting dedicated to learning, fellowship, and mutual support, where they shared experiences, knowledge, and meaningful moments that strengthened the bonds of our community.

KEY TOPICS

Psychologist Marilú Narváez worked with patients and their families through various participatory activities, highlighting the importance of teamwork, communication, and family unity as fundamental pillars for coping with the challenges associated with cystinosis.



Dr. Varón highlighted the importance of proper care when using Cystadrops®, sharing practical recommendations for its correct application and emphasizing key aspects to optimize treatment adherence and efficacy.

Dr. Greenbaum discussed nutritional recommendations for patients with Fanconi syndrome, as well as dietary and follow-up considerations after a transplant, with the goal of promoting a better quality of life and overall

health.

Dr. Stein presented information on the long-term effects of treatment with Cystagon® and shared strategies and recommendations for reducing and managing the gastrointestinal side effects associated with this medication.

Megan Morrill shared her life experience as a patient with cystinosis, inspiring attendees with her testimony. She also gave a hands-on demonstration of various

Cystinosis Family Day 2026 in Mexico City, *continued*

manual exercises focused on muscle strengthening, using different materials and therapeutic tools to illustrate the activities that are part of her rehabilitation and physical care process.

Víctor Gómez, president of the Mexican Cystinosis Association (Asociación Mexicana de Cistinosis A.C.), highlighted the success achieved through the organization of five family gatherings, which have strengthened integration, support, and the exchange of experiences among patients and their families. He also spoke about the association's hard work to ensure that people with cystinosis have timely access to quality medical services and their treatments, emphasizing the importance of maintaining adequate treatment adherence to improve health outcomes.

Finally, the organization's goals and future projects were presented,



focused on continuing to improve patients' quality of life through comprehensive support programs, access to medical care, and strengthening the community of people living with this disease.



Dr. Larry Greenbaum
Nefrologo



Dr. Adam C. Stein
Gastroenterologo



South Africa's Ongoing Struggle

By Gail Daniels, Chairperson, Cystinosis Foundation South Africa

We share our story not only to raise awareness, but in the hope that it will resonate with the global cystinosis community.

For families living with cystinosis around the world, the greatest daily challenge is often simply managing this ultra-rare disease. In South Africa, our struggle runs even deeper: it is the fight to access Cystagon, the only treatment available for cystinosis.

I know this struggle personally. Since my daughter's diagnosis in 2005, our family has faced it firsthand. It took us two full years to finally obtain the drug. The reason is simple yet devastating: Cystagon is not registered in South Africa.

Because the drug is unregistered, every prescription must go through Section 21 approval - a complicated, time-consuming process involving both the South African Health Products Regulatory Authority (SAHPRA) and each patient's medical insurer. The responsible doctor must complete a substantial stack of forms for every single application, and approval, when it comes, lasts only six months before the entire process must begin again.

Between applications, families often wait up to three months for approval - three months during which their child goes without treatment. For a progressive disease like cystinosis, where every delay carries real consequences, this is a heavy burden to place on patients, families, and the doctors caring for them, who lose significant time to administrative paperwork that should be spent on patient care.

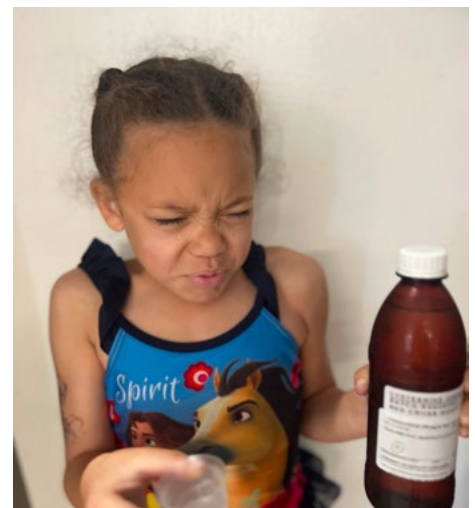
For families without medical insurance, Cystagon is simply out of reach. The cost is prohibitive without coverage. Of the 38 patients diagnosed with cystinosis in South Africa, only 14 currently have access to Cystagon. Patients in the public healthcare system rely instead on the liquid form of cysteamine - a medication so foul-tasting and foul-smelling that consistent compliance becomes its own daily battle, particularly for children.

The Cystinosis Foundation South Africa has advocated for years to

change this reality, working toward a future where every South African patient with cystinosis can access proper treatment without enduring years of delay, mountains of paperwork, or financial barriers that put a lifesaving medication out of reach.

Throughout this journey, we have been fortunate to have dedicated allies fighting alongside us. Professor Mignon McCulloch, a paediatric nephrologist, has advocated tirelessly for South African cystinosis patients for many years, lending her clinical expertise and voice to our cause both locally and internationally.

We share our story not only to raise awareness, but in the hope that it will resonate with the global cystinosis community. Every country faces its own version of this fight, and by standing together - sharing knowledge, advocacy strategies, and support - we move closer to a world where access to treatment is never determined by geography.



McKinely, taking cysteamine liquid.

Good News

Editor's note: We are so excited to share the Good News of the arrival of Jackson Sevel who joins dad and mom, Mike and Kristina, and big sister Grace.

We are also thrilled to announce the wedding of Board member Hannah Creel to Jon Logan Beadlescomb last July 18th. The CRN sends its congratulations and best wishes to both of these families.

Welcoming Jackson Sevel

We are so excited to announce the arrival of our newest little love, Jackson Michael Sevel, born June 13th, he has already brought more love and joy to our family than we could have imagined.

Our hearts (and hands!) are very full, and we feel incredibly blessed to welcome this sweet baby into our family.



Grace with new brother, Jackson.



Jackson Michael Sevel.

Good News

Hannah and Jon Logan Beadlescomb's Wedding



I wanted to share that Jon Logan and I tied the knot on July 18, 2026. It was a wonderful day of celebration! Thanks to the generosity of Frankie McGinnis and the Live Like Laura Fun Fund we were able to enjoy a wonderful honeymoon making memories as we hiked along waterfalls. Thank you to our lovely CRN friends and family for sharing your love and support, we are so grateful for this community!

With love, Jon Logan & Hannah Beadlescomb



Program Status:
OPEN APPLY NOW at
www.mygooddays.org/apply

Assistance Amount:
\$ 7,500 per year

Good Days is a national non-profit charitable organization that lifts the burdens of chronic illness through assistance, advocacy, and awareness.

We make life-saving and life-extending treatments affordable and act as your advocate, helping you navigate the system and guiding you to additional support through foundations and other organizations dedicated to those with specific, life-altering conditions.

Learn about our copay, travel and premium assistance programs to support individuals with Nephropathic Cystinosis.

Contact us for eligibility details. Ask a Good Days Patient Care Navigator for more information toll free at (855) 327-1134 or at <https://tinyurl.com/GoodDaysCyst>

Filling Our Cups: Finding Self-Care in the Rare Disease Journey

By Chelsea Meschke, Vice President of Family Support



Chelsea, Brian, Myles and Jaxon Meschke making summer memories.

I hope everyone was able to enjoy the summer months, make remarkable memories, and spend meaningful time with loved ones. Summer is one of my favorite seasons because it gives us the opportunity to slow down, enjoy time away from school, and become immersed in all the other beautiful parts of life.

For me, summer is also a reminder that self-care can come in many different forms.

Whether it's taking a vacation, spending a day at the beach, or simply jumping in puddles on a rainy afternoon, there always seems to be a long list of "We want to do this!" while the timing is right. As a family of four, we often find ourselves thinking that we have to fit everything we want to do into this small window of time. And while there are certainly times when that feels necessary, sometimes we need that gentle reminder that we

don't always have to do it all.

Are we making enough memories?

Did we take advantage of every adventure we could?

Did we do something special for this person or that friend?

Can we squeeze one more thing into an already jam-packed weekend?

There can be a never-ending feeling that we aren't doing enough. But the truth is, we are probably doing more than enough—we just don't always give ourselves credit for it.

Self-care looks and feels different for every caregiver, every patient, and every person who is part of a rare disease journey. So, when we ask ourselves, "How do we know if we're doing enough?" perhaps the answer can be found in another question:

Do you feel like your cup is being filled?

I'm a firm believer that you can't pour from an empty cup. As cliché as it may sound, it is brutally true. This rare life teaches us so much throughout its journey—how to find gratitude in the lows, grace in the highs, and appreciation for the moments that might otherwise pass us by.

I encourage every family to find those things—those moments, people, places, and experiences that make you feel like your cup is filling without even having to ask.

I would be hard-pressed to say that one of the things that fills my cup is being surrounded by our cystinosis family.

This past May, we had the incredible opportunity to gather in Jacksonville,



Attendees of the Cystinosis Family Meet-up held in Jacksonville, FL in May.

Filling Our Cups, *continued*

Florida, for a Family Meetup. Eleven families joined us for a day filled with connection, shared stories, laughter, support, and of course, learning from and spending time with the infamous Dr. Larry Greenbaum.

There is something truly special about bringing our cystinosis community together. Watching kids, adults, and caregivers connect with people who understand their journey in ways that few others can is something I will never take for granted. These gatherings create some of the sweetest moments, and sometimes they feel almost too good to be true.

To everyone who was able to join us in Jacksonville, thank you. Thank you for showing up, sharing your stories, supporting one another, and continuing to make this community what it is. Your presence matters more than you may realize.

And now, we have something even bigger to look forward to!

I am already looking forward to the 2027 Family Conference in Charlotte, North Carolina! I cannot wait to see where this next chapter takes us and, most importantly, to see so many of our cystinosis families together again.

Please keep an eye on our social media channels for more information about #CLT2027, as well as updates about our ongoing Zoom support groups.

Until then, remember to give yourself permission to slow down, to say no, to rest, and to recognize that you are doing enough.

Your cup matters, too.

Thank you all for being exactly who you are, and I hope to see you all soon!



Myles Meschke and Kennedy Kelley in Jacksonville.



The children meeting Hannah Creel's therapy dog Hope.

Financial Update

By Frank Re, Treasurer

The Cystinosis Research Network continues to utilize its financial resources to further its mission to secure a promising future for the cystinosis community through the support and funding of research grants that lead to improved treatments and ultimately a cure for cystinosis. Additionally, since 1996 CRN's vision includes enhancing the quality of life for those with cystinosis.

Thanks to tremendous grassroots fundraising efforts by the organization and Board, we were able to open

a call for research proposals this year. It has been several years since CRN was in a position to offer this opportunity, and we are excited to do so again.

CRN's current equity (assets minus liability as of September 11th, 2026) stood at roughly \$807,000, which is critical in funding additional research, as well as our next Family Conference in 2027. The CRN is a tax-exempt organization granted "501(c)(3)" nonprofit status by the I.R.S. The CRN Federal Tax ID # is 04-3323789.



Frank Re.

Almost Four Years Later: Amara's Journey to a Kidney Transplant

By Sam Nolen

In April of 2026, we got the call that we'd been waiting almost 3 years for. Amara was getting her kidney, the date was set, and our journey was coming to an end.

In January of 2026, we got the phone call that my sister-in-law had signed the paperwork and permissions to enter the partnership program available here in Australia. She would be donating a kidney to a stranger so that Amara, in return, could receive one.

It was the slowest six weeks of our life.

We got the call. June 10th, Amara would be receiving her kidney transplant.

So, we prepared as best as we could, keeping her as healthy as possible. Two weeks before transplant we pulled her out of school so that we could keep her immune system in its best condition. It was the mental preparations that had to be made too.

Almost four years ago, Amara went into the hospital for the first time. She had been sick for a long time, but many GPs had just dismissed it as common school colds and flus.

Amara got an ear infection that we couldn't get rid of, so I took her to the GP and they ordered blood labs at my insistence. The next thing we know, we are being rushed to the local children's hospital, and having scary words like leukemia thrown around.

About six months after the initial stay, we found out Amara was in renal failure, we learned the reason... cystinosis.



Amara with her mom.

We had a name for the beast and so began that fight. Getting her healthy enough that she would take a new kidney well, and the whole process that came with it became the goal.

Amara eventually needed peritoneal dialysis seven nights a week, and we had a PEG placed to help with the constant high amounts of medication.

So, when June 10th came around, Amara went into surgery in the late afternoon. We were told it would be a long surgery, approximately six hours.

We were lucky enough to be provided with family accommodations at the hospital while this was happening, so Amara's dad, sibling and I waited and waited.

It got to 11pm, so we headed to what they fondly call the "friendship

room. We call it the depression room, because it is where you wait for your child to come out of surgery, and while they try to be kind, it's just... sad. Lots of anxious and worried mums and dads.

Midnight came and went, and I started to get worried.

At 2am, a nurse came out of the door and let us know she was okay and had done extremely well.

I burst into tears. I've never been so scared in my life.

Recovery was not amazing. She was in a lot of pain, and we were in hospital for 11 days. She started getting better and better, and we thought we had passed the worst.

We were finally getting rid of Mr Beepy, a name Amara had given her

Amara's Journey to a Kidney Transplant, *continued*

dialysis machine in the early days. She would have to come back six weeks later to have her dialysis catheter and the stent in her kidney removed.

We got three blissful days at home, and then she was back in hospital again, another week-long stay and not many answers. We came home.

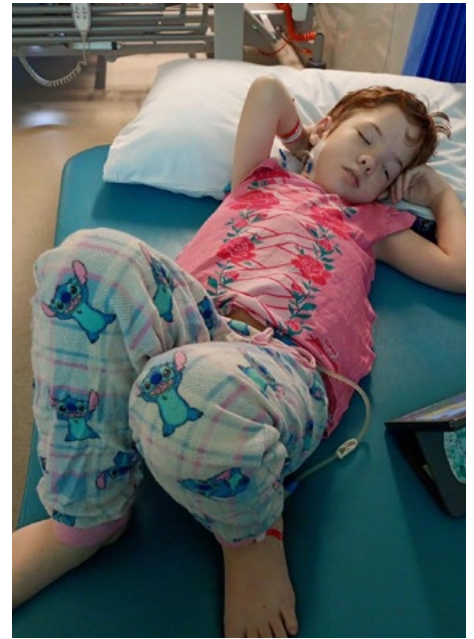
Four days later, we were back again. Another week-long stay, but this time we found the culprit.

Her stent was annoying her new kidney, so they decided to take it out early, and they also took Mr Tubey (her dialysis catheter) out at the same time. It was so weird seeing my little girl's tummy naked without her tubing that had become a part of her.

Now we are almost three months out, and honestly, I didn't know that recovery would sometimes be harder than the 'before times'. It has been a mental rollercoaster, but our little girl is **THRIVING**.

She can now swim (one of her favourite things to do) and has so much more energy. She is still on a lot of medications, but takes them like a champ. She is back at school, and we are learning what our new life and new normal is.

We can't thank her team and the whole partnership program enough. They've changed our lives, and our little girl can just be the amazing, resilient kid she is.



Amara, receives her transplant in Australia.



One in a Million

By Sam Nolen

At 10 months old, Indy was a happy, healthy baby. Just months later, she was fighting for her life in a Sydney ICU. Her father shares their family's journey through misdiagnosis, critical illness, a life-changing cystinosis diagnosis, treatment, and the day-to-day reality of raising a child with cystinosis.

Scan the QR code or visit the link to read the full story (<https://tinyurl.com/bddvd8cx>)



Living your best life with cystinosis

Everyone deserves to live their best life, including you! As a young person, you have your whole life ahead of you, so here are some ways to make the most of it:

Advocate for yourself –

help others learn more about cystinosis and what they can do to help.

Find a hobby – start doing something you enjoy and find interesting, like photography, painting, cooking, biking, writing, dancing or playing an instrument.

Help others – give back to your community and those in need by volunteering.

Make new friends – surround yourself with supportive people who make you happy.

Stay active – find a sport or outdoor activity you enjoy and your doctor approves of.

Practice positivity – a positive attitude is key to success; try meditation, mindfulness or positive affirmations.

Set goals – whether you want to go to college or start working after high school graduation, set high goals for yourself.

Share your story – inspire and motivate others by sharing your journey.

Support causes you believe in – whether it is protecting animals, equal rights or the environment, take a stand.

Participate in advocacy – use your voice to support laws relating to cystinosis or kidney disease in general. Anyone affected by kidney disease can join the American Kidney Fund's Advocacy Network, where you can work to make a difference in the lives of others.

TALKING TO OTHERS ABOUT CYSTINOSIS

It can be frustrating or awkward having to explain cystinosis to people all the time. Remember: cystinosis does not define you as a person, and it is only one part of your life. If you feel comfortable sharing your diagnosis with others, then keep it up! If you need some time before opening up to others about your condition, then that is perfectly okay and very normal.

If you do decide to tell other people about cystinosis, here are some tips to help you have the conversation:

- Explain the basics of cystinosis. It will be easier for others to remember key points, rather than a lot of detailed information at once.
- Be honest about what you may not be able to do and tell others what you can do instead.
- Let others know that you take care of yourself by taking medication and seeing a doctor.
- Inform others that you do not want to be treated differently (if that is the case).
- If you do not want to talk about cystinosis anymore, steer the conversation in a different direction.
- You do not have to answer every question if you do not feel comfortable. Instead, you can direct others toward resources like the American Kidney Fund or Cystinosis Research Network to get more answers.

Find more cystinosis resources at:
KidneyFund.org/cystinosis



AMGEN

**CYSTINOSIS
RESEARCH NETWORK**

American Kidney Fund®
FIGHTING ON ALL FRONTS

Finding Strength Through Occupational Therapy

How the Cystinosis Memorial Fund - and Daily Dedication - Helped Begin Rebuilding Hand Strength

By Whitney McElroy, 47 year old adult with cystinosis

In February, 2025 I noticed I was no longer able to “high five” my students at the elementary school where I was working as a speech language pathologist. This was difficult for me to accept, as giving my students a “high five” was a way that I could connect with them and help to establish a rapport quickly. Earlier in the year I had noticed that I also was unable to cut with scissors effectively, open water bottles, and open my front door with one hand, all things I once was able to do without even thinking. I also began to struggle at the gym holding onto weights, not because my arms were too weak for the weight, but because my hands literally could not grip the dumbbells. I attributed the loss of these fine motor skills to the muscle wasting/atrophy that is common in older adults with cystinosis.

After much thinking and procrastination, which is common with my overwhelming life as a single mom of 10 year old twins, I decided to reach out to Megan Morrill. I knew that ignoring my weakening hand muscles would be detrimental in the long run and that it would be extremely challenging to find an Occupational Therapist in Orlando, where I live, that was knowledgeable of cystinosis and the later in life effects it can have.

I knew Megan was a certified Occupational Therapist and had recently presented at the CRN conference in Grand Rapids, Michigan over the summer of 2025. Additionally, she is also an adult with cystinosis. Megan actually pointed me in the

direction of the Cystinosis Memorial Fund which was a tremendous help in funding occupational therapy sessions for 10 weeks!

Working with Megan was informative and fun! I found working with another adult with cystinosis to be validating and reassuring as she shared her struggles with cystinosis, and was able to relate to mine and vice versa. Often my weekly session was the highlight of my week!

Over the course of 10 weeks I noticed my hand strength slowly improved. I am able to complete some of the tasks I once struggled with independently. For example, I can now open store bought water bottles without using any “aids” (round rubber

circle grip). I can also cut with scissors using a single hand again and have more strength. I’m not yet able to high five my students but I’ve improvised and now “fist bump” with them.

One thing that made a lasting impression on me while working with Megan, is that the muscle wasting in my hands is not something that can be fixed overnight. It takes discipline to address. Working with Megan once a week was not enough to see a change in my hand strength. I had to dedicate time each week outside of my occupational therapy session to work on my hand strength and complete the exercises and fine motor exercises that Megan has taught me. With time I have been

“Working with Whitney was truly an honor! As someone who also lives with cystinosis, I understood many of the challenges she was experiencing. It was incredibly rewarding to combine my professional knowledge with my lived experience to provide consulting that was both practical and personal. I’m so grateful I have the opportunity to support her and be part of her journey!” -Megan Morrill



Picture of my right palm showing weakened muscles particularly on the thenar eminence (base of thumb) which is characteristically small and undefined in individuals with Cystinosis.



Therapy putty that was used weekly in my Occupational Therapy sessions.

Finding Strength Through Occupational Therapy, *continued*

able to incorporate fine motor and hand exercises as well as stretches in my daily routine. This helps me stay consistent with working to improve the muscle strength in my hands.

I would highly recommend to any adult with cystinosis having trouble with hand strength or dexterity to work with an Occupational Therapist. I believe preserving our fine motor skills is a way we can maintain

our independence for as long as possible. I think as adults with cystinosis this is one of our major goals, to be independent for as long as possible. I would encourage anyone in our community to reach out to Megan about fine motor skills and hand strength. She is passionate about her patients and knowledgeable. Megan truly cares about others, and as a patient with

cystinosis herself, she is able to understand our complex medical needs in a unique way.

The Cystinosis Memorial Fund made access to effective Occupational Therapy more of a reality and lessened the financial burden to me. I would highly recommend that any adult who is looking to strengthen their hands or body physically consider applying.

Research Update

By Heather Rothrock, Director and acting Vice President of Research

Research Community Update

Sharing this update with a slightly different voice this time, as I step in for the wonderful Kristina Sevel while her family welcomes its newest addition. The CRN team remains closely connected with the research community, and we are excited to highlight a few important updates for fall 2026:

Call for Research Proposals Coming This Year

CRN is pleased to announce that we will open a call for research proposals in 2026. This is an exciting milestone, as it has been several years since CRN has been able to offer this funding opportunity. It also reflects the incredible fundraising efforts and continued support of our broader community. Your commitment is helping make meaningful progress possible!

Scientific Symposium Scheduled for November

CRN will host a one-day Scientific Symposium in Ann Arbor, Michigan,

on November 13, 2026. The symposium will bring together medical professionals in the cystinosis community for a day of learning, collaboration, and connection. Attendees will hear about new research relevant to cystinosis, as well as updates on important initiatives already underway.

QUALIFY Study Update

We are pleased to share an update on the QUALIFY study, which focused on developing and validating a questionnaire to measure health-related quality of life for children, adolescents, and young adults living with cystinosis. The study has now concluded, and CRN is working closely with the QUALIFY team to review the findings, provide feedback, and support the finalization of related scientific papers. We are also offering input on how best to share these important findings with both the medical community and the broader cystinosis community, including patients and families.



Levi and Briley Rothrock on the first day of school, August, 2026.



Rothrock Family in Surf City, NC, at sunset, June, 2026.

“I tell my sons cystinosis is something you have, it’s not who you are.”

I choose Cystadrops because...

- It dissolves crystals across all layers of the cornea
- Provides flexibility of 4x a day dosing
- The CystaTouch dropper is easy to use

– Nicole, mother to three teenagers living with cystinosis

Indications and Usage

CYSTADROPS is a cystine-depleting agent indicated for the treatment of corneal cystine crystal deposits in adults and children with cystinosis.

Important Safety Information

- To minimize the risk of contamination, do not touch the dropper tip to any surface. Keep bottle tightly closed when not in use.
- A condition where the pressure inside the skull increases for unknown reasons has been reported with cysteamine taken by mouth or cysteamine eye drops (used at the same time as cysteamine taken by mouth). This condition went away with the addition of medicine that increases the production of urine.
- Contains the preservative benzalkonium chloride. Contact with soft contact lenses should be avoided. Remove contact lenses prior to application. Lenses may be reinserted 15 minutes following administration.
- The most common side effects are eye pain (stinging), blurred vision, eye irritation (burning), eye redness, discomfort at instillation site (sticky eyes or sticky eyelids), eye itching, watery eyes, medicine deposit on the eye lashes or around the eyes.
- **To report SUSPECTED SIDE EFFECTS, contact Recordati Rare Diseases Inc. at 1-888-575-8344, or FDA at 1 800-FDA-1088 or www.fda.gov/medwatch.**

Visit www.cystadrops.com for full prescribing information



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www.recordatirarediseases.com/us PP-CDPS-US-0245



PATIENT BRIEF SUMMARY

CYSTADROPS® (sys-tah-drops) (cysteamine ophthalmic solution) 0.37%, for topical ophthalmic use

Summary:

Read this information before you start using Cystadrops and each time you get a refill. This information does not take the place of talking to your doctor about your medical condition or your treatment.

What is Cystadrops?

Cystadrops is a prescription cystine-depleting solution used for the treatment of corneal cystine crystal deposits in adults and children with cystinosis.

What should I know about using Cystadrops?

- To minimize the risk of contamination, do not touch the dropper tip to any surface. Keep bottle tightly closed when not in use.
- Cystadrops contains the preservative benzalkonium chloride. Direct contact with soft contact lenses should be avoided. Remove contact lenses prior to application. Lenses may be reinserted 15 minutes following administration.
- A condition where the pressure inside the skull increases for unknown reasons has been reported with cysteamine taken by mouth or cysteamine eye drops (used at the same time as cysteamine taken by mouth). This condition went away with the addition of medicine that increases the production of urine.

Before you use Cystadrops, tell your doctor if you:

- are using any other eye drops
- wear contact lenses
- are pregnant or plan to become pregnant. It is not known if Cystadrops will harm your unborn baby.
- are breastfeeding or plan to breastfeed. It is not known if Cystadrops passes into your breast milk. Talk to your doctor about the best way to feed your baby if you use Cystadrops.

How should I use Cystadrops?

See the complete Instructions for Use for detailed instructions about the right way to use Cystadrops.

- Use Cystadrops as your doctor tells you.
- Use one drop of Cystadrops in each eye, four times each day.
- If you miss a dose, administer the dose as soon as feasible, and then continue the treatment with the next scheduled dose.
- Keep Cystadrops in the refrigerator until ready to use. After opening, write date on carton and after each dose, keep the bottle tightly closed and store at room temperature in the original carton.
- Discard bottle 7 days after opening even if medication is still in the bottle.

What are the possible side effects of Cystadrops?

The most common side effects are eye pain (stinging), blurred vision, eye irritation (burning), eye redness, discomfort at instillation site (sticky eyes or sticky eyelids), eye itching, watery eyes, medicine deposit on the eye lashes or around the eyes.

These are not all the possible side effects of Cystadrops. Tell your doctor if you have any side effects that bother you. You may also report side effects to **FDA at 1-800-FDA-1088**.

How should I store Cystadrops?

- **Before First Opening:** Before opening, store new, unopened CYSTADROPS in the refrigerator between 36°F to 46°F (2°C to 8°C). Keep the bottle in the outer carton in order to protect from light.
- **After First Opening:** After opening, store opened CYSTADROPS at room temperature between 68°F to 77°F (20°C to 25°C). Do not refrigerate after opening. Keep the dropper bottle tightly closed in the outer carton in order to protect from light. Discard 7 days after first opening

Keep Cystadrops and all medicines out of the reach of children.

General information about the safe and effective use of Cystadrops.

Medicines are sometimes prescribed for purposes other than those listed in a Patient Information leaflet. Do not use Cystadrops for a condition for which it was not prescribed. Do not give Cystadrops to other people, even if they have the same symptoms you have. It may harm them. You can ask your healthcare provider or pharmacist for more information about Cystadrops that is written for healthcare professionals.

What are the ingredients in Cystadrops?

Active ingredient: cysteamine 3.8 mg/mL (equivalent to cysteamine hydrochloride 5.6 mg/mL);

Preservative: benzalkonium chloride 0.1 mg/mL;

Inactive Ingredients: carmellose sodium, citric acid monohydrate, disodium edetate dihydrate, hydrochloric acid and sodium hydroxide (to adjust pH to 4.6-5.4), and water for injection.

Manufactured by: Baccinex SA, 2822 Courroux, Switzerland

Manufactured for: Recordati Rare Diseases Inc., Bridgewater, NJ 08807, U.S.A

**For current prescribing information, please visit
www.CYSTADROPS.com/PI.**

Revised: July 2024

Available for patients using CYSTADROPS *The PATIENT LIAISON (PL) PROGRAM**

Personalized one-on-one support is available for patients receiving treatment with CYSTADROPS and their caregivers. Dosing and administration information along with additional resources are within easy reach. The PL program is also available in Spanish. Enrollment is voluntary.

*Information provided by the PL program is for educational purposes only and is not intended to replace the advice of patients' healthcare providers.

To find out more – visit www.cystadrops.com/resources-faq/



Scan QR Code
to enroll now



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In Memoriam

Patrick Douglas Partington Dec 7, 2004 — Aug 29, 2026



Information adapted from the obituary published by gormleyandsons.com.

Patrick Douglas Partington, 21, died peacefully in his sleep on Aug. 29, 2026. He was deeply loved, and he is missed beyond measure.

Patrick had a shy smile that never really left him and a soft-spoken nature that stayed with him from childhood. He didn't rattle, and he didn't complain, though nobody would have blamed him if he had. He was deeply thoughtful and curious, and conversations with him could wander into the big questions of life and how to live well. But quiet was never the same thing as timid. As a boy, he was fearless about roller coasters. He wanted the front car and the biggest drop.

Patrick and his twin sister, Jenna, shared a closeness all their own. They were inseparable, and even with geographic distance, they remained each other's constants. They had a "twin test": anyone important in one twin's life had to pass muster with the other.

Cystinosis had been part of Patrick's life, but it was never the whole of it. Patrick and Jenna were diagnosed when they were small. They faced it together, and it deepened a bond already all their own. For two decades, Patrick's community funded cystinosis research in his honor, helping carry a potential cure into clinical trials. That work outlives him.

Patrick is survived by his parents, Kevin and Teresa; his twin sister, Jenna; his grandmothers, Juanita Partington and Jan Batt; and numerous aunts, uncles, and cousins.

*Donations may be made in Patrick's memory to the Jenna & Patrick's Foundation of Hope, jpfh.org, or mailed to:
JPFH c/o Kevin Partington, 400 Capital Mall, Suite 1800, Sacramento, California 95814
Make checks payable to: **Jenna & Patrick's Foundation of Hope***

Help Support the CRN's Mission – Donate Today!

Get connected! Stay informed! Together we can find a cure!

Become more active within our global network of caring families, concerned individuals and healthcare professionals working together in the fight against cystinosis. The Cystinosis Research Network's vision is the discovery of improved treatments and ultimately a cure for cystinosis. The Cystinosis Research Network is a volunteer, non-profit organization dedicated to advocating and providing financial support for research, providing family assistance and educating the public and medical communities about cystinosis. CRN funds research and programs primarily through donations from the public, grassroots fundraising events and grants. CRN provides outreach and access to resources.

We take great pride in carrying out our motto: "Dedicated to a Cure. Committed to our Community"

We are here for you, whether you are:

- A parent who needs critical resource information, support services or help in sharing the challenges of cystinosis with those who serve your child.
- An adult with cystinosis interested in information regarding medical and social issues specifically geared for adults.
- A relative or a friend who wants to increase their understanding of cystinosis and find out how you can help out or become involved.
- A Physician, Social Worker, Educator or other Professional who makes a difference in the life of a family affected by cystinosis, and want to have access to critical information to better serve your patient, student or client.

The Cystinosis Research Network is proud to provide valuable resources to the community, free of charge. Many can be found on our website: cystinosis.org.

Resources include but are not limited to:

- The latest cystinosis information through our biannual CRN Newsletter; The Cystinosis Advocate, our website (cystinosis.org), the popular online cystinosis Facebook support groups, regular email updates and other social media channels.
- CRN Family Conferences and Regional Meetups. Exchange knowledge and create friendships with other families and individuals living with cystinosis. Learn first-hand the latest discoveries about cystinosis from medical professionals.
- Rare Disease Week Scholarships. Participate in a week-long event in Washington, D.C. Let your voice be heard by legislators and policymakers who need to know why cystinosis (and other rare diseases) is important to you.
- Access to Cystinosis Research Network individuals and families near you.

Please consider donating to the CRN today to help us continue offering robust support, education and research to the global community.

Ways to donate:

- Scan the QR code using your mobile camera
- Use the envelope provided to contribute via check
- Visit our website donation page at <https://tinyurl.com/CystDonate>



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Cystinosis is a rare, genetic, metabolic disease that causes an amino acid, cystine, to accumulate in various organs of the body, including the kidneys, eyes, liver, muscles, pancreas, brain and white blood cells. Without specific treatment, children with cystinosis develop end-stage kidney failure at approximately age nine. The availability of cysteamine medical therapy has dramatically improved the natural history of cystinosis so that well-treated cystinosis patients can live into adulthood.

CRN VISION

The Cystinosis Research Network's vision is the acceleration of the discovery of a cure, development of improved treatments, and enhancement of quality of life for those with cystinosis.

CRN MISSION

The Cystinosis Research Network is a volunteer, non-profit organization dedicated to advocating and providing financial support for research, providing family assistance and educating the public and medical communities about cystinosis.