CYSTINOSIS RESEARCH NETWORK

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Fall 2025

THE CYSTINOSIS Advocate

INSIDE THIS ISSUE

CRN Family Conference 2025 a GRAND Success!

Over 250 people gathered at the Amway Grand Hotel in Grand Rapids, Michigan on July 11th-13th 2025, to attend the CRN Family Conference. Doctors, researchers, families, those living with cystinosis, and industry partners connected, shared experiences and learned the latest research updates, and had fun! Conference highlights on page 4.

THE PRESIDENT'S LETTER

Resilience in Motion: A Season of Challenge, Connection, and Hope

Elle Dicks recovering from her kidney transplant.

Dear Cystinosis Community,

This summer will be one I will never forget. While hundreds of us gathered in Grand Rapids, Michigan, for the 2025 CRN Family Conference, my own family was back home in Cincinnati facing a reality we had long since known, but could have never truly prepared for — our daughter Elle receiving her new kidney from a deceased donor. To call it a whirlwind hardly does it justice. It was a season of long nights, anxious prayers, and profound gratitude all woven together, and it underscored for me just how much this community means when life feels overwhelming.

The Grand Rapids conference itself was a landmark moment for CRN. Across three days, families, clinicians, and researchers came together for a program that blended the latest in science with the everyday realities of living with cystinosis. We heard meaningful updates from the medical and research communities, engaged in practical sessions like financial and estate planning, and found encouragement in the panels, performances, and workshops that gave voice to every member of the family. Siblings had dedicated

The President's Letter, continued

Jonathan and Finn Dicks having fun.

The Dicks family, Jonathan, Teddy, Elle, Finn and Shirley.

opportunities to be seen and heard, adults living with cystinosis found a platform for connection and advocacy, and parents and caregivers left equipped not just with knowledge, but with a renewed sense of strength. More than an agenda of sessions, it was a living demonstration of what makes our community unique: the balance of hope, connection, and progress.

This year, I experienced the conference not only as CRN's president, but also through the eyes of my son Finn, who was by my side in Grand Rapids while his sister recovered in Cincinnati. For him, the sibling support woven throughout the weekend was not abstract — it was personal. He found space where his questions mattered, where his role as a brother was recognized, and where he could connect with other kids who "just get it." Watching him be supported and uplifted in that environment reminded me that the conference isn't only about cystinosis itself, but about the entire family that carries it.

Today, Elle is home, recovering and gaining strength day by day. We are profoundly grateful for the gift her donor gave, and for the community that has walked alongside us in this journey. While the paired kidney exchange was no longer the path forward, I remain committed to honoring this journey by donating altruistically, ensuring Elle holds a kidney voucher for her future while also giving another family the chance at life today. It is both a personal decision and a reflection of what we all strive for — creating ripples of hope that extend beyond our own story.

As we look ahead, we carry with us the lessons and the momentum of Grand Rapids. We are reminded that progress is not only measured in science and medicine, but in connection, compassion, and courage shared. CRN will continue to build on that momentum: strengthening family support, expanding opportunities for education and advocacy, and standing alongside the research community as it pushes the boundaries of what's possible.

On behalf of the CRN Board and my family, thank you — for standing with us, for standing with one another, and for showing the world what it means to never give up hope.

With gratitude,
Jonathan Dicks, President
Cystinosis Research Network

CRN 2025 Family Conference Highlights

By Terri Schleuder, Director

Another phenomenal CRN Family Conference took place July 11th-13th in Grand Rapids, Michigan. The event was held at the beautiful Amway Grand Hotel, located in the heart of downtown. Filled with modern touches and 'yesteryear' elegance, it was the perfect location to gather.

While the Family Conference is a place to learn the latest information in research and in new treatments, it is also a place to connect and reconnect, a 'family reunion' of sorts. It is a gathering of people who understand without saying a word what life with cystinosis entails. That alone is everything!

Friday morning began with the Scientific Symposium where experts from around the world shared their latest findings and research.

Highlights included:

1. Taurine Therapy may correct the deficiencies of cysteamine therapy, by Dr. Jess Thoene.

2. Developments in ocular cystinosis looked at using a cysteamine filled contact lens as an alternative to topical drops, by Dr. Rachel Bishop.

3. We learned about the latest developments of CF-10, a drug which may be a treatment for cystinosis without the debilitating side effects of current cysteamine therapy, by Stephen Waldek, FRCP

Those living with cystinosis "ham it up" in a group photo.

4. There were discussions about the need for adequate pediatric to adult transition clinics in the lifelong cystinosis journey, by Lauren Suchy Marzinelli, LCSW, MSW, MPH

5. We learned what brainwaves may reveal about the cognitive effects of cystinosis related to speed, accuracy and processing, by Sophie Molholm, PhD

And so much more!

Welcome Reception

On Friday afternoon families gathered to collect their registration materials and visit with each other before the official welcome reception and dinner. There were balloons and face painting for the kids and a photo booth for everyone to enjoy.

We had many first-time attendees this year who were invited to an orientation/ networking session hosted by CRN president, Jonathan Dicks, and VP of Family Support, Chelsea Meschke. The goal was to welcome these new folks and make sure they were comfortable before dinner.

After a scrumptious meal families got a chance to introduce themselves to each other. It is always a highlight to see where everyone is from. This introduction forms a bridge to making further connections throughout the weekend.

Keynote Speaker & Research Updates

Saturday began with an inspirational talk by keynote speaker, James G. Robinson, who shared his 5-year experience parenting a child with serious heart defects. He focused on the 'truths' this experience revealed

Keynote speaker, James G. Robinson.

"Meeting everyone; the chance to see my daughter engage with people who get her, make friends and all of the information I inhaled through all of the parents, kids and adults living with cystinosis…learning I'm not alone, and she has support."

Anonymous

CRN 2025 Family Conference Highlights, continued

"We thought the conference was very organized and welcoming! Our favorite sessions were the ones led by adults with cystinosis, who are professionals in areas related to cystinosis issues. They provided relevant tips and tricks for topics like muscle wasting and managing anxiety. They inserted their own experiences, which was very helpful, but they never made it all about them. We appreciated their expertise because they have been on a journey that is similar to ours.

Thank you again for all the hard work done by the CRN!"

Jim and Paula Shal

Our expert medical panel answers attendees questions.

Megan Morril and Allie Koshen offered practical suggestions to address muscle wasting and swallowing issues in cystinosis.

to him about what it means to live, grow, and heal. It also showed him the importance of humanity in medicine. The themes he shared resonated perfectly in our rare disease community.

Much of the rest of the morning sessions provided updates on current research studies. In addition to those described at the Scientific Symposium on Friday, we learned from Melissa Wigderson, Global Clinical Head, Norvartis Global, the next steps in the gene therapy/stem cell study, which will focus on 2-5 year-olds. We also learned updates in the development of new international cystinosis guidelines, from Dr. Ewa Elenberg, and updates in the operations and policies of the U.S. kidney transplant system from Dr. Fredrick Kaskel.

Speak Up Speak Out

We were treated to an outstanding and powerful spoken word performance by many teens and adults living with cystinosis, who participated in the Speak Up Speak Out program. This dynamic workshop fosters confidence, connection, and self-expression. The works presented were heartfelt and powerful, stirring strong emotions in all present.

Before lunch everyone gathered for a group photo.

Breakout Panels

Much of Saturday afternoon was specific to attendees' individual needs through various panels.

1. A Sibling Workshop was held for the siblings of those living with cystinosis ages 6-13 years.

2. Cystinosis101 targeted the newly diagnosed families.

3. The Middle Childhood and Teens

CRN 2025 Family Conference Highlights, continued

panel addressed families of this age group.

4. Three closed sessions were also held:

   a. One for Adults living with cystinosis with a medical panel

   b. One for Adults (18+) living with cystinosis and their partners

   c. One for Parents of Adults living with cystinosis

5. Another panel addressed the needs of those approaching Dialysis and Transplant.

The afternoon ended with another conference highlight, the Medical Panel. The opportunity to hear from the experts and be able to ask them questions is invaluable to many.

Patient Centered Sessions

The Sunday sessions were more 'patient centered', addressing the needs that occur along the cystinosis journey. A handful of these sessions were led by three remarkable women living with cystinosis.

1. CRN Board member, Megan Morrill, OTD,OTR/L and Allie Koshen, MA,CCC-SLP provided practical exercises and tools to address muscle wasting/weakness and swallowing issues common in many adults living with cystinosis.

2. CRN Board member, Cheryl Simoens, MA, CCC led a session titled Brain and Body Connection: Trauma and Life with Cystinosis (for patients).

3. Hannah Creel, a licensed music educator, and her therapy dog Hope, led an interactive session titled Music and Medicine: Connecting Music therapy and Cystinosis. Hannah showed how participation in a structured music therapy setting supports core executive functions. On Sunday, Hannah also held a music therapy session for the children in daycare.

4. Katie Mesko, DrOT, OTRL led an informative session titled the Impact of Stress (for caregivers). Personally, this was eye opening, as I realized how much stress I still carry 37 years into the journey.

Speak Up Speak Out participants learn how the power of the spoken word helps in finding their own voices.

Spiderman visits Daycare, delighting the children.

Industry Partners & More

We also heard updates from our industry partners at Recordati Rare Diseases, with Anna Vorobeva; Leadient Biosciences, with Lesli King; and Amgen Rare Diseases, with Andrea Atherton and Patient Access Liaison (PAL), Gina Gough.

CRN's treasurer, Tim Wyman, CFP, JD, shared how to navigate estate and financial challenges including wills, financial and healthcare powers of attorney, living trusts, and

CRN 2025 Family Conference Highlights, continued

"...he [Oliver] was SO PROUD of his 'warrior badge' and carried it around for WEEKS, showing it off to nurses, friends, anyone who would listen. I love that he is learning about his diagnosis through this positive association."

Taylor W.

"I think the CRN conference is a unique and very special event, allowing, as it does, families and patients access to leading researchers and physicians. The format is relaxed, and this encourages chats and conversations. I think this is beneficial to both patients and those of us who work in this area. I learn something new every time I attend one (and I've been coming over for > 20 years)."

Dr. Donald Cairns

"The best aspect is equal parts community support and information presented from the doctors. Both are so crucial in this life we live, and are welcomed parts of the conference."

Anonymous

more. After that CRN's VP of Family Support, Chelsea Meschke discussed the importance of Advanced Care Directives.

Additional sessions focused on successfully navigating Children to Adult Transition of Medical Care, featuring Jack Greeley and Lauren Suchy Marzinelli, LCSW, MSW, MPH. After that, Emily Holl, Director of the Sibling Support Project, led a sibling panel discussing the impact cystinosis can have on siblings, and how to address their needs within the family.

The day ended with two highly anticipated 'expert' panels. The Parents of Children and Adults with cystinosis, and Adults living with cystinosis. This lived experience offers some of the best wisdom our community has to offer.

Dinner Dance Farewell

A fabulous, fun farewell dinner/dance concluded this wonderful conference. It provided one more opportunity to visit, share, and connect while making memories.

In closing, we would like to recognize the two years of planning and hard work it took for 3 days of 'magic', that was the CRN Family Conference 2025. Truly we are all 'Navigating the Current Together', as this year's theme suggests. A huge thank you goes to CRN's Board of Directors. A special thank you to CRN's amazing Executive Director, Clair Johnstone, and our Event Planner, Dana Marshall. Your unending efforts helped to make this conference another stellar event.

Can't wait till 2027!

For additional conference photo highlights see page 22.

Education & Awareness Update

By Marybeth Krummenacker, Vice President of Education & Awareness

I always start my newsletter articles with a reflection of where we have come from, and try to look ahead with optimism and a positive message.

I guess this one will be no different. As I write this article, we are just about a month from our overwhelmingly successful Grand Rapids Family Conference! I am still in awe, and I am so grateful to every single person who attended. It was an honor and a pleasure to meet so many new faces!

There is much work and planning that goes on behind the scenes in putting together a conference of this size. It takes collaboration and effort on the part of all the CRN Board. We all played a role in this planning. I always say it is an honor and a privilege to serve with this fantastic group of volunteers. We spend many hours discussing ideas, and planning an agenda that is both informative and helpful to each family, to help them navigate the world of living with a rare disease like cystinosis. We work very hard at the same time to offer the opportunity for families to meet, and network with each other. Just as important, we offer families the chance to speak one on one with the physicians in a relaxed environment. It is a theme we hear often, that families are grateful for this extraordinary opportunity. There is nothing like meeting another family in person who understands the life we lead and manage every single day!

The medical portion of our agenda is often overwhelming to understand, but I have said it for so many years, we in the cystinosis community are so blessed to have the brilliant minds of physicians, scientists and researchers who do understand. I have shared this story many times that I failed biology in school. Never did I think I would come to understand it in the way I do after 36 years of listening and learning. And I am still learning.

I am so grateful for our continued partnerships with industry. These relationships have strengthened throughout the years, and our community has been blessed with their generosity. We have not only been able to receive grants to help sustain all that we do including this wonderful conference, but events such as the New York Academy of Medicine meeting in 2024 would not be possible without their help.

Marybeth Krummenacker.

We have also been able to increase the amounts of our academic scholarships. This year's winners will be announced by the time we go to print with this newsletter (see pages 9-10).

The support and generosity of our industry partners have helped us become what I know to be true: a "gold standard" in nonprofits.

With the help and support of families and friends, we will continue to do great things. We represent a small, rare disease community, but we are mighty. With the help, and donations we receive from so many of you, we will continue with our message of new and improved treatments, and to offer families support, and encouragement as we continue to navigate the current together!

CRN's 2025 Board of Directors. Absent: Erica Hall.

Introducing our 2025 Academic Scholarships Winners

By Gail Potts, Director

CRN is proud to announce the winners of the 2025 Academic Scholarships. This year we had two excellent candidates for the Sierra Woodward Sibling Scholarship. Therefore, we awarded both of these individuals, Emmerson Rico and Kylie Ellerbrock this year with $2,000. Our award for the CRN Academic Scholarship for an Individual with Cystinosis, went to Abigail Monaghan. The Deanna Lynn Potts Scholarship award went to Henry Sturgis.

Emmerson Rico

Emmerson will be attending Georgia College & State University, in Milledgeville GA. She is the sister of Corbin, who received the Deanna Lynn Potts Scholarship in 2023. Emmerson is pursuing a nursing career. Aside from her high academic achievement, she was involved in volunteering with several organizations. She was active in several of her high school's extra-curricular activities, received many awards in flag football, and worked as a sales associate. Emmerson was described by her teacher as a leader with integrity, committed, disciplined and humble. She was highly recommended for this scholarship.

Kylie Ellerbrock

Kylie Ellerbrock will be pursuing a nursing career at Illinois Valley Community College in Oglesby, IL. Her academic achievement was excellent. Her letters of reference expounded on her level of maturity beyond her years, her volunteer involvement in community organizations, and her passion for helping others. They felt she was an excellent role model for others. "Kylie focuses on her goals, works very hard to achieve those goals, and always finds ways to succeed," as stated by a former teacher. She came highly recommended for this scholarship.

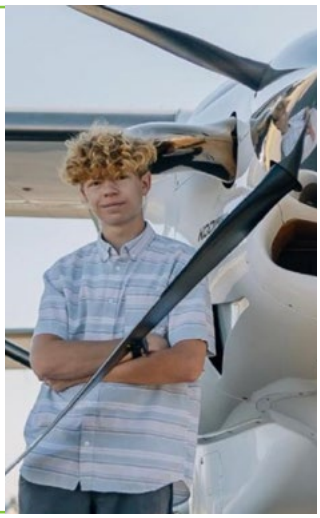
Abigail Monaghan

The CRN Academic Scholarship for an Individual with Cystinosis was awarded to Abigail "Abbi" Monaghan. She is attending Brock University in St. Catharines, Ontario. Abbi is studying to be a Child Life Specialist, so she can support children going through medical challenges, by not only acknowledging their diagnosis, but their whole experience. Her teachers indicated that she has risen above the challenges of cystinosis to find success throughout all of her school career. "Abbi is a young woman of exceptional character, strength and purpose," as stated by one of her teachers. She has accomplished much in spite of the unforeseen hospitalizations and medical complications common to those with cystinosis.

Introducing our 2025 Academic Scholarships Winners, continued

Henry Sturgis

The Deanna Lynn Potts Scholarship was awarded to Henry Sturgis, who will be attending Utah State University. His interest in aviation started at a very young age due to the numerous flights he and his dad took for medical appointments, with Henry always choosing the window seat. Flying offered a release of stress for him. Henry has worked for an aircraft company in an apprenticeship/intern position, assisting in parts delivery, inventory, and organizing, along with other tasks. He is bright, engaged, positive, and masters the tasks expected of him. He has been involved with his high school tennis team, a pilot training program, ACES and the Rotary Youth Leadership. He comes highly recommended for this scholarship.



CRN congratulates all of these exceptional young adults and wishes them well as they pursue their next adventures.

NORD®
National Organization for Rare Disorders

CYSTINOSIS

Copay & Medical Assistance

PATIENT ASSISTANCE PROGRAM

What is the purpose of this program?

Having a rare disease is difficult. Adding in the complex care required to treat or manage that disease and figuring out how to pay for it makes a rare diagnosis even harder.

NORD's Cystinosis Patient Assistance Programs offer eligible individuals financial support to pay for out-of-pocket healthcare costs that are directly related to the care and treatment of this diagnosis.

Who is eligible to apply?

This program is designed to help patients who:

- Have been diagnosed with Cystinosis.
- Are a United States citizen or U.S. resident of six (6) months or greater with evidence of residency such as a utility bill showing the patient's name and .
- Meet the program's financial eligibility criteria.

What is the application process?

Patients may be referred to the program by their health care provider, their case managers, or they may self-refer.

A NORD Patient Services Representative will guide the applicant through the application process and verify eligibility for inclusion in the Program.

Awards are based on meeting eligibility criteria, funding availability, and are made on a first-come, first serve basis.

What is NORD?

NORD, the National Organization for Rare Disorders, is a 501(c)(3) organization, is a patient advocacy organization dedicated to individuals with rare diseases and the organizations that serve them. NORD, along with its more than 300 patient organization members, is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

NORD was founded by families struggling to obtain access to treatments and whose advocacy for change led to the passage of the Orphan Drug Act in 1983. NORD assists eligible patients (those with medical and financial needs) in affording the treatments and medical services their healthcare professionals have prescribed.

Alone we are rare. Together we are strong.®

How do I get more information and apply?

Contact NORD's Cystinosis Program

Monday-Thursday 8:30am – 7:00pm ET
Friday 8:30 am – 6:00pm ET

Phone: 855-201-5087

Email: cystinosis_assist@rarediseases.org

The quickest way to apply is on NORD's website at:
bit.ly/patient-assist

Scroll down to Cystinosis and click Apply Online.

US MAIL to: NORD
Attention: Cystinosis Program
7 Kenosia Avenue
Danbury, CT 06810

What kinds of assistance can I request from NORD?

- Individuals who have health insurance may be assisted with financial assistance to cover health insurance deductibles, copayments & coinsurance costs.
- For individuals who do not have health insurance, NORD may be able to assist with out of pocket expenses incurred in managing this diagnosis

Some examples of these expenses may be

> *Cystinosis related medical office visit or consult.*

> *out-of-pocket cost for medications prescribed by your physician to manage your cystinosis diagnosis.*

> *out-of-pocket costs for lab services or radiological services.*

> *physician prescribed supportive therapies including ophthalmology, urology, and nutrition services.*

> *travel related expenses for cystinosis related medical appointments.*

Once a patient is accepted into the assistance program(s), how long are they eligible?

Awards are issued for a calendar year.

Patients are encouraged to reapply annually if continued assistance is needed.

Program assistance is dependent on funding availability.

What happens if an applicant does not meet the criteria of the Electronic Income Verification?

The NORD Patient Services Representative will offer to e-mail, fax, or mail the brief program application and disclosure forms to the patient. The applicant may then complete the application, sign the disclosure form, provide the appropriate financial documentation to verify financial need, and return them via fax, email, or USPS mail.

How does NORD demonstrate compliance with regulations required of charities?

- NORD independently designs its patient assistance programs based on the needs of specific patient communities.
- No pharmaceutical company or donor controls or influences our programs.
- Our patient assistance decisions are based on consistently applied financial eligibility criteria and diagnosis only.
- Patients have their choice of health care provider, treatment and treatment location, and can make changes at any time.
- Patients' privacy and well-being are priorities at NORD. We do not share or provide patient names or data with donors, nor do we disclose or identify donors to patients. Patients are able to make the choices that are best for them because NORD's assistance covers all FDA-approved products available for a diagnosis. Our programs also help with more than medication: patients can use their funds to pay for other physician prescribed services related to their diagnosis such as, laboratory and diagnostic testing, physical and occupational therapy, durable medical and adaptive equipment, and travel to medical appointments.

CRN Offers Memorial Fund

By Karen Gledhill, Secretary

The Cystinosis Memorial Fund was created to give individuals with cystinosis the opportunity to receive a grant of up to $1,000. The grant initially could be used to pay for a range of expenses from a college class to a new software program or a meal plan at school.

It has since added other categories such as muscle wasting prevention programs, exercise programs, nutrition counseling, various types of yoga, even home exercise equipment.

We have been pleased to award various grants this past year and hope you will consider applying.

If you are interested, please go to https://cystinosis.org/cmf.

"This award will allow me to build and maintain the physical and mental strength to continue a life of outdoor adventure and recreation with my best friend (dog), Sadie. I appreciate the help and so does my body."

"If you have the desire to pursue any type of physical fitness program or hobby, but the price stands in your way, apply! Do not let a price tag deter your hopes and dreams of living life to the fullest! It's okay to ask for help because it means more doors will open for you and your body will give many more years and miles."

Heidi Hughes

Fall 2025 Development Update: The Power of Us

By Jonathan Dicks, Vice President of Development

Dear Cystinosis Community,

As 2025 begins to wind down, I've found myself reflecting with deep gratitude on what we've accomplished this year—together. Our momentum has been undeniable. From direct-impact programming to groundbreaking advocacy, research partnerships, and community engagement, CRN has made tremendous strides—and it's because of you.

One of the most unforgettable highlights of this year was the 2025 CRN Family Conference in Grand Rapids, Michigan. Themed "Navigating the Current Together," it was more than a gathering. It was a living embodiment of our mission—bringing families, clinicians, researchers, and advocates together in one space to learn, heal, and chart a course forward. The robust programming, the emotion in every shared story, and the opportunities for connection reminded us all, of what's possible when we invest in each other.

That level of programming—the kind that brings our community together, sparks lifelong connections, and offers space for both science and support—is made possible through consistent, collective investment. Whether through major grants or grassroots giving, every contribution plays a role in shaping the experiences we offer. It's not about one person doing everything—it's about all of us doing what we can. When that happens, the ripple effects reach further than we can measure.

Giving Tuesday: Fueling What Comes Next

This Giving Tuesday, we invite you to recommit to the work we're doing. Your contributions ensure that families facing a new diagnosis receive emergency support and care packages, that young adults with cystinosis have scholarships and mentoring, and that adult patients have a stronger platform through the Cystinosis Adult Council (coming soon). It ensures that no family walks this road alone, and that our future is shaped by those most affected.

Finn Dicks.

Family love!

And we're not stopping here.

In 2026, we plan to roll out a community fundraising playbook—a resource designed to make it easier for anyone, anywhere, to start making an impact. Whether it's a birthday fundraiser, a local bake sale, a golf outing, or a company match, we want everyone to feel empowered to do something. Big or small, your effort matters—and we're going to give you the tools to make it easier than ever to act.

We Are 30 Million Strong

As members of the rare disease space, we know the numbers well—over 10,000 rare diseases, and yet

Fall 2025 Development Update: The Power of Us, continued

only 5% have an approved treatment. We also know that the burden is real—in 2019 alone, the economic impact of rare diseases exceeded $1 trillion in the United States.

But beyond those staggering numbers is a deep well of human strength.

We stand on the shoulders of those who came before us—parents, patients, researchers, and volunteers who refused to let the world ignore cystinosis. Their fight made our progress possible.

Now it's our turn to build on that legacy.

If CRN has made a difference in your life—or in the life of someone you love—please consider making a donation this Giving Tuesday. Help us keep building programs that reach hearts and move science forward.

Together, we are the difference.

With heartfelt thanks,
Jonathan Dicks
President | VP, Development
Cystinosis Research Network

Elle, Teddy and Finn having fun.

AVAILABLE IN U.S. AND CANADA

Care Package Program

CYSTINOSIS.ORG/CARE-PACKAGE

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.

"...thank you so much for the care package you sent my daughter, Myla, who was recently diagnosed with cystinosis. She has been enjoying going through all the 'surprises' and it's been making her smile! We really appreciate all that you do for this community. Thank you again!"

Kelley

Myla, enjoying summer fun.

Cystinosis Care Package Program

We're proud to share that several CRN Care Packages have been delivered since our last newsletter. These personalized packages provide much-needed support to those navigating the challenges of cystinosis.

Available: Kits Based on Points in the Journey

- Newly Diagnosed
- Kidney Transplant/Dialysis
- Wellness/Mental Wellness
- End Stage/Bereavement
  (loss of a loved one with cystinosis)

How to Sign Up

Request a complimentary package for yourself or a loved one by visiting the link below. Available for delivery within the U.S. and Canada.

cystinosis.org/care-package

Family Support Update

By Chelsea Meschke, Vice President of Family Support

Brian, Chelsea, Jaxon and Myles Meschke at the 2025 CRN Family Conference.

Jaxon and Myles Meschke picking strawberries.

Our recent conference was a beautiful reminder of what true family support looks like. The outpouring of kindness, compassion, and solidarity throughout the weekend was inspiring—not only for me and my family, but for everyone who attended. Seeing our community come together with such grace, love, and commitment truly showed the strength we have when we stand side by side.

A strong community is built on more than just connection—it's about showing up for one another. It's checking in, lending a hand, celebrating the victories, and walking together through the challenges. When we do this, we don't just create a community—we create a family. That sense of family was felt deeply by so many of us in Grand Rapids this summer.

The extended Meschke family attend the CRN Family Conference together.

As your VP of Family Support, one of my goals is to extend that spirit of connection beyond the conference. We want every individual and family touched by cystinosis to know there is always a place to share, to love, to grieve, and to grow—no matter where they are.

To continue this support, we will be hosting regular Family Support Zooms. These virtual gatherings are a chance to share experiences, provide encouragement, and remind one another that no one walks this journey alone. Keep an eye on our social media channels and website for announcements and join us — we'd love to see you there!

We also encourage everyone to take advantage of the resources available:

- PCs for People – Free computers or laptops for eligible applicants
- Patient Advocacy Network (PAN) – Support with medication copays and more
- NeedyMeds – Assistance with healthcare costs
- NORD – Financial and patient support resources (pages 11-12)

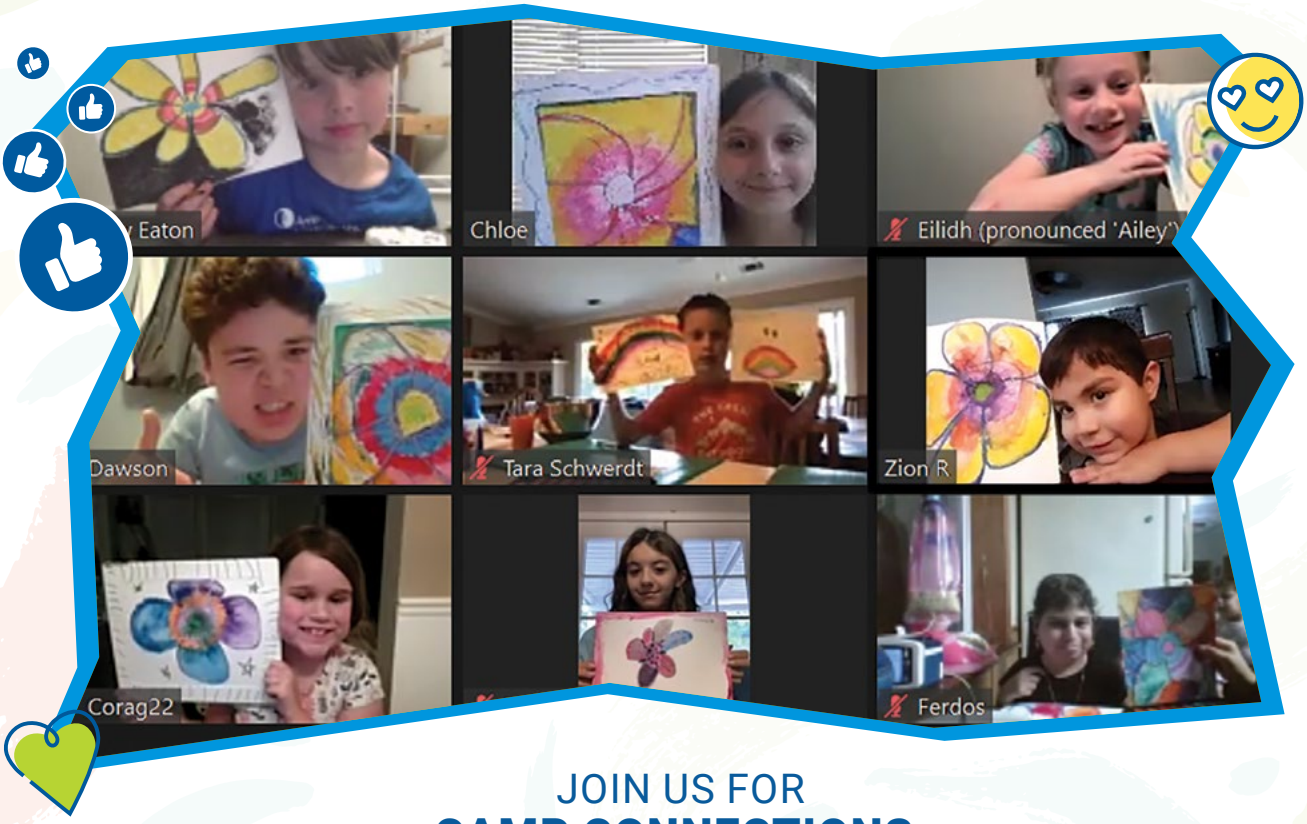
Please don't hesitate to reach out if you need guidance connecting with these programs or other forms of support. We are here for you—and I look forward to continuing this journey together.

With gratitude,
Chelsea Meschke
Vice President of Family Support

CAMP CONNECTIONS

let's go on a virtual adventure!

American Kidney Fund®



JOIN US FOR
CAMP CONNECTIONS: Cystinosis

Give your child the gift of **fun and friendship** by enrolling them in **AKF's monthly virtual adventure tailored for pediatric patients and exclusive extra activities for kids with cystinosis.**

Camp Connections: Cystinosis brings kids from across the country together through Zoom to form friendships with peers who understand their journey living with this rare disease. With interactive activities like arts and crafts, STEM projects and game nights, your child can enjoy moments of joy, creativity and connection from wherever they are. Each new camper receives a welcome swag bag to kick off camp and monthly activity kits.

Help your child find a place where they are accepted, celebrated and connected.

LEARN MORE AND ENROLL TODAY!
kidneyfund.org/camp-cystinosis

Camp Connections is supported by AMGEN and SCHATTNER FOUNDATION

Good News

Editor's note: *In this issue of our Good News section we are featuring Kacy Wyman as they celebrate her ten-year transplant anniversary. The May celebration was attended by over 180 friends, family, and supporters. Also featured is Valerie Bishop, a lovely woman who shared her over sixty-year journey of living with cystinosis, including her 1974 kidney transplant that is still functioning! We should note that Valerie's cystinosis presentation was atypical. Her symptoms were milder than what is usually seen in nephropathic cystinosis, and as a result her treatment regimen was different than what is typical.*

Cheers to 10 Years: Kidney Celebration Party

By Kacy Wyman

In May 2025, I celebrated a special milestone—10 years since receiving my kidney from my dad. To celebrate this event, and to honor all of our supporters, my family hosted a "Kidney Anniversary" celebration filled with gratitude, laughter, and love.

The event brought together 180 friends, family members, and supporters who have been part of this journey and have supported me since my diagnosis in 2006. It was a beautiful reminder of the gift of life, the power of community, and the incredible people that have surrounded me every step of the way.

While it was not intended to be a fundraiser, our community of supporters still donated upwards of $13,000. We are thankful every day for the support shown to our family and to the CRN!

Kacy and her family welcoming guests to the celelbration.

Kacy's friends and family joined her in celebrating this life-changing milestone.

Good News

50 Years and Counting!

By Valerie Bishop

Valerie at the Amway Grand Hotel, location of the 2025 Cystinosis Family Conference.

Valerie with her former pediatrician, Dr. Miriam Daly, who recently celebrated her 100th birthday.

My name is Valerie Bishop, and I am from Michigan. It was an invaluable experience for me to attend the 2025 Cystinosis Research Network Conference in Grand Rapids, Michigan. I was very challenged and moved by the cystinosis patients, parents and families, doctors, and professionals.

The Beginning

Born in 1961, I am now 64 years old, and so very grateful to be asked to share my story. I am blessed to have lived my adult life in good health. I have been able to earn several degrees, and complete my career as a high school teacher. I am married and continue to be very involved and active in all of life. In contrast, I was not well as a child, and was eventually diagnosed with cystinosis. This was relatively late, not because I didn't exhibit the disease from birth, but because it was so rare and unheard of even by medical professionals. At age 3 my local pediatrician, Dr. Miriam Daly, (who I was able to thank this year at her 100th birthday celebration), saw that I had abnormal lab results. She set me up with specialists at the University of Michigan Hospital. As I have been told, it took several years for a team of doctors to give a diagnosis. Little was known about cystinosis prior to the 60's and 70's. Few children lived to adulthood since research and information was limited, and there was no treatment. We were told in the late 60's that an intern put a name to what I had, after his extensive research.

A Perfect Match!

Thankfully, because of a diagnosis, I was given appropriate supplements and medications needed to keep me going and growing (I made it to 5 ft. tall). As in true form with cystinosis, my kidney function diminished by age 13. I went on dialysis for about 3 months while they tested my family members as potential kidney donors. We were told my sister Janet, 6 years older than I, was an A1 match, the next closest match to an identical twin!

I had a kidney transplant over Christmas break in 1974. My sister was a nursing student, and it worked out for her to donate a kidney over her school break. A donor's surgery was very extensive at that time. In fact, it seemed Janet's surgery was much more invasive than mine and her recovery was arduous. I am thankful that I have never experienced kidney rejection, nor has Janet had related complications from her kidney donation.

So, I had a month-long stay in the hospital. I continued on Imuran and prednisone for anti-rejection for many years. I never experienced any signs of rejection and have always had normal blood labs. I now take Cellcept, and a low dose of prednisone.

After my transplant in 1974, my family and I thought that was the end of my cystinosis story. I did not have a follow-up for cystinosis for years, just routine blood labs and annual University of Michigan Hospital visits at the Renal Clinic.

Good News

50 Years and Counting!, continued

Learning More

In about 1994, I heard about a cystinosis conference in the Chicago area, and decided to attend this, since it was relatively close by. There, I learned for the first time that my disease was still with me…it hadn't disappeared because of a transplant.

Through that conference, I connected with Dr. Gahl and went to the NIH to become part of his clinical study. It was confirmed that I did have cystinosis; my genetic mutation was confirmed. I had an elevated cystine level, but not as elevated as others with cystinosis. I learned that I have an intermediate form of cystinosis that keeps me from experiencing many of the debilitating symptoms associated with this rare disease. I am still doing well without the cysteamine treatment. I have been allowed to decide if the benefits of this treatment outweigh the negative side effects that this medicine causes. For now, I have decided not to take it, but to re-evaluate that decision often with the help of Dr. Elizabeth Ames, and formerly Dr. Jess Thoene, at the University of Michigan. I am reconsidering Cystadrops, since my vision seems to be worsening from the crystals in my corneas, while hoping for additional options in the future.

The Path Forward

I appreciate the knowledge and resources gained at our conference in July. It made me consider how I might be more proactive with treatment options and clinical studies if it may help others realize a longer and healthier life.

My heart goes out to so many I spoke with at the conference who said they were on their second, third, fourth, or even fifth transplant. It is only the grace of God that has gotten me this far along in life. I am not deserving of this and feel very blessed.

All I can say is...it's not fair. Children still die of this disease. Those with this rare disease live lives that are difficult and struggle through treatment, physical and cognitive challenges, and social and emotional difficulties. I am now benefitting from the knowledge gained through those who have struggled for years or have died at a young age with cystinosis. This is a breakthrough generation for this rare disease. It has been during my lifetime that the majority of the research has occurred to give many of us the ability to overcome. I have not had the same severity of this autosomal recessive genetic disorder as many of those I met at the conference. However, I too live with the realization that there is the unknown always lurking around the next corner of life that could cause "end of life" disease effects. We all must continue to live by faith and not fear what is ahead. Each day is a gift!

I want to thank you, the cystinosis patients, parents, and medical professionals. My parents' and pediatrician's proactivity is what got me the medical help at an incredible university hospital. I thank those who, since before I was born and during my lifetime, studied this rare disease, developed new and improved treatments and solutions, and paved the way for further research.

Only God knows the number of our days. I have been blessed already with 64 years of life. Even with 50 years with a transplanted kidney, I do not hold the record (I ask my nephrologist every couple of years), However, I may be one of the oldest survivors with the diagnosis of cystinosis. For this I am thankful and do not take my health for granted.

Valerie Bishop and her husband Will Dutcher enjoying the Dinner Dance on the final night of the conference.

Valerie visiting the Judean Wilderness in Israel.

Additional CRN Family Conference Photo Highlights

Board member, Cheryl Simoens.

Adults living with Cystinosis Panel.

Chelsea Meschke, speaking about Advanced Directives.

Doctors Ewa Elenberg, Joshua Zaritsky, and Fredrick Kaskel.

Dr. Rachel Bishop.

Friends at the speaker dinner.

Heather Rothrock and daughter, Briley.

Welcoming first-time attendees to the conference.

Speak Up Speak Out participants.

Making Memories in Grand Rapids

Enjoying each other at the dinner dance.

Best friends.

Dancing the night away.

Dinner dance fun!

Family introductions.

Finn Dicks with Myles and Jaxon Meschke at the 2025 CRN Family Conference.

Friends making memories.

Heidi Hughes and Emily Mello.

Martina, Jessica and Herberth Sigler.

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Twenty-Six Years Post Kidney Transplant

Reflecting on a life-changing, life-extending event I'll always be grateful for

By Steve Schleuder

Steve, pre-transplant with his brothers Chris and Eric.

Introduction

Recently, I've begun to get to know a lovely writer and beautiful person who goes by the name Veritas Knox. The first thing I read from Veritas on the Medium platform I belong to, was about her recent double transplant. It was performed with a robotic surgery machine known as Da Vinci that saved (and transformed) her life. Her passion, intelligence, and clarity hooked me immediately.

I shared with her that I just celebrated the 26th anniversary of my own organ transplant surgery (a not unheard of, but sort of rare thing in the medical world).

Reading about Veritas's current journey—and sharing a bit of my own—has made me reflective. I've shared pieces of my kidney transplant story in various writing pieces, but I'm going to attempt (as my official kidney transplant anniversary was June 21st) to share as much of the full story as I can remember and feel comfortable sharing.

An Excruciating Pain Through the Head

It was nighttime when an excruciating pain went through my head. Being ten years old, you wouldn't think I had been through all that much pain. But you'd be wrong.

When you're born with a chronic illness (cystinosis, in my case), pain becomes a frenemy—always there, even when you can't feel it—like an invisible parasite you sometimes forget is there.

Have you ever had a really bad headache? One so bad that it drives all thoughts out of your head, and the only thing that exists is the pain? Pain in its purest and most primordial form that refuses to be ignored. It demands your attention, and nothing else matters in the world, but that you and it are one and you can't escape it.

This was the invisible monster that was attacking my young body in the dead of the night.

I don't remember how long I lay in that bed feeling that pain. Looking back through the eyes of nearly forty-year-old Steve, it feels a lot longer than it probably was. Our perception of time and past events has a weird way of warping and distorting the further we get from them.

Ten-year-old Steve probably experienced this all in a flash. A white-hot searing agony took over his capacity to be, seemingly in an instant, out of nowhere. He probably burst out of bed screaming, waking his parents up.

I do remember my mom taking my blood pressure (with a machine she just happened to have purchased that day through a weird case of divine timing), and I remember she said it was too high. Everything else fades to black.

The next vague memory is of staring up at two tired parental faces in my

Twenty-Six Years Post Kidney Transplant, continued

"home away from home"—Mott Children's Hospital, in Ann Arbor Michigan.

Filling in the Blackout Blanks

Did it involve a missing tiger and Mike Tyson singing a horrible rendition of the Genesis song "In the Air Tonight"? Alas, no dear readers!

A "fun" ER visit ensued—I fainted, stopped breathing, got airlifted to the hospital by helicopter, and thankfully, the medics got my breathing going again.

Since I don't have this memory, it feels abstract to me. Like it happened to someone else. A weird part of me wishes I could remember flying in the helicopter. To this day, it's been my only helicopter flight.

After this drama-filled adventure, I was tested, and it turned out my kidneys were failing. You might think, kidneys failing at age ten, man, that's young! However, context is the key to sort of understanding…maybe.

The fact my original kidneys lasted till I was ten is a miracle in-itself-considering I was born with 50% of my kidney function gone. A miracle made possible by being part of an experimental medical trial in which I was one of a few people who was given a new drug treatment.

(Cue the subtle wink, wink, nudge, nudge to not defund vital medical research that saves, and extends lives, and increases the quality of life for millions of chronic illness patients of all ages! Wink, wink, nudge, nudge!)

It was shortly after this that my family members who were capable of being tested to see if they were a donor match were tested. At first, it appeared that no one was a match.

Steve in 2014, after college graduation.

However, by the incredible power known as "Mom love," in which compassionate doctors took pity on an overstretched, heartbroken mother, my mom was tested a second time and was a match.

Let's Get a New Kidney Like It's 1999!

My kidney transplant was unusual for 1999 (probably even unusual by today's standards). They decided it would be best to remove both my original kidneys and give my new one its own solo bachelorette pad (bachelorette because the kidney came from my mom).

The first surgery removed one kidney. After the first surgery, I was put on dialysis until I healed enough to do the second surgery. Six weeks later, the second surgery removed the other kidney and implanted the new one.

The Not So Fast Times of 4th Grade

Before the transplant, my mornings consisted of being driven by my mom to Ann Arbor (30 minutes from where we lived), then I was plugged into the hemodialysis machine for a little bit, while I watched the Christopher Reeve Superman movie, and played Super Mario World. My nurses were fantastic. They were two burly bearded guys with big smiles, hearts, and wicked senses of humor.

After dialysis, I would go to school. It was extremely exhausting, but my school system had caring, compassionate teachers. One of them offered me a couch to sleep on whenever I got too tired to work on school stuff. I developed a massive addiction to Jelly Belly jelly beans that took years to kick. There wasn't any one flavor I particularly enjoyed. I liked to grab a bunch of them at once and see what kind of monstrosity of flavor

Twenty-Six Years Post Kidney Transplant, continued

Steve, makes a "new friend" while on vacation.

I could create for my taste buds to explore. It was like Russian roulette for my taste buds.

June of 1999 came, and on June 21st the final surgery occurred. I remember a lot of pain, and eating pizza way before I was supposed to. I also developed a new childhood food addiction: the strangely wonderfully sweet, hot mix of Hawaiian Punch, a frosty, and a spicy chicken sandwich.

Reflecting on 26 Years

It's impossible to encapsulate what a momentous change that transplant was for me. Not the least because I hit puberty exactly when I got the transplant. Want to experience something wild? Try going through puberty while on new transplant drugs.

I gained the weight I needed to gain to abandon my skeletal look. My hair and eyebrows changed colors (orange, black, and finally back to blonde). At one point, the roots of my platinum blonde hair turned black. Life is strange, and drugs are weird.

I'm not going to say I haven't had hard times (or that I don't still, occasionally, have hard times), but hard times are part of being human regardless of what's going on in your life.

Life is precious, and fleeting, and beautiful, and difficult. Good things can coexist with bad things. Even though my life hasn't always been easy, I remain an optimist.

Like a good believer, I have my pessimistic moments—but they aren't my default.

If you've never almost died, it might be hard to understand how someone with a lifelong illness can always return to optimism. But when life is precious and fleeting, every moment becomes beautiful—even when it's ugly.

Thank you for reading and happy anniversary to any transplant recipient, whatever part of your journey you're on.

Carrying It Forward

My life today—messy, unpredictable, but still mine—is a direct result of the precious gift my mom gave me just over 26 years ago. I carry it with me in every breath, every laugh, every conversation (spoken or written) I have with friends and family.

Thank you donors past, present, and future. You make more futures possible.

Final Remarks

If you feel moved by this story, please consider supporting organizations that fight for chronic illness patients and fund the research that saves lives like mine. Consider being an organ donor if you're eligible. You have no idea how much that changes people's lives.

Steve's Orgs

Here are a couple of organizations that I belong to:

I am the Vice President of Advocacy and Education at Next Generation of Cystinosis (nextgencystinosis.org).

"Next Generation of Cystinosis is a non-profit, volunteer-run organization founded in 2019. Our vision is to be an organization run by and for adults 18 years and older affected by cystinosis. Our mission is to have open and honest dialogue about the plethora of issues that affect them."

I am also an active participant in various Cystinosis Research Network events (cystinosis.org).

"The Cystinosis Research Network is an all-volunteer, non-profit organization dedicated to supporting and advocating research, providing family assistance and educating the public and medical communities about cystinosis."

If you are interested in finding out more about these organizations, please visit the links provided.

Margot's Story

By Melissa Crane

"The first year is the hardest, but it gets better," they said. We heard this time and time again, always with compassion and deep recognition from those who had once been where we were. Who truly understood what we were going through. Who were familiar with the devastating feeling left lingering in the heart long after being told their child has nephropathic cystinosis.

It was October 17, 2024, two weeks into our stay at Oishei Children's Hospital, when the genetic counselor entered our hospital room in the PICU with the results. Even though we had no idea what it was, and what this truly meant for Margot, for us as parents, and for our extended family, at least we finally had an explanation for the symptoms that were leading the doctors on a wild goose chase. The same symptoms that had gotten progressively worse over the past seven months.

Bath time fun!

Something was Different

We first noticed something was "different" when Margot was around six months of age. Her interest in water seemed excessive and she began spitting up her formula more than she's ever done before. Her pediatrician assured us it was nothing to be concerned about and sent us home with a prescription for an antacid and a referral to a GI doctor. In the months that followed, Margot was treated for constipation and GERD, and our collection of medications to help with the incessant vomiting grew. She was slowly losing interest in food, especially throughout the day. She only wanted water, and the amount of diapers we were going through reflected it. At night she would wake to guzzle bottles, and rarely kept them down. Her weight was declining, though we didn't realize it. We just assumed she was tiny and would eventually plump back up! It was normal for babies to be all different sizes, is what we told ourselves. No sense in getting caught up in the comparison game, right? Perhaps that was true, and we still brought her back to her doctors to express our concerns. Once again we were told not to worry, and were sent home with yet another medication, this time one that would help relax her stomach and increase her appetite, as well as a high caloric formula to assist with weight gain.

Of course we wanted to believe that she was ok, but after another long night of screaming, crying, and cleaning up multiple bouts of puke, we knew she wasn't. Something was wrong and enough was enough. We brought her to the emergency room. She was three days shy of being 13 months old, about 28 inches long, and a mere 15 pounds. Lab tests revealed dangerously low levels of electrolytes as well as a UTI.

We had no idea!

Looking back, I wish we had gone sooner. I wish a doctor would have suggested we get her electrolytes checked, or tell us to go to the hospital, or to at least pause and think that maybe there was something else going on besides the typical stuff they saw day in and day out. I wish I would have recognized the dark circles under her eyes. I wish I would have listened to my intuition that told me a long time ago, "Something is wrong with her kidneys." The guilt was strong at times, and it was the first lesson in granting ourselves some grace, something that we would continuously have to do as we navigated the road that lay ahead.

When we were first admitted, the main goals were to treat the UTI with antibiotics, give her fluids, balance her electrolytes, and get her fed. The vomiting continued,

Margot's Story, continued

Margot at the hospital.

however, and it wasn't long before we were transferred to the PICU with a potassium level of 1.7. They didn't understand why her levels weren't stabilizing. How did the UTI fit in? What was causing all of this to happen? They did multiple tests, including a CT scan of her brain to check for any tumors, each time coming up normal. Luckily, it was early on in our stay that the nephrologist suspected Fanconi Syndrome, she just didn't know what it was secondary to. She suggested we do a genetics test to see if anything would show up.

During that week while we waited for our results we worked with the dietician to help Margot gain some weight. Her body was so depleted, and she barely took a bottle. An x-ray of her wrist revealed signs of rickets. It pained me to hear the words "Failure to Thrive" whenever a doctor would present her case. Even though I knew it was just a term that was used, it felt personal, as if it was my fault. It felt like I was failing my daughter when all I wanted was to feed her, nurture her, and see her thrive. Margot received an NG tube to help us get some food into her belly. She was on and off of IV fluids throughout each day. Her veins began to collapse from how much they needed to use the lines. It wasn't long before they went through all the available spots in her hands, arms, and feet. Her frail body looked like a canvas splattered with bruises. She needed a PICC line, which sounded super scary at the time and ended up being such a blessing.

Diagnosis and a plan

Receiving the official diagnosis helped with putting a plan together as we moved forward. Thankfully, Margot's labs were beginning to even out, and the UTI was cleared. She continued to struggle with food, though, and the caloric intake they wanted her to receive in order for her to "catch up" was a lofty goal and barely touched. This became our biggest challenge in the days, weeks, and months to come. All we could do was our best and hope that it was good enough.

They released her from the PICU onto the tenth floor where we stayed for the next few weeks. We always knew when she was feeling good because she would shine. Her smile would light up the room. She would dance to Elmo songs in her crib, and asked to go on wagon rides down the halls, around-and-around, where she'd wave to anyone and everyone on her path. During that time, we decided that a gastrostomy was in her best interest, and indeed it was. She began receiving everything through her g-tube other than water. There were even a few days following the surgery that she didn't throw up! Obviously, the winning streak didn't last but we were still making progress and headed in the right direction.

On November 1, 2024, after celebrating her second Halloween parading around the hospital as bunnies, we were discharged at last! Her labs looked good, and she had gained weight for three consecutive days. With Procysbi, supplements, and plenty of syringes in hand, it was time to go home. After 29 days we had become accustomed to seeing lab results day and night, and receiving perfectly dosed meds at their designated times. Being on our own meant that we had to trust her electrolytes would remain stable throughout the week. It meant that we had to get in the habit of doing everything ourselves. No more safety net. As much as we missed being home (our bed especially) it was definitely 'nerve-wracking' to walk away.

More Challenges!

Things went ok for a while. She wasn't losing weight, and her weekly labs were looking pretty good. She even started walking the day after Christmas! The vomiting, however, was still a huge issue. There was so much trial and error with how to administer her meds and feeds. What seemed to work for a few days would then backfire. It was beyond frustrating and didn't make sense. We tried pushing bolus feeds by hand, by pump, by gravity, you name it. We played with dosage rates and amounts she could tolerate. We did everything we could, yet continuously failed to hit that magic number the doctors and dieticians insisted we reach. Not only were we unable to reach the required amount (ml) of formula, but the formula itself wasn't even at full concentration yet. We had

Margot's Story, continued

to dilute it in-order-for her to tolerate it! We even began catching her vomit to push back in as it would happen in the middle of a feed or just after being given a med. We figured what the heck?! It was just inside of her so why not put it right back in?! Honestly, it was a nightmare and if that wasn't enough, constipation crept back in which led to more vomiting, ultimately landing us back in the hospital for a second extended stay.

An abdominal x-ray revealed a significant amount of poop, and they wanted to do a supervised "clean out." It began with a suppository, followed by 2 enemas and ended with her hooked up to a giant bag of GoLightly aka MiraLAX (which I specifically said I did not want her to have). She had a few bowel movements after receiving the first three things, but they still wanted to make sure all the poop was out. It seemed to be an absurd amount of this liquid laxative in my opinion. Once she began swelling up like a balloon I turned off the pump (against the doctor's orders which of course was frowned upon). We were told that they'd do another x-ray and if it still showed poop then they would have to turn the pump back on to administer the remaining amount. Lo and behold the x-ray was clear, but with that massive clean out came a complete reset to her system and her electrolytes went haywire. After a few days of getting her back to "normal" the discussion around calories (basically, her not getting enough) became the hot topic once again. Since it seemed as though we exhausted our options on successfully using her g-tube, it was decided that it be replaced with a gj-tube in order to have the ability to bypass the stomach and to reduce vomiting. It seemed like the ideal solution. We would use her g-tube for some small bolus feeds throughout the day and then whatever was leftover would be pumped low and slow overnight through her j-tube. We sent her off to OR for the replacement procedure and shortly thereafter she was returned to us without any instruction of what to do next. Since her GI doctor had an extremely busy off-site private practice, there wasn't the ability to easily communicate. And due to political reasons, the GI team at the hospital weren't allowed to treat her or advise us.

She was hooked up to the pump via the J-tube, and much like before it was set at a very slow rate and her formula was diluted by 50%. The plan was to increase the rate and strength of the formula incrementally until we reached the goal. When it came time to administer her first round of doses we were told to carry on as normal and deliver them via the g-tube. That evening she received Procysbi, and a newly prescribed laxative and come morning (after a second dose of laxative) she became violently ill. The happy little girl we knew and loved was pale and listless. Although she was exhausted from being up all night screaming in agony, she wouldn't allow herself to sleep. She lost interest in everything that once brought her joy. Neither the nurses nor resident doctors knew what to do for her. As we anxiously awaited instruction from GI, feelings of worry and guilt coursed through our veins.We began second guessing our actions on whether or not we did the right thing.

When the GI doc finally called, we were instructed to put Margot on complete gastric rest. From this point

Margot's 2nd Halloween, parading in the hospital with mom and dad.

on nothing was to go into her stomach other than water. All food, electrolytes, and meds were to be administered into the j-tube. This meant discontinuing certain medications that were intended for the stomach, the most important one being cysteamine. We needed to switch from Procysbi to Cystagon, and wait for a new script to be written, processed, and delivered. With Margot off this essential medication, the cystine accumulated and her tolerance dropped ultimately bringing her back to square one.

To help prevent her from throwing up we had to ration her water into 5ml sips while waiting a couple minutes between each one. We impatiently watched the second hand as it traced the clock's circumference while Margot screamed and begged for more water. It was devastating to see her like this.

Frustrations continue

The next few days were awful. I was so angry, felt abandoned, and alone. I didn't want to hear about the need to reach goals of unobtainable numbers when our baby was in clear and utter

Margot's Story, continued

Margot, trying out her sunglasses.

distress. I wanted the practitioners to understand the uniqueness that came along with having cystinosis. I wanted a comprehensive and holistic plan that was flexible and adaptable. I wanted the ability to easily communicate with our team and to be sincerely listened to. I needed compassionate and confident guidance so that I could feel safe to rest.

Learning how to work with the J-tube took time. Administering everything Margot needed through a port into an area of the body that isn't designed to stretch seemed impossible at first. The pressure in her intestines combined with the pressure in her belly from all the water she was drinking was just too much! We had to keep the pump at a super low rate. It felt like we were in the same predicament as before. Unfortunately, our ideal plan went by the wayside and no one really knew how to help us since most children who receive J tubes aren't drinking 2 liters of water a day! The only thing that offered us some relief was that we knew all the meds and food that did go in, she was indeed receiving.

We were all struggling mentally, emotionally, and physically. We were there for almost 2 weeks, and Margot was once again running out of viable veins for a line. On a positive note, her labs were looking good, and she wasn't losing weight. That said, however, we were told that we wouldn't be discharged until we were successful with running the pump at a rate that gave her enough formula and at full concentration.

We knew that this wasn't going to be simple and that she needed to go slow, so I advocated hard for us to go home. We knew what the goals were, and would undoubtedly continue working toward them, but at our own pace. Reluctantly, they agreed to let us go.

Positive progress, finally

It took us 6 months to reach the goals, but we did it! Things really began to shift once we stopped obsessing over numbers and began following our intuition. We allowed Margot's body to guide us. Rather than pushing her to get in those extra ml's of food, if it looked and felt as though she had reached her limit, we allowed ourselves to stop. We were thrilled to hear at our recent visit to the NIH that Margot grew two inches, gained weight, and was back on the growth charts. She's not only met, but has surpassed her estimated daily caloric needs allowing her more free time off the pump and room to begin utilizing her G-tube again. An x-ray revealed there is no longer any sign of rickets and her bone age is within normal limits. Overall, she's doing great!

The past year has been the most trying time of our lives. It was filled with trial and error which I'm beginning to understand is the name of this game. There isn't a "one-size-fits-all" with regards to treatment and nothing is set in stone. There are a few things I've come to realize in the short time we've been on this journey, and I need to remind myself of them every day. It is safe to trust your intuition. Adaptability is key. Acceptance offers peace. Living in the moment is the lesson. Patience is most definitely a virtue, and graceful love is the way.

"The first year is the hardest, but it gets better," they said. That it is, and yes, it surely does.

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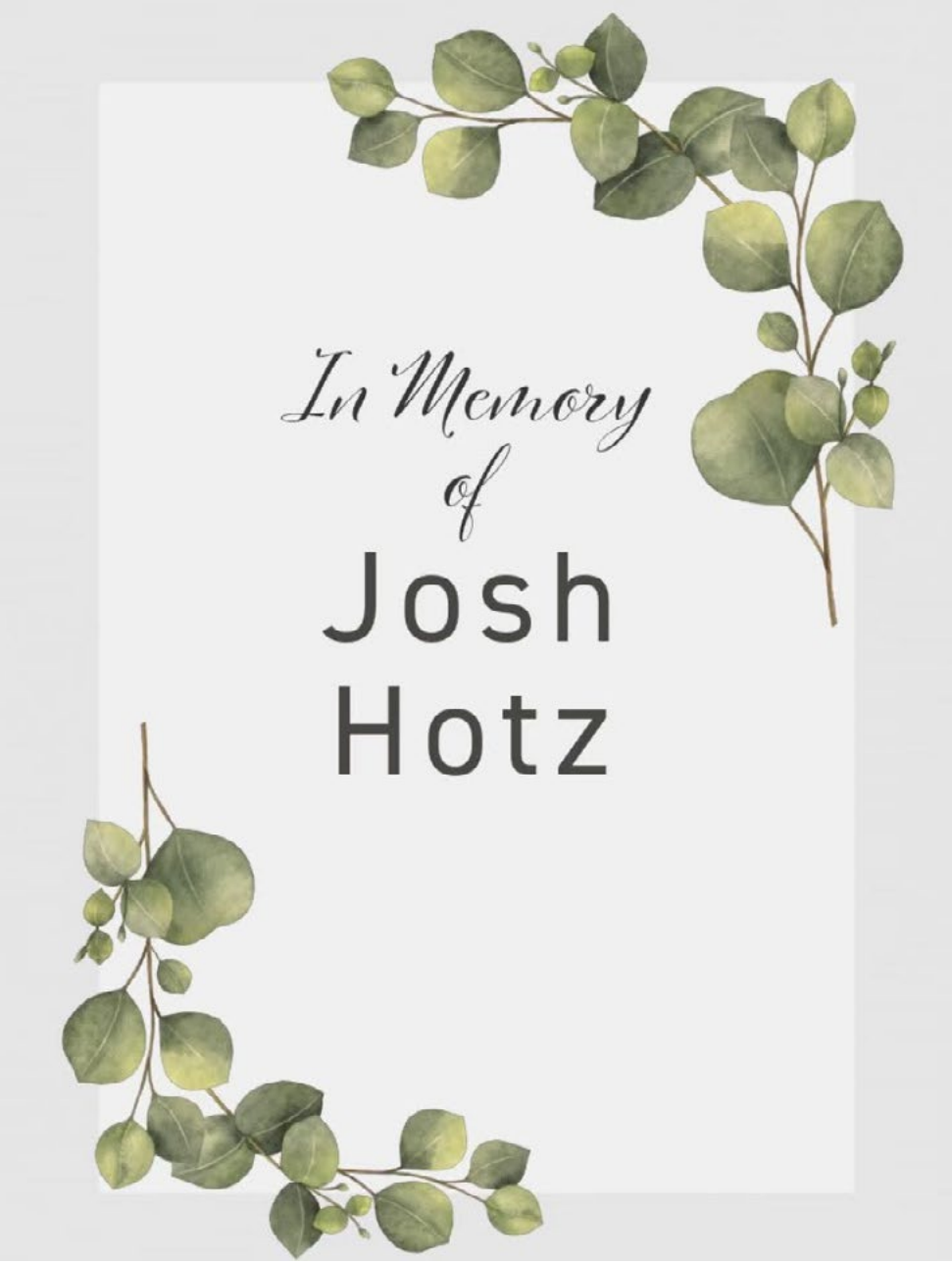
In Memoriam

Josh Hotz



The cystinosis community honors the life of a cystinosis trailblazer, Josh Hotz. Josh recently passed away peacefully at the age of 43 in California.

Josh's diagnosis in the early 1980s inspired his grandmother, Jean Hobbs Hotz, to create the first known support group for cystinosis families—a space where people could connect, share information, and realize they weren't alone. At a time when communication was limited to phone calls, fax machines, and a handful of newspaper clippings, their efforts laid the foundation for the thriving community that exists today.

Josh was part of the first generation of individuals with cystinosis whose stories helped shape a movement. His journey—and his family's vision—sparked connections, advocacy, and lifelong support for countless families.

We are deeply grateful to Josh and to Jean for their lasting contributions to the rare disease community. Their impact continues to be felt in every family that finds strength and solidarity through connection.

Our thoughts are with Josh's family, friends, and all those in the cystinosis community who are feeling this loss.

(Posted 5/15/25 on Facebook by CRN)

LIVE LIKE LAURA FUN FUND

By Lilly Grace Haynes

I am so beyond thankful for this opportunity! I knew one way or another I would be at Sam's graduation. I wouldn't miss it for anything. However, I did not know how I would make that happen. Flights are expensive, and quite frankly, I'm broke!

I told my mom a few weeks ago (multiple times), "Well, I don't know how I'm going to get these flights; they're so expensive. I'm saving to go see Sam graduate!" And coincidentally enough, I had been talking to her about it one night, and the next day she saw something about the Live Like Laura Fun Fund.

Best friends Sam and Lilly.

Cystinosis "cystas" Sam and Lilly, having fun.

She called me into the living room and said, "I know you've been saying you didn't know how you were going to afford the plane tickets to go see Sam graduate; maybe you should apply for this."

It takes a few weeks for them to get back, so I went ahead and applied.

The next morning I woke up to an email from Frankie Nance McGinnis. If you aren't in the Cystinosis community, then you probably have no idea what I'm talking about, so let me explain. Laura was a beautiful young lady who also had cystinosis, like Sam and me. She and her mom were (and still are) very active in the cystinosis community. Laura loved to travel and go on adventures. It was her favorite thing to do, and she lived her life doing what she loved. When she passed away, her mother, Frankie, started the Live Like Laura Fun Fund, which helps fund fun trips and adventures that individuals with cystinosis or their siblings want to do, but aren't able to afford.

It's a beautiful thing that I know Laura is looking down, smiling on. I didn't have to stress about money, or if I was going to be able to afford the tickets, and it was a big relief. She made it possible for me to be able to go, but also for Joe to go, so I wouldn't have to travel alone. I'm extremely thankful for Frankie and everyone involved in this fund. Because of them, I got to watch my "Cysta" (cystinosis sister) graduate high school and spend a wonderful weekend with her. And shortly after - It was a weekend full of love and fun, although we are both exhausted now. I'm thankful for these memories with my most precious friendship.

Not to mention, I'm extremely proud of Sam for graduating high school. It wasn't easy, and I know that. Now she's onto cosmetology school and is already rocking that too. Congratulations, Sam! And thank you from the bottom of my heart to Frankie and everyone involved in the LLLFF for making this happen for me!

Sam and Lilly celebrate Sam's high school graduation together thanks to the LLLFF.

Attendees of the Midwest Cystinosis Family Gathering met in Story City, Iowa, August 8–10, 2025.

The Midwest Cystinosis Family Gathering 2025

By Doretta Hoffmann

The Midwest Cystinosis group had another meaningful and heartwarming gathering, despite the rainy weather! It's great that even after 35 years, the group remains strong and we always welcome new families to join us. We met this year in Story City, Iowa on August 8th - August 10th hosted by the Hoffmann family.

During our gathering we received a recap from the CRN family conference, and the recap was interesting to everyone. We also all felt that our conversations picked up right where they left off—almost like no time had passed. Our connections are incredibly valuable for everyone.

We look forward to meeting again in 2026!

From left to right Andrew Hoffmann, Nick Wagner, Briana Dundon, and Shawn Reuter.

Cards2Warriors

Cards2Warriors provides has a variety of programs available to the cystinosis and chronic disease community. To learn more, visit cards2warriors.org.

Happy Mail is our ongoing support with a themed card 5 times per year and random handwritten cards sent throughout the year. It's fun because one never knows when a card will show up!

I'd love to encourage more caregivers to sign up for our Happy Mail as well. I'm constantly hearing moms say "someone needs it more" when they are invited to sign up.

Warrior Card Swap (www.WarriorCardSwap.com) is a monthly card swap/ penpal program for illness warriors only. Participants register once and then choose to opt-in on the months they are feeling up to it. They can choose to send digital or physical cards, make a friend (penpal), or simply brighten another warrior's day (no response expected). Send domestically or internationally.

Warriors in Crisis is our urgent short-term support for whenever an illness warrior (or caregiver) is experiencing atypical times (new diagnosis, surgery, loss of service animal, etc). They can nominate themselves or be nominated by a friend/loved one. Once approved, they will receive handwritten cards from our Card Crew members over the course of 4-6 weeks. We call it receiving a "slew of snail mail love".

"Your cards have brought more comfort and encouragement than words can fully express. Each one has arrived like a small piece of hope reminding me that I'm not alone, and that there are people out there who genuinely care."

Amy N, TN

Research Update: Cystinosis Study from The Australian Cystinosis Foundation

By Jenna McKenzie, The Australian Cystinosis Foundation

Researchers at The University of Western Australia have commenced a study investigating a potential new gene implicated in cystinosis. The project is being conducted in collaboration with the CCI Program and informed by the perspectives of individuals with lived experience of cystinosis.

Patients and carers, acting as Research Buddies, are contributing their insights and lived experiences. This collaborative approach strengthens the relevance and impact of the research for the cystinosis community.

We are excited to see what comes from the study.

THE UNIVERSITY OF WESTERN AUSTRALIA

Young Cystinosis Advocate Shares Story, Advances in Public Speaking Competition

By Jenna McKenzie, The Australian Cystinosis Foundation

Ethan, 12 years old and from NSW, Australia, recently took part in a public speaking competition at his school. He spoke about cystinosis and what it means to live with a chronic condition.

Ethan spoke incredibly well, placing in the top two in his class finals, and is now moving on to compete in the public speaking zone competition.

We can't wait to hear how he does and wish him all the best!

Link to the video of Ethan's speech (Facebook)
https://tinyurl.com/59ce2cs8

12-year-old Ethan advances in public speaking competition.

Started in 2018, Cystinosis Awareness Day was created to bring attention to our rare disease. Educating the general public and medical communities about cystinosis has the power to create a better future for the ~2,000 diagnosed and those awaiting a diagnosis.

Since its inception, Cystinosis Awareness Day has become a highly anticipated event. Each year we uncover ways to promote understanding within our personal networks and beyond. Cystinosis Awareness Day has also proven to be a successful fundraising initiative.

Thank you for continuing to fight for our cystinosis warriors on May 7th and every day! Email info@cystinosis.org for more.

Breaking Barriers: Launch of Johannesburg Chapter Brings Hope Home

By Gail Daniels, Executive Director and Co-Founder Cystinosis Foundation South Africa

A Milestone Moment

In July 2025 the Cystinosis Foundation South Africa officially launched its groundbreaking Johannesburg Chapter—marking a transformative moment for families across the Gauteng Province who have long faced the challenges of accessing support for this ultra-rare genetic condition.

As the sole organization dedicated to cystinosis in South Africa, the Foundation provides comprehensive support, advocacy, and most importantly, a sense of belonging to those who often feel isolated by their diagnosis.

Since 2010, the Foundation's Cape Town Annual Family Gathering (AFG) has been the pinnacle event for connection—a cherished tradition where families share stories, children laugh and play together, and hope is renewed. But for families in Johannesburg and surrounding areas, the 1,400-kilometer journey to Cape Town remained an insurmountable barrier to participation.

But now, in 2025, we were able to host our first AFG for our families in Gauteng.

It was a glorious day! For the first time, all nine Gauteng families gathered under one roof, breaking years of isolation with warm handshakes, heartfelt hugs, and the simple yet profound act of being understood.

The atmosphere was electric with emotion as introductions flowed, and stories were shared. Parents who had long carried their burdens in solitude discovered they were part of a larger tapestry of courage and resilience. The weight of isolation lifted as familiar struggles were met with knowing nods and supportive smiles.

The true magic of the day unfolded when young adults living with cystinosis met peers who truly understood their daily challenges—forming bonds that will undoubtedly flourish into lifelong friendships.

Meanwhile, siblings and younger children discovered the joy of play without explanation, engaging in games and activities designed by a dedicated volunteer team.

This event created safe spaces for every family member, recognizing that cystinosis affects not just the diagnosed individual, but entire family system. From discussions about treatment and compliance to carefree moments of childhood play, every

Cystinosis Foundation of South Africa, Johannesburg Chapter has their first family gathering.

Breaking Barriers, continued

aspect was designed to nurture both hope and healing.

This inaugural Johannesburg annual gathering represents far more than an event launch. It embodies the Foundation's unwavering commitment to ensuring that geography never again stands as a barrier to support, community, and hope.

The success of this milestone event signals a new chapter in South African cystinosis advocacy—one where distance dissolves, isolation transforms into connection, and families discover the extraordinary power of shared experience in overcoming life's most challenging moments.

As the Johannesburg Chapter takes root, it carries with it the promise that no family will face cystinosis alone. The Foundation continues to prove that even in the face of many obstacles and challenges, community, compassion, and collective strength can create miracles.

What started off as a financially impossible endeavour became a testimony of God's faithful providence!

The journey continues, and now it includes everyone.

Research Update

By Kristina Sevel, Vice President of Research

It was such a privilege to take part in the recent Family Conference and Scientific Symposium in Grand Rapids. The weekend brought together families, physicians, and researchers from around the world, and I left feeling deeply inspired by both the science being shared and the strong sense of community that continues to define CRN. The event reminds me of the incredible progress we have made—and how much more is possible when we work together.

I want to extend a heartfelt thank-you to all the families who traveled to Grand Rapids to be part of the event. Your presence, your stories, and your ongoing commitment to this community make every gathering meaningful and unforgettable.

One project that I am especially proud to highlight is the newly published Updated Cystinosis Patient Care Guidelines. This effort was made possible through the dedication and expertise of physicians and specialists across the globe, and it represents a significant step forward in ensuring that patients everywhere have access to the most current, evidence-based recommendations for care. The guidelines reflect not only the best available science, but also the shared commitment of our international cystinosis community to continually improve outcomes for patients and families.

Looking ahead, my goal will be expanding opportunities for research funding that directly benefits our patients. We know that every new study brings us closer to better treatments and, ultimately, a brighter future. Securing these resources is important, and I am energized by the potential we have, to support projects that will make a real and lasting difference in the lives of those affected by cystinosis.

Thank you to all the families, providers, and supporters who continue to inspire this work. Your resilience and dedication motivate everything we do, and I am grateful to share this journey with you.

Kristina Sevel, Chelsea Meschke, and Heather Rothrock, CRN Board members and friends.

Kristina, Mike and Grace Sevel at the CRN Family Conference.

Development of a patient-reported outcome to measure the health-related quality of life of children and adolescents with cystinosis

Drs. Katharina Hohenfellner and Julia Quitmann

The aim of the study is to develop and validate a questionnaire that measures health-related quality of life in children, adolescents and young adults with cystinosis.A validated, disease-specific questionnaire is an important tool for improving patient-centred care or evaluating a (new) drug or therapy.

Over the past year, we have consulted with numerous families to ascertain their views on the key aspects of cystinosis, daily life and quality of life for those affected. This information has been used to formulate preliminary statements for a questionnaire, which have been refined after feedback from the pilot test. The study will conclude this year and we hope to have information to share soon when information is reviewed.

Research Update, continued

Grant Awarded February 2021 by the Cystinosis Research Network and Cystinosis Ireland

Perturbations in the V-ATPase Pathway Drive Pathology in the Male Reproductive System in Cystinosis

Principal Investigator Professor Minnie Sarwal, Professor of Surgery, Division of Multi Organ Transplantation, University of California San Francisco (UCSF), USA and co-applicants, Dr James F. Smith, Associate Professor and Director Male Reproductive Health, Department of Urology, University of California, San Francisco and Dr Polina V Lishko, Associate Professor, Department of Molecular and Cell Biology, University of California Berkeley, USA

The cause of male infertility in Cystinosis remains unclear, resulting in morbidity for this disease. Researchers, Professor Minnie Sarwal, MD, PhD and Swastika Sur, PhD, at the University of California San Francisco (UCSF) are investigating the physiological, molecular and cellular causes of why infertility affects only the male, and not female, individuals affected by nephropathic Cystinosis. This work has been funded by the HRB and Cystinosis Foundation of Ireland.

Cognitive Control Systems in Cystinosis

Sophie Molholm, PhD Co-Principal Investigator, John Foxe, PhD Co-Principal Investigator

Seminal behavioral studies by Trauner and others in human patients have suggested the presence of cognitive dysfunction in cystinosis. Our own work suggests some behavioral and neural differences and difficulties with sensory memory in this population. However, the neurocognitive phenotype associated with CTNS mutations and its developmental path are still poorly understood, and the prime areas of neurocognitive vulnerability in this population are in need of much more thorough characterization. This is critical to developing effective therapies to compensate for or improve on areas of cognitive vulnerability. To this end, we propose to characterize different components of executive functioning (memory updating, set shifting, conflict monitoring, and inhibition) in cystinosis. Focus on this area is motivated by our previous work, the literature on cognitive weaknesses in cystinosis thus far, and by first person reports collected during interactions with patients and families. We will use high-density electrophysiology (EEG)—a non-invasive method that allows one to directly measure functional brain activity at the millisecond scale and thus reliably assess the integrity of information processing at the neural level—and standardized cognitive functional assessments to test 15 children, 15 adolescents, and 15 adults with cystinosis and the same number of age-matched unaffected healthy controls. Twenty heterozygotes and age matched controls will also be tested to examine the role that mutation versus disease plays in the cognitive phenotype of cystinosis.

Grace's grandparents join mom and dad at the CRN Family Conference.

Cystinosis Community Advisory Board/Cystinosis Network Europe

The Community Advisory Board's (CAB's) objective is to improve patient access to novel therapies and treatments. This is achieved by engaging with clinical trial sponsors at the earliest stages of their research processes. The CAB also works with pharmaceutical companies on topics like educational materials and other appropriate topics. As well as meeting with industry sponsors, the Board engages with early-stage researchers as part of PPI - Public and Patient Involvement in research. We look forward to continued partnership with

Research Update, continued

researchers and industry worldwide to improve the quality and speed with which Cystinosis treatments are developed with the patient's voice in mind. At this time the CAB is assisting with the creation of a new Standards of Care document, we hope to have this information available to share soon with our patients and their providers, it has taken months and a large cohort of providers but I am confident it will be an excellent tool.

National Institutes of Health

As a reminder, patients may contact the National Institutes of Health to be enrolled in the cystinosis protocol and for consultative care. For more information, please contact:

Joy Bryant, (301) 443-8690, bryantjo@mail.cc.nih.gov

Educational Resources

All of CRN's educational materials including brochures, guides and other publications have been updated and are available on the CRN Website. Please visit the Research page on the CRN website for updates on CRN funded studies as well as other research from the world. Also be sure to check out the many cystinosis related articles and publications available in our Publications and Guides library at https://www.cystinosis.org/support-resources/publications-guides/

The Cystinosis Warrior Impact Program is a global initiative aimed at positively impacting every single one of our 2,000+ cystinosis warriors.

Our goal is to make a difference in the lives of those affected by cystinosis by offering comprehensive support in multiple areas including but not limited to:

- Mental health and wellness
- Financial (scholarships)
- Medical (support programs)
- Educational resources
- Research opportunities
- Networking (via mentor referrals, connecting with resources to find a kidney donor, find a doctor, etc.)
- Translation services
- Advocacy and policy work…and more

Here's How to get help for yourself, your family, or other cystinosis warriors:

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.

This is a long-term goal and will only be possible with the assistance of our network of patients, caregivers, loved ones, partners, and healthcare professionals. We hope you feel empowered to reach out to us or take part in the lives of our rare disease community.

Patient Services (RARE Concierge)

Our Patient Services team provides support to individuals, care partners, families, friends, and others impacted by rare diseases. We provide information, resources and connections based on your individual needs and living experience. Together with our partners, Global Genes helps people find and build communities, gain access to information and resources, and provide hope and support for the more than 400 million people affected by rare disease around the globe.

Our Patient Services Guides are dedicated individuals whose mission is to improve the quality of life for all rare disease patients, including the undiagnosed. They understand the wide array of concerns and challenges the rare disease community experiences.

How can we help?

CONTACT US

- Help getting a diagnosis
- Locate genetic counseling services
- Help find a doctor, specialist or center of excellence
- Provide information on financial & disability resources
- Connect to mental health & social services
- Caregiving resources
- Connect with rare disease community
- Connect to patient advocacy organizations
- Identify clinical trials & research studies
- Guidance on advocacy & raising awareness

Contacting the Global Genes Patient Services is **FREE** and available to all those affected by rare diseases.

Contact us:

patientservices@globalgenes.org

globalgenes.org/rare-concierge

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Financial Update

By Tim Wyman, 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. To that end, CRN expended significant resources in 2025 for the Family Conference held in July, in beautiful Grand Rapids Michigan, which brought together hundreds in the cystinosis community along with both medical and industry partners.

Tim Wyman sharing his financial expertise at the 2025 CRN Family Conference in Grand Rapids, Michigan.

CRN's net income year to date was -$112,591; less than budgeted. It should be noted that CRN's expenses in a "conference year" are significantly higher than non-conference years and the majority of expenses were attributable to the conference, research grants and scholarships. Thanks to grants and fundraising, CRN's current equity (assets minus liability as of July 31, 2025) stood at roughly $482,000 which is critical in funding additional research as well 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.

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good days
effective compassion

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

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Leadiant Biosciences, Inc.

$25,000-$19,999
Novartis Gene Therapies

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Wyman, Tim & Jen
Dance With Your Heart
Kaskel, Rick & Phyllis
Danzig, Dianne
Manning, Lawrence & Shirley
Adelmann, Richard & Becky

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Qualley, Michael & Jennifer
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Darbee, Elaine & Calvin
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Lewis, Lori and Ronald
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Bradley, Daya
Marable, Helen
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Ferrigan, Robert
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Munch, Roxanne
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Dicks, Jonathan & Shirley
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Webb, Cecelia
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Brocks, Mary Ellen
Brouillette, Don
Campanella, Danielle
Campbell, Jean
Cardona, Lina
Carmichael, Jan
Chang, Hayley
Chavez, Lidia
Chelikani, Aswinikumar
Cochrane, Becky
Compton, Claudia
Cregin, Dennis
Crellin, Doug
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Fleming, Bob
Foxe, John
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Hallock, Chaz
Hardin, Maria
Hassan, Stephanie
Hassan, Taner
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$98-$50, continued

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Mering, Ellen
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Schubin, Kimberly
Sharkey, Melinda
Shroyer, Edward & Mary Ann
Simon, Wendy
Slagle, Jessica
Smith, Chris and Lisa
Stewart, Nichelle
Stewart, Norman & Susan
Torban, Elena
Turner, Linda
Varney, Lucas & Jessica
Verneuil, Karen
Wadnerkar, Shivani
White, Laura
Whitley, Tim
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Yamin, Alia
Zautner, Jean

$49-$25

Hunt, Cass
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Hammer, Sue
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Robinson, William
Sparling, Christine
Trifari, Caroline
Winger, Jean
Zatolokin, Ellen & David
Wallis, Jane
Thorpe, April
Uyen, Faye
Welshons, Eric
Carle, Peter
MacMaster, John
Gardella, Mary
Cobuzzi, Tracy
Renfer, Leigh
Rowe, Linda
United Way of SWPA
Carswell, Annette
Kavanagh, Bernie
Broderick, Kristine
Crane, Sarah Duatel
Hammit, Kerry
Walker, Kate
Frank, Kristin Copeland
Eckert, Charles
Foisy, Colleen
Greene, Renee
King, Kristin
Lovell, Joan
Trasborg, Patricia
Janes-Tetzlaff, Bunny
Zieminski, Julianne
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Beal, Brittni
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Diaz, Michael
Fleming, Suzy
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Johnson, Carol
Molina, Kenia
O'Keefe, Marla & Daniel
Shepard, Derrick L. & Tara
Testa, Giuseppe
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Altman, Allen
Baker, Dianne
Byrne, Rita
Fippinger, Gary & Karen
Glueckert, Eileen
Harland, Brooke
Henry, Laura
Hotchkiss, Michelle
Hub, Mariah
LaFleur, Kristi
Larsen, Erik
Morris, Chuck
Noonan Kelly, Nancy
O'Brien, Leslie
Pagano, Ralph & Marilyn
Ritchey, Janice
Rogers, Cindy & Ralph
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Schiel, Lindsey
Tarpley, Linda
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Wittenberg, Matt
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Dockery Senase, Karen
Freedman, Janet
Goode, Lester
Healy, Jana
Nelson, Paul
Noland, Jeffery
Pdeck, Katie
Scardigno, Diane
Talley, Linda
Wagenbrenner, Karen
Bertarelli, Julie
Gard, Mary
Allen-Bishopp, Crystal
Cira, Michael
Gu, Jingjing
Hogan, Shelia
Renzer, Brian
Sanders, Nikki
Schmidt, Paul
Tiger, Pamela
Wallis, Lauren
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Trevino, Meredith
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Guilford, Joy
Bowman, Kathy
Bradley, Trinity
Day, Michael and Amy
Peebles-Bonds, Paula
Westbrook, Danyelle
Day, Amber
Edmonds-Henderson, MaryJane
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Shaheen, Damon
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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 to 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 that are specifically geared for adults or you are interested in contributing ideas to new and/or legacy programming through the Adult Leadership Advisory Board
- 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 Meet Ups. 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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Please email any contact corrections to info@cystinosis.org.

CYSTINOSIS RESEARCH NETWORK

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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.