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2015 CRN Chicago "Winds of Change" Family Conference Largest Ever!



The 2015 Cystinosis Research Network (CRN) Family Conference was held July 16 - 18, 2015 at the Doubletree by Hilton – Magnificent Mile in Chicago, IL. Approximately 340 family members, physicians, researchers, and volunteers attended from 12 different countries, including 97 families and 80 individuals with Cystinosis. All attendees were able to learn about the latest updates in cystinosis research through lectures, workshops, poster sessions, and medical and patient panels. There were

many opportunities to socialize with other patients and families, which provided valuable connections and friendships that will last a lifetime. The conference theme, "Winds of Change" was clearly appropriate as the concentration of the event was on defining and measuring if the quality of lives for persons with Cystinosis is changing, and by how much?

Continued on page 10



Eighty people with cystinosis attended the Chicago conference making it the largest CRN Family Conference ever held. There were 340 attendees including 97 families from 12 countries; who joined doctors, researchers and pharmaceutical representatives to learn the latest information and to connect with each other.

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The President's Letter

Successful 2015 Will Lead CRN's Progressive Agenda in 2016

By Jeff Larimore

When the opportunity arises to address the Cystinosis community in *The Cystinosis Advocate* with the President's Letter, there is a challenge to not concentrate too heavily on past events where the readers inquire internally "what has CRN done for us lately"? Proudly during 2015, the Board of Directors, CRN Committee members and families managing with cystinosis have made tremendous contributions for the betterment of our "ultra-rare" disease community. In the forthcoming pages of this Fall/Winter edition, you will be impressed to the point of head-shaking, and possibly tears, that for nearly 20 years the Cystinosis Research Network furthers its accomplishments each year and remains structured as an all-volunteer, non-profit organization.

Within this newsletter, our captive audience will see where during 2015, CRN represented the cystinosis community on Capitol Hill, at professional medical conferences, and at cystinosis family symposiums. CRN successfully presented its 7th Cystinosis Family Conference which brought together 80 individuals with cystinosis from around the globe, the largest gathering of cystinosis individuals to date. The post-event momentum that the Conference created continues to resonate in the professional communities. The Conference opening of "Dream, Achieve, Inspire" - An Art Exhibit for Cystinosis is the first piece of the bridge to worldwide patient advocacy cooperation.

CRN's tremendous achievements of 2015 provides the framework for Strategic Imperatives in 2016. We are aware that priorities vary by age throughout the cystinosis community. For the newly diagnosed to individuals with cystinosis starting to get a bit of gray hair, and to all in between, the Cystinosis Research Network strives to provide the most comprehensive resources to improve the quality of life for all. Secondly, expanding relationships with medical professional organizations will concentrate on improving cystinosis health care education and medical care for adults. Third, broadening dialogue throughout the cystinosis and rare disease community to support clinical research initiatives that push the boundaries of what is known today. As you can see, Research is our middle name.

CRN will continue to press the need for legislative support from our local, regional and national representatives. A commitment to rare disease advocacy must be an action, not just a continuing conversation.

Highlighted in this newsletter edition, you will become aware that Global Advocacy

Outreach needs to develop. Simply stated, CRN will continue relationship building with international cystinosis advocacy organizations and representatives to develop a “one world” platform of priorities.

The Cystinosis Research Network bylaws state that a President can serve two consecutive terms, totaling five years. At the July Board Meeting, the Board honored my request for a second term. In the remaining two years of this term, I’d like to fulfill a goal I stated a year ago – “to chart the path of achievement for CRN’s future, a new era needs to begin where the mission, vision and beliefs...are delivered by individuals with Cystinosis...” A challenging goal, but an achievable one.

Our sincere thanks to Terri Schleuder for her stellar editorship skills. To everyone that continues to support the Cystinosis Research Network, our heart-felt gratitude.



CRN President, Jeff Larimore with daughter Sarah

On behalf of the Board of Directors and Committees of CRN, our warmest wishes for this holiday season.

Cheers,

Jeff Larimore, President



The Midwest Cystinosis Families met in Chicago this year in place of their traditional yearly regional gathering. This is the 26th year these families have connected with each other to share their experiences and friendship.

CRN Welcomes Four New Board Members

The Cystinosis Research Network is pleased to announce the election of four new Board members, Carol Hughes, Jennifer Loglisci, Herberth Sigler, and Stephanie Sorensen. We welcome their ideas and energy to your advocacy group. Meet them below.



Carol Hughes

Carol Hughes was born in Rochester, NY, moving to Vero Beach, Florida when she was 10. She met and married her high school sweetheart, Garry in 1978. They have two daughters, Jennell (born in 1983) and Heidi (born in 1985) who has cystinosis. They also have two granddogs.

Carol was on the Board of CRN from its inception through 2007, serving on the Family Support Committee (1 term chair), and the Conference Planning Committee for several CRN conferences. She has also exhibited with CRN for several medical symposiums over the years including University of Miami and PAS in San Diego and San Francisco.

She is honored to be invited to be on the CRN Board and excited to catch up on the many changes that have occurred over the past few years. She has a special place in her heart for connecting with the families and the education and awareness of cystinosis, which often go hand in hand.



Jennifer Loglisci

Jennifer has never been one to sit and talk about herself, but for this special occasion she would like share a few reasons why she has become an outstanding woman. By now many of you have had the chance to meet Jennifer at past Cystinosis Conferences and other meetings that have been held over the past three years. Her life completely changed from the moment she stepped into the cyber Cystinosis world.

Prior to knowing anyone else with this disease she craved to better herself and not act as if she was sick. Most people still do not know she has such a rare disease. She has always been an optimistic woman, but these days she has felt empowered knowing she has a huge family around the world who feels the ups and downs just as she does.

Prior to her first conference in 2013, she did research on her own medical history and stayed on top of her appointments at the NIH, knowing someday her numbers would help others. In between attending undergrad school and a part time job, she had a mission. With a larger Cystinosis family standing next to her today, she knows her mission is to reach out to others on a higher level and share her life story about living with Cystinosis.



Herberth Sigler

Herberth R. Sigler holds a B.S. in Civil engineering, and earned his degree at The Bolivian Catholic University. He married Jessica Magnus and they have a 6 year old girl who has Cystinosis.

The complete family is from Tarija, Bolivia. Martina started with symptoms after her first year. It took more than 12 months to find out that she had Cystinosis. They went locally in their own country, then to Argentina and finally The National Institutes of Health in Maryland which confirmed the cystinosis and there she started treatment. Martina was transferred to The Montefiore Children’s Hospital at the Bronx in New York City from the NIH. The family lived at a Ronald McDonald House

in New York for more than two years.

Fortunately for him and his family, after stabilizing, Martina is a cheerful and lively girl, taking her dozen daily medications, religiously, as her doctor told her to. Her mother is there watching her! The family has assimilated their language from Spanish to English, Herberth has validated his education and continues the constant desire of a parent for knowledgeable doctors, medicine and overall quality of life for patients who are affected by Cystinosis.

Herberth is a licensed professional engineer from Bolivia. He has been working for more than 2 1/2 years at Hatzel & Buehler, Inc., the oldest electrical contractor in the United States, based in Manhattan. His title is an engineer - cost estimator. He and his family are Catholic and attend the Holy Family church. He lives in Fresh Meadows, New York.

Certainly, Herberth and his family believe, share and have experienced firsthand the mission and vision of CRN. As a new Board member, he has initiatives, enthusiasm and all the will to disseminate, support families, spread knowledge for better quality of life and advocate to accelerate finding a Cystinosis cure.



Stephanie Sorensen

Stephanie 28 years old. She is married and has two wonderful children. They are her little miracles. She is a hair stylist at Cost Cutters. She was diagnosed with cystinosis and Fanconi syndrome around the age of one. She had a kidney transplant in 2001. Her mother raised her to be a strong, independent woman, who taught her that family is everything because they will always be there for you when you need them. She has three sisters and three brothers. She is close with them all. She has a great father and stepfather as well. Her mother passed away three years ago due to malignant melanoma. To Stephanie her mother was her rock who was a great mother and grandmother. Stephanie is grateful for her wonderful family support system.

2015 CRN Scholarship Recipients Named

The Scholarship committee is pleased to announce the winners of our two, 2015 CRN Scholarships. We had several applicants this year, making the decision a difficult one. Our CRN Academic Scholarship went to an individual with cystinosis to Garrett Thomas as he begins his college career at Fredonia State University of New York, and our CRN Sierra Woodward Sibling Scholarship went to Kasey Hohl as she finishes her Nursing Degree at Western Iowa Tech Community College. Our congratulations to both of these fine young people as they continue to achieve their academic and life goals.



Kasey Hohl

The Sibling Scholarship from the Cystinosis Research Network will help me achieve my dreams of becoming a RN. My sister Keri, who passed away in 2010, is the reason I am pursuing my RN. She led by example that she could achieve anything she set her mind to and I would like to do the same. This scholarship will help relieve some financial stress. I appreciate receiving this scholarship, which just goes to show that just because my sister has passed away, does not mean that my Cystinosis family has to disappear from my life as well.

Hi, my name is Garrett Thomas and I have Cystinosis. This article is written because I am so grateful to have been selected as one of this year's CRN scholarship recipients.

My goals are to graduate from State University of New York at Fredonia with a Bachelor's degree in Criminal Justice. This is something I have been interested in for as long as I can remember.

The scholarship money will help me to buy books for classes, and pay tuition.

Thank you for your support.



Garrett Thomas



Volunteers Needed for a Research Study of Bone Health and Muscle Strength

Children and adults with cystinosis are at risk for musculoskeletal disease. This research study, performed by Drs. Mary Leonard and Paul Grimm, will be the first comprehensive evaluation of bone structure and muscle strength in children and adults with cystinosis. The results of this research study may be used to develop clinical trials to improve bone health, muscle strength and quality of life in children and adults with cystinosis.

What Is the Purpose:



To examine bone structure and muscle strength in children and adults with cystinosis, compared to those without cystinosis.

Who is Eligible to Participate:

Children and adults ages 5 to 60 years with cystinosis.

For further information regarding questions, concerns, complaints about the research or your rights, please call 1-866-680-2906

What is Required:

- Participants will be asked to complete a single study visit, lasting about 3 hours.
- Participants will be asked to complete questionnaires, physical assessments, and bone scans.
- Participants will be asked to provide a small amount of blood, about 2 tablespoons, for laboratory studies.

Participants will be given a picture of their bone scan, an analyzed DXA report, and \$150 gift card.

This investigation was supported in whole by a grant from the Cystinosis Research Foundation.

For more information please contact:

Jessica Whalen
 jwhalen@stanford.edu
 (650) 683-5287

Megan Morrill, Living an Extraordinary Life

By Jill Morrill

Brian and I have two beautiful children.. Megan, is 19 years old and was diagnosed with Cystinosis at age 3. Tyler is 16 years old and diagnosed with Juvenile Diabetes at age 15. Our children are ordinary kids living extraordinary lives.

Megan was diagnosed with Cystinosis later than most. We joke that it was because I let her eat "tomato chips" for breakfast and pickles for lunch. One of our doctors told us she was eating exactly what she needed to make her feel better. Our story of Megan as an infant is exactly like all of your experiences with infants who have struggled for a diagnosis. Megan was a picky eater, a water guzzler, did not like the sun in her eyes, got car sick easily and was small and petit. Once diagnosed, she got her meds and hydration via a g tube. Megan had her kidney transplant just before her 13th birthday. She has not looked back. Megan remembers her difficult childhood as ordinary and fun. Imagine that! All of us parents know the ups and downs of raising a child with Cystinosis. It is really hard and exhausting.



Megan Morrill and her horse, Red. Megan has ridden horses for the past 13 years

Megan is now a freshman at Albion College and on the Varsity Equestrian Team. She is enrolled in the Health Institute and her favorite class is Biology. Megan cares 100% for herself, gets monthly lab draws and visits her specialists every 3 months. She has taken responsibility for her health very seriously. We are so proud of her character, her compassion, and her zest for life.

Megan has a passion for Horses. It began a small child. After enrolling her in dance, t-ball, swimming, soccer, art and music classes, she got on a horse. At the age of 6 1/2, Megan's favorite day of the week was the day she had her riding lesson. She told me one day that she never felt sick when she was at the barn because the horses made her feel better. Brian and I immediately signed her up for multiple lessons a week. We think because Megan had no control of her health issues, was poked and prodded so often, that when she could turn the tables and boss a 1000 lb. horse around, she felt in control of something. The gentle giants respond to her demands, the horses loved her back unconditionally and this gave her great joy. Megan began jumping fences and showing horses the summer between 2nd and 3rd grade. Although she had a

g-tube, liquid meds to take every 6 hours and didn't always feel her best in the heat, Megan never gave up her passion for horseback riding.



Megan Morrill demonstrating her equestrian passion

Megan has spent 13 years on the back of horses and has learned a lot from her experiences. Caring for and riding horses has taught her responsibility, patience and compassion. Riding gives her good exercise and a wonderful social life. Riding is her mental therapy and it's an activity she can do for a lifetime. Horses have also taught her the value of money and hard work. We are so happy she found something to be passionate about at a young age. Horses have helped Megan lead a very fulfilling life.

Raising a child with Cystinosis has been the main focus in our lives. When our son Tyler got diagnosed with Juvenile Diabetes we were forced to add another diagnosis to the mix. It was extreme-

ly difficult to digest that we had 2 children with fragile lives. We chuckle because Megan thinks poking fingers multiple times a day and wearing an insulin pump is worse than having Cystinosis. Tyler thinks the insulin pump is easy and dealing with Cystinosis, lab draws and swallowing 26 pills twice a day is worse than diabetes. Both our children are intelligent and thriving. Tyler is in 11th grade and a goalie on our high school varsity hockey team. Megan is doing what other typical healthy 19 year olds are doing at her age. Cystinosis has never held her back and I'm confident Tyler will continue to take good care of himself and lead a beautiful life too.

We have been fortunate to have great Medical Care at DeVos Children's Hospital in Grand Rapids, MI with specialists and nurses who treat us like extended family. Megan has been on the Teen Council for the Children's hospital for 3 years. She has had speaking engagements on renal disease and Cystinosis with the first year Medical Students at Michigan State University. Megan has had many other wonderful opportunities in her life because of Cystinosis.

Brian and I are very blessed and fortunate to have Megan and Tyler in our lives. Both our children have taught us so much about life and living it to the fullest. Our extended family has been our rock. Our Cystinosis family has been our hope and our children have been our passion.



Brian, Megan, Tyler, and Jill Morrill enjoying spending time together

Family Conference continued from page 1

With the support of our generous sponsors, CRN was able to provide three days of programming which was aimed at both families and professionals. Wednesday began with the CRN Board of Directors meeting. On Thursday, conference registration began. The CRN Scientific Review Board also met on Thursday to review this year's Call for Research Proposals submissions. An announcement regarding studies funded for 2016 can be found in the Research Update section of this newsletter. Families were welcomed to the conference on Thursday evening with a reception which was highlighted by the premiere of the "Dream, Achieve, Inspire" art exhibit and start of the artist in residence program. Families had an opportunity to share their journeys with one another on Thursday evening during the Family Introductions Session.



The conference keynote speaker, Bonner Paddock, with Dave Greeley

Friday was a full day focused on increasing our understanding of Cystinosis and relevant medical discussions concentrating on infant, transition and adult issues. Friday was also the opening day of the Global Genes Cystinosis Teen Adventure – Chicago, a program designed for teens to explore ways to manage the transition from pediatric to adult care while bonding with their peers. The day kicked off with an outstanding keynote address from Bonner Paddock. Bonner is the founder of the OM Foundation, which promotes the idea that any one person can make a difference if they dedicate themselves to look beyond their own personal limits to achieve their goals. His story of self-acceptance, perseverance and incredible love and support for the Cerebral Palsy community and beyond was an awe inspiring way to start the day. Everyone was provided with a copy of his book, "One More Step", which he graciously signed for all. The rest of the day included an overview of Cystinosis and then panels on the management of infants and children with Cystinosis, adolescent issues in Cystinosis, transition from pediatric to adult care, and adult issues/management of extra renal manifestations. Research was highlighted in the poster session and questions were answered during the medical panel. Finally, this year's



Christy Greeley, CRN Executive Director, presenting Maya Doyle with one of the 2015 Above and Beyond Awards

their own personal limits to achieve their goals. His story of self-acceptance, perseverance and incredible love and support for the Cerebral Palsy community and beyond was an awe inspiring way to start the day. Everyone was provided with a copy of his book, "One More Step", which he graciously signed for all. The rest of the day included an overview of Cystinosis and then panels on the management of infants and children with Cystinosis, adolescent issues in Cystinosis, transition from pediatric to adult care, and adult issues/management of extra renal manifestations. Research was highlighted in the poster session and questions were answered during the medical panel. Finally, this year's

“Above and Beyond” awards were presented to Maya Doyle and Tim Miller. Maya is a gifted and compassionate social worker and researcher, who has dedicated much of her professional career to the care and study of those with cystinosis. She serves on CRN’s Medical Advisory Board, is a perennial speaker at our family conferences, and mentors young adults through the Global Genes Cystinosis Teen Adventure Programs. Maya was the driving force behind the CRN Adult Care Excellence Initiative, a groundbreaking push towards meeting the needs of adults with cystinosis, which resulted in publication of CRN’s Cystinosis Transitioning Guide. Maya is a CRN funded researcher as well. Her publication “That eagle covering me: transitioning and connected autonomy for emerging adults with cystinosis” in Pediatric Nephrology detailed the work she completed for her PhD dissertation, a project funded in part by CRN. Tim Miller is a long time CRN board member who has become the organization’s top fundraiser, spearheading nine annual C.H. Robinson Golf Outings in Salt Lake City, UT which raised nearly a million dollars for CRN. His selfless dedication to our community, which began with a working relationship with the Woodward family and evolved into an incredible dedication of time and energy for someone with no direct tie to Cystinosis, is truly unmatched. Both Tim and Maya are well deserving of this honor.



Tim Miller received the 2nd Above and Beyond Award. Tim’s efforts chairing the C.H. Robinson golf outing fundraiser has raised nearly \$1,000,000 for CRN over the last nine years.



Physicians in attendance, answering questions and providing the most updated information on research available. Pictured above from left to right, Dr. Jess Thoene, Dr. Patrick Gipson, Dr. Richard Simon, and Dr. Rick Kaskel.

Saturday began with an update on the KDIGO Cystinosis Conference. Updates about Cystaran were provided by Sigma Tau Pharmaceuticals and Walgreens and Raptor Pharmaceuticals and Accredo provided an update regarding Procysbi. Highlights of the day included the “Parents of Children and Adults with Cystinosis Panel”, where parents of children and adults with Cystinosis answered questions and shared their experiences, and the “Living with Cystinosis Panel” presentation where teens and adults living with cystinosis shared how they live their lives to their full potential given the challenges they face. Presentations that afternoon included information on bullying, preimplantation genetics and

early pregnancy diagnosis of Cystinosis, adherence issues, and bone disease. The afternoon workshop sessions allowed attendees the opportunity to interact with the experts on an in depth level. Finally, the highlight of the conference may have been the farewell dinner dance – where we were able to celebrate our time together with our cystinosis family with music, dancing, a photo booth and a performance by ToriLynn Jones.



The next CRN Family Conference will be held in Salt Lake City, Utah at Snowbird Resort in July, 2017. We look forward to seeing everyone together again then!



ToriLynn Jones and her young fans at the Dinner Dance on Saturday night



Jan and Dan Julian



India, Victor Jr., Victor Sr., and Ina Gardener



Parent Panel, answering questions and offering the wisdom of experience to attendee about life with cystinosis.



Teens enjoying the Dinner Dance with
ToriLynn Jones



Rachel DaLomba, her daughter Maya, and
José Morales

2015 CRN Family
Conference
Chicago, IL
July 16-18
The Highlights!



Holly Reuter and Stephanie Sorensen



Dr. Galina Nesterova with Lesli King and Christy Greeley

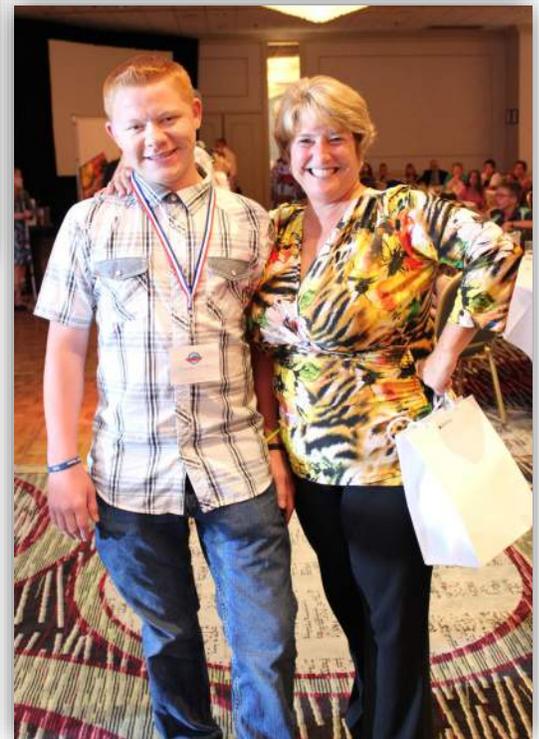




Artists in Residence collaborate with attendees to create a beautiful mural



Day care fun!



Joe and Mary Jordan



Global Genes Teen Adventure participants with Dr. Bill Gahl and Dr. Craig Langman



Spiderman visits the Day Care



Adults with Cystinosis connect with each other



Herbjorn and Heidi Gilje and family from Norway



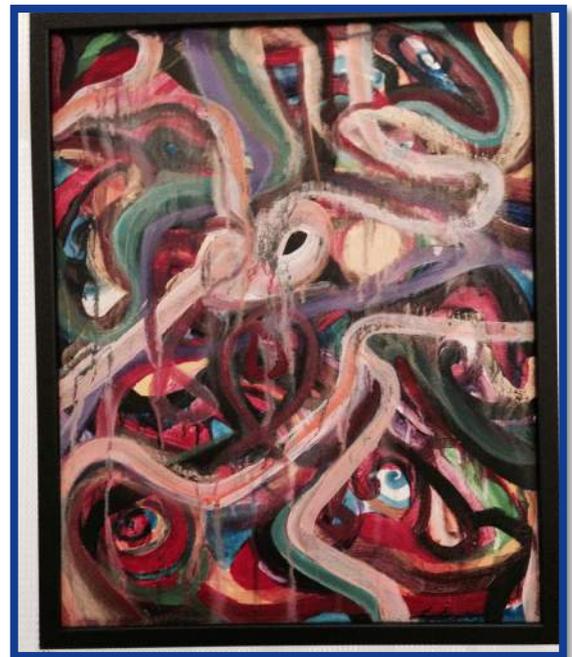
Raffle winner Mason Stilke with the Artist who donated the piece, Lee Knau



The Living with Cystinosis Panel answers questions about what it means to LIVE with this rare disease



Over 100 Artists with Cystinosis from 30 countries were featured at the Debut of the "Dream, Achieve, Inspire" traveling Art Exhibit at the 2015 CRN Family Conference, July 16-18



“Dream, Achieve, Inspire” Art Exhibit Travels to Brazil!

By Katie Larimore

Hello from Brazil!

"Dream , Achieve ,Inspire" an art exhibit for Cystinosis awareness has been very busy since its opening in Chicago at the CRN conference!

The exhibit was on display at the headquarters of Raptor Pharmaceuticals and received rave reviews from the employees and the Board of Directors.

Led by Emily Mello, the exhibit is being shared this week with families with Cystinosis in São Paulo, Brazil! The exhibit was invited to display at a meeting of Cystinosis families organized by Xenia Mota of Grupo de Suporte Cistinose Nordeste. Dr. Maria Helena Vaisbich will also be speaking. We are honored to be invited!

Exciting things are happening in the lives of our artists as well! Serena Scott of Australia has started her own business designing her button art that appears in our exhibit! She is doing quite well! You can find Serena and her latest designs on facebook. Little Adja Gruden of Slovenia was able to start cysteamine therapy! We are happy to hear she is doing very well. Our Poet Eddie Langley who wrote the passionate "Romeos Black Rose" finally received a kidney! Heal quickly Eddie! We all love you. Lee Knaus, the very popular glass artist has sold a few pieces of his art and is working on a commissioned piece! Many have asked and we are happy to report that Lee's green fish from the raffle has found a forever home with the Stilke Family!



Emily Mello and Yago Mota in São Paulo



Katie and Emily having Brazilian Pizza with Xenia Mota and her husband Jaon Cesar and their grandson Yago

"Dream , Achieve , Inspire " will continue to spread love , hope and knowledge in 2016. We have committed to our great friend Victor Gomez to display at his symposium in Mexico and a European exhibition is being planned as well. The exhibit continues to build relationships and strengthen friendships throughout the world. We are continually accepting work from artists with Cystinosis so if you would like to have your art join the exhibit just contact me! Go ahead... Dream a little!

Teens Living with Cystinosis Gather in Chicago

By Kym Kilbourne, VP, Patient Advocacy Global Genes



Alexis LeBeau, Kacy Wyman, Shannon Keizer and Kaitlynn McNeil attend the Global Genes Teen adventure as participants and mentor

Eighteen teenagers gathered for a special two-day Teen Adventure this past July in Chicago to learn about their disease from experts, discover new way to cope as they transition into adulthood and make new friends by connecting with peers who are living with Cystinosis.

Hosted by Global Genes, a leading global rare disease patient advocacy organization that specializes in custom patient education and empowerment programming, the Teen Adventure was held in connection with the Cystinosis Research Network's Family Conference.

Teens who took part in the program learned from long-time leading Cystinosis researcher and physician Dr. William Gahl, who also directs the National Institutes of Health (NIH) Undiagnosed Diseases Program. Dr. Gahl led a lively discussion among the teens to help them understand more about Cystinosis, its effects on the body and how treatments work. He also joined

the planned special collaborative art session, where teens design custom art pieces that were made into two framed pieces of art. One was gifted to Dr. Gahl, who also took part by making one on of the pieces that made up the final work. The teens gifted the other completed framed artwork to Dr. Craig Langman, Head, Kidney Diseases; Isaac A. Abt, MD, Professor of Kidney Diseases; Professor of Pediatrics, Northwestern University Feinberg School of Medicine.



Victor Gardener, Jr., Jack Kitchens, and friend having fun at the Teen Adventure

As part of the program, teens also heard from Maya Doyle, Ph.D. who discussed topics including living with a chronic illness and tactics for taking more of a role in managing the disease as teens grow into adulthood and pursue work and education. A session that focused on building a peer network and learning to handle bullies or those who don't understand the disease helped participants learn from each other and explore ways to talk about their disease and manage situations that arise with schools and friends.



One of two framed quilts designed and constructed by the teens attending the event and gifted to Dr. Wm Gahl and Dr. Craig Langman

The program is designed to balance education, information and fun! In addition to the custom expressive artwork activity, teens participating in team building activities, and an offsite excursion that allowed them to explore the sights of Chicago from up high at The Ledge at Skydeck Chicago for some spectacular views of the city.

Three young adults with Cystinosis joined us for the Teen Adventure and helped shape the experience by providing insight and guidance for the teens, the vast majority of whom had not met another peer living with the disease. Participation of the mentors, along with the opportunity to take part in one of the most inspirational sessions at the CRN Family Conference, the keynote from Bonner Paddock, who has climbed Mount Kilimanjaro and competed in the Iron Man Kona competition, all while living with Cerebral Palsy proved inspirational.

Here’s what teens said about the Teen Adventure experience:

“It was really great to talk with the other teens about the challenges we all face. I really enjoyed getting to know the mentors. It was encouraging to hear from them that it does get better. I can’t wait to be a mentor next year!”

“I was nervous to go to Chicago because I am usually pretty shy. Once I met the other teens, I quickly felt comfortable and I’m excited to keep in touch with everyone.”

“One of my favorite parts of the weekend was hearing Bonner speak. He really inspired us to believe that we can still do things, even when most people think we can’t.”

Parents were equally enthusiastic about the program:

“The Teen Adventure was such a great idea and a great schedule for the teens. This group helps the teens feel confident in themselves and their ability to monitor their disease.”



Teens enjoying the sights of Downtown Chicago during the two day Global Genes Teen Adventure event.

One hundred percent of teens reported they had a positive experience with the program and would attend again – most willing to travel a great distance to get the chance to meet and connect with other teens living with Cystinosis. One of the sessions in the program was built to help participants connect with CRN and learn more about the educational and advocacy work, that they one day will help shape and lead. Helping foster connections, building support, increasing knowledge, having fun—this are the goals we achieved with the Chicago Teen Adventure. Thank you CRN for supporting this amazing program—we are privileged to work with the Cystinosis teen community!

About Global Genes:

Global Genes’ mission is to eliminate the challenges of rare disease globally. We do that by developing resources and tools to help equip patient advocates to become successful activists for their disease; by working to build a global connected network—a platform for collaboration and success; and by funding science and technology innovations that will broadly impact patients within their lifetime.

Marybeth Krummenacker selected as a DIA Fellow

By Marybeth Krummenacker

Marybeth Krummenacker recently was selected as a DIA Fellow and attended the Annual meeting which was held in Washington, D.C. this past June. The DIA (Drug Information Association) was founded in 1964 as a neutral global membership association dedicated to improving communication and collaboration in drug development. This important organization was born out of true need to address the 1962 sweeping legislation passed in the US in response to the finding that the use of thalidomide to treat morning sickness caused birth defects. Although the problem was global, there was no organization at the time that could foster cooperative efforts across the world. Fifty one years later, the neutral posture of the DIA encourages active participation around the world. The patient advocates of today are increasingly influencing all stages of drug development and regulatory process and both industry and government are establishing and expanding patient engagement programs.

The DIA Patient Advocate Fellows Program is designed to develop, strengthen and support patient collaborations with policymakers, industry representatives, public health authorities, academia, and other health care stakeholders. By increasing knowledge and understanding of issues central to patient-centered health care, fellows are better equipped to integrate their voice and respond to changes in drug development and health care delivery. The theme throughout the five day meeting was to **Develop**, **Innovate** and **Advocate**.

CRN was a part of the exhibit space that the DIA provided and literature was exchanged and shared with all who stopped at the booth. The 20 Advocates were a big part of discussions throughout the week and were available to speak with and to share the stories that we all live and Marybeth was able to network with extraordinary individuals in the pharmaceutical industry, academia and government. As Marybeth said, "It was a wonderful experience and truly gave CRN and the Cystinosis community a "place at the table" and an opportunity to be heard".



Marybeth Krummenacker, on the right attended the annual meeting of the DIA this past June in Washington D.C.

The Art of Patient Advocacy, Seizing the Opportunity to Make a Difference

By Marybeth Kruppenacker

I was struggling with how to begin this article on the challenges that face the rare disease community in the current environment both nationally and globally until I began to think of the role of the advocate as an art form and that the art of advocating as a gift. One must not get discouraged or intimidated when beginning their journey as an advocate. President Theodore Roosevelt once said;

"It is not the critic who counts; not the man who points out how the strong man stumbles, or where the doer of deeds could have done them better. The credit belongs to the man who is actually in the arena, whose face is marred by dust and sweat and blood; who strives valiantly; who errs, who comes short again and again, because there is no effort without error and shortcoming; but who does actually strive to do the deeds; who knows great enthusiasms, the great devotions; who spends himself in a worthy cause; who at the best knows in the end the triumph of high achievement, and who at the worst, if he fails, at least fails while daring greatly, so that his place shall never be with those cold and timid souls who neither know victory or defeat."



Marybeth Kruppenacker and her daughter, Laura Kruppenacker.

This statement hit me in such a way that I began to think about how and why I became an advocate for my daughter over 23 years ago. I knew that when I was handed the diagnosis of this extremely rare diseasecystinosis, I needed to learn as much as I could so that I felt confident when it came to educating anyone who would listen to me. I knew that even though I was trying to come to terms with both the reality of my daughter's physical needs being met I knew that the emotional needs of her and the entire extended family also had to be met as well. In the case of rare a disease the advocate becomes the teacher everyone from the doctors to the individual family members. The advocate becomes the teacher, out of necessity, because if everyone is going to survive, you must get to a place where the patient, the doctor and just about anyone involved in the everyday care must figure out a way to work as a team and learn to ask questions, get answers, and even to voice an opinion and in my case, if she was going to survive in this world, I surely knew I had to open my mouth. I would have to be vocal about her to make people understand her. I felt if a person understood the disease....even a little bit..... they would understand her.

The most important lesson that I have learned is one should not be afraid to speak up for what they believe in and you feel it is something worth fighting for.....and in my case, this was my daughter's life. Slowly, as time went on I realized how rare cystinosis was and that I felt very much alone. I delved into understanding the drug being developed to understand just what the The Orphan Drug Act was and how important it was and how it could directly affect my life and more importantly my daughter's life and the realization that this extraordinary

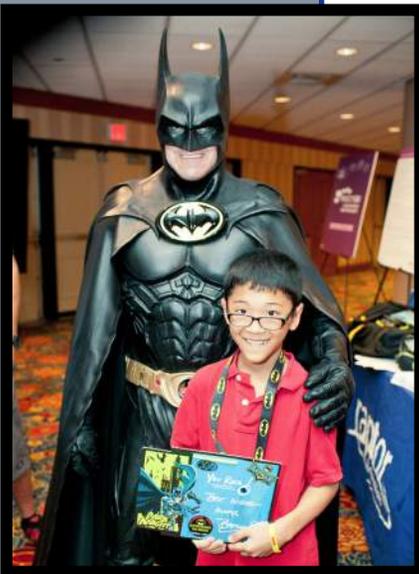
piece of legislation would have never existed without people advocating for what they believed in and the very simple fact that more research and attention needed to be paid to the many thousands of people in the United States who make up the rare disease community. This unique legislation was historic in the fact that the rare disease community was never viewed as politically significant or important. However, that community back in 1983 was a community by all estimates, at that time, to be approximately 20 million. Today that number has grown to 7,000 rare diseases affecting 30 million. Cystinosis is just ONE. But, it was only by arming themselves with information that the early rare disease advocates were able to make their voices known. They gathered collectively as one group of committed people to the cause of rare diseases. But it only takes ONE to make a difference. A smart advocate, alone is ONE, but collectively has a voice that can be heard IF the message being delivered is the same. A smart advocate is also a good listener. The true advocate will arm themselves with information after listening and learning from others and in turn this gives the individuals the confidence to speak to a particular issue and often times when dealing with a rare disease, the need to personalize the topic to make their voices heard stronger and louder.

A Tribute to Lenny 'Batman' Robinson

by Lesli King

Lenny B. Robinson - September 28, 1963-August 16, 2015

It is with great sadness that we share the passing of our friend, Lenny B. "as in BATMAN" Robinson.



Lenny Robinson, aka Batman, and friend at the 2013 CRN Family Conference in Washington, D.C.

We had the pleasure of meeting Lenny at the 2013 CRN conference in Washington D.C. and thought it was important to share a little of his story and how we had the pleasure of his company.

Lenny was a self-made millionaire who decided to use his wealth to help deliver hope, compassion, and happiness to children. He did so by becoming BATMAN. Why BATMAN? Lenny would say, "Because BATMAN has no real superpowers, he is just a guy who is trying to do good". That was Lenny, just a guy doing good things. He would answer the phone "BATMAN speaking". He wouldn't let the children see him out of costume even when events lasted longer than planned. He frequented hospitals and was sure to visit every single child there. He would go to schools to talk about bullying. He made special trips to visit children at the NIH when they were in town for treatment. All he wanted to do was make children smile. He was BATMAN.

The Theme of the 2013 CRN conference was "Heroes Among Us". There was no Better Hero to have spent time with the cystinosis Heroes than Lenny. He arrived

Continued at the bottom of page 23

Thank You!

CRN wishes to express our deepest and sincerest appreciation to all of our Corporate Sponsors. Without their generous financial contributions it would be impossible to plan, organize and execute an event of this magnitude. We are very grateful for their continued and ongoing support of our organization!



Continued from page 22

at the event in his Lamborghini Batmobile and he showered Batman themed gifts on children and adults alike. He took pictures with every single person that wanted one, even those that were not part of the conference. Everyone matter to Lenny. He was a true humanitarian who believed in people and encouraged people to believe in themselves.

Lenny always said 'At the end of the day you must ask yourself, "Self, did I make a difference?" and the answer had better be "YES!".' Lenny's hope, enthusiasm and giving nature will be greatly missed by not only the Cystinosis community, but each person he touched during his extraordinary life.



Lesli King and Ethan Bartkowski at the CRN Family Conference

Sisters Painting Picture of Rare Disease to Raise Awareness

(editors note: The following article featuring the Haynes sisters originally appeared in the Cherokee Tribune)

By Kayla Elder

Two young sisters and budding artists who both have cystinosis are using their talents to raise awareness of the rare disease.

Twelve-year-old Lily Haynes and her 9-year-old sister, Maggie, of Canton, are showcasing their artwork for "Dream, Achieve, Inspire," an exhibit created exclusively by artists affected by the disease and set for shows worldwide in South America, Europe and Mexico.

Presented by the nonprofit organization, Cystinosis Research Network, the exhibit began when Katie Larimore, mom to a 10-year-old daughter with cystinosis, was motivated by Billy Croce, an artist and young man with cystinosis. Croce died earlier this year and in memory of his life and those continuing to fight the disease, the awareness campaign was created.

Both Maggie Haynes and her sister are living with cystinosis after being diagnosed at a young age. Lily was diagnosed with cystinosis at 4 years old, while Maggie was diagnosed soon after birth.

"They were both very typical pregnancies," mother Ashley Haynes said. "Lily stopped growing at nine months. At that time, we took her to many doctors and it took a long time for her to be diagnosed. She was being treated for something at 2 years old and we weren't aware that she was misdiagnosed until she was 4. She was then diagnosed with cystinosis and began treatments."

Cystinosis is a rare, genetic, metabolic disease impacting about 2,000 people worldwide. Cystinosis causes an amino acid, cysteine, to accumulate in the kidneys, eyes, liver, muscles, pancreas, brain and white blood cells, according to CRN.

"Lily had a kidney transplant in 2011 and has been treated for both that and cystinosis since," Ashley Haynes said. "Though most children with cystinosis have to have kidney transplants, Maggie has had very few complications. She has been on medications a long time, whereas with Lily, we did not know until 4 that she had it."

Without treatment, children with cystinosis can develop end stage kidney failure at around age 9. This disease has devastating outcomes when untreated.

“Due to cystinosis being genetic, Maggie was tested right after birth and was diagnosed with it,” Ashley Haynes said. “Though she has it, she has very few symptoms thanks to the treatments from early on.”

Both girls attend Free Home Elementary School. Lily is in fifth grade and enjoys swimming and hanging out with friends. Maggie is in third grade and plays softball and basketball, according to Ashley Haynes, who is a kindergarten teacher at Free Home Elementary School.

“It has been helpful that both girls have cystinosis. There are a lot of challenges, but they are supportive of each other and know what each other are going through,” Ashley Haynes said. “As parents, my husband and I feel this has brought our family closer together and shows how strong we all are. My husband and I are very involved and we both work together. The pressures are extensive, but we all go through this together.”

The Cystinosis Research Network is comprised of family, friends and medical researchers dedicated to finding a cure, developing treatments and enhancing the quality of life for those affected by cystinosis.

“They provide support to patients. The board is made of parents and those affected by the disease,” Ashley Haynes said. “We attend conferences and keep up with them. One year we even did a small-scale fundraiser ourselves.”

As a part of the “Dream, Achieve, Inspire” exhibit, both girls contributed artwork.

Lily Haynes submitted a self-portrait piece that depicts who she is, with the word “sassy” written at the top and a photograph of herself with her hand on her hip. Maggie Haynes created her “Big City” collage with different pieces of paper.

“Lily’s piece is implicative of who she is and Maggie’s piece reminded us of Chicago,” Ashley Haynes said, noting they went to the Chicago Cystinosis Research Network Family Conference in July.

Both pieces of artwork are headed to Sao Paulo, Brazil. The exhibit will be featured by cystinosis advocacy groups in Brazil, Mexico and Europe throughout the next year. The exhibit showcases art from 107 artists living with cystinosis from 30 countries.

The collection was unveiled at the Chicago Cystinosis Research Network Family Conference to the largest



Sisters, from left, Maggie Haynes and Lily Haynes contributed artwork to the Cystinosis Research Network to raise awareness for cystinosis, a disease they both were diagnosed with when they were younger. / Special to the Tribune [slideshow](#)



Maggie Haynes and Jackson Bartkowski

attendance in conference history. The Haynes family has attended three conferences.

“It was neat to be there,” Ashley Haynes said. “There were adults around the age of 50 and 60, which was great since they did not have treatment at a young age, there were adults in their 30s and 40s and of course there were the children. The conferences are wonderful and we got a lot of information, especially in improvements in treatments.”

There are 30 million or 1 in 10 Americans living with a rare disease. Worldwide, it is estimated that 350 million people suffer from rare diseases, and half are children.

Additional information is available on Cystinosis Research Networks website at www.cystinosis.org, on Facebook at www.facebook.com/CystinosisResearch , Twitter at CystinosisCRN and Instagram at CystinosisResearchNetwork.

Read more: [Cherokee Tribune - Sisters painting picture of rare disease to raise awareness](#)

Cystinosis Patient & Caregiver Town Hall October 16 - 18, 2015

By José Morales

The Cystinosis Research Network (CRN) participated in the Cystinosis Patient & Caregiver Town Hall held in Atlanta, Georgia, October 16 – 18, 2015. CRN presented an overview of the rising importance of patient advocacy organizations in research and healthcare. The town halls are sponsored by Raptor Pharmaceuticals and enable individuals to participate in an engaging two-day program focused on Caregivers/Care Partners, Adults and Teens & Pre-Teens.

Meeting Objectives

- Improve the support and education resources available to the cystinosis community
- Identify educational information and tools to support patients and caregivers
- Exchange ideas and information that will help empower people in the cystinosis community

The program covered a comprehensive array of topics and is tailored for the participant demographics. Key Elements of the Atlanta Town Hall were:

Cystinosis Resources & Services

All About Cystinosis

Managing Cystinosis: Nutrition

My Cystinosis Journey

Scavenger Hunt

Cystinosis Advocacy

Insurance 101

Thriving With Cystinosis



Friends reconnecting at the 2015 CRN Family Conference in Chicago, July 16-18, 2015

The CRN's presentation focused on how important it is for us to become active advocates for our community. Utilizing CRN's organization, to highlight how we have to become our own most powerful advocates in three principle areas:

Research

Family Support

Education and Awareness

The recent creation of the 21st Century Cures campaign, by the Energy & Commerce Committee, provides clear evidence of the emerging power of our voice. The 21st Century cures campaign "created strategies to speed up the development and delivery of therapies to patients. There is a growing level of confidence and empowerment among patient advocacy groups in that their voice is being heard on the Hill. There have been recent strides that indicate the tides may be changing for organizations willing and able to align their interests with those of key lawmakers. Patient advocates have been the key element in shaping these drug development policies and in ensuring their inclusion in the final legislation."

The town halls are designed to provide participants with information and insights to more effectively and efficiently manage the health care of loved ones. We highly recommend everyone to take advantage of this program and make every effort to participate.

"If not us, who? If not now, when?"



<https://globalgenes.org/>

CRN Attends Global Genes RARE Patient Advocacy Summit and Tribute to Champions of Hope

By Christy Greeley

Cystinosis Research Network representatives Christy Greeley and Jose Morales were honored to attend the annual Global Genes RARE Patient Advocacy Summit and Tribute to Champions of Hope Gala September 24-26 in Huntington Beach, California. Global Genes is one of the leading rare disease patient advocacy organizations in the world. The non-profit organization promotes the needs of the rare disease community under a unifying symbol of hope – the Blue Denim Genes Ribbon™. What began as a grassroots movement in 2009, with just a few rare disease parent advocates and foundations, has since grown to over 500 global organizations. CRN is proud to participate as a member of Global Gene’s Foundation Alliance.



Bonner Paddock, inspirational speaker at the CRN Conference in June, received the 'Brave Feats of Courage Award' at the gala. He was also keynote speaker for the summit, and included his experiences with the Cystinosis community in his opening remarks.



Amy Grover, Global Genes Director Event Services and coordinator Chicago Cystinosis Teen Adventure with Christy Greeley, CRN Executive Director at the Summit

Information on the Summit and Gala from Global Genes:

RARE Patient Advocacy Summit

A rare diagnosis changes everything. It crashes plans and dreams, knocks you off your feet, and requires a continual investment of time and money as you try to determine what should be your next step. The purpose of the RARE Patient Advocacy Summit is to help patient ADVOCATES become successful ACTIVISTS and provide the discussion, insights, and tools to move down this advocacy path, equipped and prepared. The Summit offered practical advice, case studies, and networking opportunities as we learn from one another. The goal is to have patient advocates walk away with a better understanding of the challenges they will face and ways they can be the most effective in advocating for their disease/disorder.

You will:

- Hear directly from experts
- Better understand how to overcome the challenges facing caregivers
- Learn about new advances in science
- Learn ways to become an unstoppable advocate and an effective organization
- Become equipped and educated on successful lobbying
- Focus on drug development and how patients are playing a critical role
- Network, Network. Network!
- Have fun connecting with other advocates who share your passion for advocacy



Lesli King, Sigma Tau Pharmaceuticals, Christy Greeley, CRN Executive Director, and Kristina Broadbelt, Raptor Pharmaceuticals at the gala

By attending this two-day event, you will meet new friends and colleagues, gain a new perspective on the complexities and questions that need to be considered in order to become effective advocates for rare disease, and be prepared to help make advances in the rare disease space.

Tribute to Champions of Hope Gala

Each year, Global Genes hosts its annual RARE Tribute to Champions of Hope Gala, where we have the good fortune of hearing from rare disease champions from around the globe who are proving that great things can be accomplished by dreaming, planning, taking action, and believing. The results are quite inspiring.

The purpose of the RARE Tribute to Champions of Hope Gala is to recognize those who are uniquely attacking the challenges they face in the fight against rare disease; those who are thinking outside of the box and working tirelessly, not focusing on what was, but setting their sites on what can/will be.



Lesli King, Sigma Tau Pharmaceuticals and Jose Morales, CRN VP Development, attend the Summit

The 4th annual RARE Tribute to Champions of Hope was host to over 800 attendees and was in part responsible for helping in raising 1.2 million dollars towards Global Genes' educational programs and new Rare Patient Impact Grant Program. The night will be remembered for its courageous honorees, touching acceptance speeches, and amazing performances.



Cystinosis Research Network Family Forum Program, Los Angeles Family Gathering, May 9, 2015

By José Morales



Jeff Larimore, Dr. Ora Yadin and José Morales at the PAS Conference held in April 2105, in San Diego.

The Cystinosis Research Network hosted a Family Gathering on Sunday, September 27, 2015 at the W Los Angeles Hotel. Our speaker, Dr. Ora Yadin (Professor of Pediatrics, UCLA Mattel Children's Hospital) led the group through a lively discussion on a diverse array of topics. Highlights was a discussion regarding the Enteric-Coated Cystagon study results - highlighting the efficacy in reducing amino acid in the body, dosage adjustment method, a lay person's explanation of the specific biochemistry of cystinosis, etc. This led to a number of other discussions relative to the assembled group's own personal situations, issues and concerns.

CRN's Family Forum Program was conceived to provide families that are not able to participate in the biennial Family Conference opportunities to:

Access to leading medical physicians, researchers/experts

Facilitate introductions and networking between our families

Heighten the awareness and understanding of on-going research

Create sense of connectedness and community among our families

Three events were hosted in 2015:

March 7, 2015 - Charlotte, NC

May 9, 2015 - Columbus, OH

September 27, 2015 - Los Angeles, CA

Additional Family Gatherings are planned for 2016 and will be posted to the CRN website (<https://cystinosis.org>) under the Event Tab upon confirmation.

A heartfelt thank you to Dr. Yadin for sharing her extensive experience and making herself available to us.

Prairie House Corn Roast Raises over \$2,000 for CRN

By Christy Greeley



The annual Prairie House Corn Roast fundraiser was held on August 29th in Buffalo Grove, IL. The event was well attended despite rain most of the day, with live music, raffles, silent auction, games, food and drinks. The event this year benefited both the Cystinosis Research Network and Illinois Animal Rescue, generating over \$2,000 for CRN. We are indebted to owner Gary Gillam and the Prairie House for their continuing support of CRN and the Cystinosis community!



Jack Greeley and Prairie House owner, Gary Gillam

**Do you want to connect with
cystinosis families in YOUR area?**

Visit <http://www.cystinosis.org/families-in-your-area>. Send your contact info to the e-mail listed to learn who is in your area.

It's that easy!

“Paint for a Purpose” Fundraiser in Honor of Peytan Taylor Raises \$1,200 for CRN

By Autumn Palmateer

I had the honor of hosting “Paint for a Purpose in honor of Peytan Taylor,” a Paint Nite event held at the Hilton Garden Inn, Town Center in Virginia Beach, Virginia. The event was sold out raising \$1,200 for the Cystinosis Research Network. We had a few donated items auctioned off in addition to the money raised from ticket sales.

Unfortunately just before the fundraiser Peytan reached a fever of 103 and was admitted to CHKD. She spent the following 5 days, battling high fevers, stomach aches and tetany. While I visited Peytan and her mother, Channing, the Doctor came in for a checkup, they discussed different options on how to proceed with methods to treat Peytan's pain and sickness.

Channing picked the Doc-

tor's brain and suggested alternative methods. She has tirelessly spent day and nights



Bailey Shelton and family enjoy the fun!



Peytan Taylor, presents a check to CRN for \$1,200, the proceeds from a fundraiser held in her honor

researching to help Peytan. I admire her and her husband's, Jamie, strength to work together during these difficult times and still manage daily activities as well as their active 2-year-old son, Brayden. They have an amazing support system of friends and family.

Although Channing and Peytan weren't able to attend the fundraiser, we made sure they felt

included sharing photos and Peyton painted along while at CHKD. Jamie and I expressed our sincere gratitude and appreciation to all who attended the event. We look forward to hosting another fundraising event in the future.



Glen and Terri Graves



Paint with a Purpose attendees having fun at the sold out event!



Finance Update

By Jeff Larimore

**The Cystinosis Research Network, Inc
Financial Review – Accrual Basis**

For the 9 months ended September 30, 2015



Lee Knaus with his beautiful glass artwork at the CRN Family Conference

Revenues

For the nine months ended September 30, 2015, total income collected of \$556,500 was approximately 26% more than the same period in 2014. This increase was attributable to an increase in corporate grants received to support CRN's program services and Family Conference.

Expenses

Total operating expenses of \$560,700 were 300% more than operating expenses for the nine months ended September 30, 2014. Increased participation in education & awareness programs, funding of clinical research grants along with costs associated with the 2015 CRN Family Conference accounted for the increased in total operating expenses. Total General & Administrative costs were approximately \$25,000 in both reporting periods. G&A costs as a percentage of income decreased from 5.7% to 4.5%.

CRN had a net operating loss of \$4,310 for the nine months ending September 30, 2015 as compared to net operating income of \$254,000 through September 30, 2014. Successful fundraising activities and corporate support in 2014 and 2015 have provided cash resources to increase patient advocacy activities to support the cystinosis community.

Cash on hand at September 30, 2015 was \$422,516. Net change in cash through September 30, 2015 was a decrease of \$2,315. This net decrease in cash was primarily a result of total costs for the 2015 Family Conference slightly exceeding revenues secured.



Jeff Larimore at a recent Town Hall Meeting in Jersey City, New Jersey

Artist, Lee Knaus, Finds Joy in his Glass Art

By Lee Knaus

My name is Lee Knaus and I have Cystinosis, yet I live a fairly normal life. I just achieved my BFA at Jacksonville University in the glass arts. I find my art gives me joy and keeps my spirits high. The challenges of the disease are real but with the right attitude and support system anything is possible. I have found looking forward to the good days on the bad ones and enjoying the good ones when they are here keeps me going strong. I take the time to enjoy the world around me and I believe this helps my art and my attitude. There are many people much worse off than I am. I am thankful every day for the cystinosis community and my parents for their support. I feel I am comfortable in my own skin. I know who I am, I believe having Cystinosis has forced me to figure those truths out about myself at a young age. In a way Cystinosis has helped my life. I believe it has taught me how to look at others and not judge. Never knowing what someone is dealing with beneath the mask they wear. Cystinosis has made me the person I am today and I like that person.



Lee Knaus creating beautiful, original works of art in glass



Examples of Lee's unique and exquisite works of art in glass



Flippin Friends 2nd Annual CRN Pinball Tournament raised \$1,500

By Dave Greeley

Flippin Friends hosted the 2nd Annual CRN Pinball tournament in honor of Jack Greeley on October 11th. This year was extra special as Jack participated in the tournament and demonstrated his own pinball wizard skills. The event was attended by several top professionals with former world champion Dave Hegge winning the event once again. Thank you to all of the participants who helped raise \$1500 for this wonderful cause.



Jack and Dave Greeley compete at the tournament



Tournament host Jeff Hooper, Jack Greeley, and Pinball legend, Roger Sharpe

DO YOU OR SOMEONE YOU LOVE HAVE CYSTINOSIS?

Are they affected by:

Photophobia?

Eye Pain?

Foreign Body Sensation?

Squinting?

If you said "yes" to any of the above, it may be time to discuss these symptoms with your ophthalmologist.

Cystinosis causes cystine build-up in the body which may damage cells in the kidneys, liver, brain, other organs and the corneas.



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GEN10 08/15

Research Update – CRN Funds Three New Research Projects for 2016

By Christy Greeley, VP Research, Executive Director

A major focus of the Cystinosis Research Network continues to be a determined effort 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. CRN has funded over \$4 million total in research grants and fellowships. CRN funds a Cystinosis fellowship at the National Institutes of Health and has funded research and education programs in the United States and many countries around the world including Egypt, Mexico, England, Scotland, Italy, Belgium, France, Germany and much more. CRN research topics are aimed at every aspect of cystinosis with the purpose of understanding the disease and finding improved treatments and a cure. Topics include research and therapies related to neurological, genetic, ophthalmological, gastrointestinal, muscular, nephrology, pulmonary, skin, improved medications, and psychological effects of the disease.



Dave, Christy, Alex and Jack Greeley

The Cystinosis Research Network is pleased to announce its grant awardees for 2016. These three grants represent one established Cystinosis researcher in Dr. Levchenko's group in Belgium, one esteemed neuroscience lab at Montefiore in the Bronx which is expanding their work into cystinosis, and one innovative newborn screening initiative in Germany led by Dr. Hohenfellner. The grants total over \$200,000 in funding for 2016. They are:

"Mechanisms Underlying Neurocognitive Changes in Cystinosis"

John Foxe, PhD, Sophie Molhom, PhD, Steven U. Walkley, DVM PhD, Co-Principal Investigators

Departments of Neuroscience and Pediatrics and Pediatrics, Albert Einstein College of Medicine, Montefiore Medical Center, Bronx, NY

Aims for the project are to explore sensory processing and multisensory integration as potential biomarkers using high-density electrophysiological mapping techniques in individuals with Cystinosis and to determine the cell biological contributions to neuronal dysfunction in Cystinosis and their impact on neural network connectivity.

Grant Year One: \$98,010

“Postpartal Screening for Cystinosis with urine dipsticks at the age of 12 weeks”

PD Dr. med. Katharina Hohenfellner, LÄ Kindernephrologie, Kinderklinik Traunstein, Cuno-Niggelstr.3, 83278 Traunstein, Germany

Dr. med. Uta Nennstiel Ratzel MPH, Leitung Sachgebiet GE4, Präsidentin der Deutschen Gesellschaft für Screening, Bayerisches Landesamt für Gesundheit und Lebensmittelsicherheit, Veterinärstr.2, 85762 Oberschleißheim

Prof. Dr. med. Lothar Thomas, Emeritus Direktor Laboratoriumsmedizin, Klinikum Frankfurt Höchst, 65929 Frankfurt, Germany

Prof. Dr. med Erich Harms, Emeritus Direktor, Kinderklinik Münster, 48149 Münster, Germany

The objective of the present study is to determine if screening with urine strips can detect patients with Cystinosis during early childhood. This study plans to test infants born in Bavaria, during the next two years, with a urine dipstick at the age of 12 weeks. If this method yields valid as a screening method for Cystinosis, we plan to integrate it into the standard German child health care examinations.

Total grant: \$25,000

“Altered protein kinase signaling as a cause of reduced adhesion and increased motility of renal epithelial cells in Cystinosis”

E. Ivanova, L. van den Heuvel, E. Levtchenko
Katholieke Universiteit Leuven, Belgium

In this project, we will focus on the altered cytoskeleton and increased motility of cystine-deficient human podocytes and proximal tubular epithelial cells that may underlie the increased renal cell loss in cystinosis and the development of kidney damage. We will also further investigate the mechanisms of altered vesicular trafficking that can link disturbed endocytosis to protein kinase signalling. Finally, we will study the effects of cysteamine and various protein kinase inhibitors on the abnormal phenotype of cystinotic cells. The obtained results will contribute to the understanding of the pathogenesis of cystinosis and will help identifying novel therapeutic targets to improve the treatment of the disease.

Grant Year One: \$77,000

We are happy to support all three groups important work and will report results back to the community.



Dave and Christy Greeley at the 2015 CRN Family Conference in Chicago

Education & Awareness Update

By Terri Schleuder– Vice President of Education & Awareness



Terri Schleuder

The end of the year is fast approaching. As I write this the leaves are in peaked splendor with some already falling to the ground in a crunchy layer. There is a bite to the air and the taste of crisp apples, cider and warm cinnamon donuts is a delightful treat. It is also a time to look back on 2015 and the amazing things that have occurred within CRN and the cystinosis community this year.

A hugely successful event, the 2015 CRN Family Conference was held in Chicago, July 16-18th. The entire CRN Board under the incredible leadership of our conference Chairperson, Christy Greeley, worked nearly 2 years to plan this event. Our theme the *Winds of Change* fit perfectly with the 'Windy' city location. The Midwest location also heralded our largest attended conference to date with 340 people in attendance, including families, people living with cystinosis, physicians, researchers and pharmaceutical representatives from 12 countries. The latest information, catching up with old friends and making many new ones made it a memorable experience for all who attended.

Four new Board members were elected during the CRN Full Board meeting held prior to the conference in Chicago. We welcome the energy and new ideas of these wonderful people, Carol Hughes, Herbreth Sigler, Stephanie Sorensen, and Jennifer Loglisci. You may read more about them in this issue on pages 4 and 5.

CRN exhibited at the Pediatric Academic Society Conference (PAS) held in April, and the American Society of Nephrology Conference (ASN) held in November, both were in San Diego this year and provided the opportunity to network, educate and increase awareness about cystinosis with thousands of physicians in attendance.

In addition, CRN participated in Town Halls in Seattle in August, New Jersey in September, and Atlanta in October, and a Family Forum Luncheon Held in Los

Angeles in October. These events provide an opportunity to connect patients and families with each other regionally and with physicians who can answer questions and provide the most current information on research.



Steve and Carl Schleuder at the 2015 Family Conference in Chicago, with Carol Kelly, a PAM representative with Raptor, getting the latest research information at the Poster Session

In August CRN was pleased to award two \$1000 dollar academic scholarships to two deserving recipients. (Please read more about Garrett Thomas and Kasey Hohl in this issue on page 6).

CRN, represented by Christy Greeley and José Morales participated in the Global Genes Rare Patient Advocacy Summit and the Champions of Hope Gala both in held in Oct. in Huntington Beach, California.

Now available on CRN's website is a newly published **Cystinosis Transitional Management Tool** written by pediatric and adult physicians with experience treating Cystinosis patients for physicians who may not be familiar with the disease. Please check out the amazing document at this link <https://cystinosis.org/images/research/article-library/overview/Cystinosis-GuideFinal.pdf>

One of the highlights in our biennial newsletter is featuring families and adults successfully living with cystinosis each day. This issue features equestrian, Megan Morrill and her family, and Lee Knaus, an artist whose beautiful glass creations were on display at the Family Conference in Chicago. I know you will be inspired by their stories.

Many fundraising events have also occurred since our last issue was published including the C.H. Robinson Golf Tournament and Walkie Talkie 5K run, the Prairie House Corn Roast honoring Jack Greeley, Flippin Friends 2nd Annual Pinball Tournament also honoring Jack Greeley, and "Paint with a Purpose" in honor of Peyton Taylor hosted by Autumn Palmatteer,

CRN continues to do its best to serve the cystinosis community, and meet its stated vision and mission and will continue to do so as the New Year begins.

Rare Disease Day 2016 Theme Announced

NORD and its European partner, EURORDIS recently announced the theme for Rare Disease Day 2016. The theme for this year's Rare Disease Day is *Patient Voice* with the slogan, *Join us in making the voice of rare diseases heard.*

Rare Disease Day 2016 will be celebrated on February 29, 2016 and will mark the ninth year that the International rare disease community has come together to celebrate this event.

There are many ways to get involved and raise awareness about Cystinosis. Please check out the attached link for updates from NORD about Rare Disease Day.



<https://rarediseases.org/rare-disease-day-2016-theme-and-slogan-announced/>

CRN Development Update

By José T. Morales, Vice President of Development

The pace for 2015 has been torrid. There simply never seems to be a time to sit back and catch one's breath. I believe this is true for the majority of us as we continue our respective journeys. Parenting to the best of our abilities. Guiding, supporting our kids. Endeavoring to leverage our individual and collective experiences to provide the best support we can.

For the Development Committee, we also expend a lot of energy to oversee the organization's fundraising efforts in order to pursue CRN's vision and mission:

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

Mission - The Cystinosis Research Network is a volunteer, non-profit organization dedicated to supporting and advocating **research**, providing family **assistance** and **educating** the public and medical communities about cystinosis

CRN is committed to holistically serve and represent our community's needs. In conjunction with the broad scope and expanding activities, CRN expends significant efforts towards organizing and promoting fundraising on behalf of the organization. The following are the names of your Development Committee:

Development Committee

Chair – José Morales – jose.morales01@icloud.com

Corporate Sponsorship

Jeff Larimore - JLarimore@arnoldfamilycorp.com

John Maccarone - johnm@maccaroneplumbing.com

Deb Reed - dk_reed@hotmail.com

Fund Raising

Rachel DaLomba - rdalomb@my.wgu.edu

Katie Larimore - klarimore112@sc.rr.com

Katie Morrison - katiemorrison91@gmail.com

Briana Smythe - bri_ana15@hotmail.com

Jen Wyman - jwyman@comacst.net

Administration

Christy Greeley - Greeleycd@aol.com

Jen Wyman - jwyman@comcst.net

The Development Committee recently completed an internal project focused on reengineering our development policies and procedures. The project team was comprised of

Rachael DaLomba, Katie Larimore, Tai Spargo and José Morales.

Project Objectives:

- Broaden fundraising outreach and scope
- Enhance branding and marketing of CRN’s contributions
- Document and formalize fundraising policies and procedures

The policies and procedures are in the final stages of review and are planned to go into effect at the beginning of 2016.

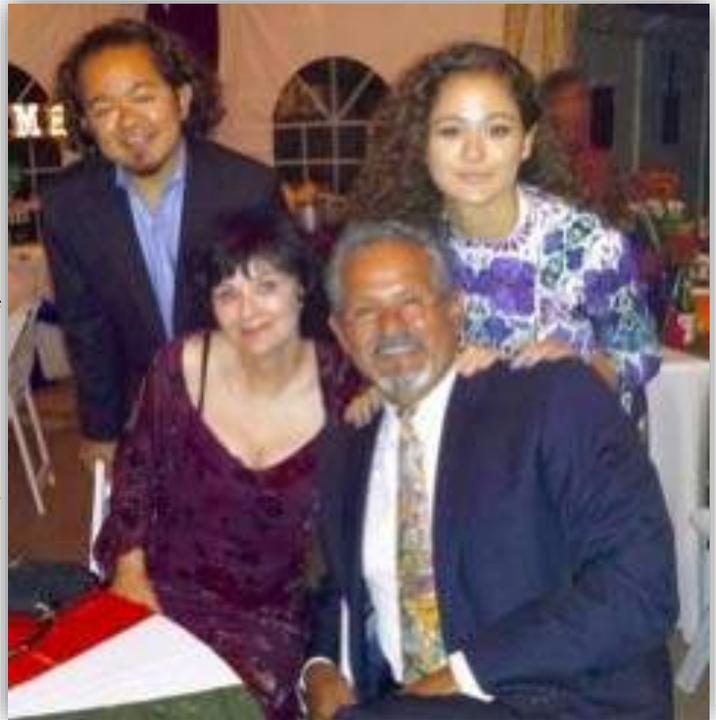
A number of fundraisers were successfully conducted since our last newsletter. Be sure to read the four articles highlighting our community’s commitment to making a difference:

Paint Night – in honor of Peyton Taylor and sponsored by the Autumn Palmateer Family

Prairie House Corn Roast – in honor of Jack Greeley and sponsored by the Prairie House Restaurant

Flippin Friends Pinball Tournament – in honor of Jack Greeley and sponsored by Flippin Friends/Jeff Hooper

C.H. Robinson Golf and Fun Run – in honor of Lola Long and sponsored by Tim Miller, Pam and Tahníe Woodward



The Morales family, from left to right Christian, Velyna, José, and Aexandra.

I would be remiss if I did not mention **Chandler’s Chance Christmas Palooza**, scheduled on December 5, 2015, in honor of Chandler Moore and sponsored by the Clint Moore Family. This will be the third consecutive year this event is being held and features music, free hot chocolate, coffee and cookies, fire truck rides, hay-rides and, of course, Mr. and Mrs. Santa Claus. What a wonderful way to begin the holiday season. Ya’ll be sure and attend if you find yourself in the vicinity of 24062 Peterkins Road, Georgetown, DE 19947.

Please reach out and thank our sponsoring families and individuals for their commitment and contributions, which enable us to continue to be powerful advocates for our children.

As always, we are interested in increasing the number of individuals involved with CRN. Please give serious consideration to engaging in some capacity with the organization. The Development Committee is available to assist you if you are interested in joining our committee or sponsoring an event. Remember, how can we expect others to advocate for us if we do not take a strong stance ourselves. Please reach out to one of our committee members or myself jose.morales01@icloud.com / 203 722-9292, to become involved.

Cystinosis Heroes in Egypt

By Professor Neveen A Soliman

The Egyptian Group for Orphan Renal Diseases (EGORD) started its journey to identify patients living with rare kidney diseases more than a decade ago. It all started with "Aya" a 6 years-old girl living with cystinosis. We were excited then that we could finally offer Aya and her family a diagnosis after quite a long and exhaustive odyssey of uncertainty. We knew there were challenges ahead in terms of medical care and access to therapy, but we never imagined such a bumpy road. In fact we stepped into a whole new world of numerous hurdles and less achievements. EGORD got to discover, through caring for cystinosis, where does caring for rare diseases stand in terms of medical awareness, society, and health care system regulations/legislations.

Through the whole journey, what kept us really going and endlessly motivated (despite the disappointments related to access to therapy, funding and pricing issues) were our amazing heroes and their dedicated families. Their courage, commitment, and above all their highly contagious smiles continue to be the driving force to improve the status of cystinosis and other rare diseases where it is desperately needed, not just nationally but hopefully regionally as well.

A huge tribute to cystinosis heroes in Egypt and worldwide!



A gift from Egypt presented to CRN



Some of the cystinosis heroes in Egypt

UCSD Announces a New Cystine Determination Test and the Opening of a Multi-specialty Cystinosis Clinic

By Dr. Bruce Barshop

The new testing:

The UCSD Cystine Determination Laboratory has developed and validated a new method for WBC cystine assay, which permits whole blood samples to be sent, without requiring special preparation of the cells at the point of care. The samples must be drawn into a yellow-top (ACD) tube, kept at refrigerator temperature (4 degrees Centigrade—not frozen), and shipped on the same day for overnight delivery, packed in a Styrofoam mailing container along with at least 3 well-frozen, medium-sized (minimum 6x6 inch) cold-packs (complete instructions available at the UCSD Cystine Determination Lab: 619-543-5260 or www.ucbdbqglab.org). **The samples must be received within 30 hours of being drawn. Note that this new test measures granulocyte cystine, and the results are different (usually higher) than the previous assay using mixed leukocytes. The therapeutic target for this new assay in granulocytes is 1.9 to 2 nmol/mg protein.**

Also, please note that the UCSD Cystine Determination Lab is also happy to continue running the assay on mixed leukocyte preparations, when the referring lab is able to promptly prepare the samples in the usual manner.

The clinic:

The physicians and specialists at the University of California San Diego have started a multi-specialty Cystinosis Clinic, including, among other specialists, Dr. Bruce Barshop and Dr. Annette Feigenbaum (Biochemical Genetics), Dr. Nadine Benador (Nephrology), Dr. Ranjan Dohil (Gastroenterology), Dr. Doris Trauner (Neurology), Dr. Ron Newfield (Endocrinology), Dr. David Granet (Ophthalmology), and Dr. Stephanie Cherqui (Laboratory Scientist). The clinics will be held on Monday mornings (every 3 months at present), at the UCSD/Rady Children's Hospital Medical/Research Offices at 7910 Frost Street, San Diego. If you are interested, please contact Denise Young, RN, at 858-576-1700 X4642, or send email to dnyoung@rchsd.org or to cystinosis@ucsd.edu. We will require information ahead of time, for insurance authorization to be arranged and for arrangements for medical records to be released and forwarded.



We are now on YouTube!
The CRN YouTube channel features new and archived videos of the Cystinosis community. You can access footage from the 2015 Family Conference including the "Dream, Achieve, Inspire" art exhibit and creation of a Cystinosis mural. To subscribe, go to www.youtube.com and search Cystinosis Research Network.

C. H. Robinson Golf Event another Great Success for CRN

By Tim Miller



Lola Long

It all started rather early the first weekend in June. It was decided by Committee to kick off the annual Cystinosis Charity Golf event of 2015 with a 5K fun walk with the hopes of creating another revenue stream. Now when I say Committee you should know whom I speak of! First there is **Pam Woodward** along with her daughter, **Tahn**ie, who have been a part of the golf event from the beginning. Actually they are the reason C.H. Robinson got involved with CRN in the first place and Tahn



CRN President, Jeff Larimore and VP of Family Support, Pam Woodward

The past two years, the event had a personal connection to C.H. Robinson. In 2013, **Lola Long**, daughter of **Jim** (C.H. Robinson of Information Technology Manager) & **Melissa Long** was diagnosed with cystinosis. I am privileged, as a member of the CRN Board, to have assisted Jim and his wife Melissa

connect with CRN to assist them in their journey in providing Lola with the best healthcare.

Then there is an extraordinary bright, driven and compassionate lady who will blush when she reads this article.

Jeannie Oki is a retired C.H. Robinson employee who has supported the golf event with several foursomes and sponsorships over the years. She is a precious gem and I'm fortunate to have found. Without her commitment to the 5K event I know it would only have been an afterthought rather than a reality. She found walkers and prizes and helped organize so many parts of the 5K fun walk.

So there you have it, the Committee who in the end are just a handful of individuals who knew that taking action and risking hours of work for an unknown outcome was worth a gamble. They also knew there was a possibility of really impacting the annual golf event in a negative way by messing with the "Karma" of this successful fundraiser. That's why hours of time was committed into looking at all of the details



Rory Boyer, Tahn

and hammering out the schedule of events.

I also have to point out three other individuals who make this all happen. **Mark Whetzel**, Director of Golf for Vanguard Golf Management was instrumental with the execution of the day. He lined up a 5K route on the golf course that kept us from having to get permits from the city. He also provided solution for keeping the golf event from being impacted as he offered serving lunches on the course! Mark's connections and willingness to keep this event rolling is nothing short of amazing.

Then there is the team at C.H.Robinson. While **Alicia Madsen** was not able to attend, she still had her finger on so many things! Without



her support

Michelle Boyer crosses the finish line of the 5-K Fun Run flanked by Tahnne Woodward and Sookie Boyer

we could not have offered up a "hole" prize and countless other "perks" for the event. The **Patrick Darby**, the new General Manager of the Salt Lake Office, was key as he provided a solid base of support both financially and physically. When we needed help moving and storing things he was always able to provide the muscle. Of course the strength of that support came from the staff at C.H.Robinson.

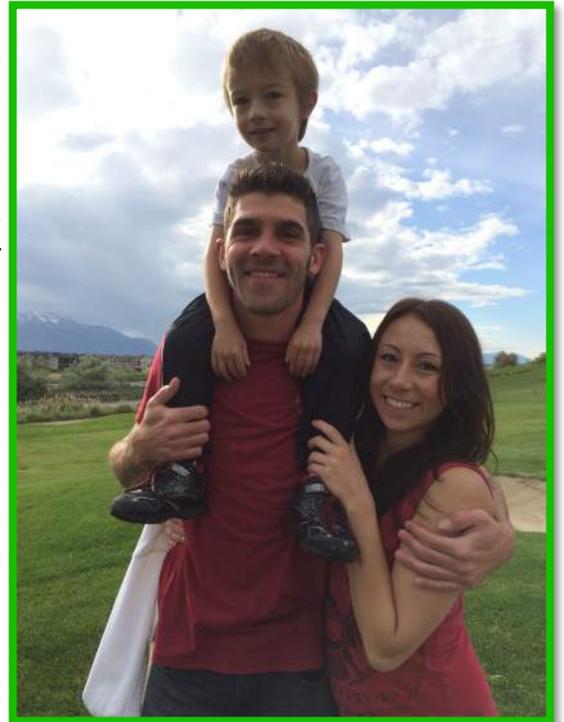
So it all started really early that June weekend without a hitch. Walkers walked and enjoyed a few treats and prizes for their efforts. Golfers, well they golfed and enjoyed



Tim Miller and José Morales

enjoyed a lunch on the course while finishing in record time. Much money was raised while smiles and laughter abounded. It was a bitter-sweet event for me as I have retired from C.H.Robinson and the tradition of this CRN event is in doubt. It was wonderful to see the support of my C.H.Robinson Family from across the country as they joined me for one last time. They brought the much needed support both financially and morally. I'll never be able to say thank you enough to all of them.

I am sincerely grateful to have been a part of a wonderful event that filled my life with a great amount of pride.



A great day of fun for all in attendance!

World Fair 2015: Lysosomal Diseases Meeting

By Sara Healy

There were many things I didn't know about my disease until now. The speakers at the meeting talked about metabolic acidosis, growth hormones and spurts, CTNS gene encoding, new WBC test (granular blood testing), the most common types of cystinosis, how to thrive with cystinosis, and new bullying threats in this day and age.

Metabolic Acidosis is one of the stages of loss of amino acids. It's basically like a person having some missing pieces. There is treatment, usually through sodium bicarbonate supplements.

Growth varies among people with cystinosis. People with cystinosis usually have a growth rate that is below average. Many people with cystinosis have to take growth hormones to keep up the growth of with normal kids their age. It doesn't always work though. In some cases growth hormones are ineffective.

CTNS genes are proteins that transport cysteine out of lysosomes. Mutations in these genes cause problems with transportation leaving cysteine to pile up into cysteine crystals. That mutation problem results in organs and blood in the body being affected, causing one of the rare diseases called cystinosis.

There has recently been a new blood test made available to people with cystinosis. It is called Granular WBC Blood Testing. Unlike the regular WBC test, this test doesn't get processed by multiple labs, this way there is less of a chance for error. It gets sent directly to the lab in California where it is tested.

I also learned there are three different types of Cystinosis. The first one is called Infantile or Nephropathic (early onset). It is the most common type affecting 95% of those with cystinosis. The second is Juvenile (late onset); only 4% to 5% get this. The last one is called Ocular or non-nephropathic (adult onset); only around 1% are affected.

The last two things I want to talk about are thriving with cystinosis and bullying. I have had personal experience with both. The need to feel normal is a really strong feeling. When my twin sister Jana and I were young our parents helped us with our meds and daily routines. It was hard for us as we never felt normal. The moment the kids found out about our disease they treated us differently. It didn't help that our drugs made our bodies along with our breath smell bad. We had a few friends but they never accepted everything about us. We were subjected to tests, labs and doctors our whole lives. We felt alone and depressed a lot, but it least we had each other.



Dr. Bradley S. Miller, M.D., PhD. was the speaker at the conference.

In high school we thought if we wrote a paper about cystinosis and explained our disease to the class that people would finally accept us and not treat us so differently. We were wrong. They were nice to our faces but they spread rumors about us behind our backs. One of the worst rumors they spread was that we were dying. I think if actual qualified doctors came and talked to our class maybe they would have actually treated us with dignity and respect. Bullying has gotten really bad, and now with social media it has even spread to the internet. One thing that will help with the bullying I think, is keeping families and friends with cystinosis informed and being sure they can always get a hold of experts and other people like them making them feel like they are not alone and that they do have support and love. This is something I have found with the Chronic Disease Fund and Cystinosis Research Network.

If we would have known about the Chronic Disease Fund and Cystinosis Research Network sooner I believe living with cystinosis would have been much easier, especially when life throws the occasional curve ball. I know recently finding these programs and meeting all these people like us has made our lives so much better.

I hope to keep learning and meeting new people and learning new things about my disease as the years go by.



Attendees of the 2015 World Fair Lysosomal Storage Conference held in September in St. Paul, Minnesota.

CRN Attends 2015 ASN Conference in San Diego



Dr. Bruce Barshop, Dr. Rick Kaskel, Tahníe Woodward, Pam Woodward, and Dr. Paul Goodyer at the 2015 ASN Conference in San Diego

CRN was represented as an exhibitor at the 2015 American Society of Nephrology Conference held in San Diego the first week of November.

Pam Woodward, CRN's VP of Family Support and her daughter Tahníe were on hand to answer questions and hand out educational materials about cystinosis to attending physicians. Included in this year's information was the newly published *Cystinosis Transitional Management Tool*, a comprehensive guide written by physicians familiar with cystinosis for those physicians treating patients who are unfamiliar with this rare disease.

It was another great opportunity for CRN to educate and raise awareness among the medical profession about Cystinosis.

CRN provides Patient Advocacy Viewpoint at Raptor Town Hall events in Seattle, WA and Jersey City, NJ

By Jeff Larimore

When Raptor Pharmaceuticals provided Cystinosis patient advocacy organizations the opportunity to participate in the Town Hall event series, the Cystinosis Research Network welcomed the invitation. In August and September, Raptor hosted Town Hall programs in Seattle, WA and Jersey City, NJ and Jeff Larimore represented CRN discussing the role and function of patient advocacy for the cystinosis community.



Jeff Larimore, President of CRN, at a Town Hall Meeting in Jersey City, New Jersey addressing families and cystinosis patients.

The 30-minute presentations at these two Town Halls discussed

CRN's mission and vision and its commitment to nearly 20 years of community support in the areas of research, family assistance and medical education for cystinosis.

Each of these two Town Halls had a large contingent of adults with cystinosis. In addition to discussing CRN's activities and resources, the dialogue addressed key subjects that are directly important, and potentially unique, to the adult community. Some of the key topics included:

- Disease Awareness for Lysosomal Storage Disorders
- Risk of Dehydration / Emergency Room Health Care Services
- Muscle Degeneration
- Long Term effects of Cystine Crystals
- Stem cell, Live cell, Gene Therapy programs
- Kidney Transplant rejection systems
- Kidney Transplant Education
- Psychological/Neurological Support

Support from Peers, Continuous Learning
Nutritional Support Strategies
Pediatric to Adult Care – Mentors
Medical Insurance Options

In addition to adult initiatives, engaging conversations were had with younger individuals with cystinosis as well as their caregivers to share the resources and relationships that CRN can provide.

CRN appreciates being a part of the Town Halls as it provides chances to share the message of the importance of patient advocacy for cystinosis and the far-reaching benefits that involvement within patient advocacy can bring.



Sarah Larimore (far right) with Emma and Gracie Patterson, enjoy time together in Seattle, Washington while attending a recent Town Hall meeting with their families.



Families gather in Seattle, Washington at a recent Town Hall meeting to learn the latest about cystinosis research and to connect with each other



Little ones having fun at the Town Hall meeting in Seattle, Washington.

Join the Cystinosis Research Network

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

Join The Cystinosis Research Network (CRN) and become part of a 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"...whether you are...

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

Joining the Cystinosis Research Network enables you to:

- Receive all the latest cystinosis information through our countless resources, including the biannual CRN Newsletter, our very informative web page www.cystinosis.org, the popular online Cystinosis Support Group, and our toll free number (1-866-276-3669).
- Attend the CRN Family Conference with other cystinosis families to exchange knowledge and create friendships. Also, find out the latest discoveries about cystinosis from the medical professionals.
- Let your voice be heard by legislators and policymakers who need to know why cystinosis (and other rare diseases) are important issues to you.
- Have access to the Cystinosis Research Network's representatives in the areas that are most relevant at any given time to you or your loved one affected by Cystinosis.

Join Cystinosis Research Network today!

Thank you for your consideration in becoming a member of Cystinosis Research Network.

Pam Woodward
VP Family Support, Cystinosis Research Network



Dedicated to a Cure. Committed to our Community.



Join the Cystinosis Research Network Today!

Immediate Family	\$20.00
Extended Family / Friend	\$25.00
Professional	\$35.00

International: (Including Canada) Base rate (see above categories) plus \$10.00 for postage.

Payable in US dollars

Please complete the form & mail with check payable to CRN to:
 Cystinosis Research Network
 302 Whytegate Ct.
 Lake Forest, IL 60045

Name _____

Street _____

City & State _____ Zip Code _____ Country _____

Phone _____ Fax _____ Email _____

Name of Child / Adult / Acquaintance / Patient affected with cystinosis: _____

Join A CRN Support Group

Looking for a way to communicate with others in the cystinosis community on a day-to-day basis?

The Cystinosis Research Network offers two email support groups for communicating with others in the cystinosis community:

The CRN Support Group is a group for parents, affected adults, caregivers, family, and friends. We also welcome researchers and medical professionals who are interested in cystinosis. This is the place to discuss the various aspects of cystinosis, and how it affects our lives, how we cope, vent our frustrations, share our fears, our hopes, and our dreams.

The CRN Teen Support Group is for teens with cystinosis and teenage siblings of children and adults with cystinosis. Connect with other teenagers who are dealing with similar issues. The posts include questions, concerns, ideas and supportive sharing.

To join a support group, visit www.cystinosis.org.

Support CRN's Mission with Your Donation

YES, I want to help children and adults with cystinosis.

Enclosed is my tax deductible contribution of: \$ _____ made payable to the Cystinosis Research Network (CRN) and mail to: 302 Whytegate Ave., Lake Forest, IL 60045

Name _____

Street _____

City & State _____ Zip Code _____

Phone _____ Fax _____ Email _____

In Honor Of _____

In Memory Of _____

You may send notification of my gift to:

Please check all that apply:

- | | |
|--|--|
| <input type="checkbox"/> Friend | <input type="checkbox"/> Individual with Cystinosis |
| <input type="checkbox"/> Parent of Child with Cystinosis | <input type="checkbox"/> Professional |
| <input type="checkbox"/> Family | <input type="checkbox"/> I am interested in volunteering for CRN. Please contact me. |

Search the Web with GoodSearch & Raise Money for CRN

Every time you use GoodSearch.com to search the Web, a donation is made to the Cystinosis Research Network! GoodSearch.com is powered by Yahoo!, so you get the same results you get from most search engines. What is unique is that GoodSearch.com has developed a way to direct money to your selected charity with every click!

To get started, go to goodsearch.com and select Cystinosis Research Network where it says "Choose your cause." Then search like you normally would!

The more people who use this site for CRN, the more money is earned. So please tell your friends and family!



Make Purchases at GoodShop & Raise Money for CRN

GoodShop.com allows you to purchase through most online retailers, and a percentage of your purchase goes to the Cystinosis Research Network! There is no additional cost to you!

To get started, go to goodshop.com, choose CRN as your charity, and click through the link on the GoodShop page to get to your favorite retailer. It's that easy!

GoodShop will donate up to 30% of your purchase to CRN. Some of the hundreds of retailers include: Best Buy, iTunes, Home Depot, Amazon, Barnes & Noble, Dell, Banana Republic, Macy's, Target, Wal-Mart, Ann Taylor Loft, Chicco, Coldwater Creek, American Eagle Outfitters, and many more!





United Way Contribution Guidelines

Identify the Cystinosis Research Network, Inc. as the agency you want to receive your contribution through the United Way Donor Choice Program.

Agency Name	The Cystinosis Research Network, Inc.
Non-Profit Tax ID #	04-3323789
Address	302 Whytegate Ct., Lake Forest, IL 60045
Telephone	1-866-276-3669 (toll free), 1-847-735-0471
Fax	847-235-2773
E-mail Address	CRN@cystinosis.org
Web Page	www.cystinosis.org
<p>The local United Way organization will contact The Cystinosis Research Network via phone, fax, or e-mail to request we prepare and submit documentation verifying our status as a non-profit organization.</p> <p>The Cystinosis Research Network prepares all necessary documentation and submits it to the respective local United Way organization.</p> <p>The local United Way organization processes the documentation and sends a check for the aggregate sum designated for the Cystinosis Research Network.</p> <p>The Cystinosis Research Network sends thank you/acknowledgement letters to recognize contributing individuals.</p>	

Donate to CRN by Selling on eBay

CRN is registered with MissionFish, the exclusive charity provider for eBay Giving Works. eBay sellers can now list items through eBay Giving Works and designate a percentage of the sales to go to CRN. The seller picks the percentage, and all money donated is tax deductible. eBay will even refund a percentage of listing and final value fees that is equal to the percentage sellers donate! Items listed with eBay Giving Works are given a special icon, so they stand out. Some sellers report 20-40% higher sale prices for the exact same item using eBay Giving Works. Give it a try, and be sure to tell established eBay sellers about this great opportunity to give to CRN!



Learn more about the CRN Amazon Smile Program by clicking on the link below:

https://org.amazon.com/npo/portal/marketing-tools/ref=org_prt_gwh_mt

Cystinosis Research Network
302 Whytegate Ct.
Lake Forest, IL 60045



Dedicated to a Cure. Committed to our Community.

Cystinosis is a rare, genetic, metabolic disease that causes an amino acid, cysteine, to accumulate in various organs of the body, including the kidneys, eyes, liver, 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.

302 Whytegate Ct.
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Editor: Terri Schleuder

CRN's Vision and Mission

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.*

Mission. *The Cystinosis Research Network (CRN) 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.*

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 [YouTube](https://www.youtube.com/CystinosisResearchNetwork)