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2017 CRN Conference set for Beautiful Snowbird Utah

The Cystinosis Research Network is pleased to announce that its 2017 Family Conference will be held July 13 – 15 at the Snowbird Ski and Summer Resort located in Utah.



We hope you will plan to join us and learn about the latest updates in Cystinosis research through lectures, workshops, poster sessions, and medical and patient panels. There will also be opportunities to socialize with other patients and families which will provide valuable connections and friendships that will last a lifetime. This is a great chance to meet others who are walking in your shoes and also renew prior friendships. CRN is committed to providing family support to those living with Cystinosis. The family conference brings families and the medical community together to share hope and support.

Located up Little Cottonwood Canyon in the beautiful Wasatch Mountains, the resort has something for everyone. The lodging will include the Cliff Lodge, which is a hotel, and also the Lodge at Snowbird which are condos equipped with kitchens. There are lots of activities including Mountain Coaster, Alpine Slide, Mountain Flyer, Vertical Drop, Ropes Course, Climbing Wall, Bungee Trampoline, Aerial Tram and Peruvian Chairlift. Getting to the Resort is easy, the Salt Lake International airport is only 30 minutes away. For more information about the resort please visit their website at www.snowbird.com

Registration materials will be available in early 2017 on the CRN website and will also be mailed to families. Special room rates have been negotiated and will be made available to conference attendees.

We look forward to seeing you in Snowbird in July 2017!



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Message from the President Jeff Larimore

Each opportunity I am given to write a president's letter spurs moments of reflection, an evaluation of the present, and an eye towards the future. Over the past four years, I reflect on the progress that the wonderful volunteers within the Cystinosis Research Network have made in raising dollars for clinical research studies around the globe with the goal to help more with Cystinosis in need. During this same time period, the Cystinosis Research Network has broadened the scope of awareness throughout the entire Cystinosis community with a target of a global community vision. In concert with growing awareness, new demands in progressive education arise, these areas of concentration are a short list of what the Cystinosis Research Network has accomplished.

In this current edition of the Cystinosis Advocate, we will provide the latest information on clinical research grants and current medical areas of interest, updates from recent medical symposiums concentrating on Cystinosis and other medical disorders that may affect the Cystinosis community. You will receive highlights of the array of fundraising activities over the last half-year that will assist CRN with future calls for research proposals. In addition, we will recap the Cystinosis communities' activities in Washington D.C. during Rare Disease Week. Engagements during Rare Disease Week presents how our elected officials can engage in progressive conversation for all those challenged with a rare disease. Looking forward, the Cystinosis Research Network's committees and its Board members will remain proactively engaged to represent the Cystinosis community. We will continue our efforts to assist in streamlining across to medical education, to represent the Cystinosis community in any capacity, to support healthcare initiatives, to engage with our medical advisors on the status on the status of cutting edge research for Cystinosis.



Jeff and Sarah Larimore

Support received in many ways is the life blood of a patient advocacy organization anytime. The dollars invested are invaluable and the time given is precious. I want to assure each of you reading this spring newsletter that the Cystinosis Research Network will remain a highly-engaged patient advocacy organization because of its committed volunteers. Be a part of the volunteer base-it is extraordinarily important to our community.

Best wishes to a fun and engaging summer,

Jeff Larimore

Dr. Galina Nesterova completes CRN NIH Clinical Fellowship, takes position in Vancouver, B.C.

Dr. Galina Nesterova has completed her 6 year CRN NIH Clinical Fellowship under the direction of Dr. William Gahl and has accepted a position at BC Children's Hospital of British Columbia, Vancouver, B.C. Patients can contact Dr. Nesterova via email at either nesterovag@mail.nih.gov or Galina.nesterova@cw.bc.ca

The Cystinosis Research Network is indebted to Dr. Nesterova for dedicating a significant portion of her career to the treatment of hundreds of individuals with cystinosis. She has become an expert in the disorder and continues to be one of our community's greatest advocates.

We are pleased to announce that she will be joining CRN's Medical Advisory Board and will thus continue her outstanding service to the cystinosis community.



Dr. Galina Nesterova



Adventures in DC with Cystinosis Research Network

By Aimee Aldemann

A year ago I couldn't imagine writing this article. A year ago I felt like I was pretty separated from the Cystinosis community. Now I feel have been reconnected. Last summer I went to a town hall meeting including RaptorCares, other people with Cystinosis and family members. It changed my outlook and reinvigorated my passion to work for change that would benefit all of us with Cystinosis. It made me realize I really could do something to help by becoming more active as an advocate. I have been working as an educator and advocate for organ donation through my job with Donate Life Northwest but I hadn't done any advocacy specific to Cystinosis.



Jennifer Loglisci, Chandler Moore and Aimee Aldemann all attended Rare Disease Week on Capitol Hill and found it to be an empowering experience.

In March of this year I got that opportunity - CRN and RaptorCares supported me to attend the 2016 Rare Disease Week on Capitol Hill in Washington DC, February 29 to March 3. The annual event is coordinated by a national collaborative, Rare Disease Legislative Advocates. It was an amazing experience! Over 350 people from around the country participated in this event, including people with rare diseases, family members, and representatives from advocacy organizations. During the week we had a chance to:

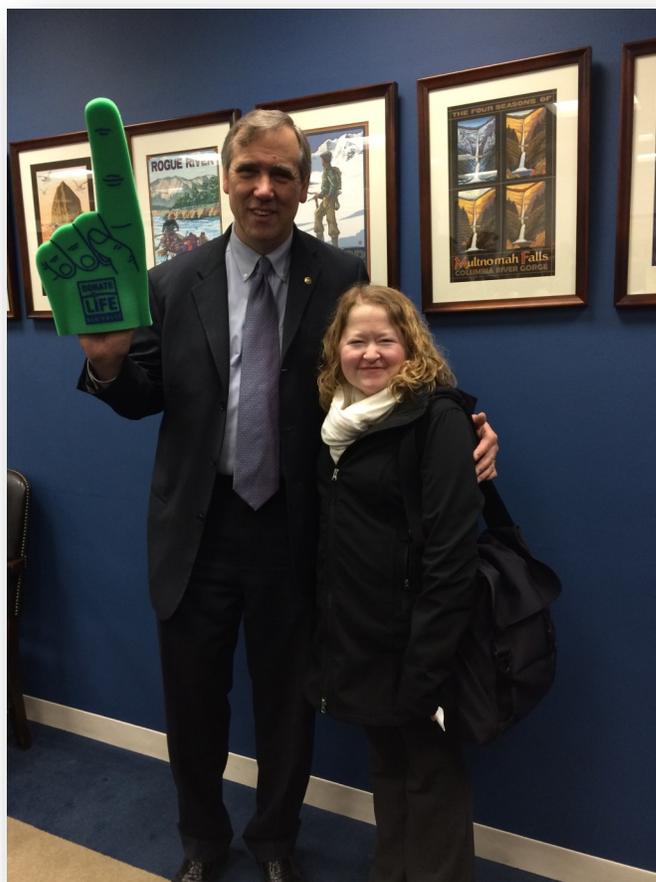
- Meet each other and learn about issues and challenges that are common to all of us, as well as some that are different because of economics, geography, and political will,
- Find out about bills currently under consideration that would improve life for people with rare diseases and allow them to get appropriate and timely care,
- Learn about how to be an effective advocate with federal senators and representatives, and,
- Meet with members of Congress to tell our stories and ask for support for specific legislation.

It was really incredible to talk to other people that had experiences that were very similar to mine (including several with Cystinosis) and to learn from those whose experience was very different. It was heart-wrenching to hear from many people who have struggled for years without treatment and/or payment options and who are looking for anything that could help them and their family members. Personally and collectively this time together increased our passion for change and helped us to understand how we can truly use our personal stories to advocate for positive changes.

Many of the Senators and Representatives were very supportive. I felt like our efforts were important in helping them understand why change is needed and that they have the opportunity to make real a difference in the lives people with rare diseases. I learned so much during this week that will help me be a better advocate. I hope others in our Cystinosis community will be able to have this experience in the future.

This experience has made me realize how lucky I am to have benefited from prior research and advocacy, and to have treatment options that have truly improved the health of those of us with Cystinosis. Now, don't get me wrong taking meds sucks, but at least we have the meds to take! I hope by getting more people involved with advocacy and education, we can have a larger impact on future generations. I really believe if we can work together there will be a brighter future for all of us!

Thanks to the work of our Cystinosis advocates on Capitol Hill, South Carolina Rep. Joe Wilson and Massachusetts Rep. Joseph Kennedy III have requested to become members of the Rare Disease Caucus. The caucus objectives include generating media attention and raising public awareness for rare diseases like Cystinosis.



Aimee Aldemann with Senator Jeff Merkley from her home state of Oregon.

If you would like more information about Rare Disease Week on Capitol Hill, you can visit <http://rareadvocates.org/rdw/>. You may also view this Rare Disease Week video at this link. https://www.youtube.com/watch?v=hYoY9uFj0_I

Contact a CRN Board member to learn more about becoming a Cystinosis Advocate.

(for more Rare Disease Week photo highlights see page 9)

Wyman's Hold 10th and Final 5K Fun Run for CRN in Honor of Kacy

By Jen Wyman



Alexis and Evan LeBeau front row. Jack Greeley and Kacy Wyman back row are enjoying the day.

Sunday, May 1 marked our 10th and final Cystinosis 5K Fun Run. It's hard to believe it has been 10 years since Kacy was diagnosed. What started out as a Bar Mitzvah project for 3 young boys in our community transpired into a very successful fundraiser. From the day we received Kacy's diagnosis our "people" stepped up for our family in countless ways. It's easy to give the numbers and report that our community has raised in excess of \$300,000 over the last 10 years and upwards of \$30,000 for this year's run, but equally important is the emotional support that we have received. It gives us wings to fly and hope to hold on to. Cystinosis has been a blessing and a curse...it has given us some challenging times and has brought wonderful people into our lives, strengthened existing friendships and bonded our family.

"They say a person needs just three things to be truly happy in this world: someone to love, something to do, and something to hope for."



Tee Shirts celebrating 10 years of 5K Fun Runs in support of CRN

We certainly have all three of those things in abundance.



Michelle Kelly, Sara Bruhn with her daughter, Emma and Jen Wyman



Jack Wyman and his friend Libby



Sisters, Tia Carmichael and Jen Wyman enjoy the day



Kacy Wyman with Jill Morrill



Steve Schleuder with José Morales

Final 5K Fun Run Highlights



Pat D'Ambrosio with Kacy



Front row: Alexis and Evan LeBeau, and José Morales, Back row: Jen Wyman, Brett LeBeau, and Jill Morrill

Wymans Host Donor Appreciation Party

By Jen Wyman



Brittney LeBeau, Jen Wyman and Christy Greeley

On April 30 the Wymans hosted a Donor Appreciation Party to honor and thank the wonderful people in our lives who have repeatedly supported our cause over the course of 10 years. It was a beautiful night of fun and dancing, a silent auction and plenty of food and drink. We are grateful to have such wonderful people in our lives.



Brittney LeBeau and Jill Morrill



Brett LeBeau, Tim Wyman and Dave Greeley



Jeff Larimore, Jennifer Loglisci, and José Morales



Annie, Chandler, and Clinton Moore with Sen. Thomas Carper from Delaware



Wisconsin Rep. Mark Pocan and Alex Schulze

Highlights from Rare Disease Week on Capitol Hill

Right- Raptor CEO, Julie Smith with Chandler Moore



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.ucbdbglab.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.

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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Rare dedication

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Financial Update

by **Jeff Larimore**

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

For the three(3) months ended March 31, 2016

Revenues

For the three months ended March 31, 2016, total income collected of \$72,000 was approximately 40% less than the same period in 2015. This decrease was attributable to a reduction in corporate grants received to support CRN's 2015 Family Conference and the Dream, Achieve, Inspire Art Exhibit. Favorably, fundraising revenues of approximately \$44,000 through March 2016 was 25% higher than the first quarter of 2015.

Expenses

Total operating expenses of \$29,700 were 8% more than operating expenses for the three months ended March 31, 2015. A slight increase in fundraising expenses to support second quarter 2016 revenues accounted for the increased in total operating expenses. Total General & Administrative costs were approximately \$4,000 in both reporting periods. G&A costs as a percentage of income increased from 4.5% to 6.0% due to the reduction in quarterly inflows.

CRN had net operating income of \$42,000 for the three months ending March 31, 2016 as compared to net operating income of \$107,000 through March 2015. Successful fundraising activities and corporate support in 2016 and 2015 have provided cash resources to increase patient advocacy activities to support the cystinosis community.

Cash on hand at March 31, 2016 was \$355,415. Net change in cash for the last three months is an increase in \$39,000. This increase approximates the amount of net operating income for the first quarter of 2016.



Jeff Larimore and Jay Greissing from Shire Pharmaceuticals attend Rare Disease Week on Capitol Hill

CRN to sponsor IPNA 2016 Cystinosis Symposium in Brazil

The Cystinosis Research Network is pleased to announce its sponsorship of the 17th Congress of the International Pediatric Nephrology Association, IPNA 2016, which will take place September 20-24 2016, in Iguacu, Brazil. www.ipna2016.com



The International Pediatric Nephrology Association (IPNA) is comprised of roughly 1,500 pediatric nephrologists and allied professionals representing 89 countries around the world. Together, they work to disseminate knowledge about kidney disease in children in the areas where care is most needed. Every 3 years, IPNA organizes an International Congress reflecting what is going on in Pediatric Nephrology in the world and presenting the best work of all subfields. Specifically, CRN will be supporting the cystinosis symposium which will take place during the 2 day pre-congress course on rare diseases and will be chaired by Dr. Levtchenko and Dr. Nesterova. There will be also a congress presentation by Dr. Cherqui on new approaches to cystinosis during the renal tubular acidosis symposium.

CRN Announces 2016 Scholarship Application Deadline

Cystinosis Research Network is pleased to offer two \$1,000 Scholarships this year. One is for an individual with cystinosis. The other is the Sierra Woodward Sibling Scholarship offered to the sibling of an individual with cystinosis. Information about, and forms for both scholarships, can be accessed on CRN's website <https://cystinosis.org/family-support/scholarships>. The deadline to receive the applications is August 15th, 2016.

Both scholarships are available to persons attending a qualifying College, University or Trade School. They are offered to those beginning their college career or already attending college. They may also be reapplied for in subsequent years.

Completed applications should be received at the address below by August 15, 2016 to be considered:

CRN Scholarships
C/O Terri Schleuder
40472 Franklin Mill St.
Novi, MI 48375



2015 CRN Scholarship winners, Kasey Hohl and Garrett Thomas

Rare Families Needed for Cox Prize Contest



2016 DAVID R. COX PRIZE FOR RARE COMPASSION



Connecting rare families with medical students

globalgenes.org/coxprize

We are thrilled to announce our 3rd annual Cox Prize for Rare Compassion contest, which encourages 1st and 2nd year medical students to get to know a rare family and write an essay based on their experiences. If the medical student does not have a rare family to work with, Global Genes will match them with a rare family in their local area. *We are asking your help in finding rare families we can match for this program as well as reach out to medical teaching institutions you work with.* This is a wonderful way to create awareness for your disorder within the medical education community and possibly create a relationship that could shape the med student's future career focus.

Please help us spread the word about our med student matching program to your community by asking them to go to https://globalgenes.org/coxprize_familyrequest to see what locations are currently asking for requests. This list gets updated weekly, so please have them check back often to see what new cities are

requested.

Global Genes Toolkit Resources

We are excited to announce that our RAREToolkits are available for order at our online store RAREhouse. These resources were developed collaboratively with patients and other rare disease stakeholders to help inform our community. You are now able to order these educational toolkits to add value and support your meetings, events and groups in bulk quantities for FREE (nominal shipping fee added). We have recently added some new toolkits online to kick off 2016, we can't wait for you to check them out!



(Click on caption link below picture for more information)



NAVIGATING EMOTIONS: A GUIDE FOR TEENAGERS WITH RARE DISEASES

- ["Navigating Emotions: A Guide for Teenagers with Rare Diseases"](#) (available for order)



HOW TO PROMOTE YOUR RARE DISEASE STORY THROUGH SOCIAL MEDIA

- ["How to Promote Your Rare Disease Story through Social Media"](#) (available for order)

- [Keys to Help Rare Disease Patients Ensure a Successful Surgery"](#)
- ["Rare Disease Registries: Advancing Disease Understanding, Treatments, and Cures"](#)

If we don't have a toolkit available for order, you can find our FREE downloadable PDF version of all our 25+ toolkits by [clicking here](#).

Carrie Ostrea Senior Manager, Patient Advocacy/Parent Advocate
 Global Genes - Allies in Rare Disease
<http://www.globalgenes.org>
 (949) 248-RARE x110



BUILDING A CARE NOTEBOOK

- ["Building a Care Notebook"](#)

Chandler Moore Meets the Globetrotters and Raises Cystinosis Awareness

By Clinton Moore

Chandler is a huge Globetrotters fan.....Who isn't....How can you not be? With all of their crowd interaction and difficult shots, it's hard not to be entertained by them. So when we heard they were coming to a town near us again, we knew we were buying tickets.



Chandler Moore with the Harlem Globetrotters and Cystinosis Awareness goes viral nationwide

The conversation that started all this was casual between my wife, Annie, and one of her coworkers, Ms. Sweetman. Christmas was nearing and Annie mentioned we were thinking of buying tickets to give to Chandler for Christmas. Ms. Sweetman commented that her father had connections with the civic center, where the game would be held, and asked us to wait a couple days to see if her father could get them cheaper. We agreed because who doesn't like saving a few bucks when you can? That was the end of it....for now.

Several days past and we hadn't heard anything back from Ms. Sweetman. When we asked, we were told that her father, Mr. Janus, was in fact getting them, but he wanted to donate them to Chandler as a gift. A very thoughtful and caring gesture coming from someone whom we've never met. We wondered why? People never just go around handing out stuff to strangers, so I couldn't help but wonder what was his reasoning. It turns out he had heard about Chandler and vaguely knew about cystinosis. Apparently Ms. Sweetman and her father had discussed Chandler before, and not only did he just want to give to Chandler....he had bigger plans in mind.

When the tickets arrived, they weren't just ordinary tickets. They were court side seats, actually sitting with the players!!! Only 8 of these tickets are sold for this particular game and we were now holding 4 of them!!! It really seemed to good too be true, but there we were, holding them. They included VIP lanyards and backstage passes to meet players. Surely this was going to be a night to remember.

Soon after receiving them I was contacted by a group known as Delmarva Media Group. They knew of the tickets and wanted to know if we were interested in allowing them to do an article about Chandler, the basketball game, and cystinosis. When I asked how they knew of the tickets, I was told that Mr. Janus was in fact the president of this media group and that he had taken particular interest in Chandler and cystinosis and wanted to help in raising awareness by doing an article in The Daily Times. Surely I'd be a fool not to agree to this. Globetrotters game and a chance to tell our story

and raise awareness. We agreed instantly!

Game night came and it was exactly what we thought it would be. We went backstage to meet players, were given Globetrotters jerseys, and then it was game time. The team welcomed us to their sideline and interacted with Chandler throughout the entire game. They did their usual crazy shots and tricks with the crowd and I think we all remember the water bucket stunt that they have been doing for years. Chandler smiled and laughed throughout the entire game.

In the days to follow the media group came to our home and did a video of our interview. They really just wanted to know what cystinosis was and how it affected Chandler. They asked about his medications, current research, and most anything that could give them a better understanding of this illness. I could tell after the interview that this was going to be a great story.

Roughly a week later it printed. Not exactly where I expected though...it was front page!!!! Front page through page 7 to be exact!!! The story was written in a way that I had never seen before. It was very descriptive and extremely informative. These reporters took more away from our interview than I ever thought they would. We were excited to think about just how many people this would reach. Awareness had been raised, but the best was yet to come.

The following morning, I received an email from the media group that said they had done a Google search on the article and found that in just the 24 hours since it printed it had been picked up by other media groups and newspapers literally from Florida to California. It had gone viral overnight!!! I searched the Web myself and found it to be true....countless websites and media groups had it in print as well. A week later 2 more of our local papers also printed the exact story!!! What had started off as a simple Globetrotters game...had now turned into literally tens of thousands of people being educated about cystinosis. All stemming from a casual conversation between my wife...and a coworker.

I think the whole point behind telling this story is this. Any chance you get tell your story, do it. You really never know just where it may lead. You may not know just exactly who you are talking to or who they may share it with. You telling your story could lead to awareness being raised by tens of thousands. It could find its way to someone who enjoys fundraising for various causes. It could fall on the ears of a doctor that has been spending the last few weeks trying to figure why a pediatric patient of his has this unquenchable thirst, which could lead to a diagnosis and start of treatment. Who knows where it will go? Many times it will go nowhere, but to whom you told it, but the next time you tell it, it could change the world.



Chandler Moore, a special guest of the Harlem Globetrotters



CRN Attends 12th Annual WorldSymposium on Lysosomal Storage Disorders

By Christy Greeley– VP Research, Executive Director

Christy Greeley represented CRN and the cystinosis community at the 12th Annual WorldSymposium February 29 – March 4, 2016 in San Diego. WORLDSymposium is designed for basic, translational and clinical researchers, patient advocacy groups, clinicians, and all others who are interested in learning more about the latest discoveries and the clinical investigation of lysosomal storage disorders.

The WORLDSymposium is a multidisciplinary forum presenting the latest information



Christy Greeley and Carrie Ostrea from Global Genes

from basic science, translational research, and clinical trials for lysosomal diseases. Originally conceived in 2004 in response to an NIH RFP for rare diseases, the Symposium is often cited as the most important scientific meeting on lysosomal molecular biology, disorders and treatment. The WORLDSymposium has become the major educational and unifying activity of lysosomal disease researchers, and has evolved into a highly interactive research activity. The underlying theme “transitioning molecular biology to human therapies” seeks to

elucidate the challenges—and highlight the successes—in bringing bench discoveries into successful clinical therapies. The main emphasis of the meeting remains the same: to assess the mechanisms, and obstacles, for taking bench research into human therapy.

The meeting kicked off on Rare Disease Day with a group photo of attendees holding the Handprints Across America logo to commemorate the day. CRN partners NORD and Global Genes exhibited with other advocacy organizations, while Raptor Pharmaceuticals exhibited to educate physicians in attendance on Cystinosis and Procsybi. Cystinosis was represented in the poster session as well, with the inclusion of Dr. Bruce Barshop's poster, "Leukocyte cysteine as a biomarker to monitor cystinosis" and CRN funded researcher Dr. Gizely Andrade's project, "Multisensory processing in lysosomal disorders: a behavioral and high-density electrophysiology investigation in Niemann-Pick disease type C and Cystinosis". Christy also took part in the Council of Patient Advocates (COPA) meeting, which brought together a variety of patient advocacy organizations to discuss the current state of lysosomal research and patient support.



Handprints Across America photo on Rare Disease Day

Jack Greeley 16th Birthday Letter Fundraiser – Total raised nearly \$300,000!

By Christy Greeley

The Greeley Family once again celebrated Jack's St. Patrick's Day birthday, 16 this year, by sending out the 14th edition of his birthday fundraising letter. As is his way, Jack has faced many challenges in the past year with his characteristic grace, courage and positive attitude. We cannot thank our friends and family enough for all of the support we have received, this year and since his initial diagnosis in 2001. Since 2003 we have raised nearly \$300,000 in honor of Jack to support CRN and the cystinosis community, thanks so much to everyone who has taken part over the years. Following is this year's letter:

March 1, 2016

Dear Family, Friends, and
Colleagues,

More, more, more. In 2016, we live in a world of "more." More wealth, more poverty, more attention, more posts, more violence, more rhetoric, more intolerance, more options, more showiness, more "look at me"... just more, more, and more. Is it good? Is it bad? Is it better? Is it worse? Is it progress? Is it regression? The answer, depending upon whom you talk with or what you talk about, is probably, well...probably.

"More" has been part of human history pretty much since forever.

The famous stories and lessons of "more" go back to Adam and Eve and exist today in politics (more power, insults, radicalism), world affairs (more people, consumption, conflict), pop culture (more options, shock, reality TV), and beyond.



Jack recovering from surgery in December 2015

For those who have heard the Greeley family story and that of our son Jack, know that his life has been filled with “more.” Jack, who will be celebrating his 16th birthday this March 17 (stay off the roads on St. Patty’s Day!), has Cystinosis, a rare metabolic, genetic disease where an amino acid called cysteine fails to leave his cells in an orderly manner. This has the potential to impact all of the organ systems in the body leading to kidney failure, muscle wasting, diabetes, blindness, pulmonary deficiency, hypothyroidism, and neurological damage. It is an incredibly tangled and complicated disease with largely symptomatic treatments, no cure, and lots of health by-products. Since Jack was diagnosed at a year old, “more” has been part of his existence:

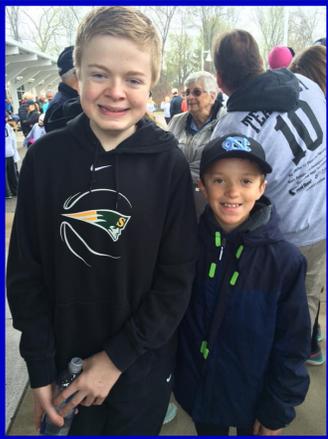
- More doctor visits – hundreds of appointments across pediatrics, orthopedics, nephrology, gastroenterology, ophthalmology, genetics, cardiology, neurology, pulmonology, and endocrinology
- More symptoms – profuse vomiting, heavy urination, muscle weakness, dehydration, imbalanced body chemistry, mobility issues, failure to grow, and more
- More hospitals – Rush Children’s Hospital, University of Chicago, UC-San Diego, Washington (DC) Children’s Hospital, Northwestern Lurie Children’s Hospital, Rehabilitation Institute of Chicago, National Institutes of Health, and Stanford Medical Center
- More treatments – CAT scan, X-ray, NG tube, G tube, leg braces, blood draws, slit lamp tests, DEXA scan, echocardiogram, swallow tests, and pulmonary function tests
- More medications – 140,000 pills swallowed to date, plus 1,000 shots...and counting
- More surgeries – four so far and a potentially significant orthopedic procedure on both feet later this year

The reality is that Jack has faced more trials and tribulations than most. Life forces him to give more with everything he does. Look at a daily routine. Sitting up in bed, walking to the bathroom, standing in the shower, getting dressed, putting on your shoes, etc. All require more effort from Jack and he will need to put forth more effort today than yesterday and more tomorrow than today. He has no choice.

As his sophomore year unfolds, Jack continues to amaze all that know him with his can-do spirit, positive mental attitude, and heart-warming smile. His days are busy and his effort starts from his heart, which has proved to be impenetrable from Cystinosis. At school, it takes more from Jack to simply walk to class, carry his backpack, or hold a pencil and write for an hour. Yet, he is unrelenting in all that he does. He has achieved High Honor Roll at one of America’s top high schools. With his extra-curricular activities from IM golf to table tennis team to archery club to student manager for both the sophomore and Varsity basketball teams, he keeps very active. Like



Student Manager Jack celebrating Stevenson HS Basketball regional championship



Jack Greeley and cousin, Joe Dillon at the 5K Fun Run in Honor of Kacy Wyman

academics, it requires more effort from Jack – traversing hills, bunkers, and greens on the links, carrying gear from the locker room, walking to the top of the bleachers to film a game, pulling back the bow and holding it steady, or moving side to side with paddle at the ready. No matter the challenge or obstacles at hand, being “one of the boys” is Jack’s reward with all his activities.

Once you know Jack, you understand that he needs to put more into life 24/7/365, but you quickly realize that he also gets more out of life and puts more back into it than just about anyone. What has amazed us over the years is how people notice this effort from Jack and we were reminded of this back in December when we contacted his teachers, his support team, and some family friends about an upcoming surgery he was to have to remove staples in his knees that were placed there from a past surgery. The responses were telling and we quote:

- Friend – Jack is such a brave guy! I’m so sorry he has to endure all of this and at such a young age.
- Friend – Jack is such a trooper.
- Counselor – He is very positive about it.
- Coach – He is an amazing asset to our Team and we hope this surgery will give him comfort and no more pain.
- Teacher – While my thoughts will be with Jack over break, I am not worried at all about his performance on the final as he has been doing exceptional this year so far.
- Coach – He is a pleasure to have around the program. His work and dedication is an inspiration considering his medical issues. I’ve never heard him complain once!
- Therapist – Your son is kind and stoic and not an easy nut to crack. Not once has he indicated any pain with his knees. I am even more humbled by his determination in light of this new information. Jack really works hard moving about the building.
- Friend – Please keep us in mind for any help. You know he has a special place in our hearts.

We have learned that people generally put energy into a room or they take it out of it... take your pick, but it is usually one or the other. With Jack, he puts life into the room, not with a dynamic, big personality or an imposing, charming physical presence. Jack simply has an unmatched attitude and a wonderful way he lives life. He finds joy, love, and happiness in everything he does. That is Jack’s secret – he gets more out of life, because he has to put more into life. That is somewhat of a paradox; although he gives more, often he is only capable of giving less in many facets of his life. Often more is not more, because quality can absolutely trump quantity. From that, we all learn and grow.

Noted English poet, William Blake wrote a famous proverb: “The road of excess leads to the palace of wisdom.” Over time and through interpretation, the meaning of that proverb has showed us that striving for extremes – pushing for the “more” that we all live and see every day – ultimately leads to a middle ground. Blake further explained, “Without Contraries is no progression. Attraction and Repulsion, Reason and Energy,

Love and Hate are necessary to Human existence. From these contraries spring what the religious call Good & Evil.”

In the crazed world of today, Jack leaves us all with a gift. He keeps life balanced and in perspective allowing those who know him to find their own middle ground. He gives more and gets more and personifies what good is. He sets an example and an approach from which we can all learn. From that, we all benefit by getting to know Jack more.

Please consider supporting the efforts of CRN, the foundation we run, by making a donation in honor of Jack and his 16th Birthday. Thank you, God Bless, and the best of St. Patrick’s Days to you and your family.

Follow Us On Social Media

Don’t miss out! Stay updated on the latest support programs, educational materials, research, and events from the CRN. It only takes a moment. Follow us today!



@CystinosisResearchNetwork



@CystinosisCRN

Town Hall Held in Tampa in February

By Clinton Moore

It was a great honor to be asked to represent CRN at the Tampa Town Hall back in February. It's always a pleasure to engage with other families, to share stories and experiences and even more of a delight to watch the cystinosis patients themselves interact with each other. The Town Hall meetings have a way of pulling true feelings from the parents and patients alike about the many aspects of cystinosis. They give everyone, especially the younger children, a sense of normalcy and a place that they can discuss their concerns or struggles. Raptor has done a fantastic job with these meetings.

Clinton Moore, right, recently hosted a Town Hall held in Tampa, Florida



CRN Exhibits at PAS Meeting in Baltimore

By Terri Schleuder

The city of Baltimore hosted the annual 2016 Pediatric Academic Societies Meeting (PAS) from April 30th to May 2nd. Carol Hughes and Terri Schleuder represented CRN at the exhibit hall. With the goal of educating and raising awareness of cystinosis to the many doctors in attendance, they handed out literature, shared personal stories and experiences to all who passed by booth 528.

This year they were very excited to be a part of celebrations honoring Dr. Rick Kaskel who received one of two prestigious ASPN Founders Awards.

It was wonderful to see many "old" faces as well as new ones and to share information, and gain insights. As always it was a very worthwhile and rewarding experience.



Terri Schleuder and Carol Hughes with Dr. Rick Kaskel, 2016 ASPN Founders Award recipient



Carol Hughes and Terri Schleuder with Dr. Joshua Zartisky from Wilmington, DE



It is always good to reconnect with our friend from Sigma Tau, Lesli King, with Terri Schleuder and Carol Hughes

Dr. Kaskel Honored with 2016 ASPN Founders' Award

[Frederick Kaskel](#), M.D., a renowned faculty member at The Children's Hospital at Montefiore (CHAM) and Albert Einstein College of Medicine, was honored during the CHAM reception at the Pediatric Academic Society annual meeting on Saturday, April 30 at the Hilton Baltimore Hotel. Dr. Kaskel was acknowledged by current and past associates for his many years of service and leadership at both institutions and for his contributions to advancing children's health and research. Dr. Kaskel was also honored by the American Society of Pediatric Nephrology (ASPN), with the 2016 ASPN Founders' Award on April 30. (More details below).



American Society of
Pediatric Nephrology

***Congratulations to the 2016 ASPN Founders' Award Recipients
Drs. Ellis Avner and Frederick Kaskel***



Frederick J. Kaskel, M.D., Ph.D. is an internationally recognized clinical investigator and educator in pediatric nephrology. He is a Professor and Vice Chairman of Pediatrics and Director of Pediatric Nephrology at the Albert Einstein College of Medicine of Yeshiva University. He is a Past President of the American Society of Pediatric Nephrology and the Congress President of the 15th Scientific Congress of the International Pediatric Nephrology Association. He received his undergraduate bachelor's degree in Biology from Monmouth College, Monmouth, Ill., and his doctorate in Physiology and Medical Degree from the University of Cincinnati College of Medicine and is a Distinguished Alumnus from both of these institutions. ...[Read full bio](#).

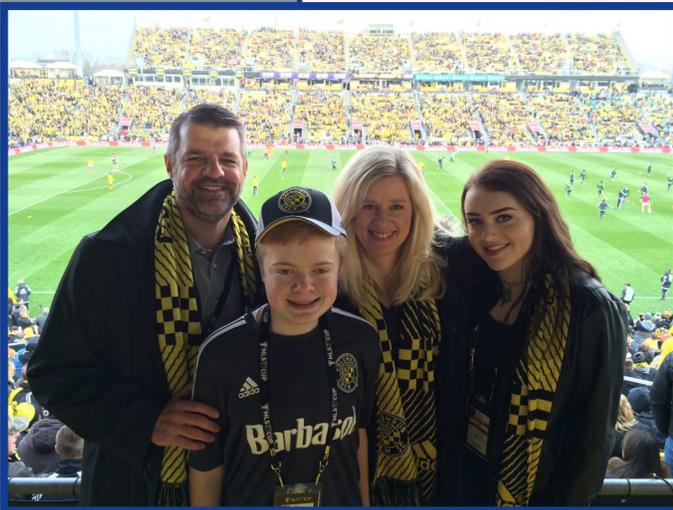


Ellis Avner, M.D. was appointed Founding Director of the Children's Research Institute and Founding Associate Dean for Pediatric Research in 2004. He retired in 2015 as Emeritus Professor of Pediatrics and Physiology at the Medical College of Wisconsin, and Attending Physician and Director, Multidisciplinary Program for Childhood Polycystic Kidney Disease in the Division of Nephrology at the Children's Hospital Health System of Wisconsin. Dr. Avner has held leadership positions in numerous professional organizations including the Society of Pediatric Research, the International Pediatric Nephrology Association, the American Society of Transplantation, and the Polycystic Kidney Disease Foundation. ...[Read full bio](#)

Research Update

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 has funded 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, Jack, Christy and Alex Greeley

CRN is currently funding three grants, with new projects in the works. These three grants represent one established Cystinosis researcher in Dr. Levtchenko'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.

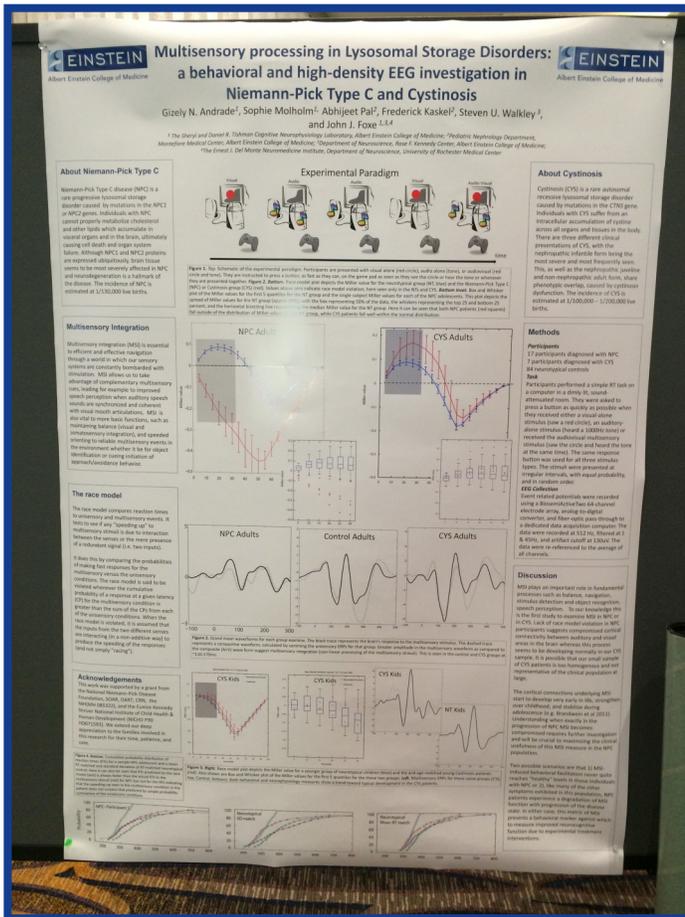
Grant Year One: \$98,010

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

PD Dr. med. Katharina Hohenfellner, LÄ Kinderneurologie, Kinderklinik Traunstein, Cu no -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



CRN Funded Research on display during the Poster Session at the WORLDSymposium 2016

“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 cysteine 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. These projects are ongoing, with updates to be posted on the CRN website in the coming months.

Brooke's Story, a Family's Journey with Cystinosis Begins

By Clay and Jill Emerson

Our story starts similar to many other families impacted by cystinosis. Brooke was born in September of 2014 and was a happy and, by all indications, healthy child. Brooke's birth weight and height put her around the 50th and 70th percentile, respectively; it wasn't until around an age of four to six months that her growth began to slow down, which we felt was cause for serious concern. Brooke had minor gastrointestinal issues, but nothing out of the ordinary for infants and nothing that was enough to explain her slow growth. None of the health care professionals we consulted shared our concern and for over a year we brought her to numerous specialists at the leading children's hospital in our area. After a great deal of misinformation and fumbling of blood tests and results,



Newly diagnosed Brooke Emerson

we decided to see one last specialist at the hospital. In December of 2015, from a simple visual examination, the doctor immediately recognized that Brooke had rickets. Blood tests and finally a urinalysis were done and, with the findings of a genetic test, in early February we received the phone call for which we had been waiting for over a year.

Like many in the community, we had never heard of cystinosis and we quickly found online resources to help cope with the diagnosis; including the CRN website. The CRN put us in touch with Clinton Moore, who quickly became our trusted friend and confidante. Clinton has provided answers to our many questions and the unique support that only another parent of a child with cystinosis could possibly provide. We are lucky to live in close proximity to the Moore family, and we had the opportunity to meet them in March. Brooke greatly enjoyed Chandler's company and we think the feeling was mutual! The Moore family has also connected us with a nephrologist experienced in treating cystinosis patients; this has been invaluable as Brooke's new doctor has been an excellent ally and advocate

in these few short months, something that we quickly learned is absolutely critical for this disease. We have also had the opportunity to talk with other families within the community, which has helped calm our fears and alleviate the alienation felt when your child is diagnosed with a rare disease. We are so grateful to have such a strong and supportive community behind us in this fight!

As we write this, we are only three months into our journey with cystinosis. While we are still awaiting Brooke's first cystine test result since starting Cystagon, we are fortunate that all her other levels have normalized with routine dosages of the usual supplements. We know that we have been relatively fortunate that Brooke has maintained her strength and energy thus far and has been a real champ in taking her medicine. She has yet to develop the extreme thirst that seems to be one of the early and characteristic symptoms of cystinosis, and fortunately has not yet been hospitalized. She enjoys playing soccer and insists on going outside by shouting "Out, Out!" and pointing at the door while often going so far as to bring mommy or daddy their shoes in hopes of an escort out the door. She continues to amaze us each day with her energy, happy and loving nature, resiliency and positivity.



Brooke, Jill and Clay Emerson



Brooke as an infant

Interestingly, since Brooke's diagnosis we have come to discover a family history of a more benign or "ocular" version of the disease, originally involving Brooke's great grandmother's siblings. They were trailblazers and were involved in early studies at the NIH in the sixties which helped contribute to the early understanding of the disease.

We look forward to meeting other families at CRN conferences in the future, and "paying it forward" and offering support to newly diagnosed families. We plan on fundraising to help contribute to the knowledge base of the disease with the goal of improved treatment and, one day, a cure.

Update from National Organization for Rare Disorders (NORD)

By **Marybeth Krummenacker**



NORD Board member
Marybeth
Krummenacker

As I sit and write this article I can't help but think how lucky the Cystinosis Community is. Not lucky in the sense that our loved ones have a rare genetic disease but lucky in the sense that our community of less than 500 in the United States (2,000 worldwide) has such a strong voice in various organizations. I am overwhelmed at times that I am privileged to be on the Board of Directors of NORD. I sit at that table and I am in awe of the people that I have had the honor to get to know and meet both in Washington D.C. and around the world.

Abbey Meyers, the Founder of NORD just wrote a book and I have combed through it and have learned so much of the beginnings of the Rare Disease Advocacy world. (www.abbeymeyers.com ; *Orphan Drugs: A Global Crusade*) I knew and understood some of the story, but this certainly helped to clarify so much for me personally and the understanding of the role so many people have played. Meeting Abbey was an honor as well and I have had the opportunity many times. She always struck me as a passionate woman about rare diseases (her children were diagnosed with Tourette's Syndrome) and she always represented herself as "just a grandmother from Connecticut!" The story of how the Orphan Drug Act of 1983 became law is extraordinary and it was really by sheer circumstance and opportunity that Abbey was able to recognize the issue and got people to listen! It is so critically important for all of us in our VERY SMALL rare disease community to understand that without people like Abbey Meyers, Jerry Schneider, Jess Thoene, Bill Gahl and a host of other individuals being in the right place at the right time.....we would have nothing. Without this extraordinary piece of legislation we never would have gotten Cystagon approved by the FDA. And as a reminder to all, Cystagon was #41 of the first 100 drugs approved as a direct result of the Orphan Drug Act of 1983. I want to share with you a short excerpt from Abbey's book to demonstrate how fortunate our community is:

*"Although the pharmaceutical industry continued to ignore orphan drugs, generic companies did not. They continued to help, and their help was invaluable to many patients and families. **The SECOND drug we asked the generic industry to adopt was cysteamine for treatment of Cystinosis**, a hereditary multisystem disorder that forms crystals in various organs of the body, especially the kidneys and eyes. Children with Cystinosis usually experience kidney failure and blindness before they die in their teenage years (*remember this was in the early 1980's).*

Dr. Jess Thoene was a researcher at the University of Michigan whose specialty was Cystinosis. Dr. Thoene and his colleagues, Dr. Jerry Schneider at the University of California, San Diego and Dr. William Gahl at the National Institute of Health (NIH), had formulated a drug called cysteamine and could dissolve the crystals. However, no

company would make the drug because it had a bad smell and there were only a few hundred children in the U.S. with Cystinosis.

Dr. Thoene knew a retired chemist and asked him if he could make the drug. The chemist converted a garage where the smell would not bother anyone and for several years cysteamine was made in the chemist's garage for the children who could not live without it.

During the late 1980's Bill Hadded (a generic drug manufacturer) put Abbey in touch with Mike Puskar who owned a generic drug company called Mylan Pharmaceuticals in West Virginia. Dr. Thoene went to visit Mylan and they arranged for the company to manufacture cysteamine. Since it was the only medicine for Cystinosis, families from all over the world were blessed with access to their treatment through Mylan. The German chemist who had manufactured the drug in his garage for several years was finally able to shut his garage shop down as Mylan agreed to produce the drug.

Eventually, Dr. Thoene and his colleagues were able to conduct the clinical studies that the FDA required for a new drug approval and in 1994 cysteamine was approved by the FDA for the American market. It is still used today under the brand name Cystagon. Dr. Thoene became so devoted to NORD's mission that he served voluntarily as Chairman of NORD's Board of Directors for more than ten years." I wanted to share this part of Abbey's book to show you just how blessed our community is even today. I am very proud and honored to be a member of the Board of Directors of NORD and as I sit at that table I have each and every family in my heart as I advocate on behalf of the Cystinosis community and rare disease as a whole.

I will leave you with one of my favorite quotes that Abbey uses in Chapter 2....."Start by doing what is necessary, then do what is possible, and suddenly you are doing the impossible." St. Francis of Assisi



**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!

3rd Annual "Chandler's Chance" Fundraiser for CRN Honoring Chandler Moore

By Clinton Moore

With any fundraiser or big event, the "unknowns" can often be the scariest part. You work tirelessly for weeks and even months to make sure every detail is covered but there always ends up being a handful of things that seem to get overlooked and some things simply cannot be planned for. Certain problems can even turn out to be a good thing. These event are always full of surprises and unknowns, and that seems to be one of the most exciting parts.

This year's Chandler's Chance event proved to be no different. Going into it for our 3rd year, we have learned many things and with the growing experience, it seems to get a little easier, however expanding this event year after year keeps it more interesting for attendees but also adds to the growing number of volunteers needed, adds to the amount of preparation time, and definitely adds to the stress level.



Chandler Moore at the 3rd annual Chandler's Chance fundraiser for CRN

This year was by far our greatest year to date. We were joined by several great organizations which greatly increased our amount of advertising and made the event

much more enjoyable. Raptor Pharmaceuticals was the first group to join us this year, not only by sponsoring the event, but also by attending and volunteering their time to make it a success. Raptor's very own, Kristina Broadbelt, is an amazing asset to this community and is just wonderful to be around. She arrived early and jumped right in to help organize and prepare for the evening. The Delaware State Police announced their arrival just about an

hour prior the event, showing up with their SWAT team and patrol cars to help direct traffic. I was also told that there were a handful of plain clothed officers in attendance. Our nephrology team from A.I.Dupont Hospital for children arrived and showed their



Attendees enjoying the day's events at Chandler's Chance

support for Chandler and the entire cystinosis community. The Lower Chapter of the National Wild Turkey Federation joined us and brought their bb gun shooting range for the kids, however I saw many adults shooting as well. The NWTF was also selling tickets for a gun raffle that they were holding in Chandler's honor. Dustin's Smoke Shack also joined us selling food and donated 100% of their proceeds to the cause. They even created a special sandwich just for the evening which was named "The Chanman".

Our goal has shifted a bit from our 1st event and as it is still a fundraiser, we also refer to it as an awareness raiser. Getting new faces here every year is key to continue raising new awareness. My personal attendee goal was to get 300 people to attend. This has been my goal since the first year and although we have come very close to it...we've never met it...until this year.

The weather was forecast to be absolutely perfect. Chilly but not freezing and most importantly..No Rain!!! Children and adults alike were entertained with thousands of Christmas lights, firetruck rides, hay rides, bb gun shooting, music, Mr. and Mrs. Santa Claus, various characters, hot chocolate, cookies, and candy. This entire event, with exception of food, is free of charge and we only ask families to donate what they can.

At the end of the evening we had confirmed that we had absolutely crushed our goal of 300 attendees. This is proof that a community can pull together and accomplish most anything. We had a final tally of 476 people attend!!! Many familiar faces as well as many new ones. I had several people come to me after announcing the total and they informed me that they recommended planning on 600 for next year as they felt the event was great and they planned on returning with more of their family next time.

Next year's event has already begun the planning stages. Recent conversations with our nephrology team has confirmed that the next event (December 3rd) will begin in the early afternoon with educational seminars for all attending cystinosis families with speakers very well known to the Cystinosis community!!!! (names to be announced) And the fundraising/awareness raising activities to begin at 5:30. This promises to be an educational as well as an entertaining day for all attending cystinosis families and the public alike. Travel and lodging assistance (for cystinosis families) may also be available. All details will be announced in probably midsummer. Save the date!!!!



Santa and Mrs. Claus join in the fun



Many community groups and members came out to support Chandler Moore and his family at their 3rd Annual fundraising event.

Education & Awareness Update

by Terri Schleuder—Vice President, Education & Awareness



Terri Schleuder, VP of Education and Awareness.

The Education and Awareness committee has been quite active the first few months of 2016. We celebrated Rare Disease Day on February 29th by sending eight cystinosis community members to Washington, D.C. to participate in Rare Disease Week on Capitol Hill, an event organized by RDLA (Rare Disease Legislative Advocates). Participants were able to tell their stories and bring the needs of those with cystinosis and other rare diseases to legislators who have the power to make a difference. It was a positive and empowering experience for all who attended. Thank you to Aimee Aldemann, Alex Schluze, Clinton, Annie and Chandler Moore, Jennifer Loglisci, Jeff Larimore and José Morales for taking time out of their busy schedules to be a part of this great event. Many of these folks were also featured in their local newspapers and television stations again raising cystinosis awareness.

In April, CRN represented by board members Carol Hughes and Terri Schleuder, exhibited at the 2016 Pediatric Academic Society Meeting (PAS) held in Baltimore, Maryland from April 30th to May 2nd. This is always an exciting event that allows CRN to provide the latest information about cystinosis to the many pediatricians in attendance who stop by our booth. This year we had the distinct pleasure to be a part of celebrations honoring Dr. Rick Kaskel, one of two recipients of the 2016 ASPN Founders Award.



Terri and Steven Schleuder "Count Down to Rare Disease Day 2016"

There have been two Town Halls since our last Newsletter, one in November in Madison, Wisconsin and one in January in Tampa, Florida. These events bring small groups of families and cystinosis patients together to share, learn and provide support to each other. We have several each year and they have been very well received by those who have attended.

CRN is again pleased to be able to accept applications for our two academic scholarships from now through August 15, 2016. Please refer to the article on page 13 for more details.

Finally, we have been busy collecting articles and compiling information for this latest this edition of the Cystinosis Advocate Newsletter. We hope the content inside provides beneficial information and updates of interest to our cystinosis community.

Planning for the next CRN Family conference in July 2017 in Salt Lake City, Utah at Snowbird Resort will begin soon. Please plan to join us. Details will follow shortly on CRN's website and in our next newsletter.



"Dream, Achieve, Inspire"

Cystinosis Research Network

CRN was honored to present the debut of a traveling ART exhibit featuring Artists of all ages, from all over the world, who live with Cystinosis everyday at the 2015 CRN Family Conference, held in Chicago, Illinois, July 16th-18th. Since then the exhibit has traveled to São Paulo, Brazil and will soon go to Mexico to be featured as part of the 8th Cystinosis Medical Symposium held in Mexico City on August 5th 2016. Learn more about the Art Exhibit and the Artists on the video link below.

https://www.youtube.com/watch?v=2GBBE1u2jYU&list=PL5t4KcgQO_ZMwqrJxXI-liPf5ehhBhyvr

Development Update

By José Morales—Vice President, Development

I am on an airplane bound for Dallas, Texas to visit my family. A primary objective of my visit is to spend time and support my sister in her efforts to fight cancer. These last few months have not been easy for the Morales family. We have had to contend with a number of issues and challenges. Through it all, we rely on our faith, our family and friends. During the darkest hours, during those times when one's fundamental values and beliefs are challenged we have relied on our faith. Faith as defined in whatever strengthens you, whatever gives you the hope to take another step, whatever drives you to believe that tomorrow will bring a better day and is worth continuing to fight.

Over the years, I have had the honor and privilege to witness first hand how hope plays itself out in the various forms from the perspective of the various of roles I have assumed on behalf of The Cystinosis Research Network. I am always taken by the fact that those with less to commit, commit the most; those with the most to lose, are willing to lose the most; those with the least to be hopeful for are the most hopeful. It is humbling and it continues to inspire me to remain committed to The Cystinosis Research Network because of the wonderful things it contributes to.....what wonderful things we are accomplishing as individuals and, collectively, as a group:

The National Institutes of Health (NIH) defines a rare disorder as any disorder having less than 200,000 diagnosed occurrences in the United States.

There are approximately 500 cases of cystinosis diagnosed in the United States

There are approximately 7,500 recognized rare disorders by the NIH. Less than 1% of these disorders have a Federal Drug Administration (FDA) approved drug

There are currently 3 drugs with FDA approval for cystinosis:

Cystaran (cysteamine ophthalmic solution) – eye drop treatment for corneal cystine crystal accumulation. Administered one drop per eye, per waking hour

Cystagon (cysteamine bitartrate) – capsules for oral administration, cysteine depleting agent which lowers the cysteine content of cells. Administered every six hours, 24 hours a day, seven days a week

Procysbi (cysteamine bitartrate) – long-acting formulation delayed-release capsule for patients ages 6 years and older. Administered every twelve hours, 24 hours a day, seven days a week

Having three FDA approved medication provides context to how fortunate we are as a

community and how successful we have been in pursuing our objectives of identifying treatments that enhance our children's quality of life and, ultimately, making progress towards identifying a cure. Research solely focused on cystinosis and the broad spectrum of existing and emerging issues continues to be promoted and supported. This is being accomplished by a broad spectrum of organizations and groups and The Cystinosis Resesearch Network is making valuable contributinons.

Over the last ten years The Cystinosis Research Network has sponsored 26 discreet research studies and committed over \$3.0 million dollars supporting researchers.

Research is just one part of the The Cystinosis Research Network'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

One of the most critical elements to achieve the before mentioned, is the raising of monies to fund a broad spectrum of programs. To achieve this, The Cystinosis Research Network has a Development Committee committed to assisting families in sponsoring fundraising events. The following are the individuals from our community that have taken on the responsibility of assisting in raising the funds on behalf of the organization:

Development Committee

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

Corporate Sponsorship

Jeff Larimore - JLarimore@arnoldfamilycorp.com

John Maccarone - johnm2maccaroneplumbing.com

Deb Reed - dk_reed@hotmail.com

Fundraising

Rachel DaLomba - rdalomb@my.wgu.edu

Katie Larimore - klarimore112@sc.rr.com

Katie Morrison - katielmorrison91@gmail.com

Briana Smythe - bri_ana15@hotmail.com

Jen Wyman - jwyman@comcst.net

Administration

Christy Greeley - Greeleycd@aol.com

Jen Wyman - jwyman@comcst.net



José Morales and family

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

Jack Greeley Birthday Letter – held in honor of Jack Greeley and sponsored by Dave and Christy Greeley

Steve Schleuder Birthday Letter – held in honor of Steven Schleuder and sponsored by Carl and Terri Schleuder

May!hem 2016 – held in honor of Sierra Ayers and sponsored by Julie Ayers

Cystinosis 5K Fun Run – held in honor of Kacy Wyman and sponsored by Tim and Jen Wyman



Velyna and Christian Morlaes enjoying the game

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.

“No one has ever become poor by giving.”
– **Anne Frank, diary of Anne Frank: the play**

“It's not how much we give but how much love we put into giving.”
– **Mother Teresa**

“Every man must decide whether he will walk in the light of creative altruism or in the darkness of destructive selfishness.”
– **Martin Luther King Jr.**

“The simplest acts of kindness are by far more powerful than a thousand heads bowing in prayer.”
– **Mahatma Gandhi**

Schleuder Letter Campaign raises \$6,000 for CRN

By Terri Schleuder

The Schleuder family would once again like to express our gratitude to all those who helped make our latest fundraising effort for the Cystinosis Research Network so successful. Over \$6,000 was raised to support CRN's Vision and Mission through our 5th Birthday Letter Event honoring our son, Steve. This effort over the last five years has raised over \$35,000.

We have been a part of this journey for 28 years now and have seen firsthand what research can accomplish. In 1989 there were no FDA approved treatments for cystinosis, only experimental drugs and research studies. It was very much a disease of despair with a grim prognosis and short lifespan for those children diagnosed with it.

But this community was blessed with some of the finest minds and most dedicated medical researchers and doctors around who made studying this very rare disease a lifelong career goal and passion. Because of their legacy and the efforts of many who followed there are now three FDA approved treatments for cystinosis that have made this disease of despair one of hope. God willing, someday research will lead to a cure.

Thank you all for helping to make a difference in the life of our son and all others who live with cystinosis every day.



(left) Aidan and Lily Schleuder, the nephew and niece of Steve Schleuder. (Above)

Update from Mexico: Great Success on First Cystinosis Family Workshop

By Victor Gomez

The first Family Cystinosis Workshop was held in Mexico on March 11-12, 2016. It was a great success. The workshop was created to promote a life plan and motivation in patients and families who live with Cystinosis. The Monterrubio family hosted the event and coordinated the agenda. Miriam Monterrubio, a teacher, and José Eduardo Monterrubio, psychologist were the speakers.

The course lasted two days. Ten families came together to participate in various activities such as teamwork. They were asked to describe the activities they enjoy doing as a family, and to make the decision to draw a dream and make it happen.



The Monterrubio family and Victor Gomez join attendees as they gather at the first Family Cystinosis Workshop to learn and support each other.



8th Cystinosis Medical Symposium in Mexico City & "Dream, Achieve, Inspire" Cystinosis Research Network Art Exhibition.

By Victor Gomez

The 8th Cystinosis Medical Symposium in Mexico City and the "Dream, Achieve, Inspire" Cystinosis Research Network Art Exhibition will be held next August 5th 2016, in Marriott Reforma, Mexico City.

This year's Symposium will include the International "Dream, Achieve, Inspire" Cystinosis Research Network Art Exhibition. We are very excited to have an incredible art creation from patients around the world.

We are happy to welcome for the first time Larry Greenbaum, MD from Atlanta, Georgia, as speaker on our Symposium.

The Mexican Organization of Cystinosis appreciates the support of the Cystinosis Research Network.



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Viernes 5 de Agosto de 2016, 8:00 a.m.

Accredo Relaunches Center Focusing on Rare Disease

By **Andy Liu, PharmD, Accredo Product Manager**

On April 19th, Accredo Specialty Pharmacy celebrated the re-launch of our Rare Disease Therapeutic Resource Center® (TRC). Accredo TRCs are “pharmacies within a pharmacy,” dedicated to specific disease states. Each TRC team is composed of pharmacists, nurses, certified pharmacy technicians, and support staff trained to work with the drugs and diseases they manage. This approach brings a deeper level of clinical specialization to each of these conditions we care for, and the Rare Disease TRC is no exception. With a brand new pharmacy space in Memphis, TN, Accredo’s Rare Disease team delivers focused attention that’s essential for each rare disease patient and caretaker. The strategic design of the new space allows these small patient care teams to sit next to each other, enhancing communication and collaboration.

As part of the celebration week, Accredo invited the community to attend. The Cystinosis Research Network (CRN), National Organization for Rare Disease (NORD), and two rare disease patients participated in the event to educate and collaborate with the staff on how they can work together to improve patient care. **Katie Morrison** of the Cystinosis Research Network delivered educational material and shared stories about the struggles of cystinosis patients.

FIVE things your Accredo Rare Disease care team wants you to know:

- If you have questions about taking your medication or side effects, we’re here for you! A pharmacist is available 24/7.
- Call us any time your prescription changes. This includes short-term changes made to your dose or titration schedule.
- It’s best to use the same one or two pharmacies to fill your prescription. By developing a relationship with the same care providers, you can help them better know what is or isn’t “normal” for your profile. This helps ensure your safety, and can make managing your medicine easier.
- On the go and missed our call? Enroll in our text program to keep track of when your next refill is due.
- Tell your pharmacist EVERYTHING you take, including herbals, over-the-counter drugs, dietary supplements, and medicine received from other pharmacies. This way, your medicine review will be complete and accurate.

accredo®

Fewer than 2,000 patients worldwide have cystinosis. It can be daunting for patients and caretakers to find clinicians who not only understand the condition, but also the medicine used in treatment. Because the Accredo Rare Disease pharmacy team focuses solely on ultra-rare conditions like cystinosis, they often see more patients in a week than most physicians will in a year. Having the ability to talk with patients with this rare disease on a daily basis gives invaluable experience and insight to the staff. The Rare Disease team at Accredo has heard, first hand, the unique difficulties each patient struggles through. Their cystinosis-specific training makes it easier for them to offer counsel and resources to each patient, whether they're newly diagnosed or already taking therapy. Directing patients to support groups and foundations such as the CRN is also a vital part of the team helping patients succeed in managing their conditions.



Pictured: Katie Morrison (left) with CRN, and Justin Gifford (right) with Accredo Specialty Pharmacy



Rare Disease TRC Relaunch Celebration

For cystinosis patients, life doesn't stop with a diagnosis. The Accredo Rare Disease care teams at the Rare Disease TRC put their heart and soul into making sure their patients get their medicine safely and reliably. The team wants to remove the stress from the process, and to allow patients and caretakers to focus on the one thing that matters most: fighting their disease.

The excitement surrounding the Accredo Specialty Pharmacy re-launch of the Rare Disease TRC is palpable, and the participation of partners like the CRN enhanced the experience for all of the staff. With partnerships between specialty pharmacies like

Accredo and foundations like the CRN, cystinosis patients are certain to get the medicine, care, expertise and attention they need.

National Association for Rare Diseases (NARD): A New Body for Rare Diseases in Egypt

By Professor Neveen Soliman



Prof. N. Soliman announcing the establishment of NARD on Rare Disease Day 2016 in Cairo "Inspiring Hearts & Minds"

The Rare Disease Day 2016 celebration in Cairo, Egypt "Inspiring Hearts & Minds" marked the establishment of the National Association of Rare Diseases (NARD). The association, first of its kind in Egypt, provides an umbrella to support and promote advocacy to all rare diseases.

The event brought together health policy makers, doctors, patients, comedian celebrity Mr. Sameh Hussein, media, NGOs and support groups to highlight the current situation and call for more collaboration between all parties to alleviate some of the suffering the patients and their families have to endure.

A roundtable discussion moderated by Prof. Neveen Soliman (Founder of NARD and Director of Egyptian Group for Orphan Renal Diseases - EGORD), and joined by key health policy makers: the Head of Egyptian Medical Syndicate (Prof. Hussein Khairy), Vice Dean of Kasr Al Ainy School of Medicine (Prof. Khaled Makeen), Deputy Director of Cairo University Hospitals (Prof. Ahmed Sobhy), Chairman of Pediatric Department (Prof. Ahmed Elbleiedy) and the Director / Deputy Director of Monira Children Hospital (Prof. Hafez Bazaraa / Prof. Ahmed Badr) as well as patients / families and media representatives. Later, a number of awareness talks on 7 diseases, cystinosis included, were presented in Arabic to audience by medical experts.

As a result a campaign was started to raise further awareness and encourage decision makers to join the rare disease community for better understanding of the challenges patients and their families face. Concrete actions to provide diagnostic tools and treatment options for rare disease patients are now being developed to be implemented soon. Hopefully this would help patients living with rare diseases to lead as normal lives as possible and integrate



NARD members and rare diseases advocates holding NARD flags in support.

themselves into productive segments of society.

Finally a family of two brothers living with cystinosis talked of their long odyssey to understand what was going on with their eldest son who was not growing well until finally diagnosed by EGORD as having nephropathic cystinosis at the age of two years. Now that they know, when their younger son was born he took the test confirming the disease. They explained how life was much easier this time as their younger son was diagnosed correctly and treated with cysteamine early enough, even before developing any symptoms. They



Discussion panel: from left Prof. A Sobhy, Prof. A Elbeleidy, Prof. K Makeen, Prof. H Khairy, Prof. N Soliman

explained how living with cystinosis was completely different in their second son whose health care is almost optimal in terms of diagnosis and treatment. The story stunned the audience and no further words needed to be said since the enhanced quality of life spoke louder than words!



Children and adults celebrating NARD and Rare Disease Day



Cystinosis advocates: from left Dr. S. Abdelrahman, Dr. H. Abdelaziz, Dr. M. Nabhan and Dr. R. Abdelhamid



Thanks are due to Prof. Hussein Khairy, the Head of Egyptian Medical Syndicate, for his enormous support to NARD and for launching a nationwide support program in collaboration.

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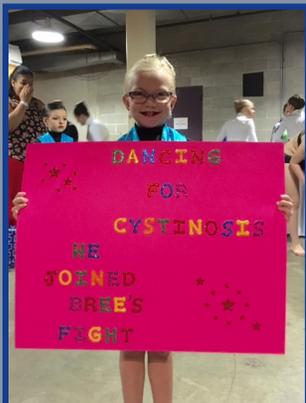
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Bree Forrester, at her "Dancing for Cystinosis" Fundraiser. Her event will be featured in our 2016 Fall/Winter newsletter



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Plan to Join Us for the
**2017 CRN Family
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Snowbird Ski and Summer
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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 website www.cystinosis.org, the popular online Cystinosis Facebook Support Groups, 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

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 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

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In Honor Of _____

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You may send notification of my gift to:

Please check all that apply:

- Friend
- Parent of Child with Cystinosis
- Family
- Individual with Cystinosis
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- I am interested in volunteering for CRN. Please contact me.

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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!





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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.
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E-mail Address	CRN@cystinosis.org
Web Page	www.cystinosis.org

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.

The Cystinosis Research Network prepares all necessary documentation and submits it to the respective local United Way organization.

The local United Way organization processes the documentation and sends a check for the aggregate sum designated for the Cystinosis Research Network.

The Cystinosis Research Network sends thank you/acknowledgement letters to recognize contributing individuals.

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.

302 Whytegate Ct.
Lake Forest, IL 60045
www.cystinosis.org

Phone: 847-735-0471
Toll Free: 866-276-3669
Fax: 847-235-2773
Email: info@cystinosis.org

Editor: Terri Schleuder

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, muscles, pancreas, brain and white blood cells. Without specific treatment, children with cystinosis develop end stage kidney failure at approximately age nine. The availability of cysteamine medical therapy has dramatically improved the natural history of cystinosis so that well treated cystinosis patients can live into adult hood.

CRN 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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