CRN Family Conference: “Winds of Change”, July 16-18, 2015 in Chicago, IL

The Cystinosis Research Network (CRN) is pleased to announce that the 2015 Family Conference will be held July 16-18, 2015, at the DoubleTree by Hilton Magnificent Mile in Chicago, IL. This will be CRN’s seventh family conference. 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.

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.

CRN has budgeted for a number of Conference Scholarships to help families/individuals

In Remembering Livia; We LivGracefully

By Kirsten Stilke

On September 26, 2014, our worst nightmare became a reality. We lost our 8 year old daughter, Livia Grace Stilke to a very rare disease known as Fibromuscular Dysplasia. Our world was crushed, ripped out from underneath us, and life as we knew it has ceased.

Livia had Cystinosis, just like her 12 year old brother Mason. The Fibromuscular Dysplasia had gone undiagnosed for her entire life, unknown to us. While we were conquering cystinosis; this extremely rare disease, was harboring itself inside her body, waiting ever so patiently to take our little girl up to Heaven. The bittersweet reality is that we fought hard against cystinosis, but then this
Message from the President
Jeff Larimore

CRN Family Conference – Can you feel the Winds?
by Jeff Larimore

The swell of conversation regarding the Cystinosis Research Network Family Conference to be held in Chicago from July 16-18, 2015 actually began shortly after the successful 2013 Family Conference held in Washington DC. Discussion circulated on what is the best format to keep the energy flowing. The exchange of ideas on how to keep all in attendance, young and not so young, professional or teen with Cystinosis, engaged and electrified, however to not lose a core focus that the Family Conference could be a new family’s first introduction to those with Cystinosis. This was the case for our family in July 2009.

CRN strives for the Family Conference to be a gathering open to debate where the possibilities of beneficial change can be borne for the betterment of the Cystinosis community. We are rewarded that research and medical professionals of many disciplines throughout the world put the CRN Family Conference on their schedule to participate. We also receive tremendous support and engagement from professional organizations such as Raptor Pharmaceuticals and Sigma Tau Pharmaceuticals who provide updates on approved treatments but also seek new information from families and physicians to enhance their research capabilities and provide the Cystinosis community with potentially better treatment alternatives.

But the true essence of the CRN Family Conference is to recognize the individuals who have to manage with Cystinosis daily. The opening reception will be exhilarating with dialogue as CRN proudly presents “Dream, Achieve, Inspire”. This exhibition will present the artistic talents of cystinotic individuals from six continents across the globe and will be a travelling, worldwide exhibit.

To the families of the newly diagnosed and the youngest generation, the Conference will provide a safe and secure daycare facility where the children will be given direct attention and endlessly entertained. The parents and caregivers will be provided the opportunity to freely converse with the highly committed medical experts in managing Cystinosis and capture the multitude of experiences of families in attendance.

For the teens, CRN has teamed with Global Genes and SmithSolve to present the 2nd Cystinosis Teen Adventure. This dynamic program will provide peer activities throughout Chicago and will include professional dialogue concentrating on Teen Transition issues.

For the adults with Cystinosis, the Family Conference becomes the setting for you to be the new mentors; to share your experiences of endurance but also to be forthright on how managing with Cystinosis can be improved. We will be listening...
The Conference will include the presentation of the Above and Beyond Recognition Award and will once again wind down with the entertaining Dinner Dance. This year’s musical guest Tori Lynn Jones will get everyone on their feet to rock the house.

My sincerest thanks to the Family Conference Committee chaired by Christy Greeley for developing a comprehensive program and agenda that will provide a feeling that the “Winds of Change” are with all of us.

CRN Advocates for Cystinosis during Rare Disease Week on Capitol Hill

In honor of Rare Disease Week in February, CRN representatives Christy Greeley, José Morales and Rebekkah Palmer attended an event sponsored by the Rare Disease Legislative Advocates organization which brought together members of the rare disease community from across the country to share their unique stories, ideas, and make our collective voice heard on Capitol Hill. We met with several Senators and Representatives from Illinois, Arizona and Wisconsin to explain the needs of the rare disease community using cystinosis as an example. The large presence of dedicated advocates helped to make this event a great success. Their enthusiasm and positive spirit helped make Rare Disease Week 2015 both productive and fun. - See more at: http://rareadvocates.org/rdw/#sthash.UUUKNe4D.dpuf
disorder, also known as FMD, crawled its way into her body at birth, and took her from us eight years later, with no indication. We have since learned we were blessed to have had 8 years with her, and not less than that. Still not fully capable of understanding the hits our family has taken, and not able to conceptualize all the why’s in our circumstances; we have come to see that Livia was beyond special. There was no warning, nothing we could do to stop it, and now here we sit with only memories. Memories we would like to share with you, as these are the things allowing us to slowly take each breath and awaken to a new day. This is the story of our family and our journey. As we remember our sweet Livia and all that she was to our family, we hope our story brings inspiration, and a message of how one little girl embraced her life to the absolute fullest.

I look back, less than a year ago, and I have to say, our lives were awesome, perfect perhaps. In my own little skewed world of perfect, I thought it was just splendid. Yes, both kids had cystinosis, yes we have the health issues, but you know what, despite all those bumps in the road, we had each other. Mason and Livia were happy, “healthy”, and enjoying life to its fullest. We have an amazing family, friends, two beautiful children, a dog, a home and good jobs. Yes, we had hit it folks. We had hit our own little world of perfection. Seven months ago, I was worried about our crazy morning routines, our after school events, our hectic weekends, but I relished in it. It was the family; the dream Dave and I both wished for. Two amazingly beautiful, smart, kind children who put smiles on our faces every time they walked into the room. Yes, this was my perfect.

Our lives weren’t always easy, far from it actually. So, when I reflect, I look at how far we had come. How many obstacles we had courageously fought, and I am proud of us. We were doing it. Every bump in the road, we picked ourselves up, dusted ourselves off, and somewhere down deep, mustered the strength, and bravery to continue forward. We all have those moments, those incredibly difficult moments in time that rock our world, and make us question everything we were taught to believe. I know that especially all of us who have kids who were diagnosed with cystinosis, that little dream of a perfect world for your kids’ changes, and at first, we do not believe that a perfect reality is possible. So, I understand that it may be hard to think that anyone could have anything resembling a perfect life. But for us, we still found our version of perfect.

Late in September 2014, as our kids were just getting into school, and just settling into their routines, we had news that would forever change our perfect world. It’s the phone call that changes your entire life. That phone call from Livia’s school telling me that she collapsed on the playground, will be forever engrained in my memory. Just two short weeks later, we would be saying good bye to our beautiful little girl Livia. Goodbye to our perfect little world. At 8 years old, my blonde haired, blue eyed, little angel, left this
world so unexpectedly, it shattered our core, and took a piece of us with her forever. Nothing could have prepared us for this tragedy.

I cannot adequately even begin to explain the pain, sorrow and emptiness we now feel. It is every parents’ worst nightmare, and we are living it. There were several months, I kept asking myself to just wake up. To get over this awful, terrifying reality, but no matter what, I couldn’t. I couldn’t wake from this, I could barely muster the strength to breathe, let alone face the reality of one of my children no longer being in our perfect world.

Every minute of every day, I think about Livia. I think about what she would be doing, what she would say, or what she would be thinking. And with every thought that I have of her, I also bear a smile. I am able to smile because of the joyful memories I have of such a wonderful, true little girl. I was beyond blessed to be her mom and we were blessed to be her family, and as I think about that statement, I remember why. I remember the stories of Livia I have deep down inside that remind me just how truly fortunate we were to raise her, teach her and just be with her in her life. Dave, Mason and I were chosen to be the ones that were lucky enough to get her in our perfect little world. We were blessed because of all the things this little 8 year old girl taught us. Yes, believe it or not, she taught us so many things.

All I have left of Livia are those memories. Memories that as I reflect upon, make me realize what a gift she obviously was, but also what she has taught me. I have always known, since her birth that she was special beyond words. I knew in my heart this little girl, would somehow form me into the person I am today. But as I am forced to have only memories, I also have this incredible reflection. I look back at the way Livia lived her life. Although the pain is real, and the reality is terrifying; I find peace in sharing with you who my daughter was, and the things she taught me. Our story unfolds with the birth of our daughter, Livia.

In 2005, Dave, Mason and I welcomed a beautiful baby girl into the world. Our threesome finally turned into our much anticipated foursome! She was radiant and just perfect. We knew that Livia needed a very special name, as we were blessed to have been given the opportunity to be parents once again. We chose her name, Livia Grace, as we felt it was “God’s Grace” that we were blessed with another chance to be parents; another precious life.

Mason was beyond excited. At 3 years old he had someone to talk to, play with and teach all the beautiful lessons of the world. Mason was diagnosed with cystinosis at 18 months old, so at this point, we had finally grasped this new path we were forced upon, and we were embracing it as much as we could. When a diagnosis like cystinosis enters your life, it forever changes you, but we were quickly forced to adapt to those changes and find a new normal. At 10 weeks old, Livia, too was diagnosed with cystinosis which was a crushing blow. Hysteria set in, once again, just as it had with Mason’s diagnosis. As if the first diagnosis wasn’t enough, the second tested every ounce of faith we had left, leaving us on the edge of ruin.

But then, like an epiphany I cannot explain.... I felt a calm, an almost sense of relief. As I sat, looking at my busy 3 year old son, gently kissing his baby sister on her cheek, I, at that moment realized that Livia’s diagnosis was actually a gift. I know, it sounds nuts, but let me explain. At that moment in time, I realized that my son, would
not be alone on this journey, this path that we were thrown upon. He had a partner. A sister, a friend to hold his hand, and to walk upon this path together. Dave and I had at this point gotten our routine down. We had our fights, our issues with meds, and we were finally at a comfortable pace with it. We knew, with all the fury and strength we had to conquer this when Mason was diagnosed. That same fury, strength and commitment came to us when Liv was diagnosed. We knew we would handle this. We would get through this all together. This was our family. Our children have cystinosis, and that bittersweet reality was going to take all the courage we could gather. But we were ready.

Their true commitment to one another persevered and was truly inspirational. Their bond started at a very young age, and through the years, and through all they have endured, their bond grew into something, I cannot fully explain. Livia just adored Mason, and it was so evident. From the time the girl was born, she would do anything she could to be by him, play with him or learn from him. If you saw both of them, you knew they shared a special bond, they were truly best friends.

Livia was such an incredible little sister and daughter. I know we’re her parents and quite biased at that, but I have to say, she was special; from her breathtaking beauty, and her delicate features, her porcelain skin and her stunning blue eyes. Her kindness and her huge heart was something you just don’t see every day. From the time Livia was born, she was angelic. Livia had a birthmark on her neck. We called it “God’s kiss”. We always told her that only a few very special people have a birthmark. It is a sign from God that she is special. She knew this, we told her often.

She was a pistol. Our spunky little lady, who loved to dress up and wear jewelry, polish her nails, and catch butterflies in the yard. Livia was a Brownie member and loved her troop and her leaders. She couldn’t wait for the next meeting and put on her sash with such pride. She still loved to cuddle up on our laps and snuggle with her blankie. She loved more than anything to swing on her swing set daddy built. “Mommy, see how high I can go”, she would always say to me. She would sing songs loudly as she went back and forth, and we would look out the window, hearing her beautiful, unmistakable voice, giggling with delight at the beauty of her being. The effervescent splendor of that hair blowing in the wind, and those beautiful long legs kicking back and forth! What I wouldn’t give for one more look at that.

Livia had passion for so many things. But most importantly, her passion was her family. Her brother was her world and although we had the sibling fights and normal sibling arguments, they were beyond a dynamic duo! Just as she adored her brother, there was another boy in her life, whom she adored just as much. It was her puppy, Reese. When we first got Reese, he would only do certain things for Livia that he wouldn’t do for anyone else. We called her our “Dog Whisperer”, and she relished in that title. In the hospital, all she talked about was coming home to Mason and her dog Reese. Her plan was to buy a bone for Reese on the way home from the hospital so when she walked in the front door, she could surprise the dog with it. Our little Livia has always been very nurturing and car-
The funny thing about Livia, is that she was always full of surprises. Liv always had her coat pockets full of goodies. Whether it was rocks, barrettes, dog treats or a treasure. I remember one particular instance, about three weeks prior to her passing. We were at Mason’s soccer game and she reached inside her pocket and pulled out these little seeds that lay in her tiny hand. I asked her what all those things were. She explained they were “tree seeds”, and she had gotten them off trees at her school. I asked her why? She said she and her friend walked around their school “planting trees”, by scattering those seeds all over the playground, in hopes that someday, a new tree would be there, and she would be the one who had planted it. She was quite excited to tell me of her plan. I, of course, thought it was an awesome plan.

I reflect on this little girl and these little stories, and I am able to remember what perfection is for me. It is the children we raise. It is who they become and how they are able to fight, be brave, and show empathy and love. It’s the home we live in, the people who surround us, and the everyday things that make us smile. It’s what gets us out of bed each and every day, to do it all over again. It’s our perfection.

A few days after Livia passed, we were tucking Mason into bed. He looked at me sadly, and asked me the most difficult question I ever heard. He said “Mom, I just don’t know what my purpose is anymore”. I sat in absolute amazement. I sobbed, and I was instantly crushed. His purpose... He felt that he didn’t have a purpose without his sister. I get that, and honestly I had been asking myself the same question the entire time. What is our purpose now in this life without Livia? I have reflected upon this so much. I have prayed, and I have hoped for a purpose. Why all this pain, what purpose could these tragedies possibly be? I may never know, but what I do know is this:

Our life, just like each of you, holds purpose. Livia’s purpose was beyond explainable. But I can say that her purpose was so much more than her constant love, her bright light, her amazing sense of humor, her beauty and her kind, gentle empathy to all those around her. Her purpose, we believe, was to also stand by her brother’s side through their journey together. Her purpose was to fight, to be courageous, to be a force that cystinosis was never going to make weak. She didn’t care about cystinosis, and didn’t live her life as if she was affected by it. She rose above it. She took her pills, all in one swallow, then immediately went onto bigger, better things. She didn’t bat an eyelash at this. This was a part of her life, but she was not going to let it define who she was.

Mason has purpose too. But of course that purpose changed to the extreme when Livia left us. Mason was the protector, the teacher. He is kind, caring, empathetic and completely hilarious! He would, for 8 years, teach her the ways of the world, in ways Dave or I could not. He taught her strength, by example. He taught her how to deal with cystinosis, and how to go on with life, even with this completely tiresome disease. He was her strength, as she was his. As the years went on, they defined their roles. Each year getting better than the one before. Each year, they got stronger, and they came out on top and never looked back. Each angst, they got back up, dusted themselves off, and continued on.

As we face our reality of tragedy and loss, we also face the fear of finding our purpose again. Our perfection of sorts. Even in this despair I am able to see that Livia would want us all to find purpose, and find joy and
happiness within these battles we all fight each day. I know this because she was my amazing daughter, who taught me how to find courage and strength when I didn’t think I could. Even though our world is crushed, torn and unmendable, I do know for a fact that my family of three is still my perfect little world. I know this because Livia is still with us, in spirit. She is watching over us, giving us the strength to continue being great parents to Mason and continue to persevere.

I write this tribute because through our pain, and through our sadness, I find inspiration in the life my daughter lived. But I also, in my pain, have realized that my story is worth sharing. If we can take anything out of this life, I pray that we can take with it the hope, desire and the bravery to continue to fight. My kids are examples for me. They teach me, and have taught me to go with the flow, and just keep trying, as best as I can, to keep my chin up, look forward and trudge through. Livia will never be far from our minds, and she will never be forgotten. I will make sure her memory is kept alive, through the way I live my life, and the way I can hopefully inspire someone else by telling her story.

We have started the “LivGracefully” movement in memory of Livia Grace Stilke. It reminds us of how she embodied life to the fullest, and how she went through life, ever so gracefully. Through our dedication to Liv, we will help give back to CRN, and all the passions this little girl loved so much. We invite you to Livgracefully.org or on Facebook to read posts, see pictures or to just be inspired. In keeping her memory alive, we hope you all find your perfect, your purpose and to live gracefully, just as Livie did.

There’s a saying that I love. It says that “it takes a village to raise a child”. It’s this “village” that consists of our friends, family, and our cystinosis community who have inspired us to continue to find passion and faith to keep moving forward. It’s this same village, who has helped us through this awful loss. We thank you all from the bottom of our hearts. I do not know where we would be without each and every one of you. From the first diagnosis, to the second, and each countless obstacle, you have all been there, willing to hold our hand as we pressed forward. Dave and I thank you for that. More than I can ever explain, we are grateful for being a part of this family and blessed to be a part of this “village”.

Livia will never be forgotten. She will never be far from our thoughts and we will allow her memory to live on. But through our grieving, we do have to continue to live and try be great parents for Mason. So, if you see us out for dinner, and we share a laugh or two, or if you see us at his soccer game, cheering Mason on, please do not think we are no longer sad or mourning for our little girl. Just know that we are trying to do the best that we can, and just because you see us laughing during the day, doesn’t mean we are not crying ourselves to sleep at night. Your support, prayers and love will continue to allow us to live a life; a different life, but a life for our amazing young man.

May the sweet memory of Livia remind us all how to ”LivGracefully”.

(To learn more about Livia Grace Stilke and how her family plans to honor her memory please visit  http://www.livgracefully.org/ )
Order, Store, & Use

To order, please call:
Walgreens Specialty Pharmacy
1-877-534-9627
Mon–Fri, 8AM–8PM EST

1. To order, please call:
Walgreens Specialty Pharmacy
1-877-534-9627
Mon–Fri, 8AM–8PM EST

2. Ships directly to your home

3. Store unopened bottles in the freezer

4. Every 7 days remove 1 bottle and thaw for 24 hours before use*

5. Discard bottle after 7 days

*NO REFRIGERATION NECESSARY AND DO NOT REFREEZE

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

by Jeff Larimore

The Cystinosis Research Network, Inc
Financial Review – Accrual Basis

For the 3 months ended March 31, 2015

Revenues

For the three months ended March 31, 2015, total income secured of $133,600 was approximately three times more than the same period in 2014. This increase was attributable to better financial results from Fundraising activities and corporate donations supporting the 2015 Family Conference.

Expenses

Total operating expenses of $55,000 were 50% more than operating expenses for the same period during 2014. Research grant expenditures in the first quarter of $27,000 was the primary reason for the increase in operating expenses as compared to the same period in 2014.

Net operating income of $74,000 for the three months ending March 31, 2015 exceeded the net income of $8,000 for the same period in 2014. Total increases in 2015 revenues exceeded increased research grant payments generating favorable financial results.

Net change in cash through March 31, 2015 was an increase of approximately $80,000. Total cash on hand was $505,000 at the end of the first quarter. This increase was derived from increases in total income received in excess of program service expenditures. Additional expenditures supporting the 2015 Family Conference are anticipated to be made in the second quarter 2015.

Jeff Larimore, speaking at a PAS discussion group entitled “Clinical and Transitional Research on Rare Disease: A Team Sport”
CRN Exhibits at the Pediatric Academic Society Conference in San Diego

By Jeff Larimore

The Cystinosis Research Network actively engaged in the Annual Meeting of the Pediatric Academic Societies held in San Diego, CA in April. José Morales and Jeff Larimore represented CRN as a Patient Advocacy exhibitor speaking over three days with a collection of medical professionals specializing in many pediatric disciplines from the U.S. and across the world.

Jeff Larimore also participated in a session discussion titled “Clinical and Translational Research on Rare Disease: A Team Sport” as a Patient

Continued on page 22

CRN Announces 2015 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, 2015.

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, 2015 to be considered:

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

2014 CRN Scholarship winners, Jack Wyman, with his sister Kacy, and Caroline Larimore
Flippin’ Friends Pinball Fundraiser Held in Honor of CRN

by Dave Greeley

Jeff Hooper, a friend of the Greeley family, hosted a fundraiser for CRN back in October 2014. The Lincolnshire, IL resident has been a lifelong fan of pinball and owns over a dozen machines. The event was a pinball tournament where 40 participants competed, including two of the best pinball players in the world. Former World Champion David Hegge won the event, while the host came in fourth place. The event was an officially sanctioned event by the International Flipper Pinball Association and was associated with flippinfriends.org, pinball tournaments for a cause.

"Hoop" hopes his tournament will become an annual charity event benefitting CRN. This past year's event raised $1,000 for CRN. Thanks, Jeff Hooper!!!
attend the Family Conference. For more information, see detailed infor-
mation in this newsletter or reference the CRN website at:

2015 CRN Family Conference

Hotel and Room Reservation Information
DoubleTree by Hilton Chicago-Magnificent Mile, 300 E. Ohio St., Chicago,
Illinois
Reservations: on-line with group booking code or by phone 1-312-787-
6100, mention CRN Family Conference for reduced rate.

Website: www.doubletreemagmile.com

Room Rate: $179.00/night plus tax ($20/night for each additional adult (17+) over 2 adults)

Check-in Time: 3:00pm / Check-out Time: 12:00pm

Parking at Hotel: $40.00/per 24-hour period

Group code: CRN

The CRN block of rooms will be released on June 24th, so plan to make your reservations before then.

Travel and Ground Transportation
The closest airports to the DoubleTree are Chicago O’Hare (ORD) and Chicago Midway (MDW) International Airports. Detailed directions from the airports to the hotel are available on the hotel’s website above or on the CRN website.

There are several options available for ground transportation:

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<tr>
<th>FROM CHICAGO O’HARE</th>
<th>FROM CHICAGO MIDWAY</th>
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<tr>
<td>Limousine</td>
<td>85.00 USD</td>
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<tr>
<td>Taxi</td>
<td>45.00 USD recommended</td>
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<tr>
<td>Subway/Rail</td>
<td>5.00 USD</td>
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<tr>
<td>Super Shuttle</td>
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Conference Registration

You can register for the conference online at https://cystinosis.org/events/conferences/2015-crn-family-conference. You can also print, complete and send it along with a check for the registration fee to the following: CRN Conference Committee - Attn: Pam Woodward, 1368 W. 2600 N., Pleasant Grove, UT 84062

Please make check payable to: Cystinosis Research Network

Registration Fee

Early bird registration fee (before June 1st): $50 per individual or household
Registration fee (after June 1st): $75 per individual or household

Childcare & Teens
Childcare will be provided during the conference for children ages 1-12 years. There will be age-appropriate activities, games, crafts, and entertainment. Snacks and plenty of water will be provided as well. Parents will be responsible for administering all medications. We are exploring opportunities for the teen group - more information will be forthcoming.

Conference Scholarships
A limited number of conference scholarships are available to individuals and families affected by cystinosis. Scholarship information and the application were mailed to homes and can be downloaded from the CRN website at https://cystinosis.org/events/conferences/2015-crn-family-conference

The deadline for submitting application materials is May 1, 2015.

Poster Session
The Poster Session will take place during the conference proceedings on Friday, July 17, 2015, from 1:15 p.m. to 2:15 p.m. This session will showcase a mix of science, medicine, industry and advocacy group, and patient experiences to provide an interactive experience for both family and professional attendees. Researchers, clinicians, industry, advocacy representatives, students, patients, and caregivers will be invited to exhibit their latest research findings, treatment breakthroughs, advocacy group updates, and real patient and family experiences. This will be an interactive session where exhibitors will be available to discuss their work or experiences with those attending. Attendees will be invited to browse the posters and take this opportunity to ask the authors and presenters questions.
## Cystinosis Research Network Family Conference

**July 16—18, 2015**

DoubleTree by Hilton Chicago—Magnificent Mile  
Chicago, Illinois

### AGENDA

#### Latest revision 4/30/15

<table>
<thead>
<tr>
<th>Wednesday, July 15</th>
<th>Room</th>
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| 3:00 pm – 8:00 pm  | **CRN Board of Directors Meeting**  
*Closed Session* | Fairbanks |

<table>
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<tr>
<th>Thursday, July 16</th>
<th>Room</th>
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<tr>
<td>12:00 pm – 5:00 pm</td>
<td><strong>Registration / Information Desk</strong></td>
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<tr>
<td>“<strong>Dream, Achieve, Inspire</strong>” art exhibit</td>
<td>Lasalle Ballroom</td>
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<td>”Dream, Achieve, Inspire” an Art Exhibit Artists with cystinosis from around the world have come together to share their creativity for global cystinosis awareness. More than 80 artists from 29 countries have united to bring cystinosis struggles to light. Each piece tells a story of strength and hope. The exhibit crosses language and cultural barriers in an effort to express the need for research, better treatments and in some countries access to life saving medication where there is none. “Dream, Achieve, Inspire” is an emotional journey through the hearts of those with cystinosis...Enjoy the beautiful ride!</td>
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| 2:00 pm – 4:00 pm | **CRN Scientific Review Board Meeting**  
*Closed Session* | Fairbanks |
| 5:00 pm – 7:00 pm | **CRN Welcome Reception**  
*Hosts: CRN Board of Directors*  
*Complimentary* | Pool Deck/Lasalle Ballroom |
| Please join us for drinks and appetizers and an opportunity to meet the families and physicians attending the conference. |

| 5:00 pm – 9:00 pm | **Artists in Residence** |
| Artists in Residence | Huron |
| Snow City Arts invites you to participate in the creation of a mural representing our conference. All attendees are encouraged to add their own twist to the project that will become part of the Art Exhibit and travel the world to other cystinosis conferences. Be a part of our artistic love letter to the cystinosis community! The final piece will be unveiled at the closing party. |

| 7:30 pm – 9:00 pm | **Family Introductions**  
*Pam Woodward, CRN Vice President Family Support* | Lasalle Ballroom |

<table>
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<th>Friday, July 17</th>
<th>Room</th>
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| 7:00 am – 8:30 am| **Breakfast**  
*Complimentary* | Lasalle Ballroom |
“Dream, Achieve, Inspire” art exhibit

"Dream, Achieve, Inspire" an Art Exhibit Artists with cystinosis from around the world have come together to share their creativity for global cystinosis awareness. More than 80 artists from 29 countries have united to bring cystinosis struggles to light. Each piece tells a story of strength and hope. The exhibit crosses language and cultural barriers in an effort to express the need for research, better treatments and in some countries access to life saving medication where there is none. "Dream, Achieve, Inspire" is an emotional journey through the hearts of those with cystinosis...Enjoy the beautiful ride!

Artists in Residence

Snow City Arts invites you to participate in the creation of a mural representing our conference. All attendees are encouraged to add their own twist to the project that will become part of the Art Exhibit and travel the world to other cystinosis conferences. Be a part of our artistic love letter to the cystinosis community! The final piece will be unveiled at the closing party.

Global Genes Teen Adventure

8:30 am - 8:35 am Welcome and Opening Remarks
Jeff Larimore, CRN President
Lasalle Ballroom

8:35 am - 8:50 am Cystinosis Research Network – Your Advocacy Group
Jeff Larimore, CRN President
Lasalle Ballroom

8:50 am - 9:35 am “Who Sets Our Limits?”
Bonner Paddock

Bonner Paddock is the Founder of the OM Foundation (OMF), 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. Bonner is the first person with Cerebral Palsy to ascend Mt. Kilimanjaro and to finish the infamous Ironman World Championship unassisted. He has since raised over $1 Million Dollars to help build and support early learning centers in the US & Africa, which provide therapy for special needs children. He has also recently launched his first book, One More Step, a memoir about his life living with Cerebral Palsy, conquering the incredibly challenging physical feats described above, and starting his own foundation. Listen to Bonner’s inspirational story as he shares with you what held him back for 30 years and how he pushed beyond his own limits to achieve success. www.1man1mission.org
Lasalle Ballroom
### Cystinosis Overview

**William A. Gahl, M.D., Ph.D., Clinical Director, National Human Genome Research Institute; Director, National Institutes of Health Undiagnosed Diseases Program**

Nephropathic cystinosis, a lysosomal storage disorder due to defective transport of cystine out of lysosomes, results from mutations in CTNS. Almost half the patients in North America and Europe are homozygous for a 57-kb deletion in CTNS.

Without treatment, children with cystinosis suffer from renal Fanconi syndrome and its complications, growth retardation, photophobia, and end-stage renal failure requiring kidney transplantation. Treatment with oral cysteamine, which can reduce cellular cystine levels by 95%, dramatically slows glomerular deterioration and normalizes growth. Based upon our examinations of 100 adult cystinosis patients between 1985 and 2006, we found striking rates of mortality (33%; mean age 29 years) and morbidity (24-75% for each complication), specifically related to hypothyroidism, hypergonadotropic hypogonadism (in men), pulmonary insufficiency, swallowing abnormalities, myopathy, retinopathy, vascular calcifications, and diabetes. In adults, long-term (>8 years) oral cysteamine therapy was associated with significantly greater height and weight, older age at renal transplant, lower serum cholesterol levels, and lower rates of morbidity and mortality. In fact, as duration of cysteamine therapy increased, the frequencies of non-renal complications of cystinosis decreased. All symptomatic cystinosis patients should receive oral cysteamine therapy, and the earlier diagnosis is made and treatment initiated, the better the prognosis. This talk will serve as background for discussions of current pursuits into cystinosis clinical and basic research.

### Management of Infants and Children with Cystinosis

**Moderator: Craig B Langman, MD**

**Setting the stage: 3 min**

- **Larry Greenbaum, MD, PhD:** A physiological approach to treatment of the Renal Fanconi Syndrome
- **Paul Goodyer, MD:** Water Conservation with Indomethacin in Cystinosis
- **Galina Nestarova, MD:** Rachitic Bone Disease
- **Neveen Soliman, MD, PhD:** Chronic Kidney Disease – Metabolic Bone Disease
- **Ewa Elenberg, MD, MS:** Gastrointestinal Manifestations

Questions to all from the audience

### Adolescent Issues in Cystinosis

**Moderator: Elena Levtchenko, M.D.**  **Panelists: Paul Grimm, M.D., Minnie Sarwal, M.D., Ph.D., Robert Kleta, M.D., Ph.D., Maria Helena Vaisbich, M.D.**

### Luncheon

**Complementary**

### Poster Session

This session will showcase a mix of science, medicine, industry, advocacy group and patient experiences to provide an interactive experience for both family and professional attendees. Researchers, clinicians, industry, advocacy representatives, students, patients, and caregivers will be invited to exhibit their latest research findings, treatment breakthroughs, advocacy group updates and real patient and family experiences. This will be an interactive session where exhibitors will be available to discuss their work or experiences with those attending. We invite you to browse the posters and take this opportunity to ask the authors and presenters questions.
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<th>Time</th>
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<tr>
<td>2:20 pm – 3:10 pm</td>
<td><strong>Transition from Pediatric to Adult Care</strong>&lt;br&gt;&lt;br&gt;<strong>Moderator:</strong> Frederick Kaskel, M.D., Ph.D.  <strong>Panelists:</strong> Richard Simon, M.D., Patrick Gipson, M.D., Jess Thoene, M.D., Robert Kleta, M.D., Ph.D., Katharina Hohenfellner, M.D., Maya Doyle</td>
<td>Lasalle Ballroom</td>
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<tr>
<td>3:15 pm – 4:05 pm</td>
<td><strong>Adult Issues/Management of Extra Renal Manifestations</strong>&lt;br&gt;&lt;br&gt;<strong>Moderator:</strong> William A. Gahl, M.D., Ph.D.  <strong>Panelists:</strong> Galina Nesterova, M.D., Rachel Bishop, M.D., Doris Trauner, M.D., Robert Kleta, M.D., Ph.D.</td>
<td>Lasalle Ballroom</td>
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<td>4:10 pm – 5:10 pm</td>
<td><strong>Medical Panel</strong>&lt;br&gt;&lt;br&gt;<strong>Moderator:</strong> Jess Thoene, M.D.  &lt;br&gt;Please join the entire group for the unique and informative opportunity to have your questions and concerns addressed by the leading physicians and researchers in cystinosis. All of the doctors who have presented at the Family Conference, all attending Medical Advisory Board and Scientific Review Board members, as well as other health care professionals involved in treating and researching cystinosis are scheduled to participate. Questions for the panel will be collected during the proceedings today.</td>
<td>Lasalle Ballroom</td>
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<td>5:10 pm – 5:40 pm</td>
<td><strong>Above and Beyond Achievement Award / Group Photograph</strong>&lt;br&gt;&lt;br&gt;<em>Christy Greeley, CRN V.P. of Research and Executive</em></td>
<td>Lasalle Ballroom</td>
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<td>7:00 pm</td>
<td><strong>Speaker Dinner</strong>&lt;br&gt;&lt;br&gt;<em>Closed Session</em></td>
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**Saturday, July 18**

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<th>Time</th>
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<tr>
<td>7:00 am – 8:30 am</td>
<td><strong>Breakfast</strong>&lt;br&gt;&lt;br&gt;<em>Complimentary</em></td>
<td>Lasalle Ballroom</td>
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<tr>
<td>8:00 am – 5:30 pm</td>
<td><strong>Registration/Information Desk</strong></td>
<td>Lasalle Ballroom Lobby</td>
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<td>8:00 am – 5:30 pm</td>
<td><strong>Child Care Open</strong></td>
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<td><strong>Teen Lounge Open</strong></td>
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<td>8:30 am – 9:00 pm</td>
<td><strong>“Dream, Achieve, Inspire” art exhibit</strong>&lt;br&gt;&lt;br&gt;&quot;Dream, Achieve, Inspire&quot; an Art Exhibit Artists with cystinosis from around the world have come together to share their creativity for global cystinosis awareness. More than 80 artists from 29 countries have united to bring cystinosis struggles to light. Each piece tells a story of strength and hope. The exhibit crosses language and cultural barriers in an effort to express the need for research, better treatments and in some countries access to life saving medication where there is none. &quot;Dream, Achieve, Inspire&quot; is an emotional journey through the hearts of those with cystinosis...Enjoy the beautiful ride!</td>
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Artists in Residence
Snow City Arts invites you to participate in the creation of a mural representing our conference. All attendees are encouraged to add their own twist to the project that will become part of the Art Exhibit and travel the world to other cystinosis conferences. Be a part of our artistic love letter to the cystinosis community! The final piece will be unveiled at the closing party.

Global Genes Teen Adventure

8:30 am – 8:40 am
**Opening Comments**
*Jeff Larimore, CRN President*

8:45 am – 9:15 am
**KDIGO Cystinosis Conference Update**
*Craig B. Langman, M.D.*
The process for a controversies conference sponsored by KDIGO will be discussed, and the one that occurred in Lisbon in December 2014 will be reviewed for topic content and methodology moving forward. An opportunity for input by the community will be reviewed. Specific highlights will be noted.

9:20 am – 9:40 am
**Raptor Pharmaceuticals/Accredo Procysbi Update**
*Eric Mosbrooker, VP, Commercial Operations, Americas*
*Raptor Pharmaceuticals and Bill Martin, Vice President, Accredo*
Eric and Bill will provide an update on the services available to Procysbi patients to make accessing the drug from the pharmacy easier and more streamlined. Eric will discuss Raptor’s Patient Support team and Bill will update our community on steps Accredo has taken to improve and expand the services available to patients. Both organizations are committed to continuously improving, and each would like to hear from us about what they can do for the cystinosis community.

9:45 am – 10:05 am
**Sigma Tau Pharmaceuticals/Walgreens Cystaran Update**
*Lesli King, Senior Product Manager, Valerie Paterno, Senior Manager Distribution Services Sigma Tau Pharmaceuticals, and Amber Walker, Director, Specialty Pharmacy Development Walgreen’s Specialty Pharmacy*
Lesli, Valerie and Amber will provide an update on the services available for obtaining access to Cystaran via Walgreens Specialty Pharmacy. Common questions and questions from the community about Cystaran will be addressed. Sigma-Tau and Walgreens are committed to the continued support of the Cystinosis community. Working together we continue to improve and streamline the processes, ensuring the patients receive the most efficient and robust services.

10:05 am – 10:20 am
**Break**

10:25 am – 11:10 am
**Parents of Children and Adults with Cystinosis Panel**
*Moderator: Jennifer Caughlin. Panelists: Jen Wyman, Brett and Brittney LeBeau, Kevin and Jackie Keizer, Jill Morrill, Don and Doretta Hoffmann, Vic and Ina Gardener, Clinton and Annie Moore*
Panel presentation during which parents of children and adults with cystinosis will answer prepared questions and address topics related to the use of coping mechanisms through the ups and downs that cystinosis brings related to not only developmental and transitional issues of daily life but also medical issues. Parents of individuals at every stage of the disease will be featured and will share how they have managed the variety of challenges they have faced. Audience participation will be encouraged.
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<td>11:15 am -</td>
<td><strong>Living with Cystinosis Panel</strong></td>
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<td>12:00 pm</td>
<td>Moderator: Maya Doyle. Panelists: Stephanie Sorenson, Paula Shal,</td>
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<td>Jennifer Logisci, Steve Schleuder, Katie Morrison, Christian Morales,</td>
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<td>Andrew Hoffmann, Shea Hammond</td>
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<td>Panel presentation during which individuals living with cystinosis</td>
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<td>will answer prepared questions and address topics related to the</td>
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<td>use of coping mechanisms through the ups and downs that cystinosis</td>
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<td>brings related to not only developmental and transitional issues of</td>
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<td>to their full potential given the challenges they have faced. Audience</td>
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<td>12:00 pm -</td>
<td><strong>Luncheon</strong></td>
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<td>1:00 pm</td>
<td>Complimentary</td>
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<td>1:05 pm -</td>
<td><strong>Bullying/Educational Issues</strong></td>
<td>Lasalle Ballroom</td>
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<td>1:35 pm</td>
<td>1:40 pm - 2:00 pm **Preimplantation Genetics/Early Pregnancy Diagnosis</td>
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<td>of Cystinosis**</td>
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<td>Jared C. Robins, MD, Associate Professor, Chief, Division of</td>
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<td>Reproductive Endocrinology and Infertility, Department of Obstetrics</td>
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<td>and Gynecology, Northwestern University Feinberg School of Medicine</td>
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<td>2:05 pm -</td>
<td><strong>Adherence Issues</strong></td>
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<td>2:25 pm</td>
<td>Ewa Elenberg, M.D.</td>
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<td>Why people are not compliant, spectrum of the problem, Relationship</td>
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<td>between compliance score and time to ESRD, Renal Fanconi Syndrome</td>
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<td>2:30 pm -</td>
<td>**Rickets, Osteomalacia and Bone Deformities in Patients with Fanconi</td>
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<td>2:50 pm</td>
<td>Syndrome of Cystinosis: Recent Data, Diagnosis and Therapies**</td>
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<td>Galina Nesterova, M.D.</td>
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<td>Children with nephropathic cystinosis invariably develop renal Fanconi</td>
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<td>syndrome with predominately proximal tubular dysfunction, leading to</td>
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<td>the loss of electrolytes and minerals, particularly calcium and</td>
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<td>phosphate. If inadequately treated with supplementations of calcium,</td>
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<td>phosphate and vitamin D, this leads inexorably to rickets/osteomalacia,</td>
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<td>with dramatic skeletal deformities during growth periods due to</td>
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<td>insufficient accretion of hydroxyapatite. We have become aware that</td>
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<td>small but chronic losses of bone mineral, not recognized by laboratory</td>
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<td>studies such as serum phosphate, calcium, and alkaline phosphatase,</td>
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<td>can produce devastating bone disease. One goal is to investigate,</td>
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<td>evaluate, and prevent bone loss and deformity due to hypercalciuria and</td>
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<td>hyperphosphaturia, and to develop appropriate therapeutic guidelines for</td>
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<td>preventing such consequences of Fanconi syndrome.</td>
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<tr>
<td>2:50 pm -</td>
<td><strong>Break</strong></td>
<td>Lasalle Ballroom</td>
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| 3:15 pm – 4:00 pm | **Workshop Session A**<br>These topic specific workshops allow families to tailor their conference experience to meet their specific needs. Families will have the opportunity to discuss and share their own experiences regarding the latest research, management techniques, and therapies with leading experts as well as with other families with similar issues and concerns. Sessions will be repeated so that participants may attend more than one workshop.  
  - Adult Session I (closed)<br>  **William A. Gahl, M.D., Ph.D., Galina Nesterova, M.D., Rachel Bishop, M.D., Doris Trauner, M.D., Robert Kleta, M.D., Ph.D.**
  - **General Issues**
  - Teen/Transition Session<br>  **Paul Grimm, M.D., Minnie Sarwal, M.D., Ph.D., Frederick Kaskel, M.D., Ph.D., Richard Simon, M.D., Patrick Gipson, M.D., Karen Waggenbrenner, Katharina Hohenfellner, M.D., Maria Helena Vaisbich, M.D., Elena Levchenko, M.D.**
  - Newly Diagnosed Families/Childhood Session<br>  **Craig Langman, M.D., Paul Goodyer, M.D., Ewa Elenberg, M.D., Larry Greenbaum, M.D., Ph.D., Jess Thoene, M.D.** | Superior I, Superior II, Superior III |
| 4:15 pm – 5:00 pm | **Workshop Session B**<br>  - Adult Session II (closed)<br>  **Moderator: Jennifer Coughlin. Panelists: William A. Gahl, M.D., Ph.D., Galina Nesterova, M.D., Jennifer Logisci**
  - **sex/reproduction/birth control issues**
  - Teen/Transition Session<br>  **Paul Grimm, M.D., Minnie Sarwal, M.D., Ph.D., Frederick Kaskel, M.D., Ph.D., Richard Simon, M.D., Patrick Gipson, M.D., Karen Waggenbrenner, Katharina Hohenfellner, M.D., Maria Helena Vaisbich, M.D., Elena Levchenko, M.D.**
  - Newly Diagnosed Families/Childhood Session<br>  **Craig Langman, M.D., Paul Goodyer, M.D., Ewa Elenberg, M.D., Larry Greenbaum, M.D., Ph.D., Rachel Bishop, M.D., Jess Thoene, M.D., Doris Trauner, M.D.** | Superior I, Superior II, Superior III |
| 5:10 pm – 5:30 pm | **Conference Wrap Up**<br>  **Jeff Larimore, CRN President** | Lasalle Ballroom |
| 6:30 pm – 10:30 pm | **Dinner Dance**<br>Complimentary, Cash Bar. Please join all conference attendees for dinner, dancing, photo booth and fun for all ages to celebrate our cystinosis community. Special performance by Tori Lynn Jones. | Lasalle Ballroom |
Advocacy Representative. The session highlighted the continuing need for patient advocacy organizations to be actively engaged with public resources such as NIH & FDA, research investigators during clinical trials and with biotech industries who pursue enhanced treatments for those managing rare diseases.

José Morales and Jeff Larimore bring Cystinosis Awareness to attending physicians at the recent Pediatric Academic Society conference held in San Diego in April.
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!

Patients Needed for Growth Hormone Study at the National Institutes of Health

Cystinosis Research Network Cystinosis Fellow Dr. Galina Nesterova and coordinator Joy Bryant are still in need of more individuals to enroll in a new research study at the National Institutes of Health. The study will look at whether growth hormone (Humatrope, recombinant human Growth Hormone) may help prevent the loss of muscle that can sometimes occur in people with cystinosis. People with cystinosis are eligible to participate if they age 18 or over, are having muscle problems related to cystinosis, are compliant with their cysteamine therapy, and do not have cancer, high blood pressure, uncontrolled diabetes or significantly impaired kidney function.

The study would last for 2 years and involve 10-12 visits to the NIH. Patients would be assigned to a group that either receives daily growth hormone injections or no treatment for the first year, then would switch to the opposite treatment the second year, so that each participant would receive growth hormone for one year. Several tests will be included such as blood and urine, MRI and DEXA scans and other physician evaluations, all of which will be at no cost to the participant.

If you are interested in finding out more, please contact Joy Bryant at bryantjo@mail.nih.gov or (301) 443-8690 or Dr. Nesterova at nesterovag@mail.nih.gov.
Research Update

By Christy Greeley, Executive Director and VP, Research

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 nearly $4 million total in research grants and fellowships. CRN funds a Cystinosis fellowship at the National Institutes of Health. CRN has funded research and education programs in the United States and many countries around the world including Egypt, Mexico, England, Scotland, Italy, Belgium, France, and much more. CRN research topics are aimed at every aspect of cystinosis with the purpose of understanding cystinosis 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.

CRN Announces 2015 Call for Research Proposals

Applicants may now submit proposals for the 2015 CRN Call for Research Proposals. Research proposals may be submitted to CRN for review and consideration. CRN utilizes a Scientific Review Board comprised of leading experts on the disease of cystinosis which reviews grant proposals and submits funding recommendations to the organization. More specifically, the Scientific Review Board provides independent, objective review and recommendations regarding each research proposal utilizing grant review guidelines established by CRN and in accordance with the mission of the organization. The Chairperson of the Scientific Review Board summarizes its recommendations and presents them to the Cystinosis Research Network which then votes on each proposed project.

Priority is given to interventional research, both clinical and basic, that will lead to improved treatments for cystinosis. New investigators are particularly encouraged to apply. CRN has a strong interest in funding projects related to advancing Newborn Screening for cystinosis – applications regarding this subject will have priority. Applicants must submit an electronic copy of their proposal to: Christy Greeley, Vice President for Research cgreeley@cystinosis.org

Jack, Christy, Alex and Dave Greeley
Submission deadline is May 31, 2015. Proposals will be reviewed in July and notification will be made to applicants in August. Detailed information on the grant submission process can be found on the CRN website at: https://cystinosis.org/research/grant-guidelines

Dr. Patrick Gipson added to CRN Medical Advisory Board
The Cystinosis Research Network is proud to announce a new addition to our Medical Advisory Board -- Patrick Gipson, M.D., Clinical Assistant Professor of Internal Medicine & Pediatrics, Division of Nephrology at the University of Michigan Medical Center. Dr. Gipson is board certified in both pediatric and adult nephrology and specializes in genetic kidney diseases and clinical research. We are thrilled to add his expertise to our advisory board, especially in light of our recent efforts in transitioning topics.

CRN proud to sponsor the KDIGO

CRN was proud to sponsor the KDIGO (Kidney Disease Improving Global Outcomes) Controversies Conference on Nephropathic Cystinosis, held December 11-13, 2014 in Lisbon, Portugal. Christy Greeley, CRN Executive Director, attended as CRN and patient advocacy representative, participating in the Adolescent Issues workgroup. KDIGO is an international organization whose mission is to improve the care and outcomes of kidney disease patients worldwide by promoting coordination, collaboration, and integration of initiatives to develop and implement clinical practice guidelines. Periodically, KDIGO hosts conferences on topics of importance to patients with kidney disease. This conference brought together approximately 50 experts from all over the world to deliberate on the current state of evidence surrounding cystinosis treatment and diagnosis. The focus was on both adult and pediatric cystinosis, and also dealt with the transition from pediatric to adult care. Patient views and concerns were a major part of the discussion. A final conference report summarizing the proceedings and deliberations will be published in a peer-reviewed journal. In addition, Dr. Craig Langman who co-chaired the meeting, will be presenting a summary of the proceedings at the CRN Family Conference in July.
Shea Hammond Receives 3\textsuperscript{rd} Kidney Transplant As Part of 70 Person Kidney Swap

By Colleen Hammond

Poet Mattie Stepanek once said "Unity is strength...when there is teamwork and collaboration, wonderful things can be achieved." Our family has witnessed the miracles that occur when great love and teamwork come together.

Shea Hammond was diagnosed with Cystinosis when he was 8 months old in July of 1990. His transplant story begins in 2006, when he was 16. At a time when most teens think of homework, hanging out with friends and dating, Shea was focused on facing a kidney transplant. I was a match and we were able to schedule the transplant for the summer before his junior year of high school.

Unfortunately, this transplant came with many complications that resulted in Shea needing another transplant within 3 years. This time, my husband Jack was the perfect match. We were able to schedule Shea’s second transplant for the summer before his sophomore year of college. All went perfectly well for five years. Shea graduated from college, was working full time, living in his own apartment in Boston. He was enjoying every aspect of life. In the spring of 2014, Shea suddenly developed kidney stones, which were routinely removed. But shortly thereafter it became clear that his new kidney, his second in less than a decade, was failing.

The prognosis was grim – dialysis and a likely more than five-year wait to get a kidney. Shea began emergent dialysis on June 1, 2014. We were all devastated. Up un-
til this point we were able to organize preemptive transplants and avoid dialysis. Dialysis took a toll on Shea’s body and he suffered many complications. Our family and friends rallied to find a third kidney. This was no easy task as with each transplant his body continued to become more sensitized to the donor kidneys. More than 50 people volunteered to be tested. But none were a match.

After months of looking for a match with no luck, we knew we had to aggressively work to try to find a donor for Shea. He was placed on the deceased donor list in the East Coast Region where we live. He also went through testing in Florida and Baltimore and was placed on their lists as well. It is important to get listed in different regions if you are highly sensitized with antibodies.

During this time, Shea’s brother-in-law, Max, and cousin, Colleen, said they would be willing to complete the testing to participate in a kidney exchange program. Both Max and Colleen endured the months of testing and both were approved to participate in the National Kidney Registry Kidney Exchange Program. Both Max and Colleen had watched Shea’s struggles with Cystinosis. Now watching his latest transplanted kidney fail, they knew that the concept of the kidney swap through the Kidney Registry would be the only viable solution with such a long waiting list ahead on a traditional list. After more testing, it was revealed Colleen would move forward with the Kidney swap. On February 19th, her kidney went to California, while Shea’s new kidney would travel from California. 70 people – 35 donors and 35 recipients – participated in this kidney exchange that occurred across the country.

Now on the mend, and through a lifetime encompassing more than 40 surgeries, Shea has maintained his sense of humor. He is driving and walking, and can now fit into his skinny jeans as the post-surgery bloat has gone down. Colleen also couldn’t be happier, saying that, without question, it’s the best gift she could ever have given. “Kidney failure halted all aspects of my life completely and was likely to reduce my life expectancy significantly. I had to stop working and stop supporting myself. Despite its benefit, I felt horrible on dialysis and had absolutely no energy to do anything on days I received it. Because of the generosity and love of Colleen, everyone involved in this chain are finally on track to get our lives back,” says Shea.

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


Send your contact info to the e-mail listed to learn who is in your area. It’s that easy!
Jack Greeley Birthday Letter Fundraiser has raised almost $250,000

By Christy Greeley

The Greeley Family once again celebrated Jack’s St. Patrick’s Day birthday, 15 this year, by sending out the 13th edition of his birthday fundraising letter. Jack has faced many challenges in the past year and we cannot thank our friends and family enough for all of the support we have received. Since 2003 we have raised nearly $250,000 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:

Dear Family, Friends, and Colleagues,

Over the past 12 years, many of you have heard about our son, Jack Greeley and the challenges he faces everyday dealing with Cystinosis, a rare genetic/metabolic disorder. While Jack does have Cystinosis, it does not have him, and we have tried to convey that in our annual letters.

The nature of Jack’s plight has been summed up well by Rabbi Harold Kushner in his #1 best-selling book, When Bad Things Happen to Good People. In it, Kushner uses the horrible death of his own young son as inspiration to address the topic. With no interest in writing about self-pity or sharing his pain, he instead wanted to affirm life and help us deal with the limits of nature, evolution, and human moral freedom, as created by God Himself. An underlying theme from Kushner is that we often do not get to choose in life, but unlike other living creatures, humans can rise above their animal nature and control their instincts. Tragedy and hardship happen and invariably lead to questions of, “Why did this happen to me? What did I do to deserve this?” These are unanswerable and pointless questions, Kushner contends, when the better question is, “Now that this has happened, what am I going to do about it?”

That – simply – is how Jack thinks and acts. Mind you, Jack has not had a choice and
he does not know differently. With his 15th birthday upon us this St. Patrick’s Day, since his diagnosis 14 years ago in spring 2001, Jack has:

- Battled Cystinosis and for 18 months after diagnosis waged a war of survival...hospitalized twice, vomited 10-15 times per day, urinated profusely, and never ate solid food.
- Had a G-tube inserted through his stomach wall allowing for nourishment while his body stabilized and he re-taught himself how to eat solid food.
- Lived a 24/7/365 medical lifestyle – swallowing around 130,000 pills, waking up overnight for 5,000 consecutive nights for meds, taking eye drops 8X per day since 2008, going to over 400 doctor appointments, wearing shoe inserts and leg braces since 2004, and facing three surgeries.
- Developed some unique and complicated orthopedic issues where his turned ankles and legs and soft bones are creating some stress on his body, which is compounded by some spinal compression. With new treatments and therapies, Jack’s legs can hopefully be strengthened and straightened and a daily injection of growth hormone should help.

While that synopsis does not fully capture Jack’s health struggles, if anyone deserved to be bitter, it would be Jack. With a future preordained by Cystinosis, the impact could potentially be kidney failure, muscle wasting, diabetes, blindness, pulmonary deficiency, hypothyroidism, and neurological damage. While we do not know what the future holds for Jack, here is what we do know. Jack is not remotely bitter. More than anyone we know, he affirms life for all of us on a daily basis. Like Kushner teaches us in his book, Jack figured it out long ago and decided that life has to be lived for something, not just against something. How does he do this? How does Jack show it?

Jack entered his freshman year at Stevenson High School this past fall and no one knew what to expect. He dove right in with his academics (all A’s in his first term!! J) and immediately found extra-curricular activities. He joined the IM golf program in the fall, he (unknowingly to anyone) participated in a chronic disease self-advocacy group, and he went on to become a student manager for the Varsity basketball team. While those activities engaged Jack and kept him busy, what really grabbed him was table tennis. While he has enjoyed the game in the past, his interest took off when he joined the school's club. It led to his #1 “ask” this past Christmas...a ping pong table. For several weeks in a row, conversations went like this:
**JACK:** (With a full disarming grin) “Mom/Dad – can we go look at ping pong tables tonight/this weekend?”

**MOM/DAD:** (With lesser disarming grins) “Yes, Jack. Dad will go when he gets back from his trip.”

**JACK:** (Upon Dad’s return from a trip, with a full disarming grin) “Dad, when can we go look at ping pong tables?”

**DAD:** (With some disarming grin) “Jack, let me unpack, say ‘Hi’ to Mom and Alex and maybe we can go later.”

**JACK:** (With a full disarming grin the next day) “Dad, good morning. Can we look at ping pong tables today?”

**DAD:** (With no disarming grin) “Jack...we’ll go look soon...promise. By the way, how is Santa going to get the thing in his sleigh? That’s pretty big...what about the chimney?”

**JACK:** (With a ginormous full disarming grin) “I don’t know. Santa is pretty smart.”

You get the idea. This went on for a few weeks and after some serious browsing at a number of stores, finally a wonderful ping pong table was procured. Jack’s interest and love of table tennis really represents him in so many ways. Can a fun, recreational game do that? Indeed...and metaphorically this came to life for Jack at a recent table tennis tournament where he and his Stevenson classmates competed against kids from other schools:

If a smile is a window to one’s heart, then Jack just might be built of reinforced glass. His endless smile underscores his warmth and sense of humor. While at the tournament, an opposing coach came up to Dave and said, “Jack is my favorite player. He played in the most exciting match all year when we played Stevenson previously.” Jack heard the story and his smile lit up the room...deservedly so!

Some people suck energy out of a room, while others fill it. Some view a glass as half empty, while some see half full. Some take, while others give. Jack is the lat-
ter in all cases. In that earlier season match, the schools’ best players flipped sides and were paired with less experienced players in a doubles match. By the time Jack’s match was done, groups of players were surrounding the table and cheering the great play and effort. The opposing kids were feeding off of Jack’s teammates, who knew and appreciated what he was giving them.

Curiosity and competitiveness drive Jack. He likes and wants to learn and excel. Whether it is school or activities, he is willing to put forth time and effort. For instance, when Jack was at this tournament, his coach came up mid-match and asked the score. Dave was quick to offer, “Two matches to one, Jack is leading…and it is 7-4 in the fourth game.” Jack, despite an intense focus on the game at hand, with his sonar ears tuned in, matter-of-factly chimed in from afar with a correction, “It’s 2-1 me and 9-5 this game…and I’m WINNING.” His coach offered, “Hear that, Mr. Greeley. Jack is always quick to make that last point.” Laughs followed.

Jack puts forth great effort and hard work in all that he does. While perhaps noble, it is more about necessity for Jack. Let’s be straight. Table tennis is a game of skill and coordination, but it is not necessarily strenuous for the average player. Yet, when tournament play is in a big gym with an open floor plan, a smooth wood floor, and tables all around, what happens when the ball hits the floor, which is every point? It tends to roll, and for Jack with his orthopedic challenges, rolling balls mean hustling to retrieve them and getting back to the table to timely play the next point. It seems easy, but not for Jack, where things are just harder; table tennis or not.

Jack is a messenger of strength, hope, and optimism for all. He affirms life everyday rather than rejects it. How many people know someone like that who has done something truly profound and meaningful in living life? Go beyond conquests and material possessions – money made, dates had, games won, cars owned, trips taken, events attended, posts liked, emails received, etc. As Kushner asks, “Are you capable of forgiving and accepting in love a world which has disappointed you by not being perfect, a world in which there is so much unfairness and cruelty…can you forgive its imperfections and love it because it is capable of containing beauty and goodness, and because it is the only world we have?” This is the only life Jack has and he gets so much out of it because he is perfectly imperfect.

Please consider supporting the efforts of CRN by making a donation in honor of Jack and his 15th Birthday. Thank you, God Bless, here’s to what St. Patrick’s Day offers, and in honor of Jack, we wish you and your family well.
CRN Education & Awareness Update

by Terri Schleuder—Vice President, Education & Awareness

On July 16-18th, 2015, CRN will host its 7th Biennial Family Conference in Chicago. The theme this year is "Winds of Change". Planning has been ongoing for over a year for this event which is always a highlight for our organization. Registration packets have been mailed and posted on CRN’s website for online registration and to make hotel reservations.

Families will have the opportunity to connect with others at all stages of the cystinosis journey, to meet and learn from doctors, researchers, and pharmaceutical representatives about all current research and treatments for this lifelong disease.

Please plan to attend. For those who have not had the opportunity to attend a conference before, it can be a life altering experience. A limited number of scholarships are available to assist with expenses. Information about them can be found on the website at: https://cystinosis.org/events/conferences as well as in mailed registration packets.

Over the past year, CRN has also hosted numerous Patient Gatherings at various locations throughout the country, including in Texas, North Carolina, California, and Ohio. This has allowed families to connect regionally with each other, to share their experiences, and to ask questions of experts about anything cystinosis.

CRN has been represented as exhibitors at medical conferences also; most recently the American Academy of Pediatrics (AAP), held in San Diego in Oct. of 2014, and the Pediatric Academic Society conference (PAS) held in April 2015 in San Diego. We feel strongly that connecting with physicians at these...
events provides another opportunity to offer information and educate those attending about cystinosis, and perhaps allow one more child to be diagnosed sooner and treated with the current best practice treatments available.

CRN is always looking for ways to better meet the needs of our community. If you have any ideas, questions or comments that would help us serve the cystinosis community more effectively please contact me at: tschleuder@cystinosis.org.

Steve and Carl Schleuder

CRN is honored to present the debut of a traveling ART exhibit featuring Artists of all ages, from all over the world, who live with Cystinosis every-day at the 2015 CRN Family Conference, in Chicago, Illinois, July 16th–18th.
2015 arrived with great fanfare. We made New Year resolutions and committed to see them through. So much promise and anticipation for a year filled with challenges, growth and success. This is a very busy time of the year for many families, mine included. School years coming to an end, graduations looming ahead, summer holiday plans to make, etc., etc. I can’t help but wonder where did all the time go? One minute you are lugging baby seats to and fro and the next you are visiting colleges to pick the perfect institution for your child. I expect it is not very much different for all of us. As we strive to take care of our children, to create opportunities for growth and to instill core values that will serve them for a lifetime.

One of the resolutions I made for 2015 was to make meaningful contributions toward the success of the Cystinosis Research Network. As I reflect on the growth and maturation of CRN, I cannot help but feel proud of the contributions it has made, is making and the potential to make even greater contributions. These achievements have been a direct result of our individual and collective actions in enabling CRN to move toward achieving its stated 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’s broad scope is challenging but necessary in order to continue to holistically represent the needs of the community with a strong voice. In conjunction with the broad scope and expanding activities comes the need to raise additional funds. This is where we all need to contribute in whatever manner we can. Raising funds can be a lot of fun. Especially when you realize whatever amount is raised is meaningful to the future of your child and the organization.

I think it is important to recognize and bring to your attention the individuals that have committed to lead our efforts in raising funds. The following are the names of your Development Committee:

**Development Committee**

*Chair* – José Morales – jose.morales01@icloud.com
2015 Fundraising Calendar

**Corporate Sponsorship**
Jeff Larimore - JLarimore@arnoldfamilycorp.com
John Maccarone - johnm2maccaroneplumbing.com
Deb Reed - dk_reed@hotmail.com

**Fund Raising**
Rachel DaLomba - rdalomb@my.wgu.edu
Katie Larimore - klarimorie12@sc.rr.com
Tim Miller - Jayhawk.miller@gmail.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 - jenwyman@comcast.net

The following is a listing of events currently schedule for 2015 (as of April 15, 2015):

<table>
<thead>
<tr>
<th>Date</th>
<th>Activity/Event</th>
<th>Honoring</th>
<th>Sponsor</th>
</tr>
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<tbody>
<tr>
<td>February 13th</td>
<td>Steven’s Birthday Letter</td>
<td>Steven Schleuder</td>
<td>Terri Schleuder</td>
</tr>
<tr>
<td>March 17th</td>
<td>Jack’s Birthday Letter</td>
<td>Jack Greeley</td>
<td>Christy Greeley</td>
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<tr>
<td>April 25th</td>
<td>Village Club of Lincolnshire</td>
<td>Jack Greeley</td>
<td>Christy Greeley</td>
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<tr>
<td>April 25th</td>
<td>Costume Crazies 5K Fun Run/Walk</td>
<td>Mason Reed</td>
<td>Deb Reed</td>
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<tr>
<td>May 16th</td>
<td>MayHem! Music Festival</td>
<td>Sierra Ayers-Mutchler</td>
<td>Ayers &amp; Mutchler family</td>
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<tr>
<td>May 3rd</td>
<td>Kacy’s 5K Fun Run/Walk for Cystinosis</td>
<td>Kacy Wyman</td>
<td>Jen Wyman</td>
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<tr>
<td>June 7th</td>
<td>C.H. Robinson Cystinosis Charity Golf Tournament</td>
<td>Lola Long</td>
<td>Tim Miller</td>
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<td>June 22nd</td>
<td>Sarah’s Birthday Letter</td>
<td>Sarah Larimore</td>
<td>Jeff &amp; Katie Larimore</td>
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<td>August 1st</td>
<td>Strike Out Cystinosis</td>
<td>Victor Gardner</td>
<td>Ina Gardner</td>
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<tr>
<td>September TBD</td>
<td>Games of Fun for Cystinosis</td>
<td>Angie &amp; Nelly Polanco</td>
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<tr>
<td>November 10th</td>
<td>18th Birthday Fundraiser</td>
<td>Anjie &amp; Nelly Polanco</td>
<td>Rachel DaLomba</td>
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<tr>
<td>November TBD</td>
<td>Run in Honor of Livia and Mason Stilke</td>
<td>Livia &amp; Mason Stilke</td>
<td>Joy Parket &amp; April Corrigan</td>
</tr>
<tr>
<td>December 5th</td>
<td>Chandler’s Chance…A Christmas Palooza for Cystinosis</td>
<td>Chandler Moore</td>
<td>Clinton &amp; Annie Moore</td>
</tr>
</tbody>
</table>

Please take time to note the families and individuals that are sponsoring these events and, if given the opportunity, acknowledge them for answering the call to serve. You will find a number of our events highlighted in this edition of our newsletter so please take time to read the articles closely.

We continue to make progress in growing the number of events and people sponsoring fundraising events. Please give serious consideration to becoming involved in some capacity with our

Continued on page 36
Keith and Billy Croce Memorial Scholarship

The Keith and Billy Croce Memorial Scholarship has been established with the Cystinosis Research Network (CRN) to provide financial assistance to families affected by cystinosis. The funds will help with travel expenses for those who wish to attend CRN Family Conferences. The conferences are held at different locations every two years and draw families worldwide. The event allows families to share knowledge, offer support to one another, learn more about new research findings, receive updates from cystinosis organizations from around the world, meet and renew friendships with other families, have the opportunity to participate in research studies, and interact one-on-one with many of the world’s expert clinicians treating and researching cystinosis.

Donations can be sent to:
Cystinosis Research Network
302 Whytegate Court
Lake Forest, IL 60045 USA
https://cystinosis.org/how-to-help/donate
Checks payable to CRN—Keith and Billy Croce Memorial

Development Update cont. from page 35

fundraising efforts. The Development Committee is available to assist you in thinking through whatever fundraising activity you are most comfortable with in sponsoring.

How can we expect others to advocate for us if we do not take a proactive stance ourselves. Please reach out to one of committee members or myself, jose.morales01@icloud.com / 203 722-9292, to become involved.

Alexandra Morales, (left)
Schleuder Letter Campaign raises $6,600 for CRN

By Terri Schleuder

This is the fourth year we have written a letter asking friends and family to help us financially support CRN by honoring Steve’s February 13th birthday and Rare Disease day, celebrated February 28th this year. Each year we have been blown away by the love and support shown to our family by supporting this cause so dear to us. This year was no different. As of this writing about $6,600 has been donated with checks continuing to come in.

Thank you from the bottom of our hearts for your generous financial support that has allowed this effort to raise nearly $31,000 over the past four years.

Our journey with cystinosis is currently 27 years long. There have certainly been ups and downs, joys and many challenges as I’m sure there will be going forward. I have always believed out of every tough situation in life there is good and beauty to be found. For us the love, prayers and support of so many people, in so many ways, over the years have helped us feel the good, to know there are lessons to be learned that only difficult situations can teach. Steve’s life and diagnosis was the fork in our road that changed our planned trip to “Paris” to “Holland”. A different world yes, but still beautiful, full of opportunities and wonderful people we would have never met in “Paris”.

Thank you so much for helping with this cause that has been a lifeline for us and others who found themselves unexpectedly in “Holland” too.
The Three Amigos

By Henry Brehm

(Editors note: We received this amazing article in late December, too late to include in the last newsletter, but were so touched by it we are including it now. Enjoy!)

Amigo is “friend” in Spanish as người bạn in Vietnamese. Drs. Elisabeth Hodson, a retired pathologist, and Rick Kaskel, a pediatric nephrologist from New York, traveled as volunteers to Vietnam this past summer on a special mission. Their very busy agenda included patient education for families dealing with Nephrotic Syndrome – related conditions and CME training for docs and nurses. Organized by the Australian NGO, Caring and Living As Neighbors (CLAN) in collaboration with The NephCure International Foundation, I saved this story for the holiday season because this is the time of the year when we reflect on those people who impacted our lives and the lives of others.

Elisabeth came out of retirement to lead. Luan, a native Vietnamese, had not returned in 25 years, shared his pathology expertise and reunited with his sister, and Rick Kaskel, one of my favorite people in the whole world, and always the first to volunteer to help patients. The Three Amigos are a reflection of the many nephrologists around the world who give their time to help others unable to pay for medical care. These nephrologists’ who treat our family members, also have families, soccer games and dance classes; yet they give their Saturdays and in this case, traveled thousands of miles, devoted preparation time and three weeks of non-stop teaching, sharing and learning. I was privileged to help organize and accompany The Three Amigos on this journey and kudos to CLAN and NephCure for their support. There are many wonderful caring docs who have devoted their lifework to improving the lives of children and adults enduring chronic kidney disease. Drs. Holzman, Smoyer, Kamil, Fornoni, Trachtman, Gipson, Huber, Greenbaum, Saleem, Minor, Bagga, Benzing, Zaritski, Reiser, Salusky, Skorecki, Shaw, Kashtan, Cochat, Lemley, Kopp, Johnson, Pollak, Falk, Pearlman, and Kaplan to name a few, yet not enough space to mention all. I salute your efforts; your dedication and your passion.

In Vietnam, 18 CME programs for 200 plus docs were held at hospitals in Hue, Ho Chi Minh City, Hanoi and the Thai Binh Province. The Three Amigos spent additional time with individual docs and visiting patients. At one hospital there were 7,000 patients waiting outside to be seen in the outpatient clinics. There is a shortage of nurses...
and hospital staff, so while the nurses work closely with patients, family members are responsible for their care.

There were six Patient Educational Programs at the different hospitals with participation over 600 with a waiting list. Many families traveled over 100 miles by bus to attend. The Three Amigos, supported by the local docs, presented, answered questions and tirelessly spent every waking moment sharing their expertise and experiences. There were questions about the different medicines—many not available in Vietnam, diet and genetics.

Due to the devastation of the war, Vietnam remains a very young country with 28% of its population under 18. Since 1979, the country has grown from 52 to 86 million, leaving this young population in need of economic structure and support. Although many organizations focus on supporting disparities of malnutrition and infectious disease control, there is a growing incidence rate of non-communicable kidney diseases prevalent in Vietnam. I can still visualize the drying laundry hanging out of windows; straw mats are set up in the outside walk ways so that a patient mom, dad or grandparent can be there for around the clock care of their sick child on dialysis. I will never forget seeing the children in pain; nor will I forget the smiles on their faces that accompanied a visit or a first bump. Most of all I will never forget the impact My Three Amigos or My Three Người bạn had on patients, families, doctors’ nurses and me.

As I reflect upon the past year, I wish you and your family a Happy Holiday Season. The holidays are about gathering together with family and friends. It is also a time of reflection. We learned that the word “impossible” is non-existent in Vietnam, but rather the common word in the medical community is “possible”. The docs and nurses are committed to doing their very best with limited resources while exceeding boundaries of what others would consider “possible”. The leadership, support and training provided by The Three Amigos will have a lasting impact which grows far beyond “HOPE”. They made a significant difference for many patients, families, doctors and nurses in Vietnam. Their willingness to share; their compassion and motivation sets the standard for the rest of us. The New Year will hold many new challenges and opportunities for all of us which will include...
Some of over the 200 doctors visiting Hue, Ho Chi Minh City and Hanoi.

Dr. Rick Kaskel and Dr. Luan Truong seein patients during their trip to Vietnam.
Dr. Nesterova Presents Clinical Update on Cystinosis in Charlotte, North Carolina

By Jeff Larimore

The Cystinosis Research Network held its 8th Dinner and Discussion on Cystinosis on March 7, 2015 in Charlotte, NC. Dr. Galina Nesterova, Staff Clinician-Biochemical Genetics and Pediatrics at the National Human Genome Research Institute located at the National Institutes of Health, was the featured speaker.

Dr. Nesterova provided her medical management advice with parents and caregivers for the six families with individuals managing Cystinosis that were under the age of 10 and provided insight and outlook for two adults with Cystinosis that were in their thirties. The common message from Dr. Nesterova was to be consistent with medication compliance, pursue the best metabolic results that can be achieved when levels are tested and to utilize the resources at the NIH and the Cystinosis Research Network website when questions arise.

Eight families were in attendance and travelled from as far as Virginia to Georgia to participate. Many of the families had communicated in social media circles but the Dinner & Discussion event was the first opportunity for a personal introduction. Families were also able to converse with representatives from Raptor Pharmaceuticals and Sigma Tau Pharmaceuticals about recent progress and availability of treatments.

Feedback from the attendees was that the CRN discussion series are providing a casual and open forum for communication about a major issue in everyone’s household. It was expressed that the continuous opportunity to talk with medical professionals and other families provides insight and guidance to improve the lives of individuals with Cystinosis.
CRN Patient Forum – Houston, Texas, December 4, 2014

By José Morales

The Cystinosis Research Network’s Patient Forum program is in its second year of existence. The program is designed to provide families from our community opportunities to meet with leading researchers and physicians specializing in Cystinosis and network with other members of the community.

The meeting in Houston, Texas was a resounding success. Dr. Ewa Elenburg, Assistant Professor, Renal Section, Department of Pediatrics Baylor College of Medicine, Texas Children’s Hospital provided a thorough overview of cystinosis, framing the symptoms and treatments by stages of development.

Our venue Churrascos River Oaks, provided an intimate setting for our dinner and meeting. There were twenty-six patients, parents, grandparents and family members attending. In addition, representatives from Raptor Pharmaceuticals, Sean Wiltse and Karen Waggenbrenner, and Sigma Tau Pharmaceuticals, Kristine David, were on hand to answer any drug specific questions from the group. It was a wonderful evening of camaraderie and fellowship.
7 Patient Forums events are targeted for 2015:
March 7, 2015 – Charlotte, North Carolina
May 9, 2015 – Columbus, Ohio

In progress of scheduling:
San Francisco, California
Orlando or Tampa, Florida
Denver, Colorado
Boston, Massachusetts

Please take advantage of participating in these events as they provide excellent opportunities to increase your insights and establish strong relationships with other families. Please visit the CRN website, https://cystinosis.org, for event specific details.

Samantha Hernandez and son at the Houston CRN Patient Forum

Attendees gather for a group photograph at the recent CRN Patient Forum in Houston.

José Morales, Dr. Ewa Elenburg, Kristine David, from Sigma Tau, and Sean Wiltse from Raptor Pharmaceuticals enjoy the evening in Houston at the CRN Patient forum recently held there.
“Chandler’s Chance” Fundraiser for CRN Honoring Chandler Moore

By Clinton Moore

This year's "Chandler's Chance" fundraiser was slightly less successful as last year's, and for one simple reason....Heavy rain. The week before the event we were watching weather predictions very closely and found that we better prepare for the worst. Many friends and neighbors donated small canopies and tents and we ended up renting a huge event tent that would accommodate 300 people. All we could do now was hope.

Literally one hour before our event the rain began and in a heavy manner. 160 people still braved the weather and came out to support the cause. The children still were smiling as they boarded the hayride and were soaked when they returned minutes later. Children and adults stood under small tents awaiting their turn riding the firetruck and the line to sit on Santa's lap was lengthy all night.

Overall, everyone had a good time and no one went home dry!!!
Your Raptor Patient Access Manager (PAM) Dedicated to You.
Helping you connect with services for nephropathic cystinosis

Here are a few ways your dedicated PAM can help:

Insurance*

Travel & Financial Assistance*

Your Link to the Cystinosis Community

How else can your PAM help you?
Get in touch now:
1-844-830-CARE (2273)

For more information, visit:
www.knowcystinosis.com

*Assistance may include the cost of transportation, lodging, and/or meals. Services provided are for eligible and/or qualified patients only. Please contact your PAM to determine eligibility.
Cystinosis Update from Egypt

By Neveen Soliman, M.D.

The Egyptian Group for Orphan Renal Diseases (EGORD) is committed to educating physicians and other healthcare professionals about rare diseases including cystinosis to promote early diagnosis and optimal treatment. We are also determined to further promote public awareness and provide creative opportunities for patients and families to share information.

In that context two key events were held in 2015:

The theme for Rare Disease Day Egypt 2015, was “Let’s Get Together” for rare kidney diseases including cystinosis, being a treatable disease. EGORD is thankful to the critical support of the Monira Children Hospital, Kasr Al Ainy School of Medicine, Cairo University, Global Kidney Academy, NGOs, and the Forsan team of Faculty of Fine Arts for an amazing and dynamic program.

http://www.rarediseaseday.org/country/eg/egypt

Highlights from Egypt!

Rare Disease Day 2015, held in February and the Inherited Kidney Disease Workshop (IKDW3) held in April, in Cairo. Both events included topics on cystinosis.

In addition, the 3rd Inherited Kidney Diseases Workshop (IKDW3) was held in collaboration with ERA-EDTA and the Egyptian Society of Pediatric Nephrology & Transplantation. In this unique regional educational event attended by national and regional physicians, pediatricians, geneticists, and nephrologists; two sessions were dedicated to cystinosis. The cystinosis professional session discussed the recent research studies in the region including some interesting case reports and highlighted the need to develop and implement KDIGO guidelines for nephropathic cystinosis. The other session was for patients and families living with cystinosis to meet the experts, get together with parallel children entertainment. Much appreciation to Prof. Pierre Cochat,
Prof. Elena Levchenko, and Prof. Meguid El Nahas for their enormous contribution to IKDW3, in particular the interactive cystinosis patient/family session.

http://www.ndt-educational.org/page-23918-0-921--the3rdinheritedkidneydiseasesworkshop.php

More Highlights from Egypt!
Costume Crazies 5 K Fun Run Honoring Mason Reed a Great Success!

By Deb Reed

Saturday morning, April 25th, 2015, the 3rd Annual Costume Crazies 5K Fun Run/Walk was held at the Oldham County Courthouse Square on “Old Route 66” in Vega, Texas. This special day is to honor Mason Reed and Cystinosis Research Network.

This year we were happy to welcome José Morales, CRN VP of Development, and Mary Patterson with Raptor Pharmaceuticals. José and Mary ran the 5K together along with 50 other participants.

The Court House Square was filled with all kinds of crazy and creative costumes, socks, hats, and funny wigs. The 5K began at 11:00 am.

Medals were given for 1st, 2nd, 3rd place for 2 divisions, along with Best Costume prizes for first and second place.

After the race the participants enjoyed drawings of many gift items donated by family and friends and businesses. We also added a silent auction and received several great items. “Clowns under Construction” a group of ladies who entertain with face painting and balloon art brought joy to the young and old alike. There was a bouncer for the children’s entertainment. A lunch of sausage wraps, hot dogs, chips, drinks and homemade desserts was served in a plastic tote.

This event is all about “FAMILY”. The Reed and Broce Families are so proud to bring the communities together for a day to celebrate “Mason and Cystinosis “. Thank you to everyone who participated and helped to make our fundraiser a wonderful success.
Highlights from Vega, Texas and the 3rd Annual Costume Crazies 5-K Fun Run/Walk fundraiser in honor of Mason Reed!
A wonderful day for all!
9th Annual 5K Fun/Run in Honor of Kacy Wyman Raises over $30,000 for CRN

By Jen Wyman

The first Sunday in May has become our most cherished day of the year. It's different from holidays and birthdays; more like all of them rolled into one. It brings our immediate and extended families together. And it brings our near and far friend families and our cystinosis family together. It's a celebration of a life well lived after a devastating diagnosis. It's a celebration of milestones passed and those that are to come. Kacy loves the day. It's different now that she is older. She recognizes the support and feels the love. In a weird way it helps her embrace her life with cystinosis. Cystinosis is a rare disease, but in many ways we are all the richer for it. It has forced a small community to come together, to depend on each other for emotional support, and to lean on each other in times of triumph and despair. It's a complicated life, but it's a good life.

On Sunday, May 3, 2015 over 200 gathered to walk/run on a gorgeous, warm and perfect spring morning. It's a busy time of year...spring sports, graduations, and weddings abound. But somehow people still find the time to join us in body or in spirit. Never in the year do we get more facebook messages, voicemails and kind notes than on this day. And with the help of many we raised over $30,000 for CRN.

"Hope is the thing with feathers that perches in the soul - and sings the tunes without the words - and never stops at all."

-Emily Dickenson

Kacy Wyman, Steve Schleuder and recently diagnosed, Gavin Gustafson know what it is like to live with cystinosis everyday. They, and others living their lives with cystinosis are our heroes and our inspiration.
Kacy’s friends Hannah Tilds (left) and Amelia McCourt (right) help her hand out Team Kacy Tee-shirts to all who attended the Fun/Run.

Kacy Wyman with her little friend Josie Joy, enjoy the beautiful day!

Kacy’s next chapter of living with cystinosis begins in May.

Tim Wyman, Kacy’s, dad fires up the crowd cheering on the participants as the Fun/Run begins. Over 200 people participated on a beautiful, spring Sunday morning.
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Miller, Joshua
Miller, Syndee and Lawrence
Mitchell, Lani and Ryan
Mitchell, Ralph J. Jr.
Moore, Clint
Morellim Rocco & Vicki
Moseley, Anne Scholl
Muscarello, Kevin
Muth, Michael & Julie Harms
Neff, Suzann M.
Nelson, Richard D. & Kristin W.
Newcomb, Susan and James
Newkirk, Bonnie & David W.
Newlin, Kelly
Nichols, Steven and Susan
Nieves, Lina
O'Brien, James & Michala
O'Keefe, Marla & Daniel
Parker, Dawn
Paschel, Katherine & Anthony
Paulson, Andrew and Catherine
Pawlukiewicz, Alexander
Payne, John P. & Elaine P Cook
Perazzo, Lauren R.
Petro, Eric and Mary
Piana, Sonja M. Earles
Pilder, Stephen H. & Susan D.
Pingel, Steve
Pizzaia, Jessica F. & Richard S.
Pletcher, R.G.
Plunkett, Amy
Polcyn, Nancy A. & David
Puig, Margaret J.
Raia, Vincent J. & Eileen E.
Reid, John and MaryAnn
Reilly, Lynn
Reinart, Kyle
Reinoehl, Paul and Barbara
Reus, William F. & Sherry L.
Rich, Thomas F. & Frances M.
Richardson, Charlene
Richardson, Tracy & Kelley
Richerson, Gertrude
Ripberger, Cynthia
Roatis, Calin V. & Florentina
Rossicone, Paul & Martha
Saks Mimi
Sandman, Steven & Beth
Sauer, Anita
Schechter, Jodi
Schechtman, Julie B.
Schlacter, Guy
Schmitke, Kurt A. & Michele R.
Schmitt-Peterson
Schnuer, Howard and Linda
Schueler, Kevin & Ami
Schultz. Greg & Rogers, Megan V.
Scott, Allison
Self, Micheal & Kelli
Serna, Sunshine
Shalkowski, Mary A. & Thomas F.
Sharp-Price, Karen
Shaw, Gary & Kathleen
José Morales (left) and Christy Greeley (right) advocating for Rare Diseases on Capitol Hill.
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
Join the Cystinosis Research Network Today!

<table>
<thead>
<tr>
<th>Category</th>
<th>Fee</th>
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<tbody>
<tr>
<td>Immediate Family</td>
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<tr>
<td>Extended Family / Friend</td>
<td>$25.00</td>
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<td>$35.00</td>
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</tbody>
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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:
_____Friend
_____Individual with Cystinosis
_____Parent of Child with Cystinosis
_____Professional
_____Family
_____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, Walmart, Ann Taylor Loft, Chicos, 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
collection through the United Way Donor Choice Program.

<table>
<thead>
<tr>
<th>Agency Name</th>
<th>The Cystinosis Research Network, Inc.</th>
</tr>
</thead>
<tbody>
<tr>
<td>Non-Profit Tax ID #</td>
<td>04-3323789</td>
</tr>
<tr>
<td>Address</td>
<td>302 Whytegate Ct., Lake Forest, IL 60045</td>
</tr>
<tr>
<td>Telephone</td>
<td>1-866-276-3669 (toll free), 1-847-735-0471</td>
</tr>
<tr>
<td>Fax</td>
<td>847-235-2773</td>
</tr>
<tr>
<td>E-mail Address</td>
<td><a href="mailto:CRN@cystinosis.org">CRN@cystinosis.org</a></td>
</tr>
<tr>
<td>Web Page</td>
<td><a href="http://www.cystinosis.org">www.cystinosis.org</a></td>
</tr>
</tbody>
</table>

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 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 adulthood.

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.