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2015 CRN Conference set for Chicago

The Cystinosis Research Network is pleased to announce that its 2015 Family Conference will be held July 16 – 18 at the DoubleTree by Hilton Chicago-Magnificent Mile.

Please plan to join us to reconnect and 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 expert clinicians treating and researching cystinosis today.

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A Gift of Life, A Gift of Love

by Jessica DeDio and Mary Jordan

This is the story of two friends with Cystinosis, their families, and one extraordinary gift. The only way to truly understand the incredible nature of this gift is to understand the story from both sides. The first

perspective is from the family of Bailey, a cystinotic teenager in renal failure. It includes **their plan for Bailey's eventual need for a kidney, his mother Jessica's frantic search for a kidney for him when none of his family could donate because of issues with their own kidneys, and the gift that came from another family who had just gone through the same experience.** The second perspective is that of the family of Joseph, another teenager with cystinosis. Joseph had just received a lifesaving kidney transplant. This story is about the **study that brought the boys together, Joseph's family's plan to address his need for a kidney, the unexpected twist that changed that plan, and how his mother Mary's preparations to save her son's life enabled her to save Bailey's life.**



Joey Jordan and Bailey DeDio

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Message from the President Jeff Larimore

Charting the Path of Achievement for CRN – Who will take the Lead? A President's Vision by Jeff Larimore

Desire, Commitment & Energy are just a few of the fundamentals needed in any public or private organization. The Cystinosis Research Network evolved when a group of parents got together out of the need to help their children. They had a strong belief and a commitment to each other to find better answers. With the goal to develop a strong patient advocacy organization, their involvement in the Cystinosis Research Network on occasion came ahead of the priorities of their own families. All of these efforts were completely on a volunteer basis.

A not-for-profit organization realizes that the value provided by long-term volunteers is difficult to measure. Most importantly, long-term volunteers become the ambassadors and recruiters for an organization. Therefore, in order to chart the path of achievement for CRN's future, a new era needs to begin where the mission, vision and beliefs of the Cystinosis Research Network are delivered by patients with Cystinosis under the mentorship of long-term volunteers. My aspiration is that the execution of ideas is not from parents and caregivers but by the swell of new volunteers who have come together to once again find better answers. Better answers to questions that at one time not long ago may have been considered unthinkable.

What is my commitment and action plan to encourage individuals with Cystinosis to increase their volunteer time with the Cystinosis Research Network?

First, listen for the primary areas of interest – what is the right role that will have a new volunteer excited to get started? Our goal is match needs and skill sets but to be also open to a request for new challenges. We want volunteer time to be important and have the experience lead to a degree or better employment.

Second, provide a commitment that our current Board and committee members will support their new roles. We do not want a feeling that new volunteers are being thrown into the water without a life preserver. Also, we want to provide opportunity to receive feedback as new members can provide new perspectives.

Third, many volunteer their time to an organization mainly to serve, but our goal is to enhance friendships and social interactions in order for new members to lock in their decision to stay with CRN for a number of years. We can secure financial resources where committees can gather to hold workshops in order to build peer relationships.

Fourth, constantly extend appreciation that giving time and energy to the Cystinosis Research Network is valued. It is my responsibility as the current President for CRN to express no matter the occasion that our members and volunteers are held in high esteem.



I am extending a personal request to those individuals with Cystinosis. Become active in the Cystinosis Research Network today and let's continue to energize the Cystinosis community together.

With gratitude,
Jeff Larimore

Cystinosis and Rare Disease Community Supports Raptor at Center for Medicaid

By Jeff Larimore



Members of the Cystinosis community were invited by Raptor Pharmaceuticals to participate in a discussion on a request for restoration of the Orphan Drug Exemption under the Affordable Care Act on April 11. The 75-minute meeting was held at the **Center for Medicaid and CHIP Services ("CMCS")** office within the Department of Health & Human Services in Baltimore.

A presentation was made to Barbara Edwards, Director of Disabled and Elderly Health Programs Group within CMCS and her staff that the Orphan Drug Exemption was inadvertently repealed by Congress during the first reconciliation of the Affordable Care Act. The implied intent of the exemption repeal was to lessen the amount of Medicaid reimbursement that would be made to pharmaceutical companies for new drug treatments that contained similar compound material to the drug it replaced.

CRN President Jeff Larimore joined Raptor's Patrick Reichenberger, Valerie Hotz with the Cystinosis Foundation, Diane Dorman from NORD, Patricia Beggato from Global Genes, Jay Greissing, Paul Kim and Dr. Patrick Lee for a conversation that concentrated on the sharing of personal stories of how recent FDA-approved medications including Procysbi have improved quality of life and health conditions of children and adult patients with Cystinosis and other medical conditions.

CRN President, Jeff Larimore at a meeting held April 11th in Baltimore, Maryland at "CMCS" office.

(Continued on page 25)

A Gift of Life, A Gift of Love (continued from page 1)

Jessica's Story

Bailey was diagnosed with a rare metabolic disease called Cystinosis on Friday August 13, 1999 at just 18 months old. That was the worst day of my life. I was told my baby boy would not live past his 10th birthday. I was told my son would not be healthy, that

he would have to take many medications every 6 hours throughout the day, even in the middle of the night and eye drops every waking hour. I was also told, the cysteine would build up all over Bailey's precious body destroying his eyes, muscles, bone marrow and vital organs and that he would need a kidney transplant soon. All that horrible information was too much to take in. Writing this letter, remembering what we have been through and how I felt, is the most unbearable pain I have ever experienced, and brings tears to my eyes just thinking and remembering it.

In 2009 we were invited to participate in a research study down in San Diego, California, which needed patients to help get a very special medication FDA approved. I was ecstatic to be invited!! It was a dream!!! The medication that was being studied

would allow our family to sleep through the night! It would allow us to do meds every 12 hours verses every 6 hours. We stayed at the hospital in San Diego for one week and returned home praying this medication would be FDA approved soon.

In 2010 we received a call that forever has changed our lives in more ways than one can imagine. We were invited to take part in a Raptor pharmaceutical study at **Lucile Packard Children's Hospital in Palo Alto, CA.** We were honored to be a part of this!!! It was a dream, a wish, a prayer answered. In the beginning it felt like we lived/ moved to Palo Alto; we were flying up there weekly sometimes more than one time per week. Constant blood draws, tests and **doctor's appointments.** I will never forget our first visit when Bailey met Joey Jordan, another child with Cystinosis. They were so cute. They looked like brothers- maybe even twins. They had a lot of the same interests and got along great!!! I was overwhelmed with joy. Bailey really never



Joey Jordan and Bailey DeDio



Mary Jordan

opened up or talked to anybody his age with Cystinosis. It was great they both had a lot of fun together even though we were spending more time at the hospital than they would have liked. Every trip the study coordinator made sure the boys were together. They grew closer and closer. We would all hang out and have lunch, dinner and appointments together. **During our 4 years of visits to Lucile Packard Children's Hospital in Palo Alto, California, for the study, Joey's kidneys started to fail he was getting weaker and sicker. It was heartbreaking to watch. His beautiful mother Mary began the transplant process at Lucile Packard Children's Hospital. She planned to donate to her son. All Mary's test came out positive for her to be Joey's donor. While waiting for the live donor kidney transplant to be scheduled, the Jordans received a call from the hospital with a cadaver kidney that was a better antigen match than Mary's kidney. Wow, what a miracle. Joey was transplanted on April 2nd, 2012.**

About 1 year later Bailey's kidneys began failing quickly. He was becoming sicker and weaker by the day. I started the process of figuring out if I was a match on my own since Bailey's doctor said it was too soon for me to check. The doctor said he won't need a kidney for a while, but I knew better. I received an awesome tip from Dr. Grimm (the study Director). He told me to go to my primary care physician and ask for a referral for a kidney ultrasound to make sure I had 2 kidneys and get my blood drawn to find out my blood type. We checked Bailey's blood Type too. Everything was great, our blood matched O-positive, and I had two kidneys. I was ecstatic. I was positive that I would be the one to save my son's life and give him life twice.

Donating my kidney to Bailey had become my main focus, since the day I found out Bailey had cystinosis and he would need a lifesaving kidney transplant. Bailey is my life, my love. He blessed my life and changed my life for the better. When he was diagnosed I quit smoking and didn't eat sweets or salt. I stopped doing everything I knew how to, that would negatively affect my health, and would not allow me to donate my kidney to my precious Bailey. In July 2014, after 5 days of driving to UCLA and extensive testing on me to see if I was healthy enough to save my sons life, I received the worst phone call. The head surgeon from UCLA called me because they knew how devastating this would be for me. He said I'm sorry, but you cannot donate your kidney to Bailey or anyone for that matter you have medullary sponge kidney. I started crying, our life was shattering all over again. I thought, who was going to donate, who was going to save my son's life ??? I didn't want to believe UCLA so I sent my results to Lucile Packard Children's Hospital as well and they agreed with UCLA. I could NOT ever donate a kidney!!! My husband tested, but UCLA denied him as well due to severe kidney stones. Some family and friends filled out the forms and turned them in to UCLA but never made it past that point. We were running out of options and Bailey was getting sicker. Dialysis was going to have to happen even though that was Bailey's biggest fear. We ran out of time, Bailey began life saving dialysis in December 2013. We could not avoid it.



Mary Jordan visits Bailey after his transplant and her donor surgery.

Meanwhile my best friend, Tanya Chilcott, wanted to donate her kidney to Bailey, but she had a different blood type which was not compatible with his. **She started a 'Bailey Believes' donate life page and posted up all of Bailey's information on Face Book in regards to our desperate search for a kidney. We lived and breathed finding a kidney for Bailey, while also trying to keep Bailey positive and healthy at the same time. Meanwhile, at one of our trips to UCLA for a blood draw for Bailey to get him listed "live" on the UNOs for a cadaver kidney, Tanya posted a picture and a description about me not being able to donate to**

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A Gift of Life, A gift of Love (continued from page 5)

Bailey and Bailey's blood type. **While sitting in the UCLA's lab I received the call that has changed our lives forever....My prayers were answered, our Angel Mary called me and said "Jessica I have O + blood..." and I said, "really?"** Mary proceed to tell me that **Lucile Packard Children's Hospital had already deemed her medically healthy to donate** a kidney and from that day Mary said it's a go! I was in shock and overwhelmed with joy... Honestly I didn't know what to say, but thank you, and I love you! Months later UCLA medically cleared Mary to donate to Bailey. On January 14, Mary Jordan gave our family the best gift ever, the gift of life. Mary Jordan freed Bailey from dialysis and lying in bed 20 hours per day depressed and tired. Looking back, this has been very painful, traumatizing, and scary, but so beautiful at the same time. We have been honored and blessed to meet our ANGEL/ HERO Mary Jordan. Mary Jordan is the most beautiful amazing woman. While visiting Bailey after surgery in the OR, Mary who was two curtains down from Bailey and in her own pain, smiled and asked me, **"how is Bailey?"** Then later on that day while visiting Mary in her room I noticed her

hand was swollen from the IV and she was dry heaving. Yet with all of this going on, she asked me with the most perfect beautiful smile, **"How is Bailey?"** I could see a glow in Mary's face. She was beautiful, just like a perfect angel.

I still have a hard time finding the perfect words to express our gratitude to our Angel/Hero, Mary, and her family. The Jordans are an amazing, wonderful and loving family. I feel so blessed to have met Mary and her family. We have been privileged to spend those years in the study with them. I also want to thank everyone involved for helping us get in the study, GOD, CRF, Raptor for putting on the study, and for Dr. Grimm and the study coordinators, because without you introducing our family to Mary and

her family we would still be desperately searching to find Bailey a kidney. I feel so blessed to have witnessed such a beautiful miracle. Love Always, Always Believe, Bailey's Mom xoxo



The DeDio family, Bailey, Jay, Stephanie and Ryder.

Mary's Story

Joseph and his twin brother Patrick were born on 7/17/1997 in Santa Cruz, Ca. Our family adopted them at birth, and our family of three became a family of five. It was one of the happiest times of our lives. Almost right away we noticed something was different with Joe. He would have two pound diapers, drink all the time, and projectile vomit. As his birth mother had done drugs, the doctors were not concerned until he stopped growing at age two and Patrick continued to shoot up. Also Pat had been sleeping through the night by age five months and Joe was three and was up every hour or two to drink, change his bedding, and clothing. Something was definitely wrong.

After the birth of our fourth child, Connor, and moving to a new city, a doctor finally listened to us. The road to a diagnosis of Cystinosis started. He was diagnosed at age four and miraculously had never been hospitalized. Though he ate and drank somewhat normally, his diet was strange, as he always wanted salt, drank gallons of water or milk, and always wanted red meat. It was a relief to have a diagnosis and not think we were crazy.

Joe was placed on Cystagon to slow the degeneration caused by Cystinosis, as well as a multitude of other drugs including indomethacin. This drug helped him not to have to go to the bathroom as much and we were **all able to sleep better. We joined the whirlwind of doctor's appointments, medicines, blood tests, etc.** of having a child with Cystinosis.

Cystagon was a life saving drug that caused Joe to vomit often and caused an ulcer in his stomach. When we heard that UCSD was starting a study that released the medication into the intestines instead of the stomach, and that Joe was accepted to participate, we were thrilled. Joe handled the new drug very well and we were disappointed when the first part of the study was over and Joe was back on Cystagon. The stomach issues returned and we waited to hear more. As soon as the study for RP103 became available we enrolled Joe.

The first day of the RP103 study at Lucile Packard Children's Hospital, Joe met Bailey, another young man with Cystinosis who was the same age, looked like him and had many of the same interests. They became close friends and were inseparable when they were together. Everyone who met them thought they were twins. This friendship became very important, as **this was Joe's first friend with Cystinosis. They were able to communicate about the disease, their medications, school, and activities.** They both found someone who understood what it was like to live with Cystinosis. Through the boys, the families got to know each other and were able to talk about having children with Cystinosis, and truly understand the rollercoaster ride that it is. Luckily both Joe and Bailey reacted well to RP103 and having to take the medication every 12 hours instead of 6 allowed the boys as well as both our families to sleep better. As a result they did better in school and were not as tired. Thankfully the doctors **always had our families at Lucile Packard Children's Hospital the same time so the friendship grew for both the boys and our families.**



Joey Jordan and his mom, Mary after his transplant surgery in April 2012.

When Joseph was 14 and a freshman in high school, he went into kidney failure. He never let it stop him and continued going to school as long as possible. Bailey and Joe continued seeing each other at Lucile Packard Children's Hospital and were able to talk about what was going on. Being Joe's mother, although he was adopted, I was being tested to give Joe one of my kidneys. I was a blood match, but there were no genetic markers. If no other kidneys became available, they were going to give him mine although it would not be the best match and Joe would have to take extra medication. Miraculously a cadaver kidney became available that was a blood match as well as having genetic markers the same as Joe. We were lucky enough to pre-empt dialysis. On April 2nd, 2012, Joe received the gift of life. The drug company allowed Joe to stay on RP103 during and after the transplant and we were very grateful. Although Joseph went through 1 1/2 years of various problems with the new kidney, he is doing well today and is a junior in high school. **(continued on page 8)**

After Joe went through his transplant, Bailey started going through kidney failure as well. **Bailey's mother, Jessica, always assumed that she would be able to give her kidney to him,** but due to unforeseen medical issues that was not to be. Although many other people were tested, they were not able to find a match. It was heartbreaking to see what Bailey was going through. As Joe was doing great and I nearing the age where it would be difficult to give a kidney, and I had already had all the tests, I agreed to see if I was a match for Bailey. UCLA was concerned about my age but they agreed to test me. At this point Bailey was on home dialysis almost all day every day. His life was not his own and he was miserable. Miraculously I was not only a blood type match but there were also some genetic markers that matched. The decision was made to go ahead with the transplant. During all of this Joe and Bailey would face time each other or text each other.

The day finally arrived and I flew down to Los Angeles from Sacramento to donate my kidney to Bailey. I was excited and happy. Interestingly I was not afraid as I knew I was doing the right thing and God would take care of Bailey and me. As I had my last test I was able to see Bailey and his family one last time before the transplant. I think everybody cried as Bailey was not doing well physically and needed the kidney ASAP. As my daughter dropped me off the next day at 4:30 AM, I said goodbye and walked **into UCLA knowing that I was giving the gift of life to one of my son's best friends.** I actually smiled the whole time as I was being prepped and waited for the surgery to

begin. On January 14, 2014, my left kidney was given to Bailey. All **went well and Bailey's mother** Jessica later told me that as soon as the kidney was stitched in place he began urinating. That was a great sign.

When I awoke after surgery I was in a great amount of pain but it was **worth it. Jessica, and Jay, Bailey's** parents, came to see me and told me how well things went. It took about one month to recuperate, but it was worth it. I would do it all over again. As a family we are blessed to have Bailey and his family in our family. As of now there is no rejection of my kidney. We look forward to having a long friendship with his family. If it

weren't for the power of friendship between two young men this would not have happened.

I want to thank Joe and Bailey for being the heroes that they are. I also want to say that you only need one kidney to live a normal life. If you cannot be a live donor, **please put the donor sticker on your driver's license.**



The Jordan family, Patrick, Rich, Joey, Mary, Caitlin, and Conner.

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Financial Update

by Jenni Sexstone—Treasurer



Jenni Sexstone and her sister, Heather Vest.

For the 3 months ended March 31, 2014

We are pleased to present the financial results of the Cystinosis Research Network through March 31, 2014. These financials represent the great efforts of all CRN members: patients, parents, family, friends and researchers focused on educating the public and medical communities and improving quality of life of those with Cystinosis.

For the three months ended March 31, 2014, total income secured is \$156,000 and is primarily driven by grants received from Raptor Pharmaceuticals. Fundraising efforts contributed to 20% of total funding. Total donations from public support from individuals and organizations such as United Way, Network for Good and Facebook represented the remaining 9% of funds secured in the first quarter of 2014.

Total expenses incurred for the three months ended March 31, 2014 were \$30,000. Administration, registration and travel costs associated with Education & Awareness amounted to approximately \$1,600 through March 2014 compared with \$11,000 through March 2013. Fundraising expenses incurred were \$10,600 through March 2014 compared to \$600 through March 2013. Finally, administrative expenses totaled \$17,400 compared with \$12,200 for the same period in 2013.

A primary objective of the Cystinosis Research Network is to provide funding for research grants. These programs focus on identifying the underlying effects of Cystinosis and expanding outreach and education domestically and internationally. Total



Josie Sexstone and Sarah Larimore.

grant payments of approximately \$157,000 will be paid throughout the year to the following institutions:

- National Institutes of Health
- University of Sunderland, Sunderland, Scotland
- University of Michigan

Throughout the newsletter will be more detailed information regarding the status and findings of each of the research programs in progress.

A comparable financial recap for Cystinosis Research Network for the three months ended March 31, 2014 and 2013 is:

	Jan - Mar 14	Jan - Mar 13
Total Income	\$ 155,980	\$ 128,630
Total Expense	\$ 29,549	\$ 76,615
Net Operating Income/(Loss)	\$ 126,431	\$ 52,015
Cash on Hand - Jan 1	\$ 95,654	\$ 102,654
Net Change in Cash	\$ 53,786	\$ 44,198
Cash on Hand - March 31	\$ 149,440	\$ 146,837

CRN Announces 2014 Scholarship Application Deadline

It is that time of the year again. From now until the deadline date of Aug. 15, 2014, CRN will be accepting applications for our two, annual \$1,000 Scholarships. The first is an Academic Scholarship for an individual with Cystinosis, and the second is the Sierra Woodward Sibling Scholarship for the sibling of an individual with cystinosis.

Both scholarships are offered to qualified individuals who have been accepted at, or are attending an accredited college, university, or vocational school.

Application information and needed forms can be accessed on CRN's

website at www.cystinosis.org/scholarships.

Completed materials should be mailed to:

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



Kole Binger, the 2013 recipient of the Sierra Woodward Sibling Scholarship.

Chandler's Chance Fundraiser

By Clinton Moore

Attending our first ever cystinosis conference in the summer of 2013 proved to be more than we bargained for. Listening to the many doctors and researchers was incredible, although for us, not the highlight. Meeting so many other people and their families that are also affected with this illness was a turning point. Listening to their stories about challenges, life, worries, and triumphs was at times heart breaking, but also inspiring. Watching my son have the chance to run and play



The Moore family's first fundraiser to support Cystinosis on December 17th, 2013.



Santa, Chandler Moore and friend.

with other children also taking medications and eye drops gave him a sense of normalcy. Watching the tears roll down his face when we got in the car to drive home was also heart breaking, but in all, an experience that will always be remembered.

On the final night of the conference during the dinner/dance, my wife and I had the honor of having a sit down chat with a very kind, but to the point, lady named Katie Larimore. She answered many questions that we had and educated us on various topics. The last question that I had for this wonderful lady was "What can we do to help?" Without hesitation or blinking an eye she quickly responded "Have a fundraiser, we need money."

Over the next few weeks as life returned to normal those words kept repeating in my mind. But what do I know about having a fundraiser? That answer is simple...nothing. As my wife and I discussed

possibilities one evening our son Chandler comes walking in and says "Hey guys, you know we sell hot chocolate every year at Christmas. Why don't we give them that money? Silence fell across the room as my wife and I just stared at him. Hot chocolate night is a night that we have done for a few years already. It's basically just 50 or so friends, family, and neighbors that gather outside, buy a cup of chocolate for 50 cents, enjoy the lights and then it's off to the store for Chandler to buy a toy with his freshly earned



Attendees enjoy the fun activities at Chandler's Chance fundraiser for cystinosis.

money. But now he is telling us to give away his money? Something he looks forward to all year? I asked him if he was sure he wanted to do that. His response... "Why not? They need it more than me." The debate of what to do for a fundraiser was over; now to execute it.

The next few months were basically just preparing, advertising and gathering volunteers. All seemed easy enough in thought. All proved difficult in reality. How can such a small scale fundraiser require so much work? Have we bitten off more than we can chew? Are we paying too much attention to detail? Or is my inexperience just shining through? We will soon find out.

When the ten day forecast finally included December 17th, it did not look good. Rain, heavy at times was being predicted by 3 of 4 different forecast sources. As the 17th drew closer, the forecast looked even more grim. Although we had a rain date scheduled, we had lots of people tell us they could only attend the original date. Then the morning arrived. It's fundraiser day, and it's NOT raining. Since last night the forecast has changed and now they say just cloudy. I guess it was meant to be.

When nighttime arrived all preparations were complete. As our guests arrived, they were greeted by Yukon Cornelius, Buddy the Elf, and many other characters. Hot chocolate, coffee, popcorn, and cookies all served at no charge. The fire truck gave all the kids free rides and the siren could be heard throughout the night. Mr. and Mrs. Santa Claus were kept busy listening to the children's wishes and handing out goodie bags. The live band entertained the older crowd. All while being lit up by thousands upon thousands of Christmas lights and the stars in the sky. We had the pleasure of greeting about 300 guests that night. All of which donated what they could, causing us to exceed our goal. At the end of the night our 18 volunteers helped us clean up, and it was over.



A great time was had by all who attended. THANK YOU!!

Looking back at it, we can see room for improvement. Next year we will make the necessary changes to even expand the event. But overall, Chandler's Chance was a success.



Adults with Cystinosis at the 2013 CRN Family Conference

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

Visit <http://www.cystinosis.org/families-in-your-area>.

Send your contact info to the e-mail listed to learn who is in your area.

Research Update

By Christy Greeley, Executive Director and VP, Research

Grants:

The Cystinosis Research Network's vision is the discovery of improved treatments and ultimately a cure for cystinosis. CRN utilizes a Scientific Review Board, comprised of leading experts on the disease, which reviews grant proposals and submits funding recommendations to CRN. More specifically, the SRB provides independent, objective review and recommendations regarding each research proposal, utilizing grant review guidelines established by CRN in accordance with the mission of the organization. The SRB also provides strategic advice and direction for the organization in terms of research topics to pursue.

A major focus of CRN continues to be a determined effort to secure a promising future for the cystinosis community through the support and funding of research grants, fellowships, and other research based activities that lead to improved treatments, better quality of life, and ultimately a cure for cystinosis. CRN has funded close to \$4 million total in research grants and fellowships. Information on all current and past funded projects can be found on the CRN website under the Research tab, including grant guidelines, updates, and study recruitment information. Current grants include:



Jack, Christy, Dave and Alex Greeley.

Extension of Genotype-Phenotype in Egyptian Patients with Nephropathic Cystinosis. Neveen Soliman, MD and Elena Levtchenko, MD, PhD, Center of Pediatric Nephrology and Transplantation, Cairo University, Egypt and Department of Pediatric Nephrology University Hospitals, Leuven, Belgium. Total Award: \$4,400

Extension of Proteomic Investigation of Cystinotic Cells and the Effects of Cysteine Treatment. Jill Jobson, Noel Carter, Achim Treumann, Ken McGarry and Rosaleen J. Anderson, Sunderland Pharmacy School, University of Sunderland. Total Award: \$55,000

Extension of the Continuation of Feasibility of Cystinosis Replacement Therapy in Cystinosis. Jess G. Thoene, MD, Director, Biochemical Genetics laboratory, Active Profession Emeritus of Pediatrics, University of Michigan. Total Award: \$81,000

National Institutes of Health Cystinosis Fellowship, 2013 – 2015, Galina Nesterova, MD, Mentor, William A. Gahl, MD, PhD. Total Award: \$130,000

As you can see, CRN has funded a variety of clinical and basic research under investigators all over the world. This work has led to many publications and advancements in the understanding of the disease, which can be found on the CRN website under the Research tab in the Article Library, which also houses the majority of the most important cystinosis publications, including the recent clinical and parent guides to cystinosis care published by CRN.

Patient Resources:

Another guide is in the final stages of completion to accompany the existing Cystinosis Parent Handbook, Cystinosis Standards of Care, and Cystinosis Transitioning Guide for Patients and Families – The Transitioning Guide for Physicians will be a handy, one page reference which will be distributed to pediatric and adult care physicians involved in the transfer of care from the pediatric to adult care worlds.

These guides are an invaluable resource which we have distributed widely. We are fortunate to have the advice and experience of our Scientific Review Board, Medical Advisory Committee, and Professional Advisory Committees who are so committed to our community and have generously donated their time and expertise to ensure that this information is well documented and available to families and physicians.

New Advisory Board Members:

CRN is pleased to announce the addition of two physicians to our Medical Advisory Committee, **Dr. Rachel Bishop**, an ophthalmologist at the National Eye Institute/NIH and **Dr. Mihir Thacker**, an orthopedic surgeon at Nemours/Alfred I. duPont Hospital for Children in Wilmington, DE. Dr. Bishop has been involved with the cysteamine eye drop protocol and in the treatment of cystinosis patients at the NIH for some time. Dr. Thacker is an orthopedic surgeon with an interest in the bone disease consequences of renal disease. Both of these physicians fill a long time unmet need on our advisory board. We are grateful for their commitment to the cystinosis community and to the Cystinosis Research Network.

NIH Cystinosis Fellow:

Finally, I would encourage all of you to utilize the expertise of our CRN Cystinosis Fellow at the National Institutes of Health, **Dr. Galina Nesterova**. She has been working closely with Dr. Gahl over the years, treating the cystinosis patients seen under the NIH protocol, participating in CRN Family Conferences, and speaking at professional meetings on cystinosis. Dr. Nesterova has become an expert in cystinosis and is available to consult with any family or health care provider who needs assistance in the diagnosis and treatment of cystinosis. She can be contacted at nesterovag@mail.nih.gov. Please feel free to contact her with any questions or concerns.



Jack and dad, Dave Greeley.

Growth Hormone Study to begin soon at NIH

Dr. Galina Nesterova, CRN Cystinosis Fellow, has obtained approval for a study which will examine the use of growth hormone for muscle wasting in adults with cystinosis. The protocol has been approved and she is awaiting approval of the patient recruitment materials by the NIH. Once these are obtained, we will provide the study information to the community as soon as possible.

Cystinosis Community Celebrates Rare Disease Day through Med Art

(The content below was first posted on NORD's website Blog on March 7, 2014)

By Clinton Moore

I started doing these med pictures for my son Chandler over a year ago. I decided to post a picture of a smiling face on Facebook with hopes of making another child, parent or caregiver smile. I had no idea what was about to follow. Jen Wyman quickly responded with a med picture of her own. And every day since, my feed has been filled with other families showing their art. It has become an inspiration to many and I believe it has given some a positive look at something that can seem very negative. Some have said they see things in in a different light because if this.

My son Chandler was diagnosed with cystinosis when he was 10 months old. He is now 8. He is amazingly healthy considering he has such a rare disease. Aside from his hourly eye drops and 65 pills a day, he lives a very normal and active lifestyle: Full of energy, full of life and full of love. Smiling and laughing all day, he is my biggest inspiration.



By Jen Wyman

Over the last couple of weeks, our community of cystinosis families has been enriched by an exchange of "med-art" that began with a Facebook post by Clinton Moore, a dad of a child with cystinosis. He shaped the morning dose of pills that his son, Chandler, takes into a smiley face. He then posted it to make other families smile. I responded to that post with a happy face of my own for my daughter Kacy, who also has cystinosis. That exchange of posts encouraged more parents to post more pictures, some with



quotes attached. I then turned those photos into a slideshow that all could enjoy. I felt it was something we could share beyond our small community of cystinosis families. It was something that other families could do to encourage each other, make their kids smile and make some fun out of a situation that isn't so fun sometimes.

Med routines can be taxing and monotonous. Making med-art for our kids and sharing it with each other is our way of looking at a part of this disease with new eyes and positivity. We are a small community of families who have children with a rare disease, but we are connected to a bigger community of families with all

different types of rare diseases. How true it is...*"We all came here on different ships, but we're all in the same boat now."* Martin Luther King, Jr.

- See more at: <http://blog.rarediseases.org/cystinosis-community-celebrates-rare-disease-day-through-med-art/#sthash.wxhyr5Tv.dpuf>



CRN's Executive Committee celebrating Rare Disease Day 2014 by participating in NORD's "Handprints Across America". Pictured from left to right, Jose' Morales, Pam Woodward, Christy Greeley, Terri Schleuder, Jeff Larimore, and Jenni Sextone.



"Living with Cystinosis: A Closer Look" Patient Video Now Available

"Living with Cystinosis: A Closer Look". This video is now available at <http://www.youtube.com/watch?v=eT8DB34Ktnk> and offers information about the symptoms, impact and management of the ocular effects of cystinosis. In addition, it provides valuable first-hand insights for people with cystinosis about how to fit the condition into their daily lives. Sigma-Tau Pharmaceuticals partnered with CRN in its creation. It may also be viewed on CRN's website at cystinosis.org.

My Story, Living Life with Cystinosis

By Stephanie Sorensen

My name is Stephanie Sorensen. I was too young to remember anything when I was diagnosed with Cystinosis. I grew up being told stories by my mother on how stressful it was to get me to take my medication. I would always spit it out all over her and knock it out of her hands, because it was the worst thing I have ever tasted in my life.

As I got older I would sometimes take my medication and sometimes I wouldn't. At that time I was in my early teens, and I didn't care what happened to me. I was just really sick of everything I had to do and I didn't feel like a normal kid, no matter how many times I was told by my doctors and mother that if I wanted to live, I had to take my medication. It was a lot of responsibility for me to take on at such a young age, and I didn't want that.

I knew that at some point in my life I would have to have a kidney transplant, but I just didn't care. When it was time for me to go and have my appointments with my nephrologist and have all of my kidney function tests done, I thought everything was going to be fine like it had been previously. Well, it wasn't. In 2001, when I was 14, I was told I needed a kidney transplant. It didn't really sink in until my mother talked to me about it and told me how important my life was and how much she loved me. That was the moment I realized if I wanted to make my mother proud and maybe have the possibility of having a family someday, I needed to grow up.

So, I started being responsible with all of my medications. Once the hospital started doing donor testing they warned me that dialysis was a possibility. My father was tested first. I found it sort of amusing that I wasn't the one to be tested on for once! My father was a match! Luckily, I did not have to get dialysis because my kidney function did not get dangerously low, and within a few months my surgery was scheduled to take place.

In August 2001, I had my kidney transplant. It was the best experience of my life. I met some great people that were going through big events in their lives as well. I also had significant support from my family and friends, which helped my mother and I in our time of need.

I have younger siblings, so there was more than just me that my mother had to worry about. Family and friends are an important part of life. If it weren't for everyone in my life, I wouldn't have the support needed to go through everything I have experienced in my life.



Stephanie and Justin Sorensen with their children Aiden and Lyla.

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Growing up with Cystinosis was very difficult for me. When I was in middle school I would always ask myself, "Why was I born with this? Why did it have to be me?" I have been through more than any person should ever have to go through. Even though I grew up with a big family it was always just my mom and I going to all of my doctor's appointments, medical tests, kidney transplant and everything else. My mother was my biggest supporter and my hero. She always **made sure I was safe and healthy. If it weren't for her, I wouldn't be who I am today.**

I was told by my doctors that I would never be able to have children, because the child could have deformities or could cost me my life, or even the baby's life. So, I gave up on the idea of ever being able to have children. However, in 2007 I found out I was pregnant...I was scared. Still, I wanted to go through the adventure I was about to experience and just hope that everything would be okay.



Stephanie and her husband Justin on their wedding day.



Stephanie with her mom, Cindi and her stepfather, Steve on her wedding day.

I had some complications throughout my pregnancy, but nothing that was too serious. The labor and delivery were horrible. My baby was having complications and so was I. Nobody knew what to expect since it is so rare for someone with Cystinosis have a baby. In September of 2007, after three days of labor, I had a beautiful, healthy baby boy. Luckily my son does not have Cystinosis!

While I was enjoying having a new baby, my mother was diagnosed with Malignant Melanoma and going through chemo. She helped me so much with my son, Aiden. He was her new favorite person. Even on her bad days, she spent time with her new grandson. Aiden and my mother were so close to each other. I have never seen a connection like that before. It was amazing!

In November of 2012, my mother passed away, and it was devastating. I am so thankful for everything she had done for my family and me. After my mother passed away, I found out I was pregnant again. I was ecstatic! I was also nervous to see how this pregnancy would go. I saw the doctors very often once again. This pregnancy was different than the first. I had more complications. This time it was affecting my kidney function levels among other things.

(continued on page 20)

My Story, Living with Cystinosis (continued from page 19)

In June of 2013, the doctors decided to deliver my daughter by c-section four weeks early due to complications. I named my daughter Lyla. When she was born she had some problems, due to being born early and by c-section. Everything turned out good in the end. Lyla and Aiden are our little miracle babies!

I am very grateful for the family I have. Now I am 27 years old, married, and have two amazing children. It has been 13 years since my kidney transplant. The doctors thought I would not live past the age of ten. Well, I have proven all of my doctors wrong in everything I have done in my life!

Cystinosis is not a death sentence anymore. It can be controlled of by taking the right medications at the right times everyday, and by keeping up with regular doctors appointments. Taking care of oneself can lead to living a great and fulfilling life. I am so grateful for all of the support that I have in my life!



Stephanie, Justin, and Lyla at her Baptism celebration.

2015 CRN Conference set for Chicago (continued from page 1)

Located only steps away from the Magnificent Mile and Navy Pier, the DoubleTree by Hilton Chicago Magnificent Mile puts you within reach of the windy city's best dining, shopping and entertainment. Our fabulous location, in the heart of Streeterville, is within minutes of Navy Pier, Chicago Water Tower, John Hancock Building, CTA Red Line, Tribune Tower, Millennium Park and the Lake Michigan. Getting to our location is easy if you are coming from the airport, since we are less than 45 minutes from O'Hare and Midway. For more information on the hotel please visit their website at www.doubletreemagmile.com.



Doubletree Hotel Chicago, site of 2015 CRN Family Conference.

Registration materials will be available in early 2015 on the CRN website and will also be mailed to families. Special room rates have been negotiated with the hotel and will be made available to conference attendees.

We look forward to seeing you in in Chicago in July 2015!

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



Schleuder Letter Campaign raises \$7,400 for CRN

by Terri Schleuder

This year is the third year we've written a letter in February to honor Steve's birthday and Rare Disease Day. This year we are also celebrating his graduation from college, a huge milestone for him and us.

We are again humbled by the incredible generous response of so many friends and family to this cause. With your help as of this writing over \$7,400 has been raised for CRN in 2014, making the three year total over \$24,000! **God Bless you!! And thank you All!**

It would not be possible without everyone's help. We are so grateful for the continued and generous support given to this appeal.

To be alive with cystinosis in the 21st century is to truly understand the power of improved treatments and medications. With continued successful research it is our hope that improved treatments will someday give way to a cure and cystinosis will be no more.

Until then, with your help, we will continue to support CRN's mission to educate and raise awareness about cystinosis, support individuals with cystinosis and their families and fund promising research studies that will provide better treatment options for those living with cystinosis.



Steve Schleuder celebrating Rare Disease Day 2014.

Jack Greeley Birthday Fundraisers Have Raised over \$200,000

By Christy Greeley – Executive Director and VP, Research

The Greeley Family once again celebrated Jack's St. Patrick's Day birthday, 14 this year, by sending out the 12th 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 over \$200,000 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 an excerpt from **this year's letter, which can also be found in its entirety on the CRN website at : <https://cystinosis.org/events/fundraisers>.**



Jack celebrating his 14th Birthday on St. Patrick's Day.

A Christmas Story is an American movie classic, one that the Greeley family enjoys each and every holiday season. One of the many memorable scenes from this film set in 1941 NW Indiana is when nine-year-old Ralphie Parker helps his Dad change a flat tire. He's beaming with pride that his old man would let him assist until he accidentally spills the lug nuts all over the side of the road. Ralphie knew he screwed up and made matters worse when he belted out, "Fudge!" as the bolts flew through the air into the dark night. Only Ralphie did not say "fudge" and met his fate with a bar of Lava soap. Ah, the good old days....

Minus the comedic edge, life imitating art took place recently for the Greeley's. As most of you know, our son, Jack has Cystinosis, a rare metabolic, genetic disease that presented itself around his first birthday in 2001. A side effect for Jack of the kidney disorder aspect of Cystinosis has been the development of rickets and softening of his bones, which has over time caused his legs to become knock-kneed and his ankles rolled in, causing extreme flat feet. Over this past Christmas break, we decided to have orthopedic surgery on his two knees (three staples were put in his left, two in his right) and two ankles (a screw was placed in each one) so that doctors could manipulate his still-open growth plates and have his legs slowly and naturally straighten out.

After Jack came out of surgery, some rehab commenced and he was not in a good place, even being an all-time trooper. With Mom, Dad, and a physical therapist helping him to stand up straight, Jack SCREAMED...the kind of piercing noise that could shatter china. What happened next made little Ralphie look tame as Jack let out a series of F-bombs, followed and alternated by a series of S-bombs that had never left his lips before, certainly not in this machine gun fashion...F-S-F-S-F-S-F-S-F-S. Mind you, Jack is a sweet kid, so the sheer volume and repetition in his language was surprising, impressive, unsettling, funny...take your pick. Given the circumstances of the pain, the morphine drip, and the anesthesia, it was all irrelevant because getting him

better was all that mattered. Never before has Jack slipped liked this with language and what else came out of his mouth was most surprising. For the first time in 13 years since his diagnosis, under faint breath, Jack mumbled some gibberish like "why did he (I) have to have this surgery." It is the closest thing to self-pity that Jack has ever uttered. So, if a kid ever deserved to belt out a few F or S bombs to fight through a challenge, it was Jack...heck, go to the top of the Sears Tower and yell as loud as possible, because he is a Champ and deserves it.

Jack is by far not the only champ out there today. So many people live and thrive with rare diseases like Cystinosis. Yes, Cystinosis has the potential to impact all of the organ systems in the body leading to kidney failure, muscle wasting, diabetes, blindness, pulmonary deficiency, hypothyroidism, and neurological damage, but we do our best to dwell on the positive and can-do spirit of Jack; that's what inspires us, not what might be. We are extremely confident of Jack's bright future and his ability to get up off the mat.

Remember, Jack was born on St. Patrick's Day, so he might get knocked down on occasion, but he is going to get back up...always. On a recent trip to the gym with his Dad, Jack wanted to do some of his rehab exercises and see if he was ready for an elliptical machine. "Dad, let's do five minutes on the elliptical, okay?" Jack asked at the end of his workout. Jack got on and pumped away, legs and arms moving. Well, five minutes became 10 minutes and you could tell the Champ was working hard. Tired, but determined, Jack proclaimed, "Let's do a mile." Around 0.95 miles, Jack panicked and grunted, "Dad, take over for me," so Dave got off his machine and got on Jack's and asked him with some concern, "Jack, what's wrong?" Jack was bent over the back cabinet of the empty elliptical machine next to his, clearly in pain. "Jack, should I help you?" asked Dave again. "NO! Go slow...I'm going to get back on...I need a break...I can't feel my arms and legs. Let (gasp) me know (grunt) when you (gasp) get to .98 (grunt)." As a parent, what do you do? Clearly, Jack wanted to cross the finish line at a mile, so Dave took it to 0.98 and turned it back over to Jack who wobbled back onto the machine. Quietly, under his breath and as he grinded out the last 0.02, Jack said, "You...can...do it. You can...do it. You can do it!" This was pure determination and focus. No morphine drip this time and the F and S bombs were swapped out for another chant, while the fighting spirit remained.

Ironically, the Winter Olympics were on TV while Jack was working over the elliptical machine. His determination in that moment of time was inspiring...not a little, but a lot. It was all guts, it was tough, it was Olympian, and it was Champion. Twenty minutes later in the car ride home, Jack said, "Dad, look. My arms are still shaking." No Gold Medalist that day or in two weeks in Sochi tried harder or got a better result than Jack that afternoon. Jack knows he has Cystinosis, but he does not care, because his normal is normal and he has too much to do, to see, to contribute, and to accomplish. That's our Champ.



Jack Greeley and his sister Alex.

CRN Education & Awareness Update

by Terri Schleuder—Vice President, Education & Awareness



Terri Schleuder, VP of Education and Awareness.

This spring has been welcome more than most after our brutal, never ending winter. It is a time of hope and renewal for our world. As we move forward it is also a time of hope and renewal for our cystinosis community. With the FDA approval of Cystaran and Procysbi we've seen firsthand the quality of life improvements new treatments offer and look forward to the progress of continued research. The recently published handbooks, *The Transition Guide*, *Cystinosis Parent Handbook*, and *Standards of Care in Cystinosis* offer a one-stop resource for parents, patients and physicians wanting to learn more about cystinosis. Over the last year "town hall meetings" held across the country, in Chicago, Missouri, and New York, have provided opportunities to learn, network and connect with each other. Rare Disease Day on February 28th brought international attention to the millions of people living with one of over 7,000 rare diseases.

This year CRN will continue its effort to bring awareness to and educate physicians at two medical conferences, The American Academy Pediatrics (AAP) to be held in San Diego, October 11th to 14th and the American Society of Nephrology (ASN) to be held in Philadelphia, November 11th to 16th.

The early planning stages have already begun for the next CRN Family Conference to be held in Chicago in July 2015. The opportunities to bring together families from all over the world with international cystinosis experts is always a highlight for us.

Through this newsletter we also try to inform and inspire those within the cystinosis community. I am especially proud of our three featured stories in this issue. The DiDeo and Jordan families demonstrate the best part of the human spirit and what love can accomplish. Our two adult stories featuring Barry Beard and Stephanie Sorensen, show what can be accomplished with a never give up attitude. Both of these life stories show clearly that cystinosis does not have the power to limit one's possibilities. I know you will be touched and inspired by these two amazing adults and how they have chosen to live life with cystinosis.



Congratulations to Steve Schleuder, 2014 graduate from Eastern Michigan University!

Nutrition in Cystinosis

CRN is proud to present dietician, Kelly Rulle's research paper entitled, *Dietary Consideration for Infantile Nephropathic Cystinosis*. Kelly is enrolled in a masters program for nutrition at MUIH (Maryland University of Integrative Health) in Laurel, Maryland. The focus of Kelly's research was to create a whole foods dietary approach to aid and complement the treatment of a disease. She selected cystinosis specifically for her research because her cousin has a child with cystinosis.

Her article may be viewed in full on CRN's web site at cystinosis.org. Click on Research, scroll down and click on article library then scroll down to Nutrition.

It may also be viewed by clicking on this link:

<https://cystinosis.org/research/article-library/nutrition>

Cystinosis Standards of Care

The Cystinosis Standards of Care were written to help individuals with infantile Nephropathic cystinosis, their families, and their medical team. The information presented here is intended to add to conversations with physicians and other health care providers. No document can replace individual interactions and advice with respect to treatment.

One of our primary goals is to give affected individuals and their families greater confidence in the future. With early diagnosis and appropriate treatment, there is more hope today for families with cystinosis than ever before. Research has led to better methods of diagnosis and treatment. Knowledge is increasing rapidly by virtue of the open sharing of information throughout the world among families, health professionals, and the research community.

We acknowledge the important contributions to the Standards of Care of Dr. Galina Nesterova and Dr. William Gahl of the National Institutes of Health, and the members of the Cystinosis Research Network's Medical and Scientific Review Boards.

Cystinosis Standards of Care are available on the Cystinosis Research Network Website at:

<https://cystinosis.org/images/what-is/Cystinosis%20Standards%20of%20Care%20June%2019%202012.pdf>

Cystinosis and Rare Disease Community Supports Raptor at Center For Medicaid (continued from page 3)

The common voice from the rare disease community was that the exemption was contrary to the original intent of the Orphan Drug Act of 1983 and that a continuation of the reconciliation amendment would potentially stifle new research and development for rare diseases. Restoration of the Orphan Drug Exemption would provide for the continuation of incentives that drive the development of improved therapeutic options for the rare disease community.

It is unlikely that Congress will amend the Affordable Care Act however the CMCS has the authority to restore the Orphan Drug Exemption. Ms. Edwards and her staff agreed to thoroughly review the presentation material provided along with additional public comments submitted. A statement of corrective action is hopeful by the end of 2014.

Town Hall Cystinosis Information Dinner Held in Bloomfield Hills, Michigan

By Jen Wyman

The Wyman Family hosted a regional dinner in Bloomfield Hills, Michigan on May 3, 2014. The dinner which was sponsored by Raptor Pharmaceuticals had 22 guests in attendance including guest speakers, Dr. Jess Thoene and Dr. Shane Quinonez, from The University of Michigan. Also in attendance was Raptor representative, Vera Toth-Fejel, Sigma Tau representative, Lesli King, CRN representative, Jose Morales and 6 cystinosis patients and their families.



Dr. Jess Thoene and Dr. Shane Quinonez are guest speakers at a Town Hall Dinner and Discussion of Cystinosis in Bloomfield Hills, Michigan with Steve Schleuder, Megan Hampton, Megan Morrill, Kacy Wyman, Shannon Keizer and Jen Gray.



Megan Hampton and her mom Shelia



Dr. Shane Quinonez, and Dr. Jess Thoene with Kacy Wyman

8th Annual 5K Fun/Run in Honor of Kacy Wyman Raises \$33,000 for CRN

By Jen Wyman

On May 4, 2014 Team Kacy rallied around our family once again. 350 participants came out on the sunny, crisp day to celebrate Kacy's life and her journey with cystinosis. It was an extra special event this year with 5 cystinosis families in attendance!! Raptor Pharmaceuticals and Sigma Tau Pharmaceuticals represented as well. Our community stepped up once again and helped us raise over \$33,000 this year, a record amount. Our hearts are full and we are grateful for the support and love the people in our life give us on this day and every single day of the year.



Volunteers help Team Kacy at the 5K Fun/Run.



Wyman family and friends at the event



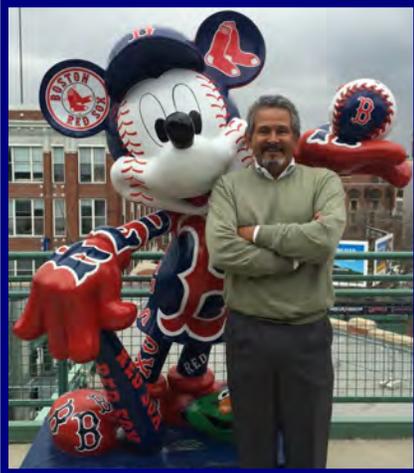
Kacy Wyman, Steve Schleuder and Jack Greeley



Kacy Wyman, Megan Morrill with their moms Jill Morrill and Jen Wyman enjoy the day at the Cystinosis 5K Fun/Run held in Kacy's honor to support CRN.

CRN Development Update

By Jose' Morales—Vice President, Development



Jose' Morales, CRN Vice President of Development.

2014 arrived and took off waiting for no one! I know I have been working hard to keep abreast of all of life's demands. You know, the things that cannot be done by anyone but yourself. The ones that will get you in trouble with the law, your spouse or your family if you do not meet the "suspense". Yes, I am referring to the IRS - 2013 taxes, medical appointments, mortgage payments, high school graduation and college applications, digging out of the 13th snow storm of the season, etc., etc., etc. Multi-tasking and running programs in parallel seems to be the norm now-a-days but I do miss the quieter days when the pace was slower and one was not so readily available.

Enough rambling, one of those "things" I am accountable and can get into trouble for is not fulfilling my responsibilities as the VP of Development for CRN. I am pleased to report that your Development Committee has been working diligently and the results of our program are impressive. It takes a lot of energy and effort to facilitate and coordinate the organization's development efforts. I am honored to be able to partner with the following individuals:

Corporate Sponsorship - Jeff Larimore, John Maccarone, Deb Reed

Fund Raising - Katie Larimore, Shannon Keizer, Tim Miller, Briana Smythe, Jen Wyman

Administration - Christy Greeley, Jenni Sexstone, Jen Wyman

We have a formidable program for 2014. That said, we can always use more families to become involved in sponsoring events as the organization's programs continue to grow and expand both nationally and internationally. We are capitalizing on the momentum we achieved in 2013 and have set high goals for 2014. Some of the focus areas we will be pursuing are carryover from 2013 but there will be new priorities as well:

- **Collaboration** - identify and enable greater interaction/partnering internally and externally
- **Community Engagement** - provide a platform for individuals to develop new skills and proficiencies via fundraising activities
- **Financial Protocols** - review and update existing Development processes and methods to increase accuracies and granularity
- **Fundraising Enablers** - identify and introduce enablers to enhance developments capabilities
- **Operating Protocols** - refine and formalize processes and methods to increase efficacy and efficiencies

(Continued on page 30)

2014 Fundraising Calendar

Date: 2014	Activity/Event	Honoring	Sponsor
February 12th	Garrett's Birthday Letter	Garrett Thomas	Lynn Thomas
February 13th	Steven's Birthday Letter	Steven Schleuder	Terri Schleuder
February 14th	Quiznos Fundraiser	Briana Smythe	Briana Smythe
March	Company Donation	Joey Jordan	Rich Jordan
March 8th	Miracle at Milleridge	Laura Krummenacker	Marybeth Krummenacker
March 17th	Jack's Birthday Letter	Jack Greeley	Christy Greeley
April	Village Club of Lincolnshire	Jack Greeley	Christy Greeley
April	Shannon's Birthday Letter	Shannon Keizer	Shannon Keizer
April 26th	Costume Crazies 5K Fun Run/Walk	Mason Reed	Deb Reed
May	MayHem! Music Festival	Sierra Ayers-Mutchler	Ayers & Mutchler family
May	Owen's Outlook - Rock Out Cystinosis	Owen Blais	Patti Blais
May 4th	Kacy's 5K Fun Run/Walk for Cystinosis	Kacy Wyman	Jen Wyman
May 4th	Pittsburgh Half Marathon	Josie Sexstone	Heather Vest
May 10th	Swings for Sarah (Golf Outing)	Sarah Larimore	Jeff & Katie Larimore
May 11th	Tomorrow Dies Concert	Patrick Hulbert	Patrick Hulbert
June 7th	C.H. Robinson Cystinosis Charity Golf Tournament	Lola Long	Tim Miller
June 22nd	Sarah's Birthday Letter	Sarah Larimore	Jeff & Katie Larimore
June 30th	College Freshman Celebratory Letter	Christian Morales	Jose' & Velyna Morales
August	Josie's Birthday Letter	Josie Sexstone	Jenni Sexstone
September	Owen's Outlook Golf Tournament	Owen Blais	Patti Blais
September	TO BE DETERMINED	Brandon & Johnny	John & Arlene Maccarone
October	Village Club of Lincolnshire	Jack Greeley	Christy Greeley
October	Golf Tournament	Peyton Paciulli	Channing & James Paciulli
November 10th	17th Birthday Fundraiser	Anjie & Nelly Polanco	Rachel DaLomba
December	Chandler's Chance...A Christmas Palooza for Cystinosis	Chandler Moore	Clinton Moore

On-going Programs

Amazon Smile Foundation
 Cystinosis T-Shirt On-line Merchandise Campaign
 Heroes in Training
 Jordan's Cystinosis Bracelets/Jewelry On-line Campaign

Honoring

Community
 Briana Smythe
 Carly
 Jordan Kulyk

Sponsor

Christy Greeley
 Briana Smythe
 Joan DeCroce
 Barb Kulyk

Development Update (continued from page 28)

We have a diverse array of events and activities scheduled for 2014:

- **Dinner Dance** - 1
- **Holiday Event** - 1
- **Letter Campaign** - 7
- **Music Festival** - 2
- **On-going Program** - 5
- **Special Occasion** - 3
- **Sporting Event** - 7



Mason Reed and friends enjoying the Costume Crazies 5K Fun/Run fundraiser in Vega, Texas to support CRN.



Kacy Wyman and Jack Greeley at the 8th annual 5K Cystinosis Fun Run supporting CRN.

Cystinosis Families Plan 25th Midwest Gathering in July

Cystinosis families living in the Midwest are busy planning their annual gathering of friends, family and fellowship.

This year marks the 25th anniversary of the event. This year's event **will be held on July 18th -20th in Cedar Rapids, Iowa.**

Anyone is welcome to attend even if you do not live in the Midwest. For more information about the event and hotel accommodations contact Doretta Hoffmann at 515-733-4437.

Many who have attended in the past return each year to renew the bonds of friendship. Please consider joining in.



Doretta and Andrew Hoffmann and friends at the 2013 CRN Family Conference.

"Miracles at Milleridge" Fundraiser Raises \$25,000 for CRN

By Marybeth Krummenacker

March 8, 2014 was another great night for the Cystinosis Research Network. The bi-annual "Miracles at Milleridge" fundraising dinner dance was held once again. It was a great event with over \$25,000 being raised for CRN. Laura Krummenacker took the lead on this event and organized and planned and was a great "ambassador" for the Cystinosis Research Network. Many of Laura's friends and family attended once again with over 200 people present. We had over 50 beautiful raffle baskets, thanks to the efforts of the Murphy and Maccarone families and friends in particular. Included also was the 50/50 raffle, the silent auction and just an evening filled with fun, family, and friends gathered for a great cause! The kindness and generosity of people is always overwhelming to Laura and I, and we



Laura Krummenacker with her good friends, Michael Lang and Jacklyn Pettas at the "Miracles at Milleridge" dinner dance.



Laura Krummenacker and friend at the "Miracles at Milleridge" dinner dance fundraiser for CRN.

know how blessed we are to have such a strong supportive network around us. We both say we can't do this alone!

The wonderful surprise of the evening was when Laura's good friends Michael Lang and Jacklyn Pettas, asked to make a special presentation to CRN in Laura's honor. The three of them have been friends since kindergarten and middle school. Mike and Jacklyn are getting married in October and Laura is one of the bridesmaids in their wedding. To demonstrate their true friendship, Jacklyn announced that she and Mike are so thankful for Laura's friendship and love that they have decided to give a donation of \$1,200 to CRN rather than buy wedding favors. Laura was shocked with this presentation and so appreciative of such a special gift. It was a wonderful moment for Laura and we are so grateful to Mike and Jacklyn for their generosity and friendship. The kindness and generosity of so many people who either attended that evening or sent a donation is overwhelming. Cystinosis is not an easy illness to live with, but Laura continues to do it with grace and love and never once does she feel sorry for herself. She continues to inspire so many and is truly an example of how to live each and every day!



Having fun at the 2013 CRN Family Conference.

raptorCares®

A patient support program for PROCYSBI® (cysteamine bitartrate) delayed-release capsules

COMMONLY ASKED QUESTIONS ABOUT PROCYSBI®

Q: What can I expect when I start PROCYSBI?

A: As with any new medicine, it may take time for your body to adjust to PROCYSBI. It is important to work with your doctor to find the best way to take PROCYSBI that works for you. For example, your doctor may start you on a lower dose and adjust it over time to a level that suits you best.

Q: Can I take PROCYSBI with water?

A: Yes, you can take PROCYSBI with water when swallowing your capsules whole.

Q: Is it ok to take PROCYSBI with food and beverages?

A: It is ok to take PROCYSBI with or without food and beverages. However, it is very important to take PROCYSBI with a similar type and amount of food every time, and to check in with your doctor as you are adapting to your new medicine.

Q: How should I take PROCYSBI if I'm using a Feeding Tube?

A: PROCYSBI can be administered through a G-Tube (or Feeding Tube). Please contact a RaptorCares nurse before starting PROCYSBI with a feeding tube or ask your doctor about a video showing G-Tube administration.

Q: What if I or my loved one have side effects?

A: With any side effect, you should notify your doctor right away. Your doctor may lower your dose and then gradually increase it to achieve your target dose. Remember not to stop taking PROCYSBI or any medication without talking to your doctor first.

Q: What financial support programs are available to help me?

A: Co-pay assistance, secondary insurance, travel, and white blood cell cystine testing support are available to eligible patients. The first step is always to contact RaptorCares at 855.888.4004.

RaptorCares is here to help.

Please check the Patient Package Insert that comes with your PROCYSBI regarding these topics as well as other helpful information.

INDICATIONS AND USAGE: PROCYSBI® (cysteamine bitartrate) delayed-release capsules is a cystine-depleting agent indicated for the management of nephropathic cystinosis in adults and children ages 6 years and older.

CONTRAINDICATIONS: Hypersensitivity to penicillamine.

RaptorCares 855.888.4004
RaptorCares.com

Monday - Friday, 8:00AM - 7:00PM CST
PROCYSBI.com

IMPORTANT SAFETY INFORMATION

What is PROCYSBI (Pro-CIS-bee)?

PROCYSBI is a prescription medicine used to manage a medical condition called nephropathic cystinosis in adults and children 6 years of age and older.

It is not known if PROCYSBI is safe and effective in children under 6 years of age.

Who should not take PROCYSBI?

Do not take PROCYSBI if you are allergic to penicillamine.

What should I tell my doctor before taking PROCYSBI?

Before you take PROCYSBI, tell your doctor if you:

- have a skin rash or bone problems
- have a history of seizures, exhaustion, sleepiness, depression, or other nervous system problems
- have or have had stomach or bowel (intestinal) problems including ulcers or bleeding
- have liver or blood problems
- are pregnant or plan to become pregnant. It is not known if PROCYSBI will harm your unborn baby. Tell your doctor right away if you think that you are pregnant. Talk with your doctor about the benefits and risks of taking PROCYSBI during pregnancy.
- are breastfeeding or plan to breastfeed. You should not breastfeed during treatment with PROCYSBI. Talk with your doctor about the best way to feed your baby if you take PROCYSBI.

Tell your doctor about all medicines that you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

Know the medicines you take. Keep a list of them to show your doctor and pharmacist when you get a new medicine.

What should I avoid while taking PROCYSBI?

Do not drive or operate heavy machinery until you know how PROCYSBI affects you. PROCYSBI can make you sleepy or less alert than normal.

What are the possible side-effects of PROCYSBI?

PROCYSBI can cause serious side-effects, including:

- **Skin, bone, and joint problems.** People treated with high doses of cysteamine bitartrate may develop abnormal changes of their skin and bones. These changes may include stretch marks, bone injuries (such as fractures), bone deformities, and joint problems. Check your skin while taking PROCYSBI. Tell your doctor if you notice any skin changes. Your doctor will check you for these problems.
- **Skin rash.** Skin rash is common with cysteamine bitartrate and may sometimes be severe. **Tell your doctor right away if you get a skin rash.** Your dose of PROCYSBI may need to be decreased until the rash goes away. If the rash is severe, your doctor may tell you to stop taking PROCYSBI.
- **Central nervous system symptoms.** Some people who take other medicines that contain cysteamine bitartrate develop seizures, depression, and become very sleepy. The medicine may affect how your brain is working (encephalopathy). Tell your doctor right away if you develop any of these symptoms.
- **Stomach and bowel (intestinal) problems.** Some people who take other medicines that contain cysteamine bitartrate develop ulcers and bleeding in their stomach or bowel.
- **Tell your doctor right away if you get stomach-area pain, nausea, vomiting, loss of appetite, or vomit blood.**
- **Low white blood cell count and certain abnormal liver function blood tests.** Your doctor should check you for these problems.
- **Benign intracranial hypertension (pseudotumor cerebri)** has happened in some people who take immediate-release cysteamine bitartrate. This is a condition where there is high pressure in the fluid around the brain. Your doctor should do eye examinations to find and treat this problem early.
- **Tell your doctor right away if you develop any of the following symptoms while taking PROCYSBI:** headache, buzzing or “whooshing” sound in the ear, dizziness, nausea, double vision, blurry vision, loss of vision, pain behind the eye, or pain with eye movement.

The most common side-effects with PROCYSBI include:

- | | | |
|--------------------------------|-------------------------|-------------|
| • vomiting | • diarrhea | • tiredness |
| • abdominal pain or discomfort | • loss of appetite | • dizziness |
| • headache | • or decreased appetite | • skin odor |
| • nausea | • breath odor | • skin rash |

Tell your doctor if you have any side-effect that bothers you or that does not go away.

These are not all of the possible side-effects of PROCYSBI. For more information, ask your doctor or pharmacist. Call your doctor for medical advice about side-effects. You may report side-effects to FDA at 1-800-FDA-1088.

Don't Give up, Don't Ever Give up!

By Barry Beard

The late Jim Valvano used these words as cancer was taking over his body. I feel these words fit my life to a tee. I've had many challenges in life including Cystinosis, rickets, kidney stones, kidney transplant, a sleep disorder, later stage cancer, ileostomy, Chemotherapy, and a marathon. I could have given up many times but I never did.

I was born on September 10, 1975 in Harrisburg, PA and diagnosed with Cystinosis 11 months later. My diagnosis came at a time long before the Internet, cell phones, flat screen TVs, or even cable television. When I was diagnosed there wasn't much known about Cystinosis other than a few paragraphs in a Physician's Medical book. There were no support groups or families willing to talk about this disease. My parents tried to learn everything they could about Cystinosis and reached out to the most knowledgeable doctors in the field. After my diagnosis I was referred to a specialist in Philadelphia. My parents were told that children with this



Barry and his three Border Collies.



Barry after his first marathon in November 2008.

disease often suffered kidney failure by age 10. They were told that if they had other children the risk would be very high that the child would also have Cystinosis. One good thing the specialist said was that an experimental drug called Cysteamine was showing signs of promise and would hopefully prolong kidney function. Experimental drugs often come with many risks and unknowns but my parents decided to put me on the medication. The drug wasn't approved by the FDA until 1994 when it was called Cystagon. This drug, along with great doctors and unbelievable parents, helped save my life. For the next 12 years my parents and I would have to drive 100 miles to Philadelphia every three months until they were able to find a specialist closer to Harrisburg. My parents played a huge role in my life and always told me I could do anything I wanted in life. As a child I did everything other kids did: played sports, video games, went to the movies, and just enjoyed being a kid. The only difference was that I had to take medicine. I would often ask my parents why me, why did I have to have this disease. I know this was probably difficult for them to explain but they would just try to explain life and listen any time I had a concern. They told me they would always be there for me through any challenges I faced. My parents along with great friends in my neighborhood helped me have a normal childhood.

My teenage years were some of the toughest of my life. Being a teenager is hard enough but being a boy and short, made it more challenging. The odor of Cysteamine was very bad and my parents tried to find ways to dilute the smell. We would try to time the doses so that I would not be in school with the smell on my breath. I was put on human growth hormone when I was around fourteen and this helped me reach a height of 5' 2". My high school years were pretty good. I was on the high school baseball team in my senior year and also went to the prom. I made some really good friends that I still keep in touch with today.

After high school I went to a local community college, but decided college was not for me. The next few years I worked a few part time jobs and tried to save money. In 1997 I got a permanent job with the Commonwealth of Pennsylvania where I still work today. I started dating more after high school and met my wife of almost 10 years in 2002. A few years before I met my wife, my Nephrologist told me my kidney function was getting bad and that I would have to start dialysis. He said I would be put on the transplant waiting list. Growing up I knew I would eventually have to get a kidney transplant, but this was still a shock. In 1998 I started Kidney Dialysis something that was very hard and drained my energy. If the dialysis nurse didn't get the needle in my vein just right, my arm would blow up and turn all black and blue. I was working full time when I started dialysis so I would have to leave at noon two days during the week to go to the hospital. I would also have to spend four hours on Saturday hooked up to the Dialysis machine. I would get the call for a transplant on June 5, 1999. I was called once before, but was kind of scared so I didn't go to the hospital.



Barry, his wife Kristen, and hall of fame jockey, Pay Day at Churchill Downs Race course in June 2006, the week of the Transplant Games.

On June 5th I had no choice because I was at the Hospital in the dialysis center. The kidney transplant surgery went well but I was in the hospital for about a week because of some complications. After a few months of recovery I was back to work full time.



2010 US Transplant Games stand. Barry won a silver medal in Badminton one week after chemotherapy treatment.

A few years after my transplant I got involved with the Gift of Life donor program doing volunteer work. I spoke about Organ and Tissue Donation and the importance of donation. I also started competing in the US Transplant games in 2002. The transplant games are an Olympic style event for individuals that have received a life saving organ or tissue transplant. The US games are held every two years in a different city. There are also International games that are held in the years the US games aren't. I've gone to the International games in Canada, and this will be my 7th U. S. Transplant games. The events I've competed in are badminton, the 5k, racquetball, bowling, table tennis, basketball, and some track/field events. I've won medals in badminton winning the gold medal twice, and silver three times. I also won a bronze medal in racquetball at the US games and badminton at the World Transplant Games in 2005. In 2002 while I

(Continued on page 36)

Don't Give Up, Don't Ever Give Up!

(Continued from page 35)

was online I decided to look up information about Cystinosis. I learned more about my disease and the great research that was happening. I also learned about the Cystinosis Research Network and the other Cystinosis organizations.

I continued to compete in the transplant games and started running distance races in 2007. Mack Maxwell inspired me to start running more after learning about his exercise program at the 2007 Cystinosis Research Network Family Conference. I also learned that he completed a half marathon (13.1 miles). I ran my first half marathon in November 2007. The race went so well that I thought, if I can run a half marathon I can run a full marathon (26.2 miles). I signed up for the Philadelphia marathon that was going to be held in November 2008. This again was a time I could have given up but I didn't. It is mile 20 in the race, and my legs are starting to get tired. Before the race I was thinking, wow this is crazy in a few minutes I'm going to be running 26.2 miles. The longest I had run before was 20 miles. Even though my legs were getting tired, I kept fighting through the pain and



Barry crossing the finish line in his first half marathon in Philadelphia, PA, in November 2007.



Picture of Barry's back before his third Marathon in Philadelphia, PA, in November 2009.

didn't give up. I finished in a time of 4 hours and 46 minutes and became the first person with Cystinosis to finish a marathon. In 2009 I ran a few more half marathons and two full marathons. I was living and enjoying life until late 2009. This is when I began seeing blood in my stool. I waited about five months (January 2010) before I went to see the doctor. I know I should have gone sooner but I didn't. The doctor advised me to have a Colonoscopy. After the procedure, the nurse told me my wife was waiting for me in a small conference room and that the doctor would be in to talk to us. I was about to face another moment of life when

I could have given up. I will never forget the words the doctor said, "We have a problem". He showed me a growth on my colon and said he was referring me to a specialist at the Hershey Medical Center. This was a very scary moment for my wife and I. When you hear the C word you think death. My

wife and I researched colon cancer all weekend reading anything we could find. My appointment with the specialist wasn't [until Monday](#) so we were thinking about this all weekend. At the appointment the specialist said I had Colorectal cancer and my heart dropped. I asked what stage of cancer because I had been reading a lot about colon cancer. He said stage 3c and again my heart dropped. I was so shocked, this was the stage right before stage 4 where the cancer has spread to other organs in the body. In stage 4 cancer there is basically no hope and it has a very low survival rate. The doctor was talking about things like chemotherapy and radiation. After the diagnosis I remember going to my parents and telling them and my grandmother to just add this to the things in life I would beat. Inside I was very scarred though and unsure of what the future would bring. A few weeks later I remember being at my father-in laws retirement party on a rainy day, standing outside looking at the beautiful trees and mountains and thinking how beautiful it was. I thought wow I'm never going to see this again. I also remember watching the 2010 Winter Olympics and thinking this was the last Olympics I would ever see. In February 2010, I had surgery to remove the tumor and to get a temporary ileostomy. After those surgeries I started Chemotherapy in March 2010. Chemotherapy was hard on my body and especially on my mind. "Chemo brain" as they call it really messes with your mind. There were many times when I couldn't concentrate or focus and didn't like being in crowds. These were things that rarely happened to me in the past. Fortunately for me I didn't lose my hair or a lot of weight something that happens to most cancer patients. Dealing with the everyday struggles of cancer was very difficult but I continued to work and fight this terrible disease. After six months of chemotherapy and a colon resection I was back to a "normal" life.



Silver Medal Barry gave to his donor's sister.

Today, four years after my diagnosis there have been no signs of cancer recurrence. I don't think cancer really ever leaves your mind, but it's not something I think about everyday. I continue to compete in the US Transplant games even competing in 2010 during the time I was on Chemo. I had decided that I wasn't going to go to the Transplant Games, but then I decided that nothing was going to stop me from going, not even cancer. The games are such a big part of my life and I love competing and seeing my transplant friends every two years. I remember competing at the 2010 games and asking a volunteer if she had any bottles of water that were not cold. She kind of looked at me weird but found one. The Chemo gives you a sensitivity to cold so I would drink warm water. I am currently training for my 4th full marathon that will be held on Columbus Day weekend in Albany New York. I am writing this article to help anyone affected by this disease, including: parents with children that have Cystinosis, children, and adults dealing with this disease.

I am very thankful to everyone that have helped me get to this point in my life including my parents, doctors, friends, family, donor family, and loving wife Kristen. I did have a chance to meet my donor family in 2006. **My donor was a 13 year old boy who died after an accident. The first time I met my donor's sister, we had dinner and had a great time.** I gave her one of my transplant games medals and told her the medal belonged to her and her family as much as it did to me. I was even invited to her wedding where I meet the rest of the family. Over the years we've kept in touch and try to meet every year. I am so grateful to my donor family for deciding to give me the gift of life.

I've had more challenges in my 38 years than most people will have in their lifetime. I want to tell you that you can do anything you put your mind to. Do not listen to people that say you can't accomplish your goals. Go out there, enjoy life, and don't ever give up! Hopefully I can inspire you as Mack and many others have inspired me. Life is not about what happens to you but how you react to happens.

Cystinosis Awareness Day 2014 Celebrated in Egypt

By Professor Neveen Soliman

Egypt celebrated the worldwide awareness-raising event "RARE DISEASE DAY". Egyptian Group for Orphan Renal Diseases - EGORD dedicated the 2014 event to build more awareness of cystinosis among public, medical communities, and health policy makers. Thanks to all (families and professionals) for making "CYSTINOSIS AWARENESS DAY" such a great and nice event!



Fun for Everyone at Cystinosis Awareness Day 2014 in Egypt,

Included were informative talks and comprehensive discussions (both in Arabic and English) on how celebrating "Rare Disease Day" in Egypt helps to shed more light on these commonly overlooked disorders. Moreover, it allowed building more awareness as to when to suspect and how to diagnose, nephropathic cystinosis; highlighting the opportunities and challenges facing the cystinosis community in Egypt to enhance the medical care offered to patients and families.



Doctor Panel at Cystinosis Awareness Day 2014 in Egypt.

The outstanding contribution and support of Professor Hussein Khairy, Dean of Kasr Al Aini School of Medicine Cairo University, to the

"Cystinosis Awareness Day 2014" is absolutely appreciated by Egyptian Group for Orphan Renal Diseases - EGORD Team and Rare Disease Advocates.



Families and Doctors attending Cystinosis Awareness Day 2014 in Egypt.

Exceptional are the disease advocates who were recognized by EGORD for their valued contribution to the success of the Rare Disease Day 2014 "Cystinosis Awareness Day".



By Deb Reed

On a cool morning on "Old Route 66" in Vega, TX, a crowd gathered for the 2nd Annual Costume Crazies 5 K Fun Run/Walk in honor of Mason Reed and Cystinosis Research Network. The event was held on Saturday, April 26th,

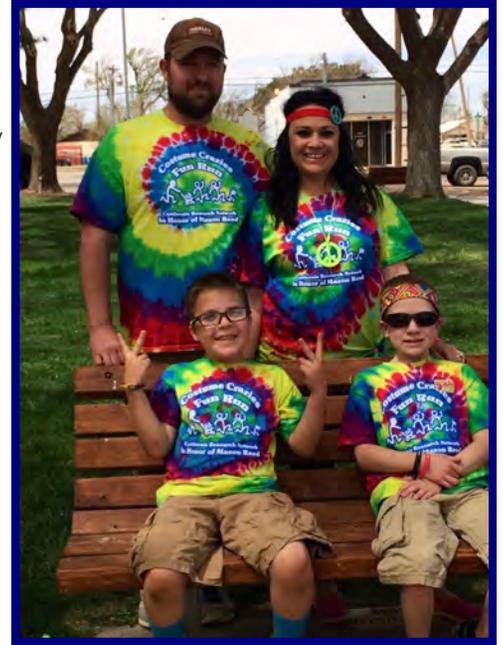


Members of the Reed Family attending Costume Crazies Fun Run.

2014 at the Oldham County Court House. We were very proud to have 117 entries which almost tripled last year's event.

This year was "extra special" as Raptor Pharmaceuticals sponsored our event and helped to make it a huge success.

As race time approached, the Court House Square filled with costumes of all kinds. We had crazy and creative costumes, socks, hats, and funny wigs. There were families dressed in match-



Mason Reed and his family enjoy a wonderful day of fun at the 2nd annual Costume Crazies Family Fun/Run for CRN.

ing costumes, The Incredible's, a six pack, and Mason's 4-H club dressed in shirts made especially for the occasion, with "We support our friend, Mason Reed!" This year we even had pets participate! We handed out gift bags for the runners with T-shirts, water bottles, and wristbands.

We were also fortunate to have join us from Dallas, TX, Mary Patterson, Office Manager with Raptor Pharmaceuticals. Mary not only came to the event to represent Raptor, she ran her first 5 K, and did an **awesome job!** Mary's husband Tim accompanied her to Vega.

After the race the participants enjoyed a drawing of gifts donated by family, friends, and businesses. "Clowns un-

der Construction" entertained the crowd with face painting and balloon art. Bouncers were also on the court house square for the children's entertainment while families enjoyed a picnic lunch.



Members of the Broce Family attending the event.

This event is all about "FAMILY". The Reed and Broce families were all in attendance to help for the day. Our extended families and friends pitched in and helped in every way to make this a very successful FUN RUN. Look out 2015, bigger and better things to come!

Join the Cystinosis Research Network

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



Jessica and Caroline Larimore at the 2013 CRN Family conference.



Terri Schleuder and Jose' Morales at the 2014 Kacy's 5K Fun/Run for CRN in Bloomfield Twp., MI

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!

Immediate Family	\$20.00
Extended Family / Friend	\$25.00
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Please complete the form & mail with check payable to CRN to:
 Cystinosis Research Network
 302 Whytegate Ct.
 Lake Forest, IL 60045

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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.



Zylar Smethurst

2013 Donor Honor Roll

Over \$50,000

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Mason Paul Reed

\$10,000-\$19,999

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\$2,500-\$9,999

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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.

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