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2017 CRN Family Conference: July 13-15 at Snowbird, Utah

The 2017 Cystinosis Research Network Family Conference will be held July 13 – 15 at the Snowbird Ski and Summer Resort located in Utah. Our theme this year is "Soaring to New Heights".

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



Attendees of the 2015 CRN Family Conference in Chicago, Illinois

#SoarwithCRN

Continued on page 10

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The Collective Spirit of the Cystinosis Community – Watch it Soar

Message from the President—Jeff Larimore

The dedicated individuals and families within the Cystinosis Research Network are working diligently on the final details for the 2017 Family Conference scheduled in July in Snowbird, Utah. This year's Conference theme of "Soaring to New Heights" recognizes the significant progress and possibilities being made in the areas of research, family support and community outreach. The CRN Family Conference is recognized worldwide as one of the most comprehensive and engaging gatherings within the Cystinosis community and we are confident that this year's event will set new heights for achievement.

The spirit of community is the key to a successful advocacy organization. Over the past few years, the Cystinosis Research Network has continued its efforts to broaden and expand its successes in eight fundamentals where:

- New families are receiving a diagnosis of Cystinosis earlier,
- Stable and well-defined treatments for our children are being provided,
- Development of a relationship network for teens and adults so they become the future of Cystinosis advocates for themselves and our community,
- Collaboration and commitment to promising and innovative research occurring and funded by worldwide Cystinosis advocacy organizations,
- Broadening worldwide relationships so that all Cystinosis patients are diagnosed and treated appropriately around the globe,
- Supporting clinical specialists committed to a career spent caring for and researching Cystinosis,
- Capture more families advocating for their children,
- Continuing to engage, advocate, and represent global rare disease communities with spirit and integrity.

The vision and mission statement of the Cystinosis Research Network will be accomplished if we are able to continuously win these important points.

In accordance with the Bylaws of the Cystinosis Research Network, my second term as President will come to an end this summer. These past five years have been a tremendous learning experience for me and the relationships will be ever-lasting.

When you are provided an opportunity to be actively involved in a patient advocacy organization like the Cystinosis Research Network, you naturally become consumed to give your best not only for your child but for all those battling with the disease. Your family expands to be inclusive and your actions are driven toward improving the lives of all. Successes are measured in progress and "not yet successful" just needs more time. In my heart, I feel we have made some progress.

Our daughter Sarah will be 12 years old at the conclusion of my term. At first, Sarah may not have known why I agreed to become CRN's President, but today, I hope she is proud of me.

To the Cystinosis community and for all those who support the Cystinosis Research Network, thank you for the opportunity to serve these past five years.

Cheers,

Jeff Larimore



Sarah and Jeff Larimore

Second Future by Design Meeting Held in Minnesota

By Jenn Loglisci

Several adults with Cystinosis held their second Future by Design meeting this past March in Minneapolis, Minnesota. It again brought in new perspectives from adults living with Cystinosis and what they want to bring to the table. Each person has care and concern about the future of the children in this community and we want to help make their lives a little better and a lot brighter. The meeting was hosted by Aimee Adelmann and Jenn Loglisci.

One of the goals of the Future by Design group is to provide support and encouragement to young adults, parents and children living with Cystinosis. This group will be featured at the upcoming family conference and would love to meet with those in attendance as mentors. They would love to sit down with parents and kids, have open honest conversations and form bonds. Aimee Adelmann will give an informal presentation



Aimee Adelmann leads a discussion of shared ideas from adults in attendance

about the Future by Design group, explain who they are, and why they desire to be a resource for other patients to help make the younger generations very successful.

The Future by Design group of adults are a generation of resilient, active adults who live with cystinosis. They want that to be the norm for the future.

"We are like building blocks and if we don't all stand together we will all fall apart." – Future by Design Group



Jenn Loglisci and Rachael Young enjoy a little fun time at the famous Mall of America located in Minneapolis

John Paul II Medical Research Institute Looking for Cystinosis Patients

JOHN PAUL II
MEDICAL RESEARCH INSTITUTE

An Iowa-based non-profit, John Paul II Medical Research Institute (JP2MRI), is looking for Cystinosis patients. Their Rare Disease Program aims to find more treatments and cures for children and patients with rare diseases.

JP2MRI is currently creating a stem cell biobank of patients with rare genetic diseases. They are currently enrolling rare disease patients through a patient registry to ultimately obtain blood or tissue samples. Participation in this research program is entirely voluntary. Your medical information will NOT go to any outside third parties. The patient registry is kept secure and confidential consistent with HIPAA regulations.

JP2MRI is not conducting clinical trials at this time. For more information, visit:

<http://www.jp2mri.org/rare-genetic-disease-program>

Horizon Nephropathic Cystinosis Scholarship (HNCS)

Horizon Nephropathic Cystinosis Scholarship (HNCS) is an exclusive funding opportunity for Cystinosis patients looking further their knowledge. HNCS gives Nephropathic Cystinosis patients the opportunity to learn something new, and empower them to grow as individuals.

Are you a patient diagnosed with Nephropathic Cystinosis? Have you always wanted to learn something new? Maybe your dream is to become a CPA, master chef, learn a new language, take a dance class, or see the world through a lens. This opportunity is designed for you to take what you are passionate about and learn more. We are offering Scholarships to help you spark an inspiration through a learning opportunity and develop yourself into a leader in the community.



to

Rules & Regulations:

- Must be diagnosed with Nephropathic Cystinosis (**download HCP form on link below**) <https://globalgenes.org/wp-content/uploads/2016/10/HNCS-Physician-Letter.pdf>
- Employees/affiliates or their children of Horizon Pharma or Global Genes are not eligible to apply
- Must submit proof of enrollment in course
- You can **apply** at the following link, <https://www.tfaforms.com/447883> retrospectively to January 1, 2017 for applicable support
- Maximum scholarship amount is \$5,000.00USD per applicant, with a maximum of \$50,000.00USD to be distributed
- Learning opportunities are open to patients of all ages in college, trade school, beauty school, vocational school, special interest courses (dance, archery, painting, pottery, etc.), or enrolled in life skill trainings and seminars.

To apply, please submit a fully completed **application** with your attached **HCP Form** and course invoice to be considered. We are accepting applications on a rolling basis, so submit your application ASAP to join in on this amazing opportunity!



Rare Disease Week on Capitol Hill 2017

By Terri Schleuder



CRN's Rare Disease Week on Capitol Hill's Cystinosis Advocates: from left to right Jeff Larimore, Emily Mello, Terri Schleuder, Eddie Langley, Jr., Steve Schleuder, Dennis Healy, and Sara Healy

What a special week! In the planning stages since late November, the CRN contingent of rare disease advocates arrived from Connecticut, Georgia, Michigan, South Carolina, Minnesota, and North Dakota into Washington, D.C. on Sunday February 26th. After settling into our rooms and getting familiar with the area near our hotel, (i.e. location of restaurants, food marts and the DuPont Circle Metro station), we headed into a very busy three days.

On Monday, February 27th we all rode the Metro subway to the NIH, a familiar place to most of us. Along with over 600 rare diseases advocates from every state and many countries, we sat through presentations from researchers, clinicians, advocacy

groups, and patients themselves. Each attendee's life had been touched in some way by a rare disease. We learned 80% of all diseases are genetic and that 50% of those genetic diseases affect children. We learned 30% of those children die by age 5. We learned of the 7,000 rare diseases only 289 have any treatment or medication available at all! Because the NIH can attract large numbers of rare disease patients, there are currently 444 clinical trials in progress. The study of rare diseases often provides a window into many other more common diseases. Of special interest to our group was a slide describing the NIH's Clinical Center's accomplishments over the last 2 decades. Number 4 on the list stated, **Cystinosis cause identified.**

After a full day, we headed to a cocktail reception and documentary screening of a film called *Up for Air*. The film chronicled the remarkable odyssey of Jerry Cahill, and his lifelong fight with Cystic Fibrosis. Jerry is 60 years old and received a double lung transplant about 5 years ago. Jerry's life demonstrates what can be accomplished through determination, dedication, perseverance, coupled with a positive attitude. At

the end of the film, Jerry appeared in person as a part of a panel to answer questions from a clearly very impressed audience. He will be our Keynote speaker at the family Conference this year in Utah.

On Tuesday, Rare Disease Day, we walked to a building just across the street from our hotel. After a delicious breakfast, our Legislative Conference began (or better described as *rare disease issues and congress 101, the advanced edition*). For eight hours, our minds were crammed with everything we would need to know on Wednesday, the actual Lobby Day, when we would meet with our states legislators.

We celebrated the passage of the bipartisan 21st Century Cures Act, passed in Dec, 2016. This legislation will help advance treatments for rare disease patients.

We learned about the Orphan Products Extension Now Act (Open Act), legislation that could potentially double the number of treatments to rare disease patents.

We learned about the importance of continued and increased funding for the NIH and FDA to further address the needs of those fighting rare disease.



Eddie Langley with legislative Correspondent William Dent from Senator Isakson's office from Georgia

We learned with the probable repeal and replacement of the ACA, the importance of making sure patient protections remain in place to protect those with pre-existing conditions, so insurance companies cannot discriminate against them because of their rare diseases.

We learned about the Rare Disease Congressional Caucus, a group of bipartisan congressional members who are interested in raising awareness about rare diseases.

In the afternoon, we broke up into groups by state, as many of us would be visiting our legislators together. In Michigan, there were 8 of us, representing 5 rare diseases who would visit the offices of Senators Stabenow and Peters. It took a little while to work out a strategy that would include everyone's voice and deliver the message we needed to deliver.

At the end of the day we met in breakout sessions to further refine our skills at successfully lobbying our congressional leaders.

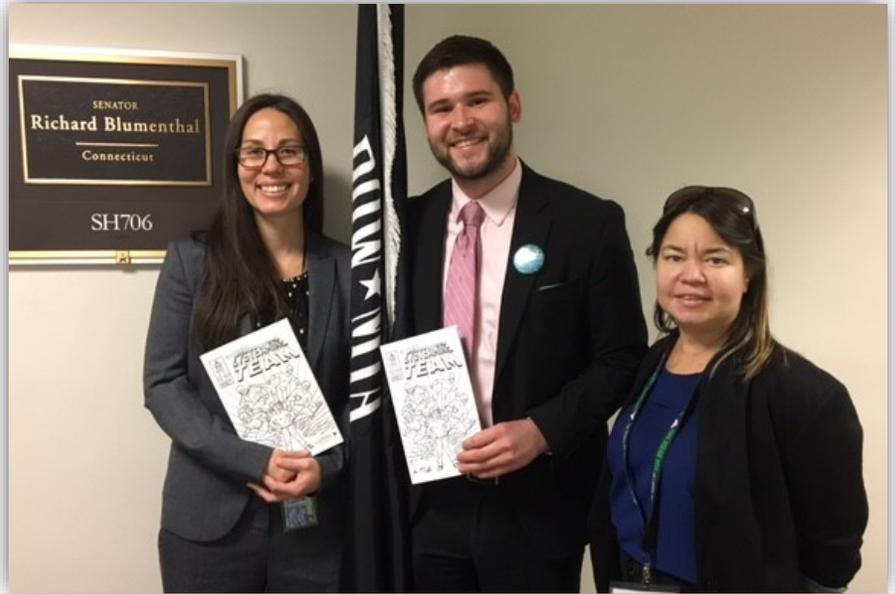
That evening our CRN group met for dinner, to visit, decompress, and connect with each other in a relaxed



Sara Healy and her dad Dennis, celebrate Rare Disease Day and raise Cystinosis awareness in D.C.

setting. The food and company was wonderful, a perfect ending to an eventful day.

All of our preparations led to Wednesday, March 1st. Dressed in our best clothes, with



Emily Mello meets with staff members from Connecticut Sen. Richard Blumenthal office advocating for the rare disease community.



Sara Healy with Rep. Rick Nolan from her 8th Congressional District in Minnesota

our packets of information and stories in hand, we traveled on the metro again to the Lobby Day Breakfast, a short walk from the Union Station Metro stop. By 8:45 we'd found our state groups and headed toward our first meetings of the day. Steve and I along with 6 other rare disease advocates from Michigan met with Lorenzo Rubaclava, the legislative assistant for Sen. Debbie Stabenow, and Devin Parsons, the legislative correspondent for Sen. Gary Peters.

We asked for the senators' support for:

- The Open Act
- Continued and increased funding for the NIH and FDA
- Keeping patient protections in place for those with pre-existing conditions with any healthcare reform legislation
- We asked our senators to consider becoming members of the Rare Disease Caucus

Our last meeting of the day was with Michigan's 11th Congressional District representative Dave Trott and his legislative assistant Bridget Sobek Dobyen. We shared our story and concerns with the congressman and were met with compassion, support and interest. He asked us several questions about Cystinosis. We left feeling hopeful. Our five other Cystinosis advocates met with their states representatives with similar results.



Steve and Terri Schleuder with Rep. Dave Trott from Michigan's 11th Congressional District.

In all, Congressional Representatives from 48 states were visited by over 600 rare disease advocates participating in Rare Disease Week on Capitol Hill. It was a good first step in this year of change.

On Wednesday evening, we all met at the Russell Senate Building in the Kennedy Caucus room for the Rare Artists Reception. Artwork from artists with rare diseases lined the walls with moving stories and biographies for each one posted by the individual works of art. For me this may have been the most poignant moment of a very emotional three days. As I viewed each incredible work of art and read about the artist, tears streamed down my cheeks. No one could not be profoundly touched by the displays in that room.

On Thursday morning, we all headed back to our homes, and families in our individual states. We all felt very good to be a part of what happened here this important week, during a very important year of change for our country. CRN wishes to thank our

advocates, Emily Mello, Eddie Langley, Sara Healy, Dennis Healy, Jeff Larimore, Steve Schleuder, and Terri Schleuder, for taking time out of their busy lives to be here representing the Cystinosis community at Rare Disease Week on Capitol Hill 2017.



Jeff Larimore and Laura Edwards from South Carolina with Sen. Tim Scott, advocating for the Rare Disease Community



A tired, but happy crew. Thank you, Eddie, Steve, Sara, and Emily for being our voices for Rare Disease Week...you all "Rock"!

2017 Family Conference continued from page 1

Registration information follows, and can also be found on the CRN website at: <https://cystinosis.org/events/conferences/2017-crn-family-conference>

Lodging: Rates quoted are per night.

Room	Single/Double Rate
Cliff Lodge - Bedroom	\$119.00
Lodge at Snowbird - Bedroom	\$99.00
Lodge at Snowbird - Efficiency	\$99.00
Lodge at Snowbird - One Bedroom Condo	\$169.00
Lodge at Snowbird-One Bedroom Condo w/Loft	\$239.00
The Inn - One Bedroom Condo	\$169.00

IMPORTANT: The CRN block of rooms will be released on June 11th- this means that these rooms will no longer be reserved for our families – PLEASE, try to make your reservations before then!!

For a reduced lodging rate, mention “CRN Family Conference” by phone OR use the group code: CRN when booking online.

To book by phone: 1-800-453-3000

** Please note, if you book by phone you will be charged ONE night of lodging at the time of booking.

To book online visit: <https://reservations.snowbird.com/default.aspx?p=&group=CRN717&arrival=07/13/2017&depart=07/16/2017&bookingstep=1>

** Please note, if booked online FULL payment will be due at time of booking.

*Check-in Time: 4:00pm / Check-out Time: 11:00am *Resort Parking is Free.

Travel and Ground Transportation:

Closest airport to Snowbird is Salt Lake City International (SLC) Airport. Canyon Transportation is the best value, round trip from SLC airport is: \$72.00 Adult (13 and up) and \$48.00 child (12 and under). Reservations can also be made by calling 1-800-453-3000.

Conference Registration:

Please complete and return the Conference Registration form along with a check for the registration fee. Mail to: CRN Conference Committee – Attn: Pam Woodward, 1368 W. 2600 N., Pleasant Grove, UT 84062
Registration can also be completed online at the CRN website - www.cystinosis.org and registration fee can be submitted online through the CRN PayPal link.

Camp CRN - Childcare & Youth Program:

Childcare will be provided during the conference for children ages 1-7 years, as well as a Youth Program for Tweens and Teens age 8-18. Youth participants will have the opportunity to socialize with peers and will be entertained with a variety of indoor and outdoor activities, games, and crafts.

Conference Scholarships:

A limited number of conference scholarships are available to individuals and families affected by Cystinosis. Thank you to those who have applied for these scholarships. The winners will be announced shortly.

Poster Session:

This year's conference will again include a poster session scheduled for Saturday afternoon. This session will showcase a mix of science, medicine, industry, advocacy group, and patient experiences to provide an interactive experience for both family and professional attendees. Researchers, clinicians, industry and advocacy representatives, students, patients, and caregivers will be invited to exhibit their latest research findings, treatment breakthroughs, advocacy group updates, and patient and family experiences. This will be an interactive session where exhibitors will be available to discuss their work or experiences with those attending. We would like to invite you, your colleagues and students to submit abstracts for the poster session. Posters will be arranged into three categories: research/clinical, industry/advocacy group, and patient experience. Poster Session Requirements for submission information can be found on the website.



Teens enjoying an outing in Chicago at the 2015 CRN Family Conference

Cystinosis Research Network Family Conference

July 13-15, 2017

Snowbird Ski and Summer Resort
Snowbird, Utah

AGENDA



DRAFT 5-25-17

Wednesday, July 12		Room
3:00 pm – 8:00 pm	CRN Board of Directors Meeting <i>Closed Session</i>	Seven Summits, Snowbird Center
Thursday, July 13		Room
12:00 pm – 5:00 pm	Registration/Information Desk	Cottonwood Lobby, Snowbird Center
12:00 pm – 5:00 pm	Snow City Arts <i>The CRN 2017 conference has designated the theme “Soaring to New Heights,” that has informed the design of a group art project. The idea is to have individuals create representations of themselves as birds in mixed media sculpture forms. Each bird will be uniquely designed. These individual sculptures will then be suspended together to create a collaborative time based hanging art installation of birds flying in a flock in an upward spiral. The upward spiral form was chosen to signify how those impacted by Cystinosis can reach new heights working as one community. The installation will only exist for one night on July 15th in Snowbird, Utah. The art project is designed to reflect the ability for individuals to become stronger, fly higher when they have shared goals and come together to connect in a meaningful way.</i>	Alpine A, Snowbird Center

2:00 pm – 4:00 pm	CRN Scientific Review Board Meeting <i>Closed Session</i>	Seven Summits, Snowbird Center
5:00 pm – 7:00 pm	CRN Welcome Reception <i>Complimentary – Sponsored by Leadiant Biosciences</i> <i>Hosts: CRN Board of Directors, Future by Design</i> <i>Kick off the conference with our Carnival in the Cottonwood Lobby! The festivities feature something for everyone including games, prizes, entertainment, drinks/adult beverages, and appetizers. Grab this chance to begin meeting families and physicians early in the conference in a relaxed, fun-filled setting.</i>	Cottonwood Lobby, Snowbird Center
7:00 pm – 10:00 pm	Family Introductions/Networking <i>Host: Clinton Moore, VP Family Support</i> <i>A great opportunity to put faces to the names of each family attending the conference. Bring your entire family as this session will include introductions AND reveal the second book in the “Team Cysteamine” comic series. Author and illustrator, Kevin McCalla, will be joined by “Team Cysteamine Live” to autograph complimentary copies of the book.</i>	Cottonwood, Snowbird Center
Friday, July 14		Room
7:00 am – 8:30 am	Breakfast <i>Complimentary</i>	Cottonwood Lobby, Snowbird Center
8:00 am – 5:30 pm	Registration/Information	Cottonwood Lobby, Snowbird Center
8:00 am – 5:30 pm	Snow City Arts <i>The CRN 2017 conference has designated the theme “Soaring to New Heights,” that has informed the design of a group art project. The idea is to have individuals create representations of themselves as birds in mixed media sculpture forms. Each bird will be uniquely designed. These individual sculptures will then be suspended together to create a collaborative time based hanging art installation of birds flying in a flock in an upward spiral. The upward spiral form was chosen to signify how those impacted by Cystinosis can reach new heights working as one community. The installation will only exist for one night on July 15th in Snowbird, Utah. The art project is designed to reflect the ability for individuals to become stronger, fly higher when they have shared goals and come together to connect in a meaningful way.</i>	Alpine A, Snowbird Center
8:00 am – 5 :30 pm	Camp CRN Children’s Group Tweens with Cystinosis (ages 8-12) Teens with Cystinosis (ages 13-18) Sibling Tweens (ages 8-12) Sibling Teens (ages 13-18)	Alpine B Alpine C Aspen Willow Studio Room
8:30 am – 9:00 am	Welcome and Opening Remarks Cystinosis Research Network Overview <i>Jeff Larimore, CRN President</i>	Cottonwood, Snowbird Center

<p>9:00 am – 10:00 am</p>	<p>Living, Breathing, and Succeeding with CF Keynote Speaker – Jerry Cahill <i>Advocate. Cystic fibrosis. Hero. Leader. Relentless. Hopeful. All of these words have one thing in common; they can be used to describe Jerry Cahill, a man who lives and succeeds in spite of his medical limitations. At 60 years old, Jerry Cahill has overcome remarkable odds and has become living, breathing proof that cystic fibrosis patients can have a normal life through firm commitment to exercise and medical compliance. Currently a CF Ambassador at the Boomer Esiason Foundation, Cahill leads its scholarship and grants division and is the founder of Team Boomer – the athletic arm of BEF dedicated to encouraging exercise among CF patients while simultaneously raising money for scholarships. He has also started the program You Cannot Fail, which includes apparel, a website, an autobiographical coffee table book, and two children’s books. In February 2016, Jerry created 60:60 and set a goal to complete 60 activities on his “living list” – an alternative to a bucket list – as a way to inspire others with CF to live their lives to the fullest. He also records educational podcasts and videos and encourages other people with CF to share their stories on Club CF. On top of all of this, Jerry invited a camera crew to follow him over the past five years, giving a unique insight into his life as a cystic fibrosis patient, pole vault coach, and advocate. The documentary Up for Air followed his journey through training, treatments, and a double lung transplant – showing a new phase of living with CF. Jerry, a strong advocate of exercise, credits pole vault and physical fitness with his success; he believes that “exercise is the single most important thing in my life that keeps me healthy with CF.” For more information on these programs, please visit:</i> www.cfwindsprints.com www.jerrycahill.com www.clubcysticfibrosis.com www.youcannotfail.com www.teamboomer.org</p>	<p>Cottonwood, Snowbird Center</p>
<p>10:00 am – 10:15 am</p>	<p>Break</p>	<p>Cottonwood Lobby, Snowbird Center</p>
<p>10:15 am – 11:15 am</p>	<p>Cystinosis Overview Joshua Zaritsky, M.D. State of the World of Cystinosis Craig Langman, M.D.</p>	<p>Cottonwood, Snowbird Center</p>
<p>11:15 am – 12:00 pm</p>	<p>Advocacy presentation</p>	<p>Cottonwood, Snowbird Center</p>
<p>12:00 pm – 1:00 pm</p>	<p>Lunch <i>Complimentary</i></p>	<p>Cottonwood Lobby, Snowbird Center</p>
<p>Panel Sessions 1, 2 & 3 <i>Panel Sessions are targeted towards different stages of the Cystinosis journey, divided into tracks by age. They will be smaller group sessions with a panel of experts presenting information, guiding discussion and fielding questions. Attendees should feel free to attend sessions of most interest to them. Please note the Adult section of Panel Sessions 2 & 3 are closed sessions for Cystinosis adults and their partners only.</i></p>		

<p>1:00 pm – 2:00 pm</p>	<p>Panel Session 1</p> <p>Caregiver/Infant & Child (0-10): Cystinosis 101 <i>Panelists: Drs. Ewa Elenberg, Paul Goodyer, Larry Greenbaum, Jennifer Simpson and Craig Langman</i></p> <p>Teenager (11-17): Cystinosis 201 <i>Panelists: Drs. Rick Kaskel, Minnie Sarwal, Katharina Hohenfellner and Neveen Soliman</i></p> <p>Adult (18+): Cystinosis 301 <i>Panelists: Drs. Bill Gahl, Galina Nesterova, Robert Kleta, Patrick Gipson and Doris Trauner</i></p>	<p>Cottonwood A</p> <p>Cottonwood B</p> <p>Cottonwood C</p>
<p>2:00 pm – 3:00 pm</p>	<p>Panel Session 2</p> <p>Caregiver/Infant & Child (0-10): Physician Q & A <i>Panelists: Drs. Ewa Elenberg, Paul Goodyer, Larry Greenbaum, Craig Langman, Galina Nesterova, Jennifer Simpson and Doris Trauner</i></p> <p>Teenager (11-17): Social Relationships and Gaining Independence <i>Panelists: Maya Doyle, MSW, PhD, LCSW, Jennifer Caughlin, Ph.D.</i></p> <p>Adult (18+): Public Awareness Campaign and Advocacy Healthcare Expectations and Self-Management/Medication Adherence <i>(closed session for Cystinosis adults)</i> <i>Panelists: Jeff Larimore (President, CRN), Drs. Rick Kaskel, Bill Gahl and Patrick Gipson</i></p>	<p>Cottonwood A</p> <p>Cottonwood B</p> <p>Cottonwood C</p>
<p>3:00 pm – 4:00 pm</p>	<p>Panel Session 3</p> <p>Caregiver/Infant & Child (0-10): Education/IEP/504 Plans, Child Life/Snow City Arts <i>Panelists: Marybeth Kruppenacker, Dr. Doris Trauner, Maya Doyle, MSW, PhD, LCSW, Allie Spicer</i></p> <p>Teenager (11-17): Transplant and Dialysis <i>Panelists: Jen Wyman (parent), Drs. Minnie Sarwal, Katharina Hohenfellner, Neveen Soliman, Rick Kaskel and Ewa Elenberg</i></p> <p>Adult (18+): Forum on Depression and Mental Health <i>(closed session for Cystinosis adults and their partners)</i> <i>Panelists: Jennifer Caughlin, Ph.D., Aimee Adelman and Jenn Loglisci</i></p>	<p>Cottonwood A</p> <p>Cottonwood B</p> <p>Cottonwood C</p>
<p>4:00 pm – 4:15 pm</p>	<p>Break</p>	<p>Cottonwood Lobby, Snowbird Center</p>
<p>4:15 pm – 5:30 pm</p>	<p>Medical Panel Host: William A. Gahl, M.D., Ph.D. <i>Please join the entire group for the unique and informative opportunity to have your questions and concerns addressed by the leading physicians and researchers in Cystinosis. All of the doctors who have presented at the Family Conference, all attending Medical Advisory Board and Scientific Review Board members, as well as other health care professionals involved in treating and researching Cystinosis are scheduled to participate. Questions for the panel will be collected during the proceedings today.</i></p>	<p>Cottonwood, Snowbird Center</p>

5:30 pm	Group Photograph	Cottonwood, Snowbird Center
6:30 pm	Meet & Greet Family Buffet Dinner <i>Hosts: Future By Design</i> Complimentary - Sponsored by Horizon Pharma Take time to refuel and relax with a casual dinner complete with good company. Share your learnings from the day and catch up with other families during our meet and greet buffet dinner. Meet the members of "Future by Design", a group of Cystinosis adults with a passion for contributing to the Cystinosis community. A primary group goal is to provide support and encouragement to young adults, parents and children living with Cystinosis. Our youth can have a positive and successful future - let us show you!	Cottonwood & Cottonwood Lobby, Snowbird Center
7:00 pm	Speaker/VIP Dinner <i>Hosts: CRN Board of Directors</i> Closed Session	The Aerie Restaurant, Cliff Lodge, 10 th floor
Saturday, July, 15		Room
7:00 am – 8:30 am	Breakfast <i>Complimentary</i>	Cottonwood Lobby, Snowbird Center
8:00 am – 4:00 pm	Registration/Information	Cottonwood Lobby, Snowbird Center
8:00 am – 4:00 pm	Snow City Arts <i>The CRN 2017 conference has designated the theme "Soaring to New Heights," that has informed the design of a group art project. The idea is to have individuals create representations of themselves as birds in mixed media sculpture forms. Each bird will be uniquely designed. These individual sculptures will then be suspended together to create a collaborative time based hanging art installation of birds flying in a flock in an upward spiral. The upward spiral form was chosen to signify how those impacted by Cystinosis can reach new heights working as one community. The installation will only exist for one night on July 15th in Snowbird, Utah. The art project is designed to reflect the ability for individuals to become stronger, fly higher when they have shared goals and come together to connect in a meaningful way.</i>	Alpine A, Snowbird Center
8:00 am – 4:00 pm	Camp CRN Children's Group Tweens with Cystinosis (ages 8-12) Teens with Cystinosis (ages 13-18) Sibling Tweens (ages 8-12) Sibling Teens (ages 13-18)	Alpine B Alpine C Aspen Willow Studio Room
8:30 am – 8:45 am	Opening Comments <i>Jeff Larimore, CRN President</i>	Cottonwood, Snowbird Center
8:45 am – 9:15 am	Walgreens Specialty Pharmacy <i>Ryan J. Atkinson PharmD, MBA, Senior Director, Health Outcomes</i> <i>Amber Walker, Director, Specialty Pharmacy Services</i> Leadiant Biosciences <i>Nancy Parsons, Vice President of Prescription Products</i>	Cottonwood, Snowbird Center

<p>11:15 am – 11:35 am</p> <p>11:40 am – 12:00 pm</p> <p>12:05 pm – 12:20 pm</p>	<p>Successful fertility treatment in male Cystinosis patient Elena Levchenko, M.D., Ph.D. <i>In general males with Cystinosis are reported to be infertile. We have performed in vitro fertilization procedure in a young male with Cystinosis known with azzospermia allowing him to father healthy twins. In our ongoing project we set out to investigate the mechanisms of male infertility in Cystinosis and to offer treatment options for the patients.</i></p> <p>Clinical studies with Procysbi. What did we learn and the end of an era Craig Langman, M.D.</p> <p>Jennifer Simpson, M.D.</p> <p>Dr. Gahl/Nesterova</p>	<p>Cottonwood, Snowbird Center</p>
<p>12:30 pm – 1:30 pm</p>	<p>Lunch <i>Complimentary</i></p>	<p>Cottonwood Lobby, Snowbird Center</p>
<p>1:30 pm – 2:30 pm</p>	<p>Poster Session <i>This session will showcase a mix of science, medicine, industry, advocacy group and patient experiences to provide an interactive experience for both family and professional attendees. Researchers, clinicians, industry, advocacy representatives, students, patients, and caregivers will be invited to exhibit their latest research findings, treatment breakthroughs, advocacy group updates and real patient and family experiences. This will be an interactive session where exhibitors will be available to discuss their work or experiences with those attending. We invite you to browse the posters and take this opportunity to ask the authors and presenters questions.</i></p>	<p>Cottonwood A, Snowbird Center</p>
<p>2:30 pm- 3:30 pm</p>	<p>Parents of Children and Adults with Cystinosis Panel Moderator: Maya Doyle, MSW, PhD, LCSW Panelists: TBA <i>Panel presentation during which parents of children and adults with Cystinosis will answer prepared questions and address topics related to the use of coping mechanisms through the ups and downs that Cystinosis brings related to not only developmental and transitional issues of daily life but also medical issues. Parents of individuals at every stage of the disease will be featured and will share how they have managed the variety of challenges they have faced. Audience participation will be encouraged.</i></p>	<p>Cottonwood, Snowbird Center</p>
<p>2:30 pm – 3:30 pm</p>	<p>Reproductive Issues Moderator: TBA <i>Closed Session for Cystinosis Adults only</i></p>	

<p>3:30 pm – 4:30 pm</p>	<p>Future by Design Adult Panel Moderator: Aimee Adelman Panelists: Jana Healy, Sara Healy, Emily Mello, Todd Bradley, Rebekah Palmer, Alison Moreno, Jenn Loglisci, Eddie Langley, Briana Dundon, Johnny Davis, Rachel Young <i>Future by Design. What does it mean? Where are we going? We are a generation of resilient adults with Cystinosis, and we want that to continue! Learn about our group’s goals, projects and commitment to our community. Let’s learn from our experiences and create a better future for generations to come. Members of the Future by Design group will present their projects and field questions from the audience.</i> <i>“We are like building blocks and if we don’t all stand together we will all fall apart.” - Future by Design</i></p>	<p>Cottonwood, Snowbird Center</p>
<p>6:00 pm – 10:00 pm</p>	<p>Farewell Dinner Dance Complimentary <i>All conference attendees – pack your dancing shoes and silly faces for our final event of the week – the dinner dance! Before saying goodbye, recharge with a delicious dinner then grab your Cystinosis crew and strike a pose in our photo booth. Finally, the conference isn’t complete until you’ve shown your moves on the dance floor. After all, this evening is to celebrate YOU and our Cystinosis community...Soaring to New Heights.</i></p>	<p>Cottonwood & Cottonwood Lobby, Snowbird Center</p>



See you in Utah!!

Muscle Strength Study at Emory University

Dr. Larry Greenbaum from Emory University will be initiating a study of muscle strength in patients with Cystinosis. Loss of muscle strength is a common complication in adults with Cystinosis. This study will measure grip strength (squeezing a grip for 5 seconds) in patients who are at least 6 years old. The goals of the study are to determine risk factors for decreased grip strength and to determine the severity of the problem in patients with Cystinosis. Describing the problem will hopefully lead to studies of interventions to slow or reverse the loss of muscle strength. Grip strength may be a good way to monitor therapies. Dr. Greenbaum previously published a study in Journal of Pediatrics showing that decreased grip strength is common in pediatric kidney transplant recipients. Study participation will be available to patients at the CRN conference in Utah and to the large number of patients with Cystinosis who are followed at Emory University. Study participation will take about 5-10 minutes.

Featured Adult: Rebekah Palmer's Story

By Rebekah Palmer

I feel an obstacle in being the patient of a rare disease, is the anticipation that time is running out. As a child and young teen, this wasn't much of a problem, but more of an incentive to live with every fiber of my being. Like most children and young teens, the day I would be an adult seemed so far away. Numbers like age 13 and age 18 and age 30 simply were numbers in a reality I had no reason to believe I would ever exist in.

At age 13, life got a little more complicated for me as I was a year out from my kidney transplant and recently in remission from B-Cell Lymphoma cancer. There were lots of moments in my day when I wondered what I would be able to do once high school was completed. Many times

I would lie on the couch, thinking what am I here to do? My body is so worn out already, will I ever be able to do all my mother does in one day? I get tired just after folding laundry! And she goes to work and washes the dishes all in the same day!

At age 18, I headed off to college with a few of my peers. Working an 8 hour shift in addition to classes created extra

fatigue and gastrointestinal problems, so I was taken down to part time hours. The lurking anxiety of "this could be my last day" was becoming more of a force to be reckoned with when my body would limit my physical activity. During student teaching, my health was again tested as working a full teaching day (8:15am-3:30pm) would cause fatigue, migraines and the ability to catch any bug floating around the school. I had better quality of life working half days. I felt like my life was over because I could not put in the hours many single adults were contributing to the work force. I began to experience heightened feelings of boredom, fear, sadness, panic and purposelessness after college graduation at the age of 23. Time had a more urgent feel to put something lasting and impactful onto the world in case I left it soon.

At age 25 I did the thing I thought would leave the most to humankind: I published a book I had written in high school. Now time felt like I had nothing more of value to offer and there were large portions of my day spent in worry and fear. I had written in a



Rebekah, (far right) with five generations of her family

freshman English paper that my life's goal was to publish a book. Instead of making more writing goals at that time, I allowed my depression and anxiety to consume me. Why get a part time job? Why write another book? Why keep up this pretense of finding a spouse? I was only going to be dead soon.

There were a few years of hope as I was in a relationship that had goals towards marriage. This only increased my fear as I felt marriage would be the last thing I ever did in this life. The relationship eventually broke up, which caused a different kind of fear as I looked towards more career oriented goals.

I began to feel utterly useless to my family as

I worked volunteer jobs instead of paid jobs. I had attempted paid part time work, but this caused financial problems with Social Security and more health problems when work was scheduled every consecutive day versus every other day or just in the evenings.

What really helped my uncomfortable and painful feelings was learning to see living in my rare disease body, not as a ticking time bomb or inconvenience to society, but as a different way of teaching other people how to love, have hope, patience and joy. How did I use my negative feelings to create more positive thoughts? Well, I ended up finding an objective therapist and with her help in realizing why these feelings caused upsetting thoughts, I was able to put forth a better effort into living in the present:

- *Having feelings like boredom, fear, sadness, panic and purposelessness forced me to think about what I truly find important in this life.
- *I realized what's truly important in this life doesn't always make money.
- *What are some things I can do with my time that those who are tied to a full time job cannot? My answer to this question varies but I find getting out into the community and giving my time to necessary local causes is a big deal for me and others.
- *What interactions and connections can I make with other people here, right now? I have time to take that tap dance class or audition for that play or attend that political rally.
- *Sharing thoughts, feelings and experiences with others can cause a great sense of accomplishment and community and reason to live.

I honestly believe a huge step in tackling the obstacle of limited time in rare disease is to have an engaging and helpful support group. As I am nearing 30, I still schedule regular appointments with my therapist. Rare disease aside, life wasn't meant to be lived in a vacuum. And if you are single like me, this doesn't mean you can't have supportive relationships. Getting out and engaging with others in friendly ways goes a long way to living your life, many times beyond what a romantic tie might bring. I still have my parents in my life, friends and along with my therapist, life as a whole has become less of an emotional roller coaster and more of a road with bumps along the way.



(from left to right) Jana Healy with her twin sister Sara Healy, Rebekah Palmer and Paula Shal at the 2015 CRN Family Conference in Chicago

A Letter from Rebekah Palmer to the Cystinosis Community

Dear Cystinosis family,

I am a 29 year old woman living with Cystinosis. I wanted to let you know a little bit about myself: I was diagnosed at the age of 5 and received my kidney transplant at age 12. I graduated high school in 2005 and went on to complete my bachelor's of science in secondary Christian education in May 2010. I pursued more classes through a writing institute and a local university. Currently, I volunteer as a tutor with Literacy Chippewa Valley, as a secretary for a local hospital's free clinic, and as a tour guide for a local history museum. I work as an author and piano/English tutor. I live at home with my parents and youngest brother, along with two cats and a beagle.

One thing I always heard growing up from adults were the following pleas: "I wish I could take those pill for you" or "I wish I was sick instead of you" and other variants between the two. I'm sure what the adults were trying to convey was comfort and support by offering to take my pain if they could. What actually came across to me was this: "You

can't live a good life like this", "Life is not fair", "God is cruel to give a child this much pain" and "I don't want you to face life's challenges, only I can do that for you".

As I have observed my own parents through the years responding to my illness, I know it is



Rebekah with her theater friends in the Cavalcade cast photo taken in August 2016. Rebekah is on the left, second row from the top, holding a hat

natural for parents to protect their children and want to make their life's experience wonderful. It's not bad to feel this way by any means, but a better way to channel those natural biological instincts is to love your child no matter how angry or sad you feel watching your child with the complications Cystinosis can bring.

Cystinosis affects every individual with it so differently. For me, it affected my ability as an adult to hold a full time paying job. If your dream for your child is a certain career and they end up elsewhere, voicing dislike for their circumstances can make them feel they have no place in society. If your dream for your child is marriage and their own children and they end up single, voicing dislike over no grandchildren can make them feel their life is a waste. This is not only advice for your child living with Cystinosis, but their sibling living without Cystinosis. Whatever the future brings to your beautiful babies, meet it with love and encouragement and creativity. A normal life doesn't look the same for everyone. Cystinosis may not even be a deterrent for your child in ways it will be for another family's child. The sneaky thing about rare disease is many times it is not the deterrent for the child whose body it affects, but the child whose body it doesn't. How are the siblings doing? Are their mental and emotional needs being met? Do you expect more from them because they have a healthy body? Don't lose hope because life isn't looking like the life you have always expected or like your neighbor's life. Be love. Inspire love to others.

If you are having a hard time with your feelings, don't let this worry or cause you fear. Talk to an objective listener about your family whether that is a professional therapist or an admired mentor. Feelings are your own and can always be changed by one's thought life. It's okay to feel differently as the care taker of a child living with illness, than the child who deals with the illness in their own body.

Hope and love to you and yours,

Rebekah Palmer



The three amigos, Stephanie Trudell Smith, Chris Holleran, and Rebekah. All were attending a Raptor meeting in California in 2015

Financial Update

By Jeff Larimore

The Cystinosis Research Network, Inc

Financial Review – Accrual Basis

For the three (3) months ended March 31, 2017

Revenues

For the three months ended March 31, 2017, total income collected of \$234,000 was significantly greater than the same period in 2016. This increase was attributable to additional corporate grant contributions received and fundraising events to support new program services.

Expenses

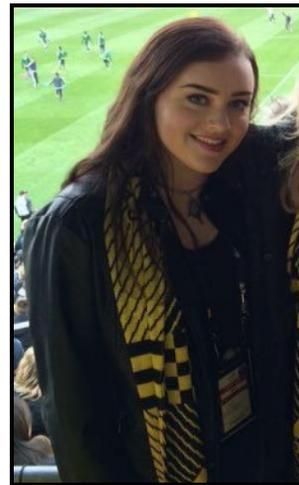
Total operating expenses of approximately \$149,000 were also significantly higher than operating expenses for the three months ended March 31, 2016. CRN has funded \$115,000 in research grant installments during the first quarter of 2017. There were no research grants funded during the three months ended March 2016.

Education, Marketing, and General operating expenditures through March 2017 of approximately \$28,000 were consistent for the same reporting period in 2016. Expenses related to members of the cystinosis community participating in Rare Disease Week activities in Washington D.C. and a CRN sponsorship to support the Cystinosis breakout session held at the Pediatric Academic Society annual conference were two of the primary, non-research grant payment, expenditures during the quarter. G&A costs as a percentage of income decreased from 7.0% to 1.5% due to the improvement in 2017 inflows.

CRN had net operating income of \$85,000 for the three months ending March 31, 2017 as compared to net operating income of \$30,000 through March 2016. Continuous fundraising activities and generous corporate support in 2017 provides cash resources to increase patient advocacy and family support activities in 2017 and beyond to support the cystinosis community.

Cash on hand at March 31, 2017 was \$335,000. Net change in cash for the first quarter 2017 was an increase of \$66,000. The increase approximates the amount of net income for the first quarter less year-end 2016 accounts payable.

CRN Announces 2017 Scholarship Application Deadline



CRN's 2016 Scholarship winners, Shea Hammond, Will Patterson, and Alex Greeley

Cystinosis Research Network is pleased to offer (at least) two \$1,000 Scholarships this year. One is for an individual with Cystinosis. The other is the Sierra Woodward Sibling Scholarship offered to the sibling of an individual with Cystinosis.

Information about, and forms for both scholarships, can be accessed on CRN's website:

<https://cystinosis.org/family-support/scholarships>. The deadline to receive the applications is August 15th, 2017.

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

Completed applications should be received at the address below by August 15, 2017 to be considered:

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

Identical twins battle disease that afflicts only 500 in the U.S.

(Editors note: The following article first appeared in the Duluth News Tribune on March 28, 2017)

By John Lundy

The 33-year-old sisters also share an organ-destroying disease that they have in common with only about 500 people in the United States. It's a lonely thing to share.

"Nobody here has heard of cystinosis," said Sara, who lives with her boyfriend in Duluth and cooks at the Benedictine Health Center. "Nobody in Fargo knew about cystinosis," she said of the North Dakota city where Jana lives and cooks at a federally supported day care.

Certainly their parents, Jane and Dennis Healy of Williston, N.D., knew nothing of cystinosis as they saw their sixth and seventh children's development virtually stop by the time they were 1.

"They were 22 pounds and thriving and doing a real good job," recalled Dennis, a retired teacher and coach, in a telephone interview from Williston. "All of the sudden, they just threw the bottles out. ... They didn't want to eat anything." Doctors advised them to give the girls Vitamin D and milk, he said, but it didn't seem to help.

When the time came that Jana couldn't stand, the Healys decided they needed to take more action. They brought the twins to a hospital in the state capital of Bismarck, where they were advised to see specialists either in Omaha, Neb., or Minneapolis. They chose Minneapolis, where they had family connections.

At the University of Minnesota Children's Hospital, the doctors told them there are 2,500 kidney diseases, and the only one they needed to worry about was cystinosis, Dennis recalled. Because Jana was too sick to handle it, Sara underwent a battery of tests, Dennis said.

The result was cystinosis.

CLINICAL TRIAL

The disease attacks the human organs, starting with the kidneys. Without treatment, the girls likely would experience kidney failure by the time they were 9, the Healys were told. But there was no cure, and no treatment.

However, a young fellow at the U of M, Dr. Thomas Wells, took an interest in the twins' case. Wells, now a pediatric nephrologist at Arkansas Children's Hospital and a

professor at the University of Arkansas College of Medicine, eventually learned of a clinical study underway at the University of California, San Diego. The Healys were able to get their daughters enrolled in the study.

"That clinical study probably saved our lives," Sara said. "We could get somewhat healthier and get on a normal routine."

The drug being tried, Cystagon, later was approved by the Food and Drug Administration for treatment of cystinosis.

Four years ago, the FDA approved Procysbi, which Sara now takes instead of Cystagon. The newer drug only has to be taken twice a day instead of four times, she said, and it doesn't come with the unpleasant side effects of Cystagon — bad breath and body odor. But Procysbi comes at an exorbitant price: \$250,000 a year, Sara said. Her insurance plan is covering it, at least for now.

Their mother, a lab technician, was constantly doing battle with insurance companies as they grew up, Sara said, particularly when coverage could be denied for pre-existing conditions. The struggle continues.

"Fighting with insurance, it's an ongoing thing," Sara said. "They don't want people with rare diseases on their insurance because that's going to cost them too much money."



Twin sisters Sara (left) and Jana Healy, 33, hang out with their dogs Angel (left) and Rosewell recently. When they were 18 months old the sisters were diagnosed with cystinosis. The rare disease causes cystine crystals to accumulate in every cell in the body. Steve Kuchera / skuchera@duluthnews.com 1 / 4

'NOT WHO WE ARE'

As the girls grew up, their normal routine didn't match

those of their schoolmates. They tired easily and weren't able to participate in sports, Sara said.

They both sang in choir. Sara continues to enjoy singing, particularly karaoke. Jana's outlet is oil painting. But they still felt isolated in their school. So they decided to do a presentation about their disease to their high school classmates "to help them understand that this disease, it's not our life," Jana said in a telephone interview. "It's not who we are." The attempt backfired.

"They took it the wrong way," Jana said. "They kind of twisted the facts, and they somehow thought that we were going to die."

Although it wasn't as dire as that, it was true that the twins were — and are — afflicted with a progressive and incurable disease. Four years ago, Jana received a kidney transplant; older sister Jennifer was the donor. Jana said she's thankful that her original kidneys functioned as long as they did.

"Most people our age (with cystinosis) have had at least one or two kidney transplants by now," Jana said.

Sara has been on the list for a kidney transplant for two years, with no living donor currently available. She was told to expect a wait of three to five years, Sara said, and she's hopeful that will happen before she requires dialysis.

Both take eye drops for a condition related to cystinosis. Jana is recovering from a bout with double vision with the help of a change in her medication, she said.

ADVOCATES

Meanwhile, the twins still felt alone with their disease. "We grew up thinking we were very rare and the only one of a kind, especially in North Dakota," Jana said.

A few years ago, they took to the internet and discovered the Illinois-based Cystinosis Research Network, which led them to a patient support group.

"To meet all those ... people our own age and younger kids and adults and some who are parents with cystinosis, it really brings up your hope," Jana said. "It really cheers you up to know that you're not alone."

It also led them to become politically involved in the cause of promoting research into rare diseases. Late last month, Sara traveled to Washington to meet with other advocates and congressional representatives during Rare Disease Week. Jana was unable to attend, she said, because her eye problems had caused her to lose too much work time. Instead, Dennis went with his daughter, advocating on behalf of North Dakota while Sara advocated on behalf of Minnesota.



Sara and her dad, Dennis Healy, advocating for Cystinosis during Rare Disease Week on Capitol Hill, February 27th to March 3rd

Sara came back energized about the issues presented in Washington. She's particularly speaking out on behalf of the OPEN Act, which was introduced in 2015 by Sens. Amy Klobuchar, D-Minn., and Orrin Hatch, R-Utah, to provide incentives to pharmaceutical companies to "repurpose" previously approved drugs for the treatment of rare diseases.

The legislation is vital, Sara said, because 95 percent of rare diseases have no FDA-approved treatment, and developing new drugs is extremely expensive and time-consuming. And although each of the particular diseases is rare, being afflicted with a rare disease isn't uncommon. More than 30 million Americans — one in 10 — are affected by one of more than 7,000 rare disorders, according to Washington-based Rare Disease Legislative Advocates. The group defines a rare disease as one afflicting fewer than 200,000 people in the U.S. An "ultra-rare" disease, such as cystinosis, affects fewer than 6,000 people.

Sara also advocates increased funding for the National Institutes of Health, which is targeted for a nearly 20-percent cut in President Donald Trump's proposed budget.

"They do a lot of clinical trials and the clinical research to help find treatments for these rare diseases," Sara said. "They can't help if they don't get the funding."

The twins' mother Jane, now retired, said she knows that being able to enroll her daughters in the clinical trial made all the difference.

"Thank God they had a medicine they were working on," Jane said. "Most of these rare diseases, there isn't anything." But according to Jane, another factor has allowed her daughters to persevere as well as they have.

"These girls have had to battle and battle and battle," she said. "They're tough little cookies. They take it in stride."

Follow Us On Social Media

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



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[@CystinosisResearchNetwork](https://www.instagram.com/CystinosisResearchNetwork)



[@CystinosisCRN](https://twitter.com/CystinosisCRN)

Kirmera Salon Holds CRN Fundraiser in Honor of Laura Krummenacker

By Marybeth Krummenacker

The owners and stylists of Kimera Salon came to Laura Krummenacker with an idea to do a fundraiser for CRN. Laura is the front end receptionist at the Salon. They called it B.Y.O.B. ...Bring Your Own Blowdryer". They picked a date of Saturday, March 18th, from 4-7 p.m. The shop was closed during that time and people were invited (via word of mouth and Facebook) to join them for a "girls night out" for a good cause.

The idea was to spend some time with the hairstylists showing you how to style your hair on your own so you look like you just came from the salon...every day! The charge was \$25 and it was advertised that all proceeds would be donated to Cystinosis Research Network. They had signs throughout their shop for about a month asking people to attend and Laura supplied them with the informational CRN brochure describing Cystinosis.

The stylists asked a few of the local merchants in the shopping center to donate some hors' devours for refreshments, which they graciously did. There were raffle baskets which were on display and any customer could purchase raffle tickets for



The owners and staff from Kimera Salon held a CRN fundraiser in honor of Laura Krummenacker (second row, second from the left next to mom, Marybeth). This first time event raised over \$1,000 for CRN

them a few days prior to the event. Between 25-30 people attended this first time event, which was a huge success, with over \$1,000 raised for CRN. Thank you very much to Laura's family and friends for attending, and to Kimera Salon and Staff for opening your door and your hearts to CRN.

New Name. Same Rare Dedication.

Continuing our support of the
Cystinosis Research Network



Formerly Sigma-Tau Pharmaceuticals, Inc.



Jack Greeley Celebrates his Golden 17th Birthday – Raises over \$300,000 from Birthday Letter Fundraiser

By Christy Greeley

The Greeley Family once again celebrated Jack's St. Patrick's Day birthday – this year a special Golden 17 on 3/17/17 -- by sending out the 15th edition of his birthday fundraising letter. As is his way, Jack has faced many challenges in the past year with his characteristic grace, courage and positive attitude. We cannot thank our friends and family enough for all of the support we have received, this year and since his initial diagnosis in 2001. Since 2003 we have raised over \$300,000 in honor of Jack to support CRN and the Cystinosis community, thanks so much to everyone who has taken part over the years.

Following is this year's letter:

March 17, 2017

LADIES & GENTLEMEN!

Welcome to the Light Heavyweight Championship of the World! Standing in this corner at 5 feet, 10 inches tall, weighing 171 pounds...a junior at Adlai Stevenson High School...from Lincolnshire, Illinois...Jack David Greeley!!

Okay, that's some hype; and, yes, Jack has always been a fighter, but after two successful years of growth hormone treatments, our Champ does now comfortably sit at the 75th percentile for height and weight for his age group. Ready for his Golden Birthday when he turns 17 on the 17th this St. Patrick's Day (cheers to all!), 16 years ago it was realistic to think Jack would



Jack Greeley with his sister Alex Greeley

never grow to 100 pounds or see his teenage years, let alone a 75th percent ranking or 17 Earth rotations around the Sun.

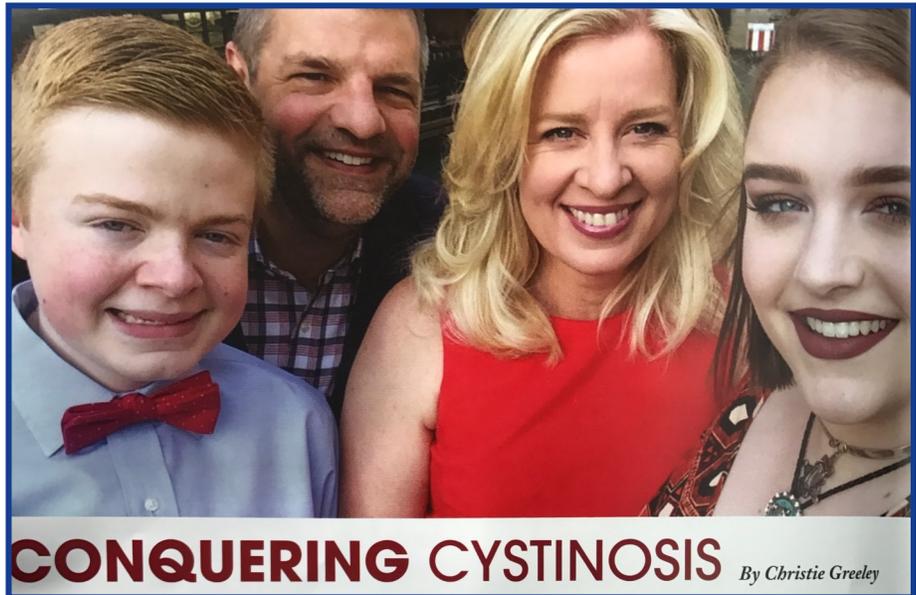
Here is what many of you know. Our son, Jack has Cystinosis, a rare metabolic, genetic disease. It presented itself around his first birthday in 2001 and we learned it has the potential to impact all of the organ systems in the body leading to kidney failure, muscle wasting, diabetes, blindness, pulmonary deficiency, hypothyroidism, and neurological damage. Kidney transplants are more common than not. Failure to thrive (grow) happens, which prompted us to start the growth hormone treatment. Jack's kidney function remains abnormally good, but over the past several years he has developed unusual orthopedic problems in his lower body. Without correction, he is likely to have much worse mobility and pain issues as he gets older. We have been delaying a lower leg surgery due to timing with other medical triggers and its invasive nature and long recovery. It appears we might now opt to have that procedure this June on his left foot and take the summer to recover. Stay tuned.

Jack continues to inspire us each and every day with the challenges he faces. While he did not choose his fight, he has accepted it. Jack's growth – which can be seen both literally and figuratively – has come from not only his fight, but also his commitment and spirit. Physically, mentally, emotionally, socially, academically, and spiritually, Jack's growth has been significant.

Physically, Jack was born above the 95th percentile. Dave remembers his delivery, standing there, seeing that meat hook of a hand follow the head...just dangling there, HUGE...“please, Please, PLEASE be a boy!!” was the coherent thought. Once Jack got sick at a year old, he began to heavily urinate and profusely vomit for about 18 months. He eventually got a gastric feeding tube and became stable over the next five years, but at that point he almost fell off the growth chart.

Jack has been picked, poked, prodded, and nagged so many times now that his stat-lines could be displayed on McDonald's marquee – 150,000 pills swallowed, 5,800 consecutive days awakened overnight for meds, 50,000 eye drop applications, and well over 500 doctor appointments. His vigilance and conformance has put him in a reasonably good situation physically, which is most pronounced with his aforementioned growth.

Mentally, Jack is so tough, even if it does not seem obvious. Because of what he deals with physically, he has a mental attitude or edge that even he does not understand or recognize. Once he fully grasps this as he continues to mature, the world will be his french fry (he doesn't like oysters). There are two anecdotes that strike us here. Whenever Jack goes to see his orthopedist, she asks him if he is feeling any pain. “Nah, not really” is what Jack usually mumbles, thus triggering Mom, Dad, and doctor to kind of smile at each other in recognition that Jack probably does not know what his pain feels like; it's just his normal. Then, maybe a couple



Article from *Heart of Lincolnshire*, winter 2016

of times a week, Jack drives himself to his 4,000 student high school. He pulls into the big parking lots and finds a handicapped spot up front (which we have chosen to take advantage of), but before he leaves the car, he must change his sneakers from the regular variety that give him full ankle motion to control the pedals to size 13.5, 4E shoes bought from the New Balance specialty store so that his leg braces will fit into them. Think about it...on display...in a high school parking lot. Here is an unsolicited tip. If you are having a bad day, think of Jack...seriously. It helps us all the time. You can do it, whatever it is, we know you can!

Emotionally, Jack is introverted and mellow by nature (how did that happen? Ha!). He might also be the happiest kid in town. If the typical person got up in the middle of the



2016-17 Stevenson High School's varsity basketball team

night to take medicine at 3 am for over 5,800 consecutive nights, how happy would he be? Jack never falters...a smile, a grin, pearly whites, and a wide, blue-eyed twinkle. Jack's increased physical stature has been a big boost for his emotional growth too. Becoming the second tallest person in his immediate family has been cause for celebration. He has gained confidence and has learned how to express himself better and when he does so now, it is eye-to-eye, which feels a little freaky (in a good way). According to big sister, Alex that self-expression is code for "sass." Mom and Dad prefer to think of it as independence.

Socially, Jack has made some nice progress with room to still grow, which will be important with college on the horizon. So much of Jack's adolescent development and general lag can be tied to his initial diagnosis 16 years ago where he fought for survival

and fell behind. He received his driver's license and short of the one-time our garage door wall abruptly got in his way backing out, he has done a nice job. He likes driving to basketball games where he remains a student manager and table tennis events. The trend lines here remain good.

Academically, Jack is a dedicated and committed student who wants to do well and is willing to put forth the work to achieve results he deems acceptable. He earns mostly A's in a curriculum you would hope your high schooler takes – Physics, Spanish, English, History, Algebra II, etc. He has begun the college application process, including ACT prep and tutoring. This next chapter will prove to be a monumental growth opportunity for Jack as he marches toward becoming a college student. There are no favorites yet for prospective schools, although Marquette seems to be an early nice fit...plus Mom and Dad really like how Jack looks in blue and gold!

Spiritually, Jack benefits greatly. Although we are not overly religious, he has a God-given spirit within him that borders on divine. Only one time have we heard Jack express self-pity for himself. In full disclosure, when he did it, he was on a morphine drip post surgery. The Bible is filled with scripture on self-pity and it hints that it is common for people who feel sorry for themselves to be miserable and there is no one we know who is less miserable than Jack.

Jack's growth inspires us and knowing that there are others like him, it drives our efforts at the Cystinosis Research Network (CRN). Like Jack, CRN continues to grow and evolve. Just this past December, CRN represented its community at an international conference in Salzburg, Austria attended by over 40 international doctors and scientists who gathered to address key, emerging issues in the treatment of Cystinosis, including bone development that affects Jack. To date, CRN has also raised over \$4MM to fund research for improved treatments (and ultimately a cure) and support of families dealing with Cystinosis. In fact, this Greeley letter writing campaign has raised over \$250,000 for that cause due to support of wonderful friends and family like you.

The fight never fades for Jack. Like so many others who are dealing with their own challenges in life, we all get into the ring every day. For us, we have had a ringside seat for 16 years where the now light heavyweight, Jack has taught us what the pop star, Christina Aguilera belts out in her hit song, *Fighter*. "...makes me that much stronger... makes me work a little bit harder...it makes me that much wiser. So thanks for making me a fighter. Made me learn a little faster...made my skin a little bit thicker...makes me that much smarter..."

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

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

Send your contact info to the e-mail listed to learn who is in your area. It's that easy!

Research Update

By Christy Greeley, VP Research, Executive Director

A major focus of the Cystinosis Research Network continues to be a determined effort to secure a promising future for the Cystinosis community through the support and



Dave, Christy, Alex and Jack Greeley

funding of research grants that lead to improved treatments and ultimately a cure for Cystinosis. CRN has funded over \$4.25 million total in research grants and fellowships. CRN has funded a Cystinosis fellowship at the National Institutes of Health and has funded research and education programs in the United States and many countries around the world including Egypt, Mexico, England, Scotland, Italy, Belgium, France, Germany and much more. CRN research topics are aimed at every aspect of Cystinosis with the purpose of understanding the disease and finding improved treatments and a cure.

Topics include research and therapies related to neurological, genetic, ophthalmological, gastrointestinal, muscular, bone, nephrology, pulmonary, skin, improved medications, and psychological effects of the disease.

CRN is currently funding three grants. These three grants represent one established Cystinosis researcher in Dr. Levtchenko's group in Belgium, one esteemed neuroscience lab at Montefiore in the Bronx led by Drs. Foxe, Molhom and Walkley which is expanding their work into Cystinosis, and one innovative newborn screening initiative in Germany led by Dr. Hohenfellner. Extensions for an additional year of funding have been approved for Dr. Levtchenko's study as well as for the group at Montefiore, including additional travel funding for the Montefiore study to enable more patients to be enrolled.

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Grant Updates:

Layman's Explanation: Altered protein kinase signaling as a cause of reduced adhesion and increased motility of renal epithelial cells in Cystinosis

By Elena Levtchenko, MD, PhD

Short report

Cystinosis is a genetic disease manifesting early in life (\approx 6-12 months) with progressive kidney disease resulting in renal failure early during childhood if not treated. In Cystinosis the metabolism of the amino acid cystine is defective leading to its accumulation in the kidney and other organs. This cystine accumulation results in cellular damage, but the direct mechanisms beyond this phenomenon are largely unknown. Some harmful cellular events in Cystinosis might not be directly related to cystine accumulation and are the subject of our research project. In our previous work we demonstrated that both cellular motility and adhesion were altered in cultured human podocytes derived from Cystinosis patients.

We tried to explore the mechanisms beyond this cellular loss. In cystinotic podocytes we found an increased expression of activated or phosphorylated Akt kinases compared to control cells. This could explain, at least partially, the abnormal phenotype.

We studied the adhesion of different podocyte cell lines (both cystinotic and control) with the use of different adhesion surfaces such as collagen IV, laminin and fibronectin. Interestingly different cell lines preferred different surfaces for adhesion as the control podocytes adhered more to collagen while cystinotic podocytes preferred fibronectin. We also analyzed the protein structure of different adhesion molecules through the proteomic analysis by mass spectrometry in both control and cystinotic podocytes, but these experiments are still ongoing.

We further plan to knock-down the CTNS gene in control podocyte cells (the knocking-down protocol is currently being established in our lab), and compare the gene expression profile of many genes involved in motility and adhesion in the cells before and after knocking down of the CTNS. And we are going to perform the proteomic analysis for the knocked-down cells in comparison with the controls. Then we are going to combine the genetic expression data with the proteomic data to explain the mechanism behind the altered motility and adhesion in the cystinotic epithelial cells.

We also established a new zebrafish animal model expressing many of the Cystinosis manifestations including cystine accumulation and the early tubular and glomerular dysfunction [Elmonem et al, Scientific Reports, 2017]. We believe that this zebrafish model can be suitable for the study of the disease pathology, and can be also suitable for the detailed study of toxicity and efficiency of new potential drugs that can improve the disease therapy.

Call for Proposals 2017:

Applicants may now submit proposals for the 2017 CRN Call for Research Proposals.

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

Priority is given to interventional research, both clinical and basic, that will lead to improved treatments for Cystinosis. New investigators are particularly encouraged to apply. CRN has a strong interest in funding projects related to advancing Newborn Screening for Cystinosis – applications regarding this subject will have priority.

Applicants must submit an electronic copy of their proposal to:

Christy Greeley, Vice President for Research cgreeley@cystinosis.org.

Submission deadline is June 1, 2017. Proposals will be reviewed in July and notification will be made to applicants in August.

Detailed information on the grant submission process can be found on the CRN website at: <https://cystinosis.org/research/grant-guidelines>

Research Volunteer Opportunities:

Three studies will be conducted at the CRN Family Conference in July in Snowbird, Utah. One will examine grip strength in Cystinosis patients, one will seek to understand how the brain processes and integrates sensory information in children diagnosed with Cystinosis using EEG testing, and one will screen for CTNS nonsense mutations by taking saliva samples with the goal of developing new treatments. Information is forthcoming regarding the grip strength study and will be sent to conference attendees and posted on the CRN website. See page 19 for more details. Information on the other two projects are on pages 39 and 41.

CRN to sponsor Cystinosis Session at May American Society of Pediatric Nephrology/ Pediatric Academic Societies Meeting

The Cystinosis Research Network was proud to sponsor a session that was held during the ASPN Annual Meeting in San Francisco, CA, May 6-9, 2017 entitled, Cystinosis: The Evolution of a Rare Disease. Session description as follows:

Researchers in Cystinosis have made tremendous scientific advancements in the pathophysiology and management of this rare disease. Today, most children with Cystinosis are living beyond the pediatric phase of their lives due to early administration of cysteamine therapy, as a result there may be an increasing prevalence of the long-term complications of Cystinosis. In addition to cysteamine therapy, there are efforts to eradicate the disease altogether through novel stem cell therapy. This session will provide a broad overview of the history of cystinosis, the current state, and the innovative research that will change the path forward.

CRN is proud to support Cystinosis education for professionals and involvement in the PAS 2017 meeting was an ideal way to fulfill that portion of our mission. Representatives from CRN also be exhibited at the meeting to further educate the pediatric nephrology community about Cystinosis.

HAS YOUR CHILD BEEN DIAGNOSED WITH CYSTINOSIS?



If so, he/she may qualify for a research study.

Our goal is to understand how the brain processes and integrates sensory information in children diagnosed with Cystinosis. We will use EEG to observe the reaction of your child's brain while he/she listens to sounds and views images.

We will be collecting data at the 2017 CRN Family Conference!

If you would like to participate in our study or to know more about it, please call us at: (718) 862-1824 or e-mail us at:
ana.alvesfrancisco@einstein.yu.edu

Participants are compensated \$15/hour

The Sheryl and Daniel R. Tishman Cognitive Neurophysiology Lab
Albert Einstein College of Medicine, Van Etten, 1st Floor, C-Wing
1225 Morris Park Avenue, Bronx, NY 10461

www.cognitiveneurolab.com



CRN Exhibits at PAS Meeting in San Francisco May 6-9

By Terri Schleuder

Carl and Terri Schleuder had the privilege of “manning” CRN’s exhibit booth at the Pediatrics Academic Societies Meeting 2017 in San Francisco from May 6-9. As always, the opportunity to raise awareness about Cystinosis among the many attending physicians was very satisfying.

In addition, they were able to attend a Cystinosis Symposium, partially funded by CRN, titled: ***Cystinosis: The Evolution of a Rare Disease***,

moderated by Dr. Ewa Ellenberg and Dr. Christian Hanna. The symposium offered four presentations by researchers that were informative and even groundbreaking that offered hope to the Cystinosis community. The presentations included:



Carl and Terri Schleuder exhibit for CRN at the 2017 PAS meeting held in San Francisco, May 6-9



Dr. Stephanie Cherqui, Dr. Mary Leonard, Dr. Jennifer Simpson, and Dr. Daryl Okamura

- ***History of Cystinosis*** by Dr. Daryl Okamura,
- ***Nanowafer: A New Therapeutic Intervention for Corneal Cystinosis*** by Jennifer Simpson
- ***An Update on the Long term Bone and Muscle Manifestations of Cystinosis*** by Mary Leonard
- ***Stem Cell Therapy in Cystinosis*** by Stephanie Cherqui



New Cystinosis Study from McGill University Montreal Children's Hospital (CTNS Nonsense Mutation Screen - CyNoMuS)

The purpose of this message is to invite cystinosis patients to join our upcoming study concerning a specific type of genetic alteration called a “Nonsense Mutation”. Although cystinosis is caused by many different disruptions of the CTNS gene, we are particularly interested in “nonsense mutations” which trick the cell into stopping production of Cystinosis protein. Several pharmaceutical companies are working hard to develop medications related to a well-known antibiotic (gentamicin) that permits the cell to disregard nonsense mutations.

Nonsense Mutations are estimated to account for about 10-15% of cases in other genetic diseases, but the prevalence in cystinosis is unknown and there is some evidence that CTNS Nonsense Mutations are clustered in certain regions. Looking forward, cystinosis patients may wish to know whether or not they carry a nonsense mutation. We would like to survey the cystinosis community and characterize the prevalence and distribution of Nonsense Mutations among cystinosis patients in North America and Europe. This would simply involve (prepaid) mailing a sample of saliva to our research group at McGill University Children's Hospital in Montreal. We would analyze the sequence of your *CTNS* gene and let you know for future reference whether or not you carry a nonsense mutation.

Please contact us by phone: (514) 412-4400 ext. 22953 or by email:
Murielle.akpa@muhc.mcgill.ca

Principal Investigator:

Paul Goodyer, M.D.

Professor of Pediatrics at McGill University

Department of Pediatric Nephrology, Montreal Children's Hospital

Study Coordinator:

Murielle M. Akpa, PhD

4th Annual "Chandler's Chance" Fundraiser for CRN Honoring Chandler Moore

By Clinton Moore

On December 3rd, 2016, we hosted our 4th annual Chandler's Chance Christmas Palooza for Cystinosis. This event has become much easier to plan and execute over the years and this year was our best one yet. This year, we are proud to say, was very well attended at just a few shy of 500 people. We had 10 Cystinosis families join us as well. All of the children were entertained with firetruck rides, hay rides, Santa Claus, characters, cookies, games, and much more; while the adults were entertained by a wonderful local band known as the Dirt Road Outlaws.

It is always a very entertaining and emotional evening with family, friends and new faces each year. It's fulfilling knowing that you are raising awareness and funds at the same time. Next year's event is already being planned, and with some new additions, should be our largest and best year to come.



Sarah Larimore visits with Santa at Chandler's Chance Christmas Palooza for Cystinosis



(Left)- Chandler Moore with Michelle Frey



(Above)- An array of characters enjoy the fun at Chandler's Chance Christmas Palooza for Cystinosis

2nd Annual Nemours Cystinosis Family Education Day Held Dec. 3rd

By Clinton Moore

On December 3rd we co-hosted the 2nd annual Nemours Cystinosis Family Education day right here in our hometown of Georgetown, Delaware, with the help of our wonderful friends, Jill and Clay Emerson. Jill's daughter, Brooke, and my son, Chandler, are seen and treated at the same nephrology clinic located at A. I. Dupont Hospital for children in Wilmington, Delaware. We worked and coordinated this year's event along with Dr. Zaritsky and Dr. LaRosa.

It was well attended by 10 Cystinosis families along with representatives from Horizon and Leadiant Pharmaceuticals and Caughlin Research.

Dr. Paul Grimm gave a wonderful Overview of Cystinosis lecture and keyed in on some facts that I think were new to all of us in attendance. We then heard from Dr. LaRosa as he presented a fabulous lecture about kidney transplantation and the processes involved with it. To wrap things up we heard a very touching few words from the mother of a young adult with Cystinosis, Ms. Marybeth Krummenacker. Marybeth spoke about her own personal story, as well as touched on just how far we've come in this journey with



Clint and Annie Moore and Jill and Clay Emmerson, co-hosts of the event



Children enjoying each other at the Nemours Cystinosis Family Education Day

Cystinosis. She also stressed how important families are to one another. She mentioned how important it was for her to have support from other families and urged us all to continue to advocate for our children.

Everyone in attendance had time over lunch to visit with each other and meet new families. After the meeting, all attendees had a few hours to relax before attending the 2nd part of the day, which was the 4th annual Chandler's Chance Christmas Palooza for Cystinosis which was held that evening.

Thank you to all who attended and hope you all return next year.

And a very huge heartfelt thank you to Jill Emerson for your dedication and thought that you put into this to help make it such a huge success.

Education & Awareness Update

by Terri Schleuder—Vice President, Education & Awareness



Terri Schleuder, VP of Education and Awareness

The first half of 2017 finds the CRN Board of Directors in full conference planning mode. The theme this year of “Soaring to New Heights”, is figuratively and literally true, as this year’s CRN Family Conference will take place at Snowbird Ski and Summer Resort in the beautiful Wasatch Mountains of Snowbird, Utah. Registration packets were mailed out in early April. Scholarships have been offered to help with expenses. Participants may also register online on CRN’s website at:

<https://cystinosis.org/events/conferences/event/142-2017-crn-family-conference>

Please plan to join us for an unforgettable, life changing experience. Reconnect with old friends and meet new ones. Know on this Cystinosis journey you are never alone.

In late February and early March seven members of the Cystinosis community headed to Washington D.C., to participate in Rare Disease Week on Capitol Hill. The opportunity to raise awareness for so many rare diseases with congressional members was very empowering.

Hundreds of attendees met with congressional representatives from 48 states on Wednesday, March 1st. We shared our concerns and asked for their support on issues critical to this vulnerable population. Time will tell if our collective voices were loud enough to be heard and followed through with positive legislative action.

In addition, since our last newsletter, CRN has exhibited at the American Society of Nephrology (ASN) meeting held in Chicago, from November 17-19 and the Pediatric Academic Society (PAS) meeting held in San Francisco, May 6-8. If even one physician



Steve and Terri Schleuder enjoy the Rare Artists’ exhibit at Rare Disease Week on Capitol Hill in Washington, D.C. on March 2nd

learns about Cystinosis and how to treat it, as a result of raising Cystinosis awareness at these meetings, then our goal has been a success.

Our adult Cystinosis population is becoming more engaged in the community as they discuss and address their needs. They met in March in Minneapolis at the 2nd Future by Design meeting.

As 2017 pushes on, CRN will strive to continue to support our community, provide Cystinosis education and awareness to the medical and lay communities we meet, and financially support research projects that promise to improve treatments and ultimately find a cure for Cystinosis. As always we remain committed to the Cystinosis Community.



"Dream, Achieve, Inspire"

Cystinosis Research Network

CRN was honored to present the debut of a traveling ART exhibit featuring Artists of all ages, from all over the world, who live with Cystinosis everyday at the 2015 CRN Family Conference, held in Chicago, Illinois, July 16th–18th, 2015. Since then the exhibit has traveled to São Paulo, Brazil and Mexico, featured as part of the 8th Cystinosis Medical Symposium held in Mexico City on August 5th 2016. It is now available for viewing at: www.DreamAchieveInspire.org.

Development Update

By José Morales—Vice President, Development

Time - “a non-spatial continuum that is measured in terms of events which succeed one another from past to present to future”. I remember family and friends saying enjoy the moments with your children, as it will pass too quickly. Today, when my children ask me what gift I would like for my birthday, Christmas, etc. my response is: “your time and undivided attention”. The most valuable gift one has to give is time. We allocate it to a diverse array of things...simple things to keep the household going...to more complex things like deciding how to provide for your children’s education and security. One of the things I dedicate time to is serving on the Cystinosis Research Network’s (CRN) Board. Some people may ask why I dedicate time to CRN given all other competing priorities. The answer is simple really; CRN was there for us when we first began on this journey of learning how to cope with Cystinosis. CRN has provided us with an extended network of resources to learn from and leverage, as we contend with the diverse array of challenges that present themselves as our child grows into an adult. CRN gives us hope the future will be better, and one day bring us a cure. We remain committed to CRN’s vision and mission:

Vision - The Cystinosis Research Network's vision is the acceleration of the discovery of a cure, development of improved treatments and enhancement of quality of life for those with Cystinosis.

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

CRN has a comprehensive set of programs and initiatives in place to achieve its mission under the stewardship of various committees.

Core Committees Representative Responsibilities:

- Education and Awareness
 - Education/ awareness execution
 - Key non-profit partnerships
 - Public Relations
 - Website Oversight
- Family Support
 - Support programs execution
 - Internet Support Group Oversight

- Reference Documents Archives
 - New Families Onboarding
 - Research
 - Research Prioritization
 - Advisory Committees Management
 - Grant Application Oversight
 - Development
 - Fundraising Stewardship
 - Marketing Programs
- Corporate Alliances / Relations

The broad number of initiatives and activities in place require support in the form of volunteers and funds. The Cystinosis Research Network’s Development Committee is committed to assisting families in sponsoring fundraising events. The following are individuals from our community that have taken on the responsibility of assisting in raising the funds on behalf of the organization:



Velyna, Christian, Alex, and José Morales enjoying family time

Development Committee

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

Corporate Sponsorship

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

Fund Raising

- Rachel DaLomba - rdalomb@my.wgu.edu
- Katie Larimore - klarimore112@sc.rr.com
- Briana Dundon- bri_ana15@hotmail.com
- Jen Wyman - jwyman@comcst.net

Administration

- Christy Greeley - Greeleycd@aol.com
- Jen Wyman - jwyman@comcst.net



Congratulations to Christian Morales, who graduated from college on May 21st, 2017

Be sure to read the articles highlighting our community's continuing commitment to making a difference:

Chandlers Chance - held in honor of Chandler Moore and sponsored Clinton and Annie Moore (see page 42).

Bring Your Own BlowDryer - held in honor of Laura Krummenacker and sponsored by Antonio Vozzolo, Kimēra Salon (see page 30).

Jack Greeley Golden 17th Birthday Letter –held in honor of Jack Greeley and sponsored by Dave and Christy Greeley (see pages 32-35).

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

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



Christian and Alex Morales

“It reminds us that we are judged not by how we treat the powerful, but how we care for the least among us.”

— Joseph Kennedy III

Cystinosis Bone Meeting held in Salzburg, Austria, December 8-10, 2016

By Christy Greeley

Christy Greeley, CRN Executive Director and Vice President, Research, attended the Cystinosis Bone Meeting in Salzburg, Austria in December as an invited patient representative on behalf of CRN and the Cystinosis community. The conference was hosted by Dr. Katharina Hohenfellner, Cystinose-Zentrum, Traunstein, Germany. Dr. Hohenfellner is a CRN funded investigator. Her group is working on postpartal screening for Cystinosis with urine dipsticks in 12 week old infants, among other Cystinosis projects.



Christy Greeley, Dr. Bill Gahl and Dr. Rachel Bishop at the recent bone meeting in Salzburg, Austria



Cystinosis Bone Meeting attendees in Salzburg, Austria in December, 2016

The goal of the meeting was to set international guidelines for preventing and treating bone and muscle problems in Cystinosis patients. Forty-five clinicians, researchers and patient advocates attended the meeting from countries all over the world. A variety of topics were addressed and workgroups met with the end goal of publishing guidelines for diagnostics, treatment and monitoring of Cystinosis bone disease and recommendations for future clinical and experimental studies. CRN will make the meeting report available on our website as soon as it is published.

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The Gardner family at the 2015 CRN Family Conference

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Join the Cystinosis Research Network

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

Join The Cystinosis Research Network (CRN) and become part of a global network of caring families, concerned individuals and healthcare professionals working together in the fight against cystinosis. The Cystinosis Research Network's vision is the discovery of improved treatments and ultimately a cure for cystinosis. The Cystinosis Research Network is a volunteer, non-profit organization dedicated to advocating and providing financial support for research, providing family assistance and educating the public and medical communities about cystinosis. CRN funds research and programs primarily through donations from the public, grassroots fundraising events and grants. CRN provides outreach and access to resources. We take great pride in carrying out our motto:

"Dedicated to a Cure. Committed to our Community"...whether you are...

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

Joining the Cystinosis Research Network enables you to:

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

Join Cystinosis Research Network today!

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

Clinton Moore
VP Family Support, Cystinosis Research Network



Dedicated to a Cure. Committed to our Community.



Join the Cystinosis Research Network Today!

Immediate Family	\$20.00
Extended Family / Friend	\$25.00
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 Payable in US dollars

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

Name _____

Street _____

City & State _____ Zip Code _____ Country _____

Phone _____ Fax _____ Email _____

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

Join A CRN Support Group

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

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

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

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

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

Support CRN's Mission with Your Donation

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

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

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- Family
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- I am interested in volunteering for CRN. Please contact me.

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

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

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

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

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

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





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

Agency Name	The Cystinosis Research Network, Inc.
Non-Profit Tax ID #	04-3323789
Address	302 Whytegate Ct., Lake Forest, IL 60045
Telephone	1-866-276-3669 (toll free), 1-847-735-0471
Fax	847-235-2773
E-mail Address	CRN@cystinosis.org
Web Page	www.cystinosis.org

The local United Way organization will contact The Cystinosis Research Network via phone, fax, or e-mail to request we prepare and submit documentation verifying our status as a non-profit organization.

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

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

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

Donate to CRN by Selling on eBay

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



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

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

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



Dedicated to a Cure. Committed to our Community.

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

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

Editor: Terri Schleuder

Cystinosis is a rare, genetic, metabolic disease that causes an amino acid, cysteine, to accumulate in various organs of the body, including the kidneys, eyes, liver, muscles, pancreas, brain and white blood cells. Without specific treatment, children with cystinosis develop end stage kidney failure at approximately age nine. The availability of cysteamine medical therapy has dramatically improved the natural history of cystinosis so that well treated cystinosis patients can live into adult hood.

CRN Vision and Mission

Vision. *The Cystinosis Research Network's vision is the acceleration of the discovery of a cure, development of improved treatments, and enhancement of quality of life for those with cystinosis.*

Mission. *The Cystinosis Research Network (CRN) is a volunteer, non-profit organization dedicated to advocating and providing financial support for research, providing family assistance and educating the public and medical communities about cystinosis.*

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