CRN Family Conference Set for July 16-19 in Atlanta, Georgia

The Cystinosis Research Network is pleased to announce its 2009 Family Conference will be held July 16-18, 2009 at the Marriott Evergreen Conference Resort located in Stone Mountain Park, just outside Atlanta, Georgia. This will be CRN’s fourth family conference, with previous events held in Orlando, Florida (2003), Salt Lake City, Utah (2005), and San Antonio, Texas (2007).

Please plan now to join us as we celebrate the many ways in which our community has gone “Above and Beyond...” You’ll learn about exciting new research findings, receive updates on cystinosis organizations from around the world, meet and renew friendships with our inspirational families, have the opportunity to participate in research studies, and interact one on one with many of the world expert clinicians treating and researching cystinosis today. Be a part of what we anticipate will be the largest ever gathering of individuals with cystinosis in the world!

More information on the resort and the park can be found on their websites, www.evergreenresort.com and www.stonemountainpark.com. Registration materials will be available in early 2009 on the CRN website at www.cystinosis.org and will also be mailed to all families. Our conference planning committee -- especially our gracious host families in Atlanta, represented by Elva Smith, Cheri Friend, and Lorna Smith -- is hard at work to make this the most enjoyable and informative conference ever. If you have any questions or suggestions, please feel free to contact the planning committee at crn@cystinosis.org.

CRN Revamps Logo and Website

The Cystinosis Research Network partnered with Altos Marketing Group to revamp its current logo, tagline, and website. The goal of this redesign project was to bring CRN to the next level from an image perspective.

CRN’s new image will help us be more aggressive when securing donations from personal and corporate relationships and will make navigating our site more user-friendly and visually appealing for all users. CRN is the only Cystinosis support organization that funds research projects as well as supports families living with cystinosis. The new logo, tagline, and website supports this unique attribute of our mission.

Christy Greeley, José Morales, and Paula Shal from CRN worked with Tony Matos, Valerie Hart, and Tracy Nadeau from Altos Marketing on the three month plus project. Continued on page 5
Dedicated to a Cure.
Committed to our Community.

It has been an eventful summer for CRN, with many changes as we continue our efforts to improve our ability to achieve our vision and mission. The changes you have probably already noticed involve a complete redesign of our logo and website. This process was undertaken with the goal of enhancing accessibility to the comprehensive studies, medical documents, and general information available on our website by families, professionals, and donors. I strongly encourage you to visit our new and improved website, www.cystinosis.org, and judge for yourself! We think the new website design, along with the new logo, colors, and motto, “Dedicated to a Cure. Committed to our Community” better showcase CRN’s vision and mission to the world. We remain committed to funding research focused on bringing better treatments and ultimately a cure for cystinosis to the community which we serve.

This issue of The Cystinosis Advocate will bring you up to date on what is happening in the world of cystinosis. CRN in particular has made significant progress on a number of fronts. Most notably, CRN has been accepted as a national organization member of the National Organization for Rare Disorders. NORD is a federation of voluntary health organizations and individuals with rare “orphan” diseases. To qualify for national organization membership in NORD, a non-profit health organization must meet a variety of stringent requirements. CRN prepared the application this spring, assembling the required materials and utilizing NORD’s advisory capacity to further enhance our operational structure, procedures, and policies. In June we were honored to become one of the select few rare disease organizations to receive this level of recognition from NORD. There are many benefits to CRN and the entire cystinosis community that are to be derived from this membership, which are detailed later in this newsletter. We are honored to have had our accomplishments recognized in this way by such an esteemed organization as NORD and we look forward to a long and rewarding relationship with them.

We are also pleased to welcome four new members to our Board of Directors. They are Cheri Friend, Kathy Mandrell, Lorna Smith and Jen Wyman. You can find more information about each of these committed individuals in this newsletter. Please help us welcome them and thank them in advance for their service to our community.

Our five standing committees have been equally as busy. Highlights for each committee:

Finance – CRN completed its annual audit earlier this year. The audit confirmed the financial health of our organization and assisted in our continuing efforts to streamline our financial and accounting processes. The latest financial reports are presented for your review. Please note that our Donor Honor Roll for 2008 will be published in the next newsletter, Spring/Summer 2009.
Development – Many innovative fundraisers took place this summer, look for detailed reports as well as our fundraising calendar.

Research – We are proud to report that, as a result of this year’s Call for Proposals, three new grants will be funded by CRN, totaling almost a quarter million dollars in new research funding. These investigators are all new to CRN – we continue to emphasize the importance of attracting new researchers to the field of cystinosis. The studies are:

- Leticia Belmont-Martinez, MD: Determination of Intraleucocitary Cystine by High Performance Liquid Chromatography (HPLC) in Patients with Cystinosis
- H.J. Blom, M.M.C. Wamelink, E. Levchenko: Newborn Screening of Cystinosis
- Dr. Francisco Emma and Anna Taranta, PhD: Functional Characterization of Cystinosis-LKG

Detailed information about each of these studies, as well as updates on our currently funded research projects, is included in this edition.

Dr. Doris Trauner and her team have made their entire library of educational, neurological, and neuropsychological research and guidelines available to CRN. This collection is available on our website and should prove to be an invaluable tool for parents who need the information to help negotiate their children’s educational needs as well as for those individuals who might be experiencing neurological symptoms.

Family Support – We have been contacted by six families with children newly diagnosed with cystinosis this year. Our Family Support Committee strives to not only welcome them into the community and provide the emotional support they need, but also to provide them with the vital medical information and contacts they require. We are proud of our efforts to shepherd these families through the incredibly difficult process of diagnosis and treatment of cystinosis – our goal is to make their journey as easy as possible.

Planning is underway for the 2009 Family Conference, to be held July 16-19, 2009 at the Marriott Evergreen Conference Resort located in Stone Mountain Park, just outside Atlanta, Georgia. The theme for this year’s conference is "Above and Beyond…", a sentiment which we feel truly summarizes our efforts as an organization and on both a personal and community level to go beyond the traditional expectations of those who live with a rare disease. We all know that individuals with cystinosis can and have accomplished anything they put their minds to and that we as a community can advocate effectively for the advances in treatment we require and deserve. We will strive to exemplify this spirit next July at Stone Mountain and hope you will plan to join us there. Look for more detailed information, including agenda and registration information, in early 2009.

Education and Awareness – Updates on CRN’s advocacy activities are included, as well as reports from other cystinosis organizations from around the world. CRN sent representatives to the 5th International Cystinosis Conference in Dublin, Ireland in June, as well as the Genetic Alliance Conference in Washington, D.C. in July. These activities serve to broaden the reach of our message and connect us with those who work towards the same goals and objectives. Look for more specific reports in this edition.

As always, we remain dedicated to finding improved treatments and an eventual cure for cystinosis while at the same time never losing sight of the importance of supporting our community. I strongly urge you to consider becoming involved in the important work we do – whether it be holding a fundraiser for CRN, joining a committee, serving on our Board of Directors, or attending the Family Conference, we need your help and you can make a difference.

Please feel free to contact me or any of our Board of Director members if you have any questions or concerns.

Warmest Regards,

Christy Greeley
President and Executive Director
CRN Welcomes New Board Members!

The CRN Board of Directors, by unanimous vote, would like to announce that Cheri Friend, Kathy Mandrell, Lorna Smith, and Jen Wyman have been elected to the CRN Board of Directors.

The Board cannot thank our outgoing board members enough for their service to CRN—Sandy Glaize, Dave Greeley, Kathleen Harrison, Carol Hughes, and Lynn Thomas.

Please take a moment to read the bios of our new board members below. We look forward to a busy and exciting 2009.

Cheri Friend

Cheri served as a past CRN secretary and is now rejoining the board on the Family Support committee. Cheri received her degree in Business Administration from Branell College of Business. She has been employed with SunGard Data Systems for the past 17 years and is currently working as a Senior Project Manager. Cheri lives in Suwanee, Georgia with her husband, Matt and their two daughters - Laurin and Morgan. Morgan underwent a steroid-free kidney transplant at the National Institutes of Health in December, 2003. Cheri's step-daughter, Jennifer, was the gracious donor for Morgan's transplant. Morgan is doing remarkably well and continues to thrive.

Kathy Mandrell

Kathy Mandrell was born and raised in West Bolton, Vermont and spent several years after high school as an Army musician. She attended the University of Vermont and graduated magna cum laude from the University of Missouri – St. Louis with a BS in biology in 1995. Since then, she has worked for Pfizer, Inc. as a research biologist in their Pharmacokinetics, Dynamics and Metabolism division. Kathy and her husband, Scott, live in St. Louis County with their two children, Eleanor and Hugh, their two Newfoundland dogs, Bison and Mattie, and the Snowshoe cat who is still nameless. Eleanor, age 10, was diagnosed with cystinosis at nine months of age and Hugh, age 7, was diagnosed by amniocentesis. Their nephrologist is Ellen Wood at Cardinal Glennon Children’s Medical Center in St. Louis. The family has taken several trips to San Diego to participate in both Dr. Dohil’s and Dr. Trauner's studies. Kathy is pleased to be able to take a more active role in CRN.

Lorna Smith

Lorna is a 38 year old mother of two. She was born and raised in Maine. She served four years in the US Navy. Lorna currently works as a Pre-K Teacher. Lorna met her husband of 11 years, Eric, in the Florida Keys. Their two children are Rachel, who is almost 7 years old, and Mitchell, age 5. Mitchell was diagnosed with Cystinosis in November, 2004 at 17 months of age. Mitchell has responded well to medication and is a happy little boy in kindergarten. Lorna and her family have lived in Lawrenceville, Georgia for the last eight years.

Jen Wyman

Jen Wyman lives in Bloomfield Hills, Michigan with her husband, Tim, sons Matt (14), Jack (13), and daughter Kacy (6). Kacy was diagnosed with Cystinosis in September 2006 at the age of four. Jen has her K-8 teaching certificate from Albion College and a Master’s in Educational Psychology from Eastern Michigan University. She taught for four years in Tecumseh, Michigan before getting pregnant with their first child. She has been a stay at home mom since her children were born. In the last two years she has become more involved with CRN and is looking forward to serving on the board.
CRN Revamps Logo and Website (cont.)

In May, the National Organization for Rare Disorders (NORD) approved CRN's application to become a National Member.

NORD offers membership to organizations on two levels -- National Member and Associate Member. To become a NORD member requires meeting certain specifications and going through a process of review by their board of directors.

CRN's Executive Committee spent many months this summer gathering information and further refining it's policies and procedures in order to meet the strict guidelines set forth by NORD. This effort included the creation of CRN's first ever Annual Report, which can be viewed on the CRN website at http://cystinosis.org/07annual.pdf. An article ran in NORD's August edition of their newsletter, "Orphan Disease Update" welcoming CRN as a new member organization. This can also be viewed on the CRN website at http://cystinosis.org/news090208.pdf.

CRN will also receive access to disease specific networking with other groups and individuals dealing with related disorders, guidance on non-profit governance, NORD’s member only publications, and access to professional development opportunities for our board members through NORD's Annual Conference and regional meetings, which are coordinated along with the NIH Office of Rare Diseases.

CRN will have access to post our research funding opportunities and conference and special event promotions on NORD's website, which receives approximately 140,000 visits per month. Finally, CRN will now be eligible to vote for the NORD Board of Directors and other agenda items, further extending our voice within the rare disease community. We are proud to have received this recognition of our efforts from this esteemed organization and look forward to becoming an active member of NORD.

The project involved several phases including:

- **Rebranding**--With input from the CRN team, Altos created a new logo, tagline, and messaging strategy

- **Website design**--Altos provided a premium, professional home page and subsequent pages that differentiate CRN from other cystinosis organizations

- **Website development**--Altos and CRN collaboratively developed a site plan that organized the content of the former website into a more user-friendly and modern format. The site will continue to educate the user about CRN's mission and vision as well as compel visitors to support CRN through online donations, store purchases, research, events, and volunteering.

- **E-mail Marketing**--Part of the project included implementing the ConstantContact e-mail marketing solution. This functionality will allow CRN to important notifications, such as newsletter and conference information via an e-mail list. Altos developed a template that includes design elements from the site redesign.

- **Website Hosting**--Altos will leverage website hosting service through their host partner, Hostgator. Services include e-mail services, web statistics, ePanel service, and 24/7 support and consultation.

CRN believes that our new website and image will allow us to continue to provide information on all things cystinosis that is just one click away for our community and will support our continuous growth as an organization.
CRN Financial Update

By Brittney LeBeau, Treasurer

As 2008 winds down, I would like to highlight a few of the latest updates from a financial perspective.

Since my last update, CRN has undergone its first annual audit of its financial statements for fiscal year 2007 as required by the State of Illinois. Many challenges arose during this process, which was quite labor intensive yet necessary. Although the opinion of the independent auditor expressed that CRN fairly represented its financial statements, they did suggest that we upgrade the accounting software we utilize. I have completed this transition for financials to date for 2008 and going forward for 2009.

The 2008 profit & loss statement indicates that our fundraising activity has raised nearly $200,000 in income as of September 30. Our research grant payments made to date are nearly $75,000. Another $30,000 was expensed for our upcoming biennial Family Conference to be held in 2009. Details of the profit & loss statement can be found on page 7.

I am looking forward to seeing how the remainder of this year unfolds and fully anticipate CRN achieving its fundraising goals. I anticipate another year of financial success and taking CRN to another level in awareness and possibilities beyond our community.

Financially Yours,
Brittney LeBeau
Treasurer

CRN Education and Awareness Update

By Paula Shal, Vice President, Education and Awareness

2008 has been a busy year for all CRN Committees, and Education & Awareness is no exception. Many of the activities that fall under the Education & Awareness umbrella are highlighted in detail throughout this edition of the newsletter. Here is a summary:

The Newsletter Itself
The newsletter has undergone quite a transformation from its state one year ago. First, it was redesigned, and now the colors have been changed to match the new logo. Don’t be surprised if the design changes again in the not-so-distant future as CRN works to align all of our marketing materials.

The Website
CRN is proud of the new website, logo, and tagline Altos Marketing has designed for us. We feel fortunate to have benefited from Altos’ design expertise, cutting edge technology, and tremendous follow-through. Please be sure to take some time exploring the site at www.cystinosis.org.

Presence in the Medical Community
My husband, Jim, and I attended our first Genetic Alliance Conference. Not knowing what to expect, we were quickly embraced by the Genetic Alliance community. The conference was unique in that it brought together diverse groups of people from medical professionals to advocacy groups. We were excited to be able to educate other individuals about cystinosis, and we were thrilled to be able to participate in the GINA victory celebration. Read more about this conference on page 16.

Marybeth Krummenacker, Pam Woodward, and Elva Smith just returned from the ASN (American Society of Nephrologists) Renal Week in Philadelphia. The medical professionals who visited the CRN booth expressed great interest in learning more about cystinosis. More information on this meeting will be in an upcoming newsletter. For a complete list of upcoming medical meetings, see page 14.
## CRN Financial Update – Profit & Loss Statement

**Ordinary Income/Expense**

<table>
<thead>
<tr>
<th>Jan - Sep 08</th>
<th>61230 · Miscellaneous</th>
<th>455.49</th>
</tr>
</thead>
<tbody>
<tr>
<td>43400 · Direct Public Support</td>
<td></td>
<td></td>
</tr>
<tr>
<td>43410 · Corporate Contributions</td>
<td>16,000.00</td>
<td></td>
</tr>
<tr>
<td>43450 · Indiv, Business Contributions</td>
<td>29,005.52</td>
<td></td>
</tr>
<tr>
<td>43400 · Direct Public Support - Other</td>
<td>152,616.47</td>
<td></td>
</tr>
<tr>
<td><strong>Total 43400 · Direct Public Support</strong></td>
<td>197,621.99</td>
<td></td>
</tr>
<tr>
<td>44800 · Indirect Public Support</td>
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<td></td>
</tr>
<tr>
<td>44820 · United Way, CFC Contributions</td>
<td>10,109.60</td>
<td></td>
</tr>
<tr>
<td>44830 · Missionfish</td>
<td>86.45</td>
<td></td>
</tr>
<tr>
<td><strong>Total 44800 · Indirect Public Support</strong></td>
<td>10,196.05</td>
<td></td>
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<tr>
<td>45000 · Investments</td>
<td></td>
<td></td>
</tr>
<tr>
<td>45030 · Interest-Savings, Short-term CD</td>
<td>4,399.68</td>
<td></td>
</tr>
<tr>
<td><strong>Total 45000 · Investments</strong></td>
<td>4,399.68</td>
<td></td>
</tr>
<tr>
<td>46400 · Other Types of Income</td>
<td></td>
<td></td>
</tr>
<tr>
<td>46430 · Miscellaneous Revenue</td>
<td>14.74</td>
<td></td>
</tr>
<tr>
<td>46400 · Other Types of Income - Other</td>
<td>18.58</td>
<td></td>
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<tr>
<td><strong>Total 46400 · Other Types of Income</strong></td>
<td>33.32</td>
<td></td>
</tr>
<tr>
<td><strong>Total Income</strong></td>
<td>212,251.04</td>
<td></td>
</tr>
</tbody>
</table>

**Expense**

| 60900 · Business Expenses | | |
| 60920 · Business Registration Fees | 150.00 |
| 60900 · Business Expenses - Other | 15.00 |
| **Total 60900 · Business Expenses** | 165.00 |
| 61000 · Functional Expenses | | |
| 61020 · Return item chargeback | 95.00 |
| 61050 · Printing | | |
| 61060 · Frame-Festival of Hope | 381.12 |
| **Total 61050 · Printing** | 381.12 |
| 61100 · Food and Beverage | | |
| 61101 · Food and Beverage-Fest of Hope | 5,350.00 |
| 61110 · Loedown Hoedown | 3,000.00 |
| **Total 61100 · Food and Beverage** | 8,350.00 |
| 61200 · Facility charges | | |
| 61201 · C.H. Robinson | 14,439.15 |
| **Total 61200 · Facility charges** | 14,439.15 |
| 61220 · Travel | | |
| 61221 · Airfare-Wyman fun/run | 360.62 |
| **Total 61220 · Travel** | 360.62 |

**Total Expense** | 165,251.22 |

**Net Ordinary Income** | 46,999.82 |

**Net Income** | **46,999.82**

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Support CRN by purchasing the Rock Out Cystinosis CD for just $12.00! (plus $1.50 for shipping)

100% of the proceeds go to the Cystinosis Research Network!

Visit [www.rockoutcrn.org](http://www.rockoutcrn.org) to order.

See page 21 for the story behind the CD.
It is with great pleasure that I present to you the latest additions to CRN's research team! CRN and the cystinosis community are so very fortunate to have the experience and expertise of these dedicated researchers included in our pursuit of a better life for the cystinosis patient.

Leticia Belmont Martinez, M.D., Universidad de Genetica de la Nutricion, Instituto Nacional de Pediatria, Mexico. "Determination of Intraleucocitary Cystine by High Performance Liquid Cromatography (HPLC) in Patients with Cystinosis"; one year study; total grant: $31,972.00.

Henk J. Blom, M.D. et al, VU University Medical Center, Amsterdam, The Netherlands. "Newborn Screening of Cystinosis"; 18 month study; total grant: 45,000 euros, approximately $67,500.00.

Francisco Emma, M.D. and Anna Taranta, Ph.D., Bambino Gesu Children’s Hospital and Research Institute, Rome, Italy. "Functional Characterization of Cystinosin-LKG"; 2 year study; total grant: $114,480.00.

CRN is very proud to welcome these new researchers! They join that unique group of professionals who have been involved in past and on-going research studies funded by CRN. It is because of the dedication and perseverance of all these researchers that so much progress has been made in the treatment and improved outlook for our cystinosis patients. The addition of these research grants brings CRN's current research commitment to a total of almost $900,000.00! It is because of your continued support, and that of the cystinosis community, through direct contributions and fundraisers that CRN can make this research possible.

In addition to these new studies, I would like to report on the status of the current research sponsored by CRN. More detailed information about these studies can be found on our website: www.cystinosis.org

CRN has received the final report from Doris Trauner, M.D., University of California at San Diego, for the study "Early Intervention Trial for Visual Processing Deficit in Cystinosis". Start date: Aug. 2003 – ongoing; total award: $112,724.00.

We have also received the 2nd progress report from Prof. Donald Cairns, School of Pharmacy and Life Sciences at the Robert Gordon University for the study "Design and Synthesis of Novel Prodrugs for the Treatment of Cystinosis". Start date: May 2005; total award: $97,929.00.

We have received five interim reports on CRN’s on-going research grants:


Dr. Kalatzis and Kremer have submitted two progress reports, 6 and 12 months, since our last newsletter.


Catherine Tuleu, PhD, Univ. of London, School of Pharmacy (other names on grant: Ken Nischal, Olufemi Rabiu, Rajnish Sekhri, Wm Van’t Hoff and Bola Lawal). "Development of cysteamine in situ gelling system for the topical treatment of corneal crystals in cystinosis", Grant awarded January 2007 for 3 years, 1 Mar 2007 – Feb. 2010. Total award: Pounds 103,000.00, approximately US$203,500.00.

Donald Cairns, PhD, School of Pharmacy, The Robert Gordon Univ., Aberdeen, Scotland, with Dr. Rachel Knott, and Dr. Graeme Kay, "Evaluation of Novel Prodrugs for the Treatment of Nephropathic Cystinosis". Grant awarded: 1 October 2007 for 1 year, Fall 2007 – Fall 2008. Total award: Pounds 35,000.00 (approximately US$70,000.00).
Other research grants that are still under study are:

Jess Thoene, MD, Univ. of Michigan, Ann Arbor, “Tissue Repository for Cystinosis” Grant awarded: 30 August 2005, interrupted immediately by hurricane Katrina. New start date: 21 Nov. 2006 – ongoing

Total award: $26,206.00


Total award: $97,928.00

CRN has been notified by Dr. Viki Kalatzis of a publication that was partially funded by our research dollars:

Claire Hippert, Grégor Dubois, Carole Morin, Olivier Disson, Sandy Ibanes, Chantal Jacquet, Reto Schwendener3, Corinne Antignac, Eric J Kremer and Vasiliki Kalatzis

“Gene Therapy May Be Preventive but not Curative for a Lysosomal Transport Disorder”; August 2008 © The American Society of Gene Therapy

Institut de Génétique Moléculaire de Montpellier, CNRS, Montpellier, France; 2Universités Montpellier I & II, Montpellier, France; 3Institute of Molecular Cancer Research, University of Zurich, Zurich, Switzerland; 4Inserm U574, Paris, France; 5Université Paris Descartes, Faculté de Médecine René Descartes, Paris, France

We at CRN, along with the entire cystinosis community, are indebted to Dr. Doris Trauner for her years of research and experience with neuropsychological research. Her expertise has yielded a rich library of materials addressing the many questions that often arise with regard to educational, neurological, and psychological issues related to cystinosis. Dr. Trauner and her team at the University of California, San Diego, have collected and compiled a wealth of information for us which you will find listed below. In fact, you’ll not find this information pulled together in this fashion anywhere else.... CRN is proud of our role in encouraging her to assemble this material.

Our interaction and relationship with our investigators and with the cystinosis community at large exemplifies the spirit of CRN. The full text of these articles and publications can be found on the CRN website at www.cystinosis.org/news092008.html. The titles include:

"Classroom Accommodations for the Child with Learning Differences"

Ballantyne, Scarvie, and Trauner: "Academic Achievement in Individuals with Infantile Nephropathic Cystinosis"

Spilkin and Ballantyne: "Behavior in Children with a Chronic Illness: A Descriptive Study of Child Characteristics, Family Adjustment, and School Issues in Children with Cystinosis"

Delgado, Schatz, Nichols, Appelbaum, and Trauner: "Behavioral Profiles of Children with Infantile Nephropathic Cystinosis"

Williams, Schneider, and Trauner: "Global Intellectual Deficits in Cystinosis"

Ballantyne and Trauner: "Neurobehavioral Consequences of a Genetic Metabolic Disorder: Visual Processing Deficits in Infantile Nephropathic Cystinosis"

Trauner, Chase, Scheller, Katz, and Schneider: "Neurologic and cognitive deficits in children with cystinosis"

Spilkin, Ballantyne, Babchuck, and Trauner: "Non-Verbal Deficits in Young Children with a Genetic Metabolic Disorder: WPPSI-III Performance in Cystinosis"

Scarvie & Ballantyne: "Visuomotor Performance in Children with Infantile Nephropathic Cystinosis"

Trauner, Spilkin, Williams, and Babchuck: "Specific Cognitive Deficits in Young Children with Cystinosis: Evidence for an Early Effect of the Cystinosin Gene on Neural Function"

Rare Disease Day is
February 28, 2009

CRN has partnered with NORD to support Rare Disease Day. Rare Disease Day is a global effort to focus attention on rare diseases as a public health effort.

Please visit www.rarediseases.org/rare_disease_day/rare_disease_day_info to learn how you can bring attention to this day.
CRN/NIH Seeks Fellowship Applicants

CRN continues to assist Dr. Bill Gahl in the identification of the best possible candidate for fellowship training at the National Institutes of Health, Department of Health and Human Services. Dr. Gahl would like to train an expert in nephropathic cystinosis to provide the next generation with an authority in the disease.

The successful candidate will work primarily on cystinosis, but may also perform research into related disorders with renal manifestations. Optimally, the fellowship training would follow a renal fellowship, last approximately 3 years, and provide a springboard to an academic career in cystinosis and related fields. Geneticists may also be interested in this career track.

The Cystinosis Research Network will support this fellowship through an NHGRI Gift Fund. For further information, an interested party may contact Dr. Gahl at bgahl@helix.nih.gov or 10 Center Drive, Building 10, Room 10C-103, NHGRI, NIH, Bethesda, Maryland 20892-1851 (phone 301-402-2739). The Department of Health and Human Services and the National Institutes of Health are equal opportunity employers.

Teaching Medical Students and Doctors About Cystinosis

The Metabolism Service at Boston's Floating Hospital for Children has launched the Metabolism Outreach Service. This innovative program represents the largest and most comprehensive program to date to educate medical students and physicians about metabolic disease. At this time, it serves 6 academic medical centers in the northeastern U.S. and provides:

- Seminars and workshops about metabolic disease and abnormal test findings;
- Opportunities for patients/families to help teach medical students and doctors;
- Consultative assistance to physicians as they assess their patients;
- Long distance support for these medical centers during more urgent situations.

We are looking for patients and families to become involved in the teaching process!

If you are interested in speaking to a medical audience as part of the Metabolic Outreach Service, or if you will allow you/your child's record to be reviewed and used as a teaching case (anonymously) during an Outreach workshop, please let us know!

Please call:
Isabel Matos
Tufts Medical Center
Metabolism Service
617-636-5443

Or visit our website:
http://www.floatinghospital.org/OurServices/Genetics_Metabolism/theMetabolicOutreachService_
Participate in Cystinosis Research

Cystinosis Research Network, Inc.
302 Whytegate Court, Lake Forest, IL  60045
Toll Free: (866) 276-3669
Tel:  (847) 735-0471  Fax: (847) 235-2773
CRN@cystinosis.org   www.cystinosis.org

To patients and parents of children with cystinosis:

A tissue repository for renal tissue from patients with cystinosis is being established, with funding from CRN, at the University of Michigan. The existence of this repository will enable ongoing study of cystinotic tissues, which is currently hindered by lack of available tissue.

If you want to participate, please request that appropriate tissue samples be forwarded by the pathologist who analyzed you or your child’s renal tissue, whether obtained at renal biopsy during management of the disease, or at transplantation, and who has custody of the sample, to the repository at the University of Michigan. The samples will be maintained confidentially. The identity of each patient will be unknown to University of Michigan Repository staff, but will be marked with an identifier so that samples can be retrieved if future clinical needs dictate. Acceptable samples include unstained slides, or portions of paraffin blocks, depending on the amount of tissue available. All donations must comply with applicable hospital and State laws and requirements.

If you are interested, please ask your nephrologist or pathologist (if known) to contact Dr Jess Thoene at the University of Michigan, Division of Pediatric Genetics, 734-272-5573 who will cover details of sample coding and clinical summary to preserve confidentiality and ensure appropriate shipping. Please do not contact Dr. Thoene directly, as this will violate confidentiality.

Funds are available to cover the costs of shipping of the specimen.

The Cystinosis Research Network is an all-volunteer, non-profit 501(C)3 organization, Federal Tax ID # 04-3323789.
Participate in Cystinosis Research

Dr. Jerry A. Schneider and his team at the University of California San Diego are conducting a research study to learn more about how to prepare the blood samples used for the determination of white blood cell cystine levels. For this study, they are looking for cystinosis patients (12 years and older) who would travel to San Diego for this study. Flight cost will be paid for. Preference will be given to patients 18 years and older, who can fly to San Diego and back home on the same day. Patients do not have to currently be taking Cystagon. However, patients have to be able to provide recent laboratory test showing that their red blood cell count is not below normal.

White blood cell cystine levels need to be measured occasionally to be certain patients are taking the correct dose of Cystagon. When we started to study cysteamine many years ago we tried to find a way to have whole blood shipped to our laboratory so that we could both prepare the white blood cells and measure the cystine. We couldn't make this work. We only got reliable results if the white blood cells were made promptly after the blood was drawn.

Although preparing the white blood cells is not very difficult, it is very time consuming and labs often find the preparation challenging, especially if a lab only does this every few months. We have some ideas of how we might stabilize the white blood cells so that the blood could be shipped and our laboratory could prepare the white blood cells. To test these ideas we need some cystinosis patients who are willing to donate blood. Patients volunteering for this study will be asked to come to the UCSD (University of California, San Diego) Medical Center in San Diego where a blood sample will be taken. Depending on the age and blood count of the patient, up to 10 tablespoons of blood will be taken (150 mL). A "unit" of blood that is taken at a blood bank is three times as much (450 mL). Of course smaller patients will have less blood taken. Patients may be asked to not take Cystagon for 24 hours before the blood draw. Participating in research is entirely voluntary. If you are interested in participating and would like more information, please contact the University of California, 200 West Arbor Drive, MC 8450 San Diego, CA 92103-8450 Phone (619) 543 2049.

Cystinosis Research Foundation Update

IRVINE, Calif.--(BUSINESS WIRE)--The Cystinosis Research Foundation of Irvine, Calif., announced it has funded or made commitments of $1,316,361 for nine new scientific research studies in the United States and Europe aimed at finding better treatments and a cure for cystinosis, a rare metabolic disorder that afflicts about 500 children and young adults in the United States and 2,000 worldwide.

"With more than $6.5 million committed to cystinosis research by the CRF to date, our foundation is the leading funding source for bench and clinical investigations worldwide. These efforts have produced a greater understanding of cystinosis as well as the most significant advancements yet toward improved treatments and a cure," said Nancy Stack, CRF president.

Four of the nine new studies are for research efforts in France. One study is in Italy, one is in Belgium and three are in the United States at the University of California at Irvine, The Scripps Research Institute in La Jolla, Calif., and State University of New York in Buffalo.

This past year has been a year of new research developments and significant advancements in the treatment of cystinosis. The research funded at the University of California, San Diego (UCSD) has resulted in the development of a slow-release form of cysteamine. Late last year, Raptor Pharmaceuticals Corp.'s subsidiary, Bennu Pharmaceuticals, Inc., acquired the world wide license for the slow-release form of cysteamine from UCSD. They are committed to improving and advancing the slow-release drug and applying to the FDA for approval of the new drug in 2009. The slow-release medication will be a reality for all of our children in the near future.
Ashley Shaw Receives Sierra Woodward Sibling Scholarship

Ashley C. Shaw was awarded the 2008 Cystinosis Research Network Sierra Woodward Sibling Scholarship. Ashley graduated in May, 2008 from East High School and is now attending the University of Utah studying to become a pediatric nurse. She is the daughter of Gary and Kathy Shaw and has 2 sisters with Cystinosis Amy Shaw Black and Emily Shaw.

The Sierra Woodward Sibling Scholarship was established to provide supplemental financial assistance to a student who has a sibling diagnosed with Cystinosis and is enrolling or is enrolled in a regionally accredited collegiate or vocational program.

CRN would like to congratulate Ashley and wish her good luck with her future education.

Congratulations College Graduates!

Individual with cystinosis can accomplish great things! CRN is aware of three young adults with cystinosis who graduated from college in 2008. They give hope and inspiration to us all.

Kevin McCalla

graduated from the University of Texas in May with a Bachelor of Arts in Studio Art. Kevin received a kidney transplant five days after his college graduation!

Christian Sproedt

received his Bachelor of Administration in German and English from the Hogeschool Zeeland, Netherlands. He is now working on his Masters in Technical Logistics at the University Duisbrug/ Essen. He plans to do an internship in the production industry in 2009.

Tahnie Woodward

graduated from the University of Utah with a Bachelor of Science in Human Development and Family Studies with a Child Life emphasis. She will be doing a Child Life Internship at Lutheran General Children’s Hospital in Chicago, IL from January, 2009 to May, 2009. Child Life Specialists make sure the child experiences the least amount of stress possible while in the hospital.
CRN Participates in International Cystinosis Conference in Dublin, Ireland

The Fifth International Cystinosis Conference was held June 26-28, 2008 in Dublin, Ireland. The conference was organized by the Cystinosis Foundation Ireland, chaired by Mick Swift and Anne Marie O’Dowd. CRN was honored to have been invited to participate in the conference and to have been included in the poster session, which included patients, researchers, pharmaceutical companies, and other advocacy groups. This presented an opportunity not only to update the international community on the progress CRN has made this year but also to network with those organizations and investigators from around the world who were in attendance. Christy Greeley, President and Executive Director, and José Morales, President Emeritus represented CRN at the event.

We were very pleased to use this opportunity to strengthen relationships with our existing colleagues and partners such as Marc Tewey and Lesli King from Sigma Tau Pharmaceuticals; several of CRN’s board members and sponsored researchers who were in attendance including Drs. Schneider, Levchenko, Wilmer, Kleta, Trauner, Sarwal, Gahl, Goodyer, Dohil, Cairns, and Katy Freed from the Southwest Foundation in San Antonio; Orphan Europe representatives and AIRG board member Francois Couppey; Victor Gomez and Leticia Belmont from Cystinosis Mexico; Jean Hotz from Cystinosis Foundation USA; and Jonathan Terry from Cystinosis Foundation UK.

We also had the pleasure of meeting some of the international investigators we had not had the opportunity to meet in person in the past, such as Dr. Van’t Hoff, and new contributors to the cystinosis community including Ted Daley, President, Raptor Pharmaceuticals who are working to develop a sustained release cysteamine formulation. Since the meeting in Dublin, CRN has worked with Raptor and their marketing firm, Engage Health, to provide cystinosis prevalence data for their FDA submission.

CRN also attended a presentation by a representative from the Irish Health Research Board regarding European Union funding for rare disease research. The 7th EU Framework Programme Ireland funds collaborative research in Europe with rare disease research featuring annual work programs. Many of our international funded researchers would qualify for involvement in this program which could greatly increase their research funding by joining forces with other researchers interested in similar rare disease projects.

The opportunity to network with an international gathering of cystinosis researchers, patient groups, pharmaceutical companies, and families to make CRN’s mission, goals and accomplishments made known to a wider audience was truly invaluable. We look forward to continuing to strengthen these relationships in the future, and particularly hope that many of those we met in Dublin will be able to join us next July in Atlanta for CRN’s conference.
Families “Catch Up” at Midwest Regional Gathering

By Dena Gard

The Midwest Cystinosis Gathering was held in Casey, Illinois on July 4-6, 2008. Eric, Dena, Nikolaus, and Mikaela Gard were the hosts this year to about five families from the Midwest. One family even had a foreign exchange student from Spain. Most families arrived on Friday July 4th, and a supper of spaghetti and garlic bread was served that evening. After dark, we went in town to watch the fireworks display and then visited and caught up on what had been happening in everyone’s lives.

Saturday morning we had a nice parents meeting, and the children enjoyed games and activities at our local church. Usually our parents meeting is parents only, but this year we were joined by one of our children, Andrew Hoffmann, who had recently had a kidney transplant and was willing to share his experiences. It was very informative and nice to hear from his point of view. Thank you, Andrew, for sharing!

Saturday afternoon was a time for each family to just be on their own doing whatever they chose. Then Saturday evening we had a cookout at our host family’s home and played croquet and had a nice time visiting with everyone. We had a new family this year, Eric and Lacy Cooperider, with their children Camden, 3, with cystinosis and Coco, 1, without. Everyone went home Sunday, and we said our good-byes. It was a great time, and we are looking forward to having a great visit again next year.

Since having the Gathering two members of our group have had kidney transplants; Amelia ‘Douglas’ Miller and Keri Hohl, both are in their early 20’s and

Congress of the International Pediatric Nephrology Association (IPNA)

August 29 – September 2, 2010

The International Pediatric Nephrology Association (IPNA) has almost 1500 members in 89 countries in the world. The mission of IPNA is to promote the optimal care of children with kidney disease worldwide by advocacy and education. The goal and the purpose of the Association is to disseminate the knowledge about kidney diseases in children all over the world. To live up to this goal and purpose, it organizes an international congress every three years and frequent workshops and symposia in various areas of the world. The congresses serve basic science, clinical science and clinical practice.

Teaching obligations are organized in various parts of the world. The next IPNA Congress convene in New York City, New York at the Hilton Hotel, August 29 – September 2, 2010. Attendance is projected to hit record levels, 1,500 registrants, with the contingent from North America to exceed 500. The Cystinosis Research Network has assumed the responsibility for mutually developing a scientific symposium on cystinosis that would bring together experts worldwide to interact at this unique gathering of the world’s pediatric nephrology community. The format is under development and includes a broad spectrum of possibilities: an entire day of basic and clinical research symposia, a plenary speaker, a pre-meeting workshop or any combination of the educational endeavors.

The Cystinosis Research Network has committed to support funding the cystinosis symposium and has assigned Marybeth Krummenacker and José Morales to coordinate our activities with the IPNA. Please contact us at crn@cystinosis.org if you have an interest in becoming involved and assisting with the planning.
Jim and Paula Shal attended the Genetic Alliance Conference at the Bethesda Marriott in Bethesda, MD July 11-July 13, 2008. Genetic Alliance is an organization that is dedicated to improving the quality of life for everyone living with genetic conditions through education, novel partnerships, and the improvement of health systems. The Genetic Alliance Conference brings together genetics advocacy groups, policymakers, health professionals, researchers, industry representatives, and community leaders.

Jim and Paula had the opportunity to educate attendees about cystinosis, gather helpful information for advocacy groups, and learn new ideas from the genetics world through networking and attending workshops. They attended a ½ day symposium on Transformational Leadership, which encouraged participants to search within themselves in order to be a leader, and a ½ day symposium on Organizational Development, which focused on fundraising and strategic planning.

They also attended “In the Family: Exploring Issues Related to Predispositional Genetic Testing.” The National Society of Genetic Counselors and filmmaker Joanna Rudnick partnered to discuss issues surrounding genetic testing. The workshop included clips and behind-the-scenes information from Joanna’s film In the Family, which aired on PBS and emphasized the importance of family history, the benefits and limitations of genetic testing, the genetic counseling process, genetic discrimination, cultural implications, and more. Other workshops Jim and Paula attended included Patient Empowerment: Research and Experience and Healthcare Reform: Is Universal Coverage Enough?

One of the highlights of the conference was the GINA Victory Celebration. The Genetic Information Nondiscrimination Act (GINA) was passed in May, 2008 and protects individuals from genetic discrimination by employers and insurance companies. The bill was first introduced to the House of Representatives in 1995, and it has been a long road to its passage. The evening included a video outlining the journey of GINA. Several Congressional heroes and their key staff members were honored for their role in the passage of GINA, including Congresswoman Louise Slaughter (D-NY), who first introduced the bill in 1995, Senator Olympia Snowe (R-ME), who introduced a similar bill in 1996, Congresswoman Judy Biggert (R-IL-13), Senator Mike Enzi (R-WY), and Senator Edward Kennedy (D-MA).

The night was capped off by the "GINA Rocks" band led by Dr. Frances Collins, who recently resigned from his post as Director of the National Human Genome Research Institute (NHGRI). They performed a celebratory song, "G-I-N-A," which he wrote for the occasion. After a brief demonstration of the dance steps corresponding to the "G-I-N-A" song—an adaptation of YMCA—the musical number kicked off an evening of entertainment, as the crowd danced the night away in celebration of this victory that impacts every individual in the nation.

For more information about Genetic Alliance and GINA, visit www.geneticalliance.org.
MEXICAN ASSOCIATION OF CYSTINOSIS

ANNOUNCEMENT

5th Medical Cystinosis Symposium
will be held in Mexico City
January 2009
Complete information and agenda coming soon at:
www.cystinosismexico.org
info@cystinosismexico.org

5th International Cystinosis Conference – Dublin

By Anne Marie O’Dowd
Cystinosis Foundation Ireland

Cystinosis Foundation Ireland was honored to host the 5th International Cystinosis Conference in Dublin from June 26th to 28th this year. As a Foundation this was a huge undertaking for us and the committee worked for over a year to as we looked forward to welcoming all our international delegates, families and professionals. We had over 220 delegates from 20 countries represented and the conference provided a great opportunity for sharing of research information and networking between doctors, researchers and families.

We were honored that Mary Harney, TD, Minister for Health and Children addressed our conference especially as Minister Harney launched Cystinosis Foundation Ireland in 2003 when she was Minister for Health and Deputy Prime Minister.

The theme for the conference was ‘from dreams to reality’ and both the dreams and reality were expressed over the days of the conference. The bravery of those who have taken part in research and continue to do so was acknowledged. Through their determination, we have seen so many of the advances in cystinosis. For the 2008 conference, over 20 abstracts of original research were received, the most in the 10 years the international conference has been in existence. This represents genuine hope for all those living with cystinosis. Eight of those abstracts were presented verbally, and the rest were showcased in the poster session during the conference.

A full program of events ran during the conference. High level scientific symposiums took place as well as family/patient sessions. Highlights included a special session for young people to talk with Dr. Gahl without parents present. A session also took place for the younger children with Dr. William van’t Hoff, of Great Ormonde Street Children’s Hospital. William was Scientific Chairman of this year’s conference. An important part of the conference was building networks and friendships, and events such as the bowling session and barbeque cemented the bonds so necessary for supporting each other in this journey with cystinosis.

Dr Jerry Schneider received a Lifetime Achievement Award for his work for many years in the field of cystinosis. Dr Schneider gave the opening keynote speech at the conference.

Special thanks must go to all those who helped organize the conference – everyone involved with Cystinosis Foundation Ireland, the International Scientific Committee who worked on the scientific program, all the sponsors and supporters, and especially the doctors, researchers, families and patients who participated and made the conference a success.
Development Update

By Jill Morrill, Vice President, Development

The Cystinosis Research Network is one amazing organization. One may feel serves as a vital life line by providing us with continued support, an amazing and informative web site, top notch research, and this biannual newsletter which allows us to share our unique families with others.

Without this organization, we would lack guidance and knowledge. We would feel alone and uncertain. Our loved ones may not thrive as well as they do today. With this in mind, I must also remind you that The Cystinosis Research Network is only as successful as we make it. We must give to receive. Fundraising is what keeps us moving forward and helps our community live a quality life.

For those of you who have helped financially through private donations, attended a fundraiser, or hosted an event, thank you. As the year draws to an end, I hope you all can commit to go "Above and Beyond" for the 2009 calendar year. CRN depends on everyone to give back in some way. Big plans are being made for the future of our children and adults suffering from cystinosis, but we can only succeed if we all hop on board and work together. The Cystinosis Research Network is your organization, your life line, your family.

Please visit the CRN website for some fun and easy ways to raise money for cystinosis research this holiday season, including:

- Donate through United Way
- Use Goodsearch as your search engine
- Shop with GoodShop
- Print brochures with your own pictures
- Order CRN logo items from Cafe Press
- Order the Rock Out Cystinosis music CD

Make an Online Donation Through PayPal

Did you know that you can make a secure online credit card donation to the Cystinosis Research Network through PayPal?

- You can dedicate a donation to someone specific. PayPal passes along your dedication to CRN.
- You will receive a record of your contribution for tax purposes.
- Go to www.cystinosis.org and click on the “Donate” link to begin!
# 2009 Fundraising Calendar

<table>
<thead>
<tr>
<th>Date</th>
<th>Event</th>
<th>Location</th>
<th>In Honor Of</th>
</tr>
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<tbody>
<tr>
<td>January 16</td>
<td>3rs Annual “Shoot for the Cure” Pancake Supper</td>
<td>Whitesboro High School, Whitesboro, TX</td>
<td>Mason Reed</td>
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<tr>
<td>February</td>
<td>Wyman Bowling Fundraiser</td>
<td>Bloomfield Hills, MI</td>
<td>Kacy Wyman</td>
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<td>February 28</td>
<td>Miracles at Milleridge</td>
<td>Jericho, NY</td>
<td>Laura Krummenacker</td>
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<td>February/March</td>
<td>Road Rally</td>
<td>Bloomfield Hills, MI</td>
<td>Kacy Wyman</td>
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<tr>
<td>March</td>
<td>Ricardo, Inc. Jeans for a Cause</td>
<td>All U.S. Ricardo, Inc. Offices</td>
<td>Steve Schleuder</td>
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<tr>
<td>March</td>
<td>Jack Greeley Birthday Letter Campaign</td>
<td>N/A</td>
<td>Jack Greeley</td>
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<tr>
<td>May</td>
<td>5K Fun Run/Walk</td>
<td>Bloomfield Hills, MI</td>
<td>Kacy Wyman</td>
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<td>June 13</td>
<td>C.H. Robinson Golf Tournament</td>
<td>Lehi, UT</td>
<td>Tahnie Woodward</td>
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<td>July</td>
<td>Bowling for a Cure</td>
<td>Independence, OH</td>
<td>Victor Gardner</td>
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<tr>
<td>August</td>
<td>Special Kids Network Golf Tourney</td>
<td>Long Grove, IL</td>
<td>Jack Greeley</td>
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<tr>
<td>September</td>
<td>Festival of Hope</td>
<td>Downer’s Grove, IL</td>
<td>Alexis and Evan LeBeau</td>
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<td>October 18</td>
<td>Family Fun Day</td>
<td>Lincolnshire, IL</td>
<td>Jack Greeley</td>
</tr>
<tr>
<td>November</td>
<td>Wyman Holiday Letter Campaign</td>
<td>N/A</td>
<td>Kacy Wyman</td>
</tr>
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The CRN Executive Committee met in Aurora, IL for 2009 budget planning. Committee members include from left to right: Elva Smith, Pam Woodward, Paula Shai, Christy Greeley, Jill Morrill, Brittney LeBeau, and Jen Wyman.

Jill Morrill, Jen Wyman, and Brittney LeBeau look at the 2009 fundraising calendar while at the Executive Committee meeting in Aurora, IL.
A Festival of Hope Lives up to Its Name

A Festival of Hope was held on September 18, 2008 at Carlucci Restaurant in Downers Grove, IL in honor of Alexis and Evan LeBeau. The event attracted over 350 attendees and raised over $55,000 for the Cystinosis Research Network (CRN).

Ashley Kazian, Miss United States 2007, attended the event. Ashley is the National Spokesperson for CRN and is raising awareness for this disease around the country.

At the event she greeted guests, signed autographs, and also took pictures with the guests. We appreciate her volunteering her time to attend the event.

In addition to many silent auction items, there were raffle donations. Colorful necklaces were sold for $20.00. Each necklace had a key attached that opened one of three prizes.

All key holders had a chance to unlock the grand prize, a 42” Plasma flat screen TV.

Another hit was the wine table. Each bottle had been donated, wrapped, and had a gift card attached with a value of $15.00 to $50.00. All bottles were sold for $20.00, and we sold out quickly.

The evening’s entertainment was the premier acoustic duo in the Chicago land area, Frank and Dave. They played outside in the patio area where some listened, some sang along, and some even danced.

The focus of the night was brought front and center when Brett and Brittney LeBeau, parents of Alexis and Evan, took center stage and shared their story. The LeBeau’s shared their fight in battling a disease that wants to attack their children’s bodies and why CRN has been instrumental in overcoming the many challenges they face. The Festival of Hope was a chance for all of those who know and love them and for others who have been touched by their story to come together to make a difference in the fight for a cure.

Dana Drury-Brabeck was the Event Director. She with the help of her team of volunteers did an amazing job planning every last detail of the event. Our heartfelt thanks to the time and effort she sacrificed to make this an unforgettable evening. We are already looking forward to next year’s event! Stay tuned for more details.

Our thanks and sincere gratitude go to all of the event sponsors for their generosity.

Platinum Sponsor - Gorton Seafoods (Larry and Pat LeBeau)

Gold Sponsor - Dr. Sam Akmakjian of Long Grove Dental

Silver Sponsors – American Legion Auxiliary (Post 18 – Lockport, IL)

Eclipse Mailing Services, Inc. (Schaumburg, IL)

John and Jennifer Plummer
Local Bands “Rock Out Cystinosis”

The Morrill family hosted a fundraiser featuring local bands in the Grand Rapids area to help spread awareness and raise money for CRN.

Brian and Jill Morrill have two children, Megan and Tyler. Twelve-year-old Megan suffers from cystinosis, and Brian Morill has had the opportunity to record albums with many Grand Rapids’ musicians and is the drummer for the band Troll for Trout. Together, local musicians donated songs and performed at Founder’s Brewing Co. in downtown Grand Rapids. The event was a big hit, and the Brewery was packed with friends, family, and even Megan’s doctors. One of Megan’s best friends since preschool, Sally Marsh, stood up and talked about what it is like growing up with a friend suffering from cystinosis. She led the group right in to a slide show. There were many damp faces. The perspective of this disease from the eyes of a peer was very humbling for Megan.

Mackinaw Harvest Music compiled the donated songs and produced a compilation CD which was sold at the event. Brian Morrill is the drummer on all but a couple songs. The CD is called "Rock Out Cystinosis," and 100% of the proceeds from sales go directly to The Cystinosis Research Network. Pictures of children with cystinosis are featured in the CD lining.

The CD is still available and can be purchased at www.rockoutcrn.org. One of the songs on the CD can be heard here as well. The CDs make great stocking stuffers and great gifts for clients, teachers, and doctors.

Order the “Rock Out Cystinosis” CD today!

100% of the proceeds go to CRN!

Order at www.rockoutcrn.org.

Monoghans Sponsor “Pennies for Abbi”

On November 15, Terry and Danielle Monaghan (Abigail Monaghan’s grandparents) held a fundraiser to benefit several charities. CRN was one of the beneficiaries. Bracelets were sold with the proceeds going to CRN, and the evening kicked off “Pennies for Abbi”, where 100% of the proceeds are going to CRN. The Monaghan’s have a goal of collecting one million pennies ($10,000) in the next year. Donation sites are set up in their community of St. Catherine’s, Ontario, Canada.

Abigail Monaghan
The Wyman’s Community, School, and Family Support CRN

2nd Annual 5K Fun Run in honor of Kacy Wyman

May 4 marked the second annual 5K Fun Run with proceeds going to the Cystinosis Research Network. It was a gorgeous day, and about 300 runners came out to support the Wymans. Ashley Kazian, Miss United States 2007, was in attendance.

The Fun Run raised close to $30,000, up from $11,000 the year before. The Wyman’s community of friends and family continues to support the family and cystinosis research time and time again. The outpouring of love and support just grows each time an event is held.

Read-a-Thon for Cystinosis

Kacy’s brother, Jack Wyman, and his 6th grade class held a Read-a-thon for Cystinosis during the 2007-2008 school year. Students received pledges and collected donations for each page read in a 2.5 hour time frame. They managed to raise $2500 in that short period of time. What a beautiful gift.

Family Fun Day

Kacy’s Step Grandfather held a Family Fun Day in August to honor Kacy Wyman. Friends and family gathered for games, a pig roast, cookout and lots of laughs and conversation. The event raised about $7,000.

Women In the Neighborhood (WIN)

This group of amazing women in the Wyman’s community has chosen to host an event for the second time in honor of Kacy with proceeds going to CRN. The event will take place in early 2009.
Uncle Tim’s Fundraiser Raises Over $2400 for CRN

This year Tim Kitchens, Jack’s uncle, outdid himself from the last fund raiser. Live music was provided by Samurai Trout and they were fantastic. We had delicious BBQ and hotdogs prepared and served by David Jordan. Ann (Nanny) and Dean (Papa) Kitchens, Jack’s grandparents, provided an area in their front lawn to setup the band and the BBQ stand. Nanny also prepared delicious desserts for those attending. Hayrides were provided to carry people the ½ mile down to Uncle Tim’s where they could see camels, tortoises, antelope, and a variety of exotic birds. The kids especially enjoyed the hay-ride. We also appreciate the Smith family from Lawrenceville, Ga taking time to attend and help us raise awareness. The event was a lot of fun and we raised over $2400 towards cystinosis research!

We greatly appreciate the efforts of all who dedicated their time and effort towards this event and special thanks to Uncle Tim. We love you.
Travis, Bette, Jack (w/c) and Ava Kitchens

Communities Rally Around the Stilke Family

Kirsten and Dave Stilke are raising awareness for cystinosis in their current community and the communities where they were raised. The Stilkes have two children with cystinosis—Mason (5) and Livia (2).

Kirsten Stilke was invited to speak to the Ashippun Lion’s Club in Ashippun, WI (her hometown) to educate them about cystinosis. She had no idea that the Lion’s had sponsored a raffle for a trip to Las Vegas. At the end of Kirsten’s presentation, they rewarded her with a check for $625.00 for CRN! They also gave her a check to help with Mason and Livia’s medical expenses.

Kirsten expressed in her presentation that it is her job to be Mason and Livia’s voice in the fight against cystinosis.

Kirsten also spoke at the Zion Lutheran Church in Ashippun. Her presentation generated discussion, awareness, and lots of hugs and tears. The church presented Kirsten with a check for $1000 for CRN, and several church members made additional donations.

In addition to the Lion’s Club and Zion Lutheran Church, the Minneiska Water Ski Team donated $150.00 to CRN by collecting donations at their weekend shows. Mason and Livia’s grandparents own the marina that has supported the water ski team for many years. When the team found out their grandchildren suffered from cystinosis, they wanted to give back. CRN thanks the Ashippun Lion’s Club, Zion Lutheran Church, and the Minneiska Water Ski Team for their generous support and donations!

“Kirsten Stilke has made it her mission to spread the word about cystinosis...”

Mason and Livia Stilke

Rachel Smith, Mitchell Smith, and Jack Kitchens at Uncle Tim’s Fundraiser

Ava Kitchens and Rachel Smith—both girls have brothers with cystinosis

The Minneiska Water Ski Team
Golf Outings Honor Jack Greeley

16th Annual Herb Didier Memorial Golf Outing Raises Money for CRN and Make-A-Wish

The Lincolnshire Morning Star Rotary Club sponsored the 16th Annual Herb Didier Memorial Golf Outing on August 7, 2008 in Buffalo Grove, Illinois. Over the years, this event has raised tens of thousands of dollars for worthy causes in memory of one of its charter members and longtime Lincolnshire, Illinois area residents, Herb Didier. This year, LMSRC chose to support CRN, Make-A-Wish Illinois Chapter, and their local charitable fund which provides funding for a host of service projects that Rotary is known for throughout the world.

LMSRC President Alan Nerad knew about cystinosis and CRN as his son, Alex was in Jack Greeley's second grade class last year. Alan graciously offered to not only support CRN via a portion of the proceeds from this event, but to also nominate Jack to have his wish granted by Make-A-Wish. The event was a great success, with beautiful weather and a fabulous turn out of support for all three organizations. LMSRC to date has made a donation of $5,000 to Make-A-Wish. Jack has asked for a trip to Disney World and the opportunity to work behind the scenes at Sea World for a day, learning how the keepers care for the animals there and getting to participate in their care. He is anxiously looking forward to the trip and his family is most grateful for this very special experience for him.

LMSRC has donated $9,000 from the event to CRN, which coupled with $2,610 in private donations related to the event, brings the total funds raised for CRN to $11,610! The Greeley family is incredibly appreciative of all the work the LMSRC, most notably Alan Nerad and Sue Lacey, event chair, have put into this wonderful event and their thoughtfulness to support CRN.

Gerry Veles Foundation Golf Outing Benefits CRN

by Mike Harrold

The Gerry Veles Foundation was started two years ago to keep my mother’s name alive and to help people in need. My mother raised four boys by herself and struggled most of her life financially and later in life physically. After my mother passed away our family decided to have a golf outing each year with our family and friends to raise money for someone in need.

My son Tommy goes to school with Jack Greeley and has played basketball, soccer and baseball together. When we received Jack’s Birthday Fundraiser Letter we knew that The Gerry Veles Foundation would help Jack this year. The golf outing took place on June 28, 2008 in Mundelein, Illinois. The Gerry Veles Foundation was able to donate $2,000 to the Cystinosis Research Network.

LMSRC President Alan Nerad

Evan and Alexis LeBeau with Alex and Jack Greeley

Mike Harrold presents a check for $2000.00 to Jack Greeley

Evan and Alexis LeBeau with Alex and Jack Greeley

LMSRC has donated $9,000 from the event to CRN, which coupled with $2,610 in private donations related to the event, brings the total funds raised for CRN to $11,610! The Greeley family is incredibly appreciative of all the work the LMSRC, most notably Alan Nerad and Sue Lacey, event chair, have put into this wonderful event and their thoughtfulness to support CRN.
2nd Annual C.H. Robinson Golf Tournament Honors Tahnie Woodward

On September 6, 2008, the 2nd Annual C.H. Robinson Golf Tournament took place at the beautiful Johnny Miller signature course at Thanksgiving Point located in Lehi, Utah.

The event raised over $27,000 for CRN!

Golfers and/or volunteers included:

• About 25 C.H. Robinson Worldwide employees from CHRW-Salt Lake City, Southwest Produce Division, Savannah, Atlanta, Wichita, Los Angeles International Branches, and National Accounts
• Mijken Cassidy, Robyn, Tahsha and Kenley Kofford from Kofford Trucking
• Scott Tucker and Michael GARlick from Rocky Mt. Music
• Frankie and Laura McGinnis, Rory Boyer, Rock, Pam, and Tahnie Woodward representing Cystinosis Research Network
• A special appearance by Ashley Kazian Miss United States 2007!

A total of 136 golfers participated in the event. Each participant received a custom golf bag filled with all the golfing essentials. The winners also received Jazz tickets and passes for future use at Thanksgiving Point. There was also a raffle, which included numerous prizes donated by C.H. Robinson customers, local merchants, or Cystinosis families. So everyone was a winner!

It was a beautiful fall day and a good time was had by all who attended. We are also pleased to announce that C.H. Robinson will be hosting a 3rd annual golf tournament scheduled for June 13, 2009.

Thank you for fundraising!

Miss United States Ashley Kazian sports a candy necklace Laura McGinnis made for her at the golf tournament.

Do you GoodShop?

Place an online order through goodshop.com and raise money for CRN! See page 30 for more details!
What an amazing experience this past year has been as Miss United States 2007 and the National spokesperson for Cystinosis. I have met some wonderful people, had some amazing experiences, and made so many once-in-a-lifetime memories – all while going to school full time!

Last fall I had the opportunity to do a newspaper interview with the McGinnis family whose daughter, Laura, has Cystinosis. Laura is my inspiration and through this contact, my Cystinosis family has grown by leaps and bounds. During Eastside High School’s week of fundraising for Cystinosis in October 2007, I was honored to be named the Cystinosis National Spokesperson. What a great opportunity to help educate and help raise awareness (and money) for this devastating disease. In December, I went with Laura to the National Institute of Health in Bethesda, Maryland for her annual checkup. I was able to meet Dr. Gahl and get a first hand peek at what these children endure while they are at NIH.

In January, Laura and I were on Your Carolina with Jack Roper and Kimberly Kelley. We had the chance to talk about Cystinosis and how the community can help and get involved. In May, I traveled to Bloomfield Hills, Michigan where I got to meet Kacy Wyman and her family and participate in the Cystinosis Run and a Princess Party. It was so heartwarming to see how the community of Bloomfield Hills has rallied together for this cause.

In September, I went to Salt Lake City, Utah for a Cystinosis Golf Tournament put on by the Woodward Family. My final event as Miss United States 2007 was in Downer’s Grove, Illinois on September 18th at the Festival of Hope. I got to meet Alexis and Evan LeBeau who both have Cystinosis and were very inspiring. I can’t think of a better way to have ended this amazing year.

During my 396 days as Miss United States, I traveled over 51,533 miles, helped raise over $350,000 for Cystinosis and each time I signed an autograph, my card had the Cystinosis link on it. I will continue to spread the word and do my part to help put an end to this terrible disease. Thank you to my Cystinosis family for this opportunity and for sharing your love with me. Your angels make the world a better and brighter place.

Much Love,

Ashley Kazian
Miss United States 2007
Ashley Kazian’s Cystinosis Scrapbook

August, 2007
Ashley is crowned Miss United States, 2007.

October, 2007
Ashley is named spokesperson for CRN at the Eastside pep rally for Wade Hampton Week.

December, 2007
Ashley travels with Laura McGinnis to the NIH and meets Dr. Gahl.

May, 2008
Ashley signs autographs and poses with the winners at the 2nd Annual 5K Fun Run in honor of Kacy Wyman.

May, 2008
Ashley and Kacy Wyman at the Princess party before the fun run. Photos were taken with Ashley by a photographer and sold to raise money for CRN!

January, 2008
Ashley and Laura appear on "Your Carolina" with Jack Roper and Kimberly Kelley.

September, 2008
Ashley with the golf team from Rocky Mt. Music at the 2nd Annual C.H. Robinson Golf Tournament.

September, 2008
Ashley tries her crown on Alexis LeBeau at a Festival of Hope—her last appearance as Miss United States.

Thank you for your support, Ashley, and best of luck in the future!
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 (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. CRN funds research and programs primarily through donations from the public, grassroots fundraising events and grants.

CRN provides outreach and access to resources. We take great pride in carrying out our motto:

“Dedicated to a Cure. Committed to our Community”...whether you are ...

- A Parent who needs critical resource information, support services or help in sharing the challenges of cystinosis to those who serve your child.

- An Adult with cystinosis interested in information regarding medical and social issues that are specifically geared for adults.

- A Relative or a Friend who wants to increase their understanding of cystinosis and find out how you can help out or become involved.

- A Physician, Social Worker, Educator or other Professional who makes a difference in the life of a family affected by cystinosis, and want to have access to critical information to better serve your patient, student or client.

Joining the Cystinosis Research Network enables you to:

- Receive all the latest cystinosis information through our countless resources, including the biannual CRN Newsletter, our very informative web page www.cystinosis.org, the popular online Cystinosis Support Group, and our toll free number (1-866-276-3669).

- Attend the CRN Family Conference with other cystinosis families to exchange knowledge and create friendships. Also, find out the latest discoveries about cystinosis from the medical professionals.

- Let your voice be heard by legislators and policymakers who need to know why cystinosis (and other rare diseases) are important issues to you.

- Have access to the Cystinosis Research Network’s representatives in the areas that are most relevant at any given time to you or your loved one affected by Cystinosis.

Join the Cystinosis Research Network today!

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

Pam Woodward
VP Family Support, Cystinosis Research Network
Cystinosis Research Network Membership Form:

Immediate Family: .......... $20.00       Extended Family / Friend: .... $25.00
Professional: ................... $35.00          International: (Including Canada) Base rate
(see above categories)
plus $10.00 for postage. Payable in US dollars

Please complete the form & mail with check payable to CRN to:

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

Name____________________________________________________________________________
Street____________________________________________________________________________
City & State__________________________________________ Zip Code__________ Country ____
Phone__________________________Fax_______________________Email____________________

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

Join A CRN Support Group

Looking for a way to communicate with others in the Cystinosis Community on a day-to-day basis?

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

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

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

To join a support group, visit www.cystinosis.org.
Please Support CRN’s Mission with Your Donation

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

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

Name_________________________________________________________________
Street_________________________________________________________________
City & State___________________________________   Zip Code_________________
Phone___________________Fax____________________Email__________________
In Honor Of_____________________________________________________________
In Memory Of___________________________________________________________
You may send notification of my gift to:
______________________________________________________________________

Please check all that apply:
  _____Friend     _____Individual with Cystinosis
  _____Parent of Child with Cystinosis   _____Professional
  _____Family     _____I am interested in volunteering for CRN. Please contact me.

Search the Internet Using GoodSearch and Raise Money for CRN

Every time you use GoodSearch.com to search the Internet, a donation is made to the Cystinosis Research Network! GoodSearch.com is powered by Yahoo!, so you get the same great 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 www.goodsearch.com. Be sure to select "Cystinosis Research Network" where it says "Who do you GoodSearch for?" Then search like you normally would! It costs you nothing.

CRN hopes you will give it a try and support Cystinosis Research. The more people who use this site for CRN, the more money is earned. So please tell your friends and family!

Make Online Purchases using GoodShop and Raise Money for CRN

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

To get started, simply go to www.goodshop.com, choose CRN as your charity, and be sure to 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, Chicos, Coldwater Creek, Christopher and Banks, American Eagle Outfitters, and many many more!
### United Way Contribution Guidelines

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

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

### Buy Cystinosis Research Network Gear at the CRN Store

CRN has teamed up with cafepress.com to offer CRN merchandise. Items include t-shirts, sweatshirts, hoodies, tanks, camis, hats, bags, housewares, buttons, printed material, and more. Clothing is available for men, women, children, and even pets! The best part is that up to 30% of your purchase goes back to CRN!

All items have recently been reduced by 15% and include the new CRN logo.

Some items are available in bulk. Clothing and accessories are great to wear to cystinosis fundraisers.

Access the CRN Store at:
http://www.cafepress.com/cystinosis

Shop til’ your heart’s content, and be sure to tell your friends and family!

### 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!
CRN Vision and Mission

Vision. The Cystinosis Research Network’s vision is the discovery of improved treatments and ultimately a cure for 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.