

When your child's future is uncertain, hope, community and prayer sustain you.

CYSTINOSIS RESEARCH FOUNDATION

Thank you for your love, your generosity and for sustaining us.



Dear Family and Friends,

As the holidays approach, our family has so much to be thankful for. This year has been one of significant scientific progress and exciting growth for the Cystinosis Research Foundation (CRF). We are humbled by the love, support and generosity that all of you continue to display.

We have come so far in the last few years and we are moving closer to our mission: to find better treatments and a cure for cystinosis. We have much to be thankful for...

- Our annual **Natalie's Wish** event in May raised more than \$1.8 million for cystinosis research thanks to the generosity of our family and friends. In just six short years we have funded 45 research studies and fellowships totaling over \$6.9 million dollars. The CRF is funding quality research in five countries around the world.
- For several years the CRF has supported **Dr. Dohil and Dr. Schneider's research** at the University of California, San Diego to find a slow-release medication. We are pleased to announce that we have reached a significant goal! Drs. Dohil and Schneider discovered a slow-release form of the current medication and patented their discovery. In December, 2007, Raptor Pharmaceuticals bought the worldwide license for EC Cysteamine. Raptor Pharmaceuticals is reformulating EC Cysteamine and will initiate clinical trials for patients with cystinosis in Spring, 2009. Raptor will seek FDA approval in 2009 and anticipates that the new slow-release medication will be approved in 2010. The CRF will support and work with Raptor through the clinical trial phase. A slow-release medication is on the way for every child and adult with cystinosis!
- In October, we announced a \$750,000 **global call for cystinosis research proposals and fellowships**. After the CRF Scientific Review Board has evaluated each proposal and made its recommendations, we will announce the approved grants.
- The **Cystinosis Research Fellowship Program**, created to encourage new investigators to study cystinosis, is now supporting seven research fellows. The scientists are working every day to find better treatments and a cure for cystinosis.
- The first CRF International **Cystinosis Research Symposium** was held on April 3 and 4, 2008 at the Arnold and Mabel Beckman Center of the National Academies of Sciences and Engineering. The symposium was chaired by Dr. Ranjan Dohil and Dr. Jerry Schneider, and was attended by CRF-funded researchers from around the world. The symposium provided the attendees with an opportunity to present their research, share information and initiate collaborations. The abstracts presented were recently published in the *Pediatric Nephrology* journal and are available on our website at www.cystinosisresearch.org in the *Research* section under *Published Studies*.
- We are pleased to announce that **Jason Grier** of the Hope for Holt Foundation has joined the CRF Board of Directors. Jason and Chrissy Grier are parents of three beautiful children; Mary Logan, Jack and Holt. Their three-year old son, Holt has cystinosis. The Griers and their community have been actively raising funds for cystinosis research and working in partnership with CRF since 2007.
- We are pleased to announce the **redesign of the Natalie's Wish website**, www.cystinosisresearch.org. The site is a primary source of information for our research community, as well as a reference point for the cystinosis community. Our new website is more comprehensive and easier to navigate, and for the first time offers individuals and corporations a way to make online donations. We've also added a feature that provides CRF partners and volunteers with ideas and resources to assist in their fundraising efforts.

Since 2003, the CRF has funded 45 research studies and currently has 25 ongoing bench and clinical studies. CRF-funded researchers are seeking to understand what causes damage to the kidneys, eyes and brain. We remain focused in our quest to find a cure and better treatments for all who suffer from this devastating disease.

We are grateful to each and every one of you for your generosity. You have given Natalie and the other children and young adults with cystinosis the hope they need to sustain them as they dream of a future free from this disease.

We will be thinking of you this Thanksgiving as we count our blessings and remember all of you who have given so much to make Natalie's wish – and the dream of all children with cystinosis – a reality.

*Thank you so very much,
Nancy, Jeff, Alexandra and Natalie Stack*

SAVE THE DATE!
Friday, May 8, 2009

Eighth Annual
Natalie's Wish Event
Balboa Bay Club,
Newport Beach, CA

CYSTINOSIS RESEARCH FOUNDATION

Spring 2008 Research Studies Funded

Corinne Antignac MD, PhD

Hospital Necker, Paris, France
"Characterization of the Interaction of Cystinosin with Galectin-3 and Vacuolar H+ - ATPase"
\$230,000 – 2-year study

Stephanie Cherqui, PhD ■ Daniel Soloman, MD

The Scripps Research Institute, La Jolla, California
"Treatment of Cystinosis Nephropathy using Ureteral Injection of Adeno-associated Virus Expressing CTNS"
\$249,128 – 1-year study

Francesco Emma, MD ■ Anna Taranta, PhD

Bambino Gesù Children's Hospital and Research Institute, Rome, Italy
"Identification and Analysis of Cis- and Trans-acting Elements that Activate the CTNS Gene"
\$125,140 – 2-year study

Bruno Gasnier, PhD, Mentor ■ Xiong Chen, PhD, Fellow

Institut de Biologie Physico-Chimique, Paris, France
"Molecular Anatomy and Physiology of Human Cystinosin"
\$223,200 – 3-year study

Vasiliki Kalatzis, PhD ■ Eric J. Kremer, PhD

Institut Génétique Moléculaire Montpellier, Montpellier, France
"Gene Transfer Studies for Cystinosis"
\$66,900 – 1-year study

Vasiliki Kalatzis, PhD, Mentor ■ Claire Hippert, Fellow

Institut Génétique Moléculaire Montpellier, Montpellier, France
"Gene Transfer Studies for Cystinosis"
\$63,300 – 1-year study

Elena Levchenko, MD, PhD; Lambertus van den Heuvel, PhD;

Francesco Emma, MD, Mentors ■ Wilmer Martijn, Fellow
University Hospital Leuven, Belgium
"Pathogenesis of Renal Disease in Nephropathic Cystinosis"
\$165,000 – 2-year study

Jennifer Simpson, MD ■ James Jester, PhD

University of California, Irvine
"Evaluation of Novel Corneal Imaging and Therapeutics in the CTNS Knockout (Cystinosis) Mouse Model"
\$89,399 – 2-year study

Mary L. Taub, PhD

State University of New York at Buffalo
"Mechanisms Underlying the Fanconi Syndrome in Cystinosis"
\$104,294 – 1-year study

Total Committed Research Studies Spring 2008: \$1,316,361

Upcoming CRF Community Events

■ St. John Fashion Show for CRF on November 18, 2008 at 6 pm

St. John has announced a new label under the moniker SoCa. The collection will launch at the South Coast Plaza store, with a percentage of the opening day's proceeds benefiting CRF.

We hope you will join us on November 18 at 6 pm for this event. Please RSVP to retail.marketing@sjk.com by November 15.

■ Jay Strongwater at Neiman Marcus on November 21 from 4 to 6 pm

Jay Strongwater will appear at the Fashion Island Neiman Marcus to introduce his designs for the holiday season, offering treasures that range from breathtaking miniature boxes to elaborate mirrors entwined with crystal-studded flowers.

10 percent of the proceeds will benefit CRF.
Please RSVP to (929) 223-7610 by November 17.

CYSTINOSIS RESEARCH FOUNDATION

18802 Bardeen Avenue ■ Irvine, CA 92612-1521 ■ (949) 223-7610

100 percent of all donations go directly to cystinosis research. Your gift is tax deductible.

The Cystinosis Research Foundation is a non-profit, tax-exempt entity pursuant to Section 501(c)3. Federal Tax ID # 32-0067668

Families Around the Country Support CRF Research

Our growing success is due to the love and support from cystinosis families around the country who have embraced the cystinosis cause and are enthusiastically raising funds for research.

Their prayers, financial support and commitment have brought us closer than ever to extraordinary changes in the lives of children with cystinosis. This community is making great strides to help fund research and to finding a cure.

- **Denice and Mark Flerchinger** and their family of Clarkston, Washington, created a heart-warming letter-writing campaign chronicling their daughter Tina's life with cystinosis. The campaign raised over \$11,000.
- **Chrissy and Jason Grier** of Charlotte, North Carolina, with their family and community, have raised over \$102,000 through their **Hearts for Holt** dinner event, **Carolina Speed Football Game**, and the **Change for Change** event. They have held two golf tournaments in honor of Holt. www.hopeforholt.org
- **Teresa and Kevin Partington** of Sacramento, California presented a check for \$200,000 from the **Jenna and Patrick Foundation of Hope** at the Natalie's Wish event in May. And their June golf tournament was over-sold raising \$225,000. www.jennaandpatrick.org
- **Tricia and Brian Sturgis** of Sandpoint, Idaho, along with their community, organized a bike-a-thon, **24 Hours for Hank**, which raised \$42,000. www.24hoursforhank.org



Lula Halfacre of Traditional Jewelers with Jeff and Nancy Stack

Traditional Jewelers Annual Watch Faire Raises \$10,000 for CRF

Traditional Jewelers and *Riviera* magazine celebrated the store's sixth annual Watch Faire on September 18. Lula Halfacre and Traditional Jewelers have been longtime supporters of Natalie's Wish and CRF.

BOMA Golf Tournament Raises \$9,065 for CRF

The Building Owners and Management Association (BOMA) held their annual golf tournament on May 13, raising \$9,065 for CRF. This was the fourth year the CRF was the recipient charity bringing total donations from BOMA to more than \$49,185.

David Yurman and CRF Host an Exclusive Evening to Celebrate the New David Yurman Fragrance

David Yurman, America's renowned fine jewelry designer introduced his first signature fragrance on Thursday, July 24 at South Coast Plaza with proceeds benefiting Natalie's Wish and CRF. CRF Board Member, Renee Carter chaired the festivities, which raised \$7,500.



Renee Carter with friend Jeanine Veldhuis