

AUGUST 2011

## You Buy It, You Drive It!

Saturday, August 13, 2011 ★ Crash and Crush for a Cure!



Ben's Asphalt of Orange County has generously donated a car to Natalie's Wish to be auctioned off at the upcoming Orange Crush Demolition Derby at the OC Fair. The winner of the auction will have the opportunity to drive Natalie's big red car in the derby and experience the thrill of road rage legally or designate someone to take their place.

Please plan on joining us and invite your friends for this exciting and once in a lifetime event. Tickets are available online

<http://www.ticketmaster.com/event/OB004648A47F48CD?artistid=1554900&majorcatid=10004&minorcatid=25>

### SAVE THE DATE – Monday, November 14, 2011

#### Fourth Annual Fore a Cure Golf Tournament

We are fortunate to once again have Vince Ciavarella as our chairman and he is surrounded by an enthusiastic group of committee members to ensure the best tournament experience in Orange County. We expect the event to be a sellout and encourage sponsors to sign up early. For sponsorship information contact Zoe Solsby, (949) 223-7610 or [zsolsby@cystinosisresearch.org](mailto:zsolsby@cystinosisresearch.org)



CURE CYSTINOSIS  
INTERNATIONAL REGISTRY

The CCIR is an essential tool for the global cystinosis research community and the data it provides is critical to new research studies and the development of novel therapeutic treatments.

Patients from 30 countries have already registered to help

accelerate cystinosis research progress. Be part of the cure! Register at [www.cystinosisregistry.org](http://www.cystinosisregistry.org).

Questions? Contact CCIR Curator, Betty Cabrera ([curator@cystinosisregistry.org](mailto:curator@cystinosisregistry.org)).

CYSTINOSIS  
RESEARCH  
FOUNDATION

949-223-7610

[cystinosisresearch.org](http://cystinosisresearch.org)

### RESEARCH NEWS

#### CRF Awards \$1,070,458 In Grants For Cystinosis Research

We are pleased to announce that five scientific awards were granted to researchers in the United States and Belgium. They include continued funding for Stephanie Cherqui, PhD for her breakthrough progress in stem cell therapy. Because of our generous friends and community, the CRF has funded and committed \$12.9 million to researchers and scientists around the world who are working to find better treatments and a cure for cystinosis. For a list of all of the new research grants click here

<http://www.natalieswish.org/page.aspx?News-Info/75>

#### CRF Researcher Elena Levchenko, MD, PhD Published in Prestigious Journal

This week, Elena Levchenko, MD, PhD was published in the prestigious *Journal of Pediatrics* for her research paper "Cysteamine Toxicity in Patients with Cystinosis." CRF is proud to have supported this important study. Be sure to read her article it is both interesting and informative.

### RESEARCH GRANT UPDATES *(click on the titles to view each update)*

#### ■ Molecular Study of Lysosomal Transporters Involved in the Cystine-Depleting Effect of Cysteamine – Two-year study – Grant awarded: September, 2010

**Bruno Gasnier, PhD**, Principal Investigator, Institut de Biologie Physico-Chimique, Paris, France  
**Ellen Closs, PhD**, Co-Principal Investigator, Gutenberg University Medical Center, Mainz, Germany

#### ■ Molecular Anatomy and Physiology of Human Cystinosis

Three-year study – Grant awarded: October, 2008

**Bruno Gasnier, PhD**, Principal Investigator, Institut de Biologie Physico-Chimique, Paris  
**Xiong Chen, PhD**, Post-Doctoral Research Fellow, Institut de Biologie Physico-Chimique, Paris, France

#### ■ Studying Podocyte Function in Nephropathic Cystinosis

Two-year study – Grant Awarded: January, 2011

**Elena Levchenko, MD, PhD**, University Hospitals Leuven, Belgium

#### ■ Cysteamine Effects on Extracellular Matrix Accumulation in Chronic Kidney Disease

Three-year study – Funded July, 2008 - Final Report

**Allison Eddy, MD**, Seattle Children's Hospital Research Institute, Seattle, Washington  
**Daryl Okamura, MD**, Seattle Children's Hospital Research Institute, Seattle, Washington

### CYSTINOSIS FAMILIES UNITED FOR THE CURE

**October 8, 2011 – Portland, Oregon** – Matt Gillis is committed to Henry Sturgis of Sandpoint, Idaho. Matt says, "If a lot of people do a little thing, the net effect is a big thing," and Matt plans to once again be running to demonstrate his philosophy really works, this time in the Portland Marathon. He figures it will take 52,800 steps to complete the race and he is asking sponsors to donate \$1 a step! To help Matt step closer to his goal visit <http://www.payinitforward.com/index.php>



**Waterloo, Canada – Update** – *The Real Men Can Cook* event was organized by a dedicated committee who brought community leaders together to offer up a tasty evening of food, fun and fundraising. This year's gathering netted \$30,334 for cystinosis research in honor of Gabbie Strauss. Click to read about this fun filled evening <http://www.kwnow.ca/npps/story.cfm?nppage=1784>

**Kente Kiwanis Club – Canada** – The local Kiwanis Club has focused on a fundraising drive in honor of Gabbie Strauss. Last week, a \$500 check was presented to Gabbie's parents Trevor and Jody Strauss who founded the Cystinosis Awareness & Research Effort Foundation.

<http://www.emcbelleville.com/20110630/News/Cystinosis+research+may+help+little+Gaby>

The mission of the Cystinosis Research Foundation is to support bench and clinical research that is focused on developing better treatments and ultimately a cure for cystinosis.

Cystinosis is an "orphan" disease affecting approximately 500 people – mostly children – in the U.S. and 2,000 worldwide.

Cystinosis is a metabolic disease that slowly and eventually destroys the organs in the body including the kidneys, liver, eyes, muscles and the brain. Although medication is available to control some of the symptoms, cystinosis remains incurable.



If you would like to receive a copy of *Cystinosis Magazine* and are not already on our mailing list, email [nstack@cystinosisresearch.org](mailto:nstack@cystinosisresearch.org)

CRF is committed to raising awareness about cystinosis, expanding our network of supporters, and releasing current news through social media sites.

Help us by joining our groups, adding us as a friend, and inviting others to do so!

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