

New Clinical Research Study At Ronald Reagan UCLA Medical Center



Research Study of Bone Marrow Transplant for Cystinosis Patients From Matched Related Donor >

We are pleased to announce that recruitment of patients has begun for the first stem cell pilot trial. We are thankful to each and every one of you for your support and commitment to the cystinosis community. This is the beginning of a new chapter in the treatment of cystinosis, congratulations to all of you for giving the cystinosis community the gift of hope.



The current issue of **Cystinosis Magazine** is now available. If you aren't on our mailing list and would like a copy, please email your address to Nancy Stack at nstack@cystinosisresearch.org

NEW CLINICAL RESEARCH STUDY AT RONALD REAGAN UCLA MEDICAL CENTER

The first allogeneic (donor) stem cell transplant was approved by the UCLA Investigational Review Board (IRB) and the recruitment process has begun.

The stem cell protocol was designed by a team of leading stem cell experts and cystinosis experts who formed the CRF Cystinosis Gene Therapy Consortium. The Consortium members are:

Dr. Corinne Antignac (Hospital Necker, Paris, France), **Dr. Stephanie Cherqui** (Scripps Research Institute), **Dr. William Gahl** (NIH), **Dr. Don Kohn** (UCLA), **Dr. Ted Moore** (UCLA), **Dr. Dan Salomon** (Scripps Research Institute) and **Dr. Jerry Schneider** (UCSD).

If you would like additional information about the pilot study, please view the ad on the CRF web site <http://natalieswish.org/files/11-003240CystinosisUCLASTudy-5-8-12.pdf>, or visit the Cure Cystinosis International Registry, CCIR .

If you are interested in learning more, please contact your primary care physician and ask him/her to contact Zoe Solsby at CRF. Zoe's email address is zsolsby@cystinosisresearch.org and her phone number is (949) 223-7610.

Congratulations to SRB Member Julie Ingelfinger, MD for receiving this year's American Society of Pediatric Nephrology's (ASPEN) Founder's Award. This honor is given to persons who have made a unique and lasting contribution to the field of pediatric nephrology.



CURE CYSTINOSIS
INTERNATIONAL REGISTRY

Participation in the registry offers every member of our community a role in cystinosis research and the quest for a cure. More than 321 cystinosis patients from 31 countries around the world have registered to help accelerate cystinosis research progress! **Join today and register at www.cystinosisregistry.com**

CRF WELCOMES NEW SCIENTIFIC REVIEW BOARD MEMBERS

Pierre J. Courtoy, MD, PhD

Board certified Internist; Full Professor of Cell biology and General Pathology and past-President of the Department of Biochemistry and Cell Biology at the Louvain University Medical School, Head of Cell Biology Unit at de Duve Institute (CELL/ICP); Head of the Platform for Imaging Cells and Tissues (PICT), UCL and ICP, UCL-7541, Brussels, Belgium

Lisa M. Guay-Woodford, MD

Hudson Professor of Pediatrics, Associate VP for Clinical and Translational Research, The George Washington University; Director, Center for Clinical and Community Research; Director, CTSI-CN , Children's National Medical Center, Washington, DC

Martin Konrad, MD

Department of General Pediatrics, University Children's Hospital, Muenster, Germany

William E. Smoyer, MD, FASN

C. Robert Kidder Chair, Vice President, Clinical and Translational Research Director, Center for Clinical and Translational Research The Research Institute at Nationwide Children's Hospital, Professor of Pediatrics, The Ohio State University, Columbus, Ohio

CRF SRB

The Scientific Review Board is composed of leading cystinosis scientists and experts from around the world.

We are indebted to the SRB for their guidance and commitment to helping our children, please join us in welcoming our newest members.

PUBLISHED RESEARCH STUDIES

■ **Cystinosin is a Melanosomal Protein that Regulates Melanin Synthesis** – A research paper by **Robert Ballotti, PhD**, INSERM, Nice, France, has been accepted for future publication in *The FASEB Journal*.

RESEARCH UPDATES

■ Molecular Study of Lysosomal Transporters Involved in the Cystine-Depleting Effect of Cysteamine

Two-year study – Funded September 2010

Bruno Gasnier, PhD, Principal Investigator, Université Paris Descartes, Paris, France

Ellen Closs, PhD, Co-Principal Investigator, Gutenberg University Medical Center, Mainz, Germany

CYSTINOSIS FAMILIES UNITED FOR A CURE

The Fourth Annual Wine, Stein & Dine – held in honor of Tina Flerchinger was attended by more than 250 guests and raised more than \$90,000 for cystinosis research. Congratulations to the Flerchinger family and the dedicated volunteers who created a night to remember!

Saturday, June 16 and Sunday, June 17, 2012 – Pepsi Racing Power Cup Challenge, Chamberlain, South Dakota

Friday, June 22, Saturday, June 23 and Sunday, June 24, 2012 – Idaho Regatta, Snake River, Burley, Idaho

Saturday, July 7 and Sunday, July 9, 2012 – Western Divisionals, Black Lake, Olympia, Washington. Jade Yount and Team Toothpick Racing continues it racing season in honor of 3-year-old Mary Head. You won't want to miss one of these exciting hydroplane races being held around the country. www.teamtoothpickracing.com

Friday, July 6, 2012 – Hookstown, Pennsylvania – First Annual Lots of Love for Landon Charity Golf Outing to be held at the Ponderosa Golf Course in honor of 2-year-old Landon Hartz. For more information contact Jason Whitfield at (412) 535-0022 or jwhitfield@allenshariff.com.



Saturday, August 11, 2012 – Crash and Crush for a Cure, in honor of Natalie Stack, will be held at the Orange County Fairgrounds and is once again generously sponsored by Ben's Asphalt of Anaheim. You won't want to miss this fun-filled family event, tickets are available online. <http://ns.ocfair.com/ocf/calendar/calendar.asp>
We look forward to seeing you there!

