



FASTER TRACK

APRIL 2010

Emil's Blog:

Thanks to all our Partners and Friends for your calls to Senators Brownback and Kohl — it is working!

It was exciting to see grassroots politics in action through your 100+ calls in support of our \$10 million request to create a specific review division at the FDA. The respective offices were inundated with calls from very motivated patient community leaders, and the numerous passionate callers were noticed.

Read more on Emil's blog at <http://www.kakkis.org/blogs/>

Add Your Organization to our Letter Requesting an Appropriation for Rare Disease Drug Review

Please join our growing list of patient organizations signing onto our letter in support of providing additional resources to the Food and Drug Administration to create a more specialized drug review by experts who understand rare diseases. We are specifically requesting \$10 million in the fiscal year 2011 Ag-Rural Development-FDA Appropriations bill directing the FDA to establish a new review division for Biochemical and Genetic Diseases within the Center for Drug Evaluation Research, Office of New Drugs. If you would like to add your organization or company to the list, please respond via email to jjenkins@kakkis.org by close of business, Friday April 30th.

CURETHEPROCESS™ Campaign Exceeds 100 Partners

We are pleased to announce that the American College of Medical Genetics (ACMG) and EURORDIS have endorsed our Campaign. ACMG provides education, resources and a voice for the medical genetics profession. Their mission is to make genetic services available to improve the health of the public. ACMG promotes the development and implementation of methods to diagnose, treat and prevent genetic diseases. EURORDIS is a non-governmental patient-driven alliance of patient organizations and individuals active in the field of rare diseases in Europe, representing more than 400 rare disease organizations in 40 different countries. It is the voice of the 30 million patients affected by rare diseases throughout Europe. Our Campaign to CURETHEPROCESS now has more than 100 partner organizations. The Foundation recently held a conference call with the partners to give an update on the campaign. Click to [View](#) or [Listen](#) to the presentation. If your organization would like to endorse our campaign and partner with us to CURETHEPROCESS, please contact ssmith@kakkis.org. To see our complete list of partners go to: <http://curetheprocess.org/partners>

Support the Medical Food Equality Act – Write Your Congress Member

The National PKU Alliance is seeking co-sponsors to SB 2766/HR 2766, the Medical Foods Equity Act. This bill will help ensure that more than 20,000 children and adults who suffer from more than 25 rare metabolic disorders get access and coverage for the medication they need to stay healthy. In order to remain healthy, children must drink a medical formula and eat foods modified to be low in protein every day for the rest of their lives. These special medical foods cost hundreds of dollars each month and are rarely covered by insurance. [Click Here to Write Your Congress Member.](#)

The Kakkis EveryLife Foundation Endorses the “Where Are The Cures?” Campaign

WhereAreTheCures.org is a patient advocacy initiative of the Myelin Repair Foundation, dedicated to educating all Americans about the current state of the medical research enterprise, the barriers that are slowing the delivery of patient treatments, and the innovators who are challenging the status quo. It provides a meeting place where patients, their families and all others can collectively raise their voices and gain momentum for change. Join Dr. Kakkis in signing the Patient's Manifesto today at www.WhereAreTheCures.org.

Now You Can Follow Us on Facebook & Twitter

To receive CURETHEPROCESS™ Campaign Action alerts please follow us on Twitter.

To receive Foundations updates, join our Group on Facebook.



In Other Rare Disease News:

A Win for Rare Disease -- "Ideas for Change in America"

Catherine Calhoun of Louisiana has been named one of the 10 winners in the country in the “Ideas for Change in America” competition. The “Ideas for Change” competition is held by www.Change.org and will help develop a national campaign to promote rare diseases with the help of its 1 million members. Catherine's son Billy has a rare condition, called McCune-Albright syndrome,

which has an incidence rate of one in a million. Catherine is a volunteer activist on behalf of those with a rare disease and will be working with Change.org to develop a campaign to educate Congress about rare diseases. Congratulations, Catherine, the Children's Rare Disease Network and to all those who voted to ensure rare diseases made the Top 10.

Senator Specter's Cures Acceleration Network (CAN) Passes in Healthcare Reform

[CAN](#) was created in the Patient Protection and Affordable Care Act (Section 10409) and signed into law on March 23, 2010. The law creates CAN at the National Institutes of Health (NIH) to help bridge the scientific "Valley of Death": i.e., to fund translational research to move high need medical cures through the development pipeline faster – by cutting the time between scientific discovery and the development of promising new drugs and therapies. With the CAN authorization now in law, Congress needs to appropriate funds so that NIH can begin implementation. Senator Arlen Specter (D-PA) is requesting that the Senate Labor-HHS Appropriations Subcommittee include \$500 million for CAN. The Kakkis EveryLife Foundation is one of more than 80 organizations supporting this appropriation request.

National MPS Awareness Day on May 15, 2010

The National MPS Society is recruiting members, families and friends to contact their U.S. Senators to garner support for National MPS Awareness Day on May 15, 2010. Senator Lindsey Graham (SC) is sponsoring the resolution, and would love to have other Senators co-sponsor with him. Please contact the National MPS Society at (919) 806-0101 or email: ernie@mpssociety.org before May 7th if you would like additional information.

FASTER TRACK to CURETHEPROCESS™

Through our newsletters, we hope to keep you informed of our efforts to get treatment development for orphan diseases back on the fast track through science-based regulatory reform. We encourage your questions, input and involvement on our mission to CURETHEPROCESS. Please email us at info@curetheprocess.org or call us directly at (415) 884-0223 to see how you can help.