



FASTER TRACK

AUGUST-SEPTEMBER 2009

FASTER TRACK to CURETHEPROCESS™

The second installment of the Kakkis EveryLife Foundation e-newsletter bears the name "FASTER TRACK." Thru our newsletters, we plan to keep you informed on 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 on 415 884 0223 to see how you can help.

Kakkis.org launched!

Please check out our new website at www.Kakkis.org. You will soon be able to make donations to help support our Foundation, endorse the CURETHEPROCESS Campaign, and learn about our efforts through NAMA to improve treatment of rare diseases.

Coming soon: Write your Congress member to ask them to support the CURETHEPROCESS campaign.

Dr. Kakkis joins in the Health Care Reform debate in this month's blog.

Dr. Kakkis shares his thoughts on how to bring down health care costs. Check out Emil's Blog by clicking here www.kakkis.org/blogs.

Kakkis EveryLife Foundation Art Contest

The Foundation invites artists to participate in the first annual EveryLife Art Contest. The contest hopes to empower **artists affected by rare diseases** to express their unique struggle with the disease. The EveryLife Art Competition is open to all artists affected by a rare disease ages 5 and older. Artists may have a rare or undiagnosed disease or may be close friends, family members, or care takers of those with a rare or undiagnosed disease. Grand Prizes will be awarded in each of the 3 age categories.

Entries must be received by 5pm on Friday October 30, 2009.

To find out more information on the Prizes and how to enter, please visit <http://www.kakkis.org/Contest/Contest.aspx> or email info@kakkis.org

Thank you to our Partners in the CURETHEPROCESS Campaign

As we work on creating science driven public policy change, we are asking Patient Organizations to partner with us to endorse our goals with their membership, and when the time is right, to reach out to the FDA and Congress to help implement new policy that will improve the regulatory process for new treatments targeting rare biochemical and genetic disorders. If your organization would like to endorse our Campaign and partner with us to CURETHEPROCESS please contact Julia Jenkins at jjenkins@kakkis.org or 415-884-0223.

We are very excited to announce that the following organizations have endorsed our efforts to CURETHEPROCESS:

Advocacy for Patients with Chronic Illness www.advocacyforpatients.org

Alpha-1 Association <http://www.alpha1.org/contact/index.php>

American Heart Association www.americanheart.org

ARPKD CHF Alliance <http://www.arpkd.org/>

Children's Rare Disease Network www.theprojectcharity.org

Cystinosis Research Network www.cystinosis.org

Fabry Support & Information Group www.fabry.org

International Pemphigus and Pemphigoid Foundation www.pemphigus.org

John F. & Aileen A. Crowley Foundation <http://www.crowleyfamily5.com/index.htm>

Macular Degeneration Support www.mdsupport.org

Myelin Project www.myelin.org

National MPS Society www.mppsociety.org

National Organization for Rare Disorders www.raredisease.org

National PKU Alliance <http://npkua.org/>

NOMID Alliance www.nomidalliance.net

Kakkis EveryLife Foundation Scientific Advisory Committee Formed

The Foundation has created a Scientific Advisory Committee (SAC) to work on exploring creative and cutting edge solutions to the design and analysis of clinical studies for rare diseases. The Committee is tasked with creating new statistical models to validate the use of small clinical trials for very rare diseases and identify new methods for validating the use of biochemical surrogate endpoints. The Committee will be working closely with the NIH and plans to present its findings to the FDA in a workshop in 2010. The SAC members will be announced soon and will include prominent experts in clinical study design and analysis for rare diseases.

Genetic Alliance Annual Conference: Discovering Openness in Health Systems

The Kakkis EveryLife Foundation sponsored an information booth at the 2009 Genetic Alliance in July. Speakers included FDA officials as well the newly appointed NIH director Dr. Francis Collins. You can find additional speakers and more information about details of the presentations at the Conference, by visiting the Genetic Alliance <http://www.geneticalliance.org/conference09>.

If your organization would like to share CURETHEPROCESS Campaign materials at your upcoming event or if there are event sponsorship opportunities please contact jjenkins@kakkis.org

September Presentations:

International Congress of Inborn Errors of Metabolism Conference

Dr. Kakkis will talk about the Future Prospects of Enzyme Replacement Therapy

9/2/09; Manchester Grand Hyatt San Diego, San Diego CA.

****CURETHEPROCESS Roundtable Discussion****

The Manton Center for Orphan Disease Research at Children's Hospital Boston

Dr. Kakkis will be the Manton Center's first visiting scientist. The event is open to the public, patients, families, students, and health care providers are welcome.

9/9/09, refreshments at 1:00 followed by Dr. Kakkis' talk at 2:00 PM
Joseph B. Martin Conference Center at 77 Avenue Louis Pasteur Boston, MA.

International Lysosomal Disease Meeting

Dr. Kakkis will present on Enzyme Replacement Therapy for Lysosomal Disease

9/26-28/09; Nagoya, Japan

***If you would like to Dr. Kakkis or a representative of the Kakkis EveryLife Foundation to speak at your upcoming event, please contact info@kakkis.org.*

In other Rare Disease News:

Partnership to Enhance Research Related to the Management of Chronic Disease Survey

Advocacy for Patients with Chronic Illness received a grant from the National Institutes of Health in conjunction with the University of Michigan Center for Managing Chronic Disease to conduct a study of patients with chronic illnesses. This study looks at the challenges that people and their caregivers face when they have a chronic disease, how they cope with these challenges, and how they get information about their disease and its treatments.

If you have a chronic disease or a caregiver for a person with a chronic disease, please visit <http://chronicdisease.survey.sgizmo.com> or call Advocacy for Patients with Chronic Illness at (860) 674-1370 to arrange for a scheduled phone interview.

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