



## FASTER TRACK OCTOBER-NOVEMBER 2009

### FASTER TRACK to CURETHEPROCESS™

Read how busy we've been in the 3<sup>rd</sup> installment of the Kakkis EveryLife Foundation e-newsletter "FASTER TRACK." Through our newsletters, we plan 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](mailto:info@curetheprocess.org) or call us directly at 415 884 0223 to see how you can help.

### Brownback Brown Amendment for Rare and Neglected Diseases

The amendment directs the FDA to assemble two teams to evaluate various aspects of the regulatory process for rare and neglected diseases and provide a report to the Commissioner. This amendment is a first step toward establishing what the problems are and what changes are needed at the FDA for rare and neglected diseases. The passage of the Brownback/Brown amendment is an important step forward and an important first success for our CURETHEPROCESS campaign. We look forward to working with FDA and others on scientifically sound improvements to regulatory policy.

The amendment was created after Dr. Francis Collins (before becoming NIH director) spoke with Senator Brownback (R, Kansas) regarding rare and neglected disease regulation. Senator Brown (D, Ohio) co-sponsored the amendment and it was attached to the 2010 FDA appropriation bill. Dr. Kakkis met with Senator Brownback to support strengthening the amendment.

You can read more about it on Emil's blog at <http://www.kakkis.org/blogs/>

### Legends of Golf Tournament

The Kakkis EveryLife Foundation is proud to announce that the Children's Rare Disease Network, the National MPS Society and the Ryan Foundation will co-host the Inaugural Legends of Golf Tournament. The Golf Tournament will be held at the **Presidio Golf Course in San Francisco on Sunday January 10, 2010**, right before the start of the JP Morgan Healthcare Conference. For more information go to <http://www.kakkis.org/golf/>

### **Thank you to our Partners in the CURETHEPROCESS Campaign**

We now have over 60 partners in our Campaign to **CURETHEPROCESS**. Our Partners endorse our three goals that will help implement new policy to improve the regulatory process for new treatments for 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](mailto:jjenkins@kakkis.org) or 415-884-0223.

To see our list of partners go to: <http://curetheprocess.org/partners>

### **The EveryLife Art Contest – Deadline Extended to December 31<sup>st</sup> 2009**

We have received some incredible art, but it's not too late to submit yours. The Kakkis EveryLife 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 Contest 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: Children 5-11, Teens 12-17, & Adults 18+. [Employees or Board Members of the Kakkis EveryLife Foundation or their immediate family members are not eligible to participate.]

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](mailto:info@kakkis.org).

### **Where's Emil**

Our President and Founder, Dr. Emil Kakkis, is busy spreading the word on how to cure the process for rare disease drug development; that's why we've launched a new page to keep you informed of where he's been and where he will be next. You will see that he's been racking up the frequent flyers miles this month attending the Institute of Medicine Meeting for Accelerating Rare Diseases Research and Orphan Product Development, NORD's Executive Board & Corporate Council Meetings, and The Burrill Personalized Medicine Meeting. You can find him in December at the CNS Manifestations of Inborn Errors of Metabolism Workshop in Washington DC and the 7th International and Latin America Congress in Cancun, Mexico.

Get more info at Where's Emil <http://www.kakkis.org/Events.aspx>.

### **In Other Rare Disease News**

#### **Extraordinary Measures : The Official Trailer**

Extraordinary Measures is inspired by a Wall Street Journal article recounting John Crowley's remarkable efforts to save his children from a life threatening disease. Harrison Ford and Brendan Fraser star as the brilliant but unconventional scientist and the passionate father who form an unlikely alliance in pursuit of a medical miracle. Keri Russell portrays the mother, Aileen Crowley. Harrison Ford is executive producer and in fact brought the story to producers

after reading Pulitzer prize-winning writer Geeta Anand's Wall St Journal article on John and Aileen Crowley, who have two children living with Pompe Disease. The Movie is set for release January 22, 2010.

Watch the Trailer on YouTube <http://www.youtube.com/watch?v=bUCXtdTIUrk>