



KAKKIS EVERYLIFE FOUNDATION NEWSLETTER

JUNE 2009

We've been busy in our drive to accelerate innovation for rare diseases through an improved regulatory process. We've highlighted some items of interest, including [Emil's Blog](#). Check it out by clicking here www.kakkis.org/emilsblog. 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.

NORD'S Partners in Progress Summit

Dr. Kakkis presented his experiences in rare disease drug development and reasons for the CURETHEPROCESS campaign at the May 14th "Partners in Progress Summit". The Summit was part of an ongoing collaborative effort with the National Organization of Rare Diseases (NORD) as they create a national agenda for the Rare Disease Community. To view a video of Dr. Kakkis' presentation go to <http://curetheprocess.org/summit>

Select Speakers at the NORD summit included:

Tommy Thompson, former governor of Wisconsin and former Secretary of Health and Human Services;

Francis Collins, MD, PhD, Director of the National Human Genome Research Institute;

Janet Woodcock, MD, Director of the Center for Drug Evaluation and Research at FDA.

You can find additional speakers and more information about details of the presentations at the Summit, by visiting the NORD [website](#).

FDA Urged To Improve "Predictability" For Orphan Drugs By Social Security Commissioner Astrue

On June 4, 2009, Foundation representatives attended the National Chamber of Commerce discussion on Orphan Drug policy in Washington D.C., where the Social Security Administration Commissioner shared a similar perspective of the CURETHEPROCESS campaign. Echoing the call for process improvements targeting rare disease treatments, Commissioner **Michael Astrue** was quoted as saying "FDA's new leadership should focus on improving the *regulatory predictability* for orphan drug development".

Other Speakers included:

Dr. Stephen Groft, NIH Director, Office of Rare Diseases Research;

Jean R. Slutsky, Director of HHS Center for Outcomes and Evidence, Agency for Healthcare Research and Quality;

Dr. Barry H. Thompson, Medical Director, American College of Medical Genetics.

Diane Dorman, NORD Vice President, Public Policy.

To view the webcast please visit: http://nfc.uschamber.com/orphan_drugs/

CURETHEPROCESS Regulatory Advisory Committee Formed

The CURETHEPROCESS campaign has created the Regulatory Advisory Committee to help provide expert guidance in the development of regulatory change at the FDA specifically for the review and approval of treatments for rare diseases. The team consists of:

Peter Saltonstall, President and CEO, National Organization for Rare Disorders;
Marlene E. Haffner, MD, MPH, CEO of Haffner Associates;
Frank J. Sasinowski, M.S., M.P.H., J.D., Attorney, Hyman, Phelps & McNamara, P.C.;
Patti A. Engel, President & CEO Engage Health, Inc.,

By adding an experienced group of professionals with knowledge of all aspects of the regulatory process, the Foundation intends to optimize its efforts with regulators and work closely with leading patient organizations. The Regulatory Committee will be focused on advising the Foundation on the nuances of regulatory process and administrative change.

18th Annual Alpha One National Education Conference

On June 5th, Kakkis EveryLife Foundation Patient Advocacy Liaison, Steve Smith joined over 450 attendees at the Annual National Education Conference, to share the CURETHEPROCESS Campaign goals with partner patient organizations. For more information about Steve's work and the conference go to <http://www.alpha1.org/education/nateduconf.php>.

If you have an event or conference where participants would benefit by hearing about the CURETHEPROCESS Campaign please contact Julia Jenkins at jjenkins@kakkis.org.

Rare Disease Art Contest

The Kakkis Foundation is sponsoring the Rare Disease Art Contest, to find original work by artists with rare diseases. If you are an artist affected by a rare disease and would like more information about our upcoming contest, please sign up at www.curetheprocess.org/contact_us, or send an email to info@curetheprocess.org.

In other Rare Disease News:

From the NIH:

The NIH has launched a \$24 million initiative called The Therapeutics for Rare and Neglected Diseases Program (TRND, pronounced "trend") that aims to develop new treatments for rare and neglected diseases. TRND will focus on diseases that private companies have not been working on and stimulate collaborations between academic scientists to advance the process of preclinical research and product development. Relationships with patient advocacy organizations, disease-specific foundations and pharmaceutical companies will also be emphasized to efficiently move promising drugs to the clinical trial stage of development. The TRND Program will be overseen by the NIH Office of Rare Diseases and administered by the National Human Genome Research Institute in collaboration with the NIH Chemical Genomics Center. Additional information is available at: <http://www.genome.gov/27531962>

Rare Disease Congressional Caucus Announced:

NORD Board of Directors Vice Chairman and CURETHEPROCESS Advisory Committee Member Frank Sasinowski announced the formation of a Rare Disease Congressional Caucus to seek creative solutions to problems faced by patients and to serve as a forum for discussion of issues related to access, research, and increased innovation. The Caucus will also seek to increase funding for two government agencies; the National Institutes of Health Office of Rare Diseases Research and the FDA Office of Orphan Products Development. In addition, it will address regulatory issues facing the pharmaceutical, biotechnology, and medical device industries.

What's Next?

Keep checking back as we move our campaign forward and begin to develop draft guidance documents for the use of surrogate endpoints and new clinical trial design for ultra rare diseases. We also hope to bring you news on our discussions with NORD, legislators, key opinion leaders and our CURETHEPROCESS Partners.

Please let us know your thoughts by visiting our website www.curetheprocess.org and emailing your feedback to info@curetheprocess.org.

WWW.CURETHEPROCESS.ORG