



## FASTER TRACK

FEBRUARY 2010

The Kakkis EveryLife Foundation is a proud partner of



<http://globalgenesproject.org/>



February 28, 2010

<http://rarediseaseday.us/>

### Emil's Blog:

#### Rare Disease Day February 28, 2010: Time to remember who we really are

World Rare Disease Day is on the 28<sup>th</sup> of February, and I wonder about people who think this doesn't matter to them, and whether they understand anything about rare diseases and their own genetics. It has been estimated that everyone has about 4-6 defective genes, but few know this or understand its implications. So while some may think that rare diseases are things that happen to other people, the truth is that it has already happened to all of us. . . .

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

### The EveryLife Art Contest - Winners Announced!

We are pleased to announce the winners of the Inaugural EveryLife Art Contest.

#### Grand Prize Winners

Children 5-11: Lauren Lachance & Noah P. Thomas  
Teens 12-17: Kassidee Williamsen & Olivia Book  
Adults 18+: Judith Shirreff, Franky Dolan & Lisa Iannello

#### Special Artistic Merit

Children 5-11: Emma Charney-Sullivan, Ellen Marie Madsen, Tia Piziali & Sarah Elizabeth Brandstater Shaw  
Teens 12-17: Grant J. Bowen, Nathalie Dirnfeld, Hannah Juliette Madsen, Gerald (J.T.) McNeil, III & Lucas Montgomery,  
Adults 18+: Jackie Davenport, Holly Lee, Beth Meador, Bithiah Sanchez, Macksi Warner & Tami Weinert

To see their amazing artwork, go to: <http://www.kakkis.org/Contest/>

Our Board reviewed more than 125 works of art. Winners will receive Visa gift cards and Flip Video™ cameras.

### The Brownback/Brown Amendment H.R. 2997 §740 Review Teams Formed

We applaud the Food and Drug Administration (FDA) for timely implementation of the Brownback/Brown Amendment for Rare & Neglected Diseases, which was passed in last Fall's 2010 FDA Appropriations Bill. This Amendment requires formation of review teams that will submit a report to Congress on the current issues for drug development and approval for rare and neglected diseases. Dr. Tim Cote, Director, Office of Orphan Products Development will lead the rare disease team with FDA staff coming from a variety of locations within the Agency. The team will begin work in March 2010. Many thanks to the FDA for pushing this effort ahead. We appreciate their desire to dig in and look at ways to make things better for patients with rare diseases.

### Thank You to Our Partners in the CURETHEPROCESS Campaign

We are pleased to announce that the Society of Inherited Metabolic Disorders (SIMD) has endorsed the Campaign. Launched in February of 2009, our Campaign to CURETHEPROCESS now has 88 partner organizations. Our Partners endorse our three goals:

- 1) Establish a new Office of Drug Evaluation for Genetic and Biochemical Diseases, consolidating expertise to review treatments to ensure they are safe and effective.
- 2) Devise new clinical study design and analysis paradigms for rare diseases that properly account for clinical heterogeneity and disease complexity to properly capture treatment effects.
- 3) Create a new standard for the surrogate and biomarker endpoints used for rare disorders, to allow treatments for these diseases to have full access to the accelerated approval pathway.

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 complete list of partners go to: <http://curetheprocess.org/partners>

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### **Inaugural Legends of Golf Tournament – Reaching Out to Industry and Investors**

As thousands of investors, bankers, and biotech company executives poured into San Francisco for the Annual JP Morgan Healthcare Conference, the Kakkis EveryLife Foundation hosted its inaugural Legends of Golf Tournament to bring attention to the need to accelerate biotech innovation for rare diseases and spur investment in the industry. The Golf Tournament was co-hosted by the Children's Rare Disease Network, the National MPS Society and the Ryan Foundation. Our sponsors included the Biotechnology Industry Organization, BioMarin, FoldRx, Michael Lee, RedMile Group and Adams Golf. More than 70 golfers enjoyed 18 holes at the Presidio Golf Course, wine tasting, dinner and some celebrity entertainment including Rodney Dangerfield and Tiger Woods impersonators. The double-takes when "Tiger" showed up on the course to visit foursomes were priceless. Ryan Thorpe demonstrated his fastest club in the world and wowed the crowd with 400 yard drives. One of the lucky raffle winners went home with a "six-pack" of Opus 1 wine worth more than \$1,000. Some participants called it the best tournament they have ever attended. To see the pictures go to [www.kakkis.org/golf](http://www.kakkis.org/golf).

### **Dr. Kakkis Featured on the "PERSPECTIVES" Podcast Series**

The regular podcast series, "Perspectives," presented by the Children's Rare Disease Network, featured an interview with Kakkis EveryLife foundation President, Emil Kakkis. Dr. Kakkis discusses how he plans to improve drug development for rare disease. [Click here](#) to listen to Dr. Kakkis' interview.

### **Battling Rare Diseases in a Blockbuster World**

The California Healthcare Institute and PricewaterhouseCoopers released the 2010 California Biomedical Industry Report, which highlights Dr. Kakkis and some of our Partners in their efforts to improve treatment for rare disease. [Click here](#) to read the special section.

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

#### **FDA Announces New Position in CDER "Associate Director for Rare Disease"**

The Associate Director for Rare Diseases will serve as CDER's (Center for Drug Evaluation and Research) focal point to the rare disease drug development community and assist stakeholders and developers of drug and biologic products in navigating the complex regulatory requirements for bringing safe & effective treatment to patients in need. Dr. Anne Pariser has been selected as the new Acting Associate Director; and the agency says it will move quickly to fill the position. We congratulate NORD in its leadership in working with the FDA to create this new position. This is a positive first step toward creating an improved and more specialized FDA review for rare diseases.

#### **PBS TV Special - Rare Disease Diagnosis Through Technology**

The PBS program, "To The Contrary," hosted by award-winning journalist Bonnie Erbe, has produced a three-part documentary series of field pieces and discussion segments on genetic technologies and their impact on diagnosing rare disease. Don't miss Part 3, which airs March 26-28, please check local listings for the exact date and time.

The series describes the evolution of DNA sequencing and will show how DNA may be an effective tool in understanding diseases. This series also showcases the potential for DNA diagnostics in advancing patient care while building awareness and understanding of this innovative technology and its impact on the rare disease community. After the series airs, free copies of the show will be made available on DVD by request through the Children's Rare Disease Network at [www.crdnetwork.org](http://www.crdnetwork.org). This program was made possible by a generous grant from the Life Technologies Foundation, in partnership with Children's Rare Disease Network. [Click here](#) to view an online video of the documentary.

#### **Social Security Announces 38 Additional Compassionate Allowance Conditions**

Michael J. Astrue, Commissioner of Social Security, announced that the agency is adding 38 more conditions to its list of Compassionate Allowances. This is the first expansion since the original list of 50 conditions - 25 rare diseases and 25 cancers - was announced in October 2008. The new conditions range from adult brain disorders to rare diseases that primarily affect children. The expansion means tens of thousands of Americans with devastating disabilities will now get approved for benefits in a matter of days rather than months and years.

Compassionate Allowances are a way of quickly identifying diseases and other medical conditions that clearly qualify for Social Security and Supplemental Security Income disability benefits. It allows the agency to electronically target and make speedy decisions for the most obviously disabled individuals. In developing the expanded list of conditions, Social Security held public hearings and worked closely with the National Institutes of Health, the Alzheimer's Association, the National Organization for Rare Disorders, and other groups. For more information about the agency's Compassionate Allowances initiative, go to [www.socialsecurity.gov/compassionateallowances](http://www.socialsecurity.gov/compassionateallowances).

#### **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](mailto:info@curetheprocess.org) or call us directly at (415) 884-0223 to see how you can help.