



FASTER TRACK

August 2010

In this issue:

- **Announcing the Launch of RareArtist.org an Online Gallery for Artists Affected by Rare Diseases**
- **A Great Win for Rare Diseases in U.S. Senate Appropriation Bill**
- **Dr. Kakkis Testifies at Congressional Hearing on Rare & Neglected Diseases**
- **FDA Public Hearing Regarding the Review and Regulation of Treatment for Rare Diseases**
- **Emil's Blog: Turning Science into Medicine – a Political Perspective**
- **Kakkis EveryLife Foundation Registers as PDUFA Reauthorization Stakeholder**
- **Launch of Congress Web™ Action Center**
- **Rare and Neglected Disease Congressional Caucus Forms**
- **\$1 Billion Increase for NIH, includes \$50 Million for CAN & \$50 Million for TRND**
- **Children's' Rare Disease Network – Provides Slate Card for Pepsi Refresh Projects**

Announcing the Launch of RareArtist.org an Online Gallery for Artists Affected by Rare Diseases

Art is the expressive medium that we all use when capturing the trials and triumphs of our human existence. For rare disease patients, life's meaning takes on the greater significance that comes with struggling day to day with a devastating disease and unanswerable questions.

Why me?

Why this disease?

What does my future hold?

Art as an expression of these challenges in life and in living each day, can be a particularly powerful medium to guide all of us, in our struggles and successes. Artists affected by rare disease communicate their pain, frustration, optimism and joy, and through their work we can see more about ourselves.

We decided to highlight the important value of art from those living with the personal strain of a rare disease that may otherwise get lost in the noise and flash of a twittered society. We set up an art contest to bring forward that art and honor its value to all of us. The outpouring of great artwork and personal stories made it all the more clear that we had to bring this experience to a broader audience.

Our decision was to create RareArtist.org, an online gallery dedicated to artists affected by a rare disease.

RareArtist.org creates a permanent, free, virtual space where “rare artists” can express themselves and

provide an outlet for more people to view their work. The Gallery is intended to showcase the artwork and the Artist, in order to bring awareness to the rare disease community. The first collection is comprised of artwork submitted to our EveryLife Art Contest, and we encourage the rare disease community to continue to contribute their work to the site to grow the collection. At rareartist.org you can:

- View exceptional works of art by artists of all ages
- Upload your own art and share your story
- Send FREE e-cards of your favorite art

The Foundation has a [Free Widget](#) to put on your website to promote the Gallery. Please contact info@kakkis.org if your organization would like to receive a widget and partner with us to showcase artists affected by rare diseases.

[CURETHEPROCESS™ UPDATES](#)

[A Great Win for Rare Diseases in U.S. Senate Appropriation Bill](#)

The FY 2011 Agriculture, FDA, and Rural Development Appropriations Bill ([S. 3606 Section 741](#)) has been introduced and includes language supporting two of our [CURETHEPROCESS](#) Campaign's goals. Specifically, the Bill supports the creation of new guidances that could improve the scientifically sound use of surrogate endpoints and new clinical study designs and analysis. The language builds on the Brownback/Brown Amendment language that was included in the FY 2010 Appropriations Bill (Section 740). The Bill includes specific funding for the Office of the Associate Director for Rare Diseases in the Center for Drug Evaluation and Research (CDER). Funding for this office is increased by \$1,000,000 to hire additional staff with specific expertise in facilitating the development and review of products to treat rare diseases. The Bill also includes the first increase for the Orphan Product Development Grant program since FY 2005. The program is increased by \$2,000,000 for a total grant level of \$16,035,000. We are especially grateful to Senator Sam Brownback (R-KS) for his leadership on this issue and to Senator Herb Kohl (D-WI) for his support. [Click here](#) to read the press release.

[Dr. Kakkis Testifies at Congressional Hearing on Rare & Neglected Diseases](#)

On June 23, 2010 Chairman Herb Kohl (D-WI) and Ranking Member Sam Brownback (R-KS) of the Agriculture, FDA, Rural Development and Related Agencies Appropriation Subcommittee convened a Congressional Hearing on the **“FDA's Review Process for Rare and Neglected Diseases.”** Dr. Kakkis presented the [CURETHEPROCESS](#) Campaign goals. [Click here](#) to watch the hearing or download copies of the testimony from all the speakers.

On July 21, 2010, the Senate H.E.L.P. (Health, Education, Labor, and Pensions) Committee held a Congressional Hearing on **“Treating Rare and Neglected Pediatric Diseases: Promoting the Development of New Treatments and Cures.”** Chairman Tom Harkin (D-IA), Ranking Member Mike Enzi (R-WY) and Senator Sherrod Brown (D-OH) chaired the hearing. Alex Silver, a parent of a child with Epidermolysis Bullosa (EB) - a rare skin disease, Diane Dorman from NORD, John Crowley, CEO of Amicus Therapeutics and parent of two children with Pompe, a rare neuromuscular disease, testified

along with American Academy of Pediatrics, Doctors without Borders, FDA and NIH representatives. [Click here](#) to watch the hearing or download copies of the testimony from all the speakers.

FDA Public Hearing Regarding the Review and Regulation of Treatment for Rare Diseases

On June 30, 2010 the FDA's Rare Disease Committee created by the Brownback/Brown Amendment for Rare and Neglected Diseases in the FY 2010 FDA Appropriations Bill (HR 2997 Section 740) wrapped up its first public meeting. Many voices from the rare disease community, including patients, parents, academia and industry, presented their suggestions on how to improve the drug review process. Major themes of flexibility, sensitivity, collaboration on the FDA's use and interpretation of the laws and regulations emerged. Our **CURETHEPROCESS** campaign goals, including the need for guidances on surrogate biomarker endpoints and the use of small clinical trial designs as well as the specialized drug review office for rare diseases, were repeated in many of the presentations and supported by both patient organizations, academics and industry alike. The Committee must submit a report to Congress in March of 2011 on the recommended changes for rare disease drug development & review and then provide guidances to address these problems by September 2011. The next public meeting will be held in January. [Click here](#) to watch Dr. Kakkis' presentation.

Emil's Blog: Turning Science into Medicine – a Political Perspective

Guest Writer: Julia Jenkins, Director of Public & Government Relations

Our Government has an immense capability to help advance treatments and cures for rare diseases through incentives for industry, research grants, and improving regulatory conditions; however, our politicians often lack public support to challenge the status quo and make good changes to the system. [Read more](#)

Kakkis EveryLife Foundation Registers as PDUFA Reauthorization Stakeholder

The Kakkis EveryLife Foundation will be participating as a stakeholder in the Prescription Drug User Fee Act (PDUFA) reauthorization process. PDUFA permits FDA to collect fees from companies that produce certain human drug and biological products. Orphan designated products are exempt from these fees. PDUFA stakeholders include patient and consumer advocacy groups, health care professionals, and scientific and academic experts. The statutory authority for PDUFA IV expires in September 2012. At that time, new legislation will be required for FDA to continue collecting user fees for the prescription drug program. The Federal Food, Drug, and Cosmetic Act requires that FDA consult with a range of stakeholders in developing recommendations for the next PDUFA program. The act also requires that FDA hold frequent discussions with patient and consumer advocacy groups during FDA's negotiations with industry. The agency expects to finalize its proposed recommendations for PDUFA V to Congress in September 2011. These recommendations will also be posted for public review and comment. A final set of FDA recommendations on PDUFA V is due to Congress in January 2012 with a deadline for reauthorization in September of 2012.

TAKE ACTION

Launch of Congress Web™ Action Center

You can now write your Congress Member via our **CURETHEPROCESS** Website. It only takes two minutes.

- [Congress Web](#) will identify your Congress Member and Senators by zip code
- You can personalize your letter or just use the template provided
- Send you letter via the web or download and print your letters to fax or mail

Congress needs to hear from you. You can also help spread the word and automatically link the letter to your Facebook page. On the Congress Web page there are [Free Widgets](#) for our **CURETHEPROCESS** campaign partner organizations to post on their websites. Just cut and paste the code and you can let your members send letters directly from your website. **Ask your Congress Member to support:**

Appropriation for Rare Disease Drug Review

Our request for an additional \$10 million to fund a new division does not appear possible in Budget Year 2011, however we feel it is important for Congress to fully understand the issues, and the broad benefits of our proposed changes both for rare diseases and the FDA as a whole. We urge you to continue to contact your members of Congress on the need for new funding for rare disease drug review. [Click here](#) to write your letter today!

OTHER RARE DISEASE NEWS

Rare and Neglected Disease Congressional Caucus Forms

The bipartisan and bicameral Caucus was formed in May and announced at the NORD Gala. The Caucus Co-Chairs are Congress Members Joe Crowley (D-NY) and Fred Upton (R-MI), and Senators Sherrod Brown (D-OH) and John Barrasso (R-WY). The co-chairs will focus on:

- Bringing Congressional attention to the 6,800 known rare diseases that currently have no approved therapies
- Ensuring sufficient funding for research and orphan product development
- Exploring ways to incentivize companies to create new drugs, biologics and humanitarian use devices
- Providing an opportunity for Members of Congress, families and advocacy groups to exchange ideas and policy concerns

Congress Members Crowley, Upton, Brown and Barrasso are looking to you to encourage your congressional representatives to become a member of this important Caucus. [Click Here](#) to find your Congress Members. [Click here](#) to read the House “Dear Colleague” letter.

\$1 Billion Increase for NIH, includes \$50 Million for CAN & \$50 Million for TRND

The NIH has long funded basic science; however there is now a shift in focus to also help turn that basic science into life saving treatments. Last year’s Economic Stimulus package created a new NIH grant program, [Therapeutics for Rare & Neglected Diseases](#) (TRND). Funded with \$24 million dollars in its first year, TRND has funded five projects that will help bring these treatments further along in the development process and make them more appealing for companies to develop the treatments.

Read more about TRND and its pilot projects in a recent Wall Street Journal article: [NIH Takes On New Role in Fight Against Rare Diseases](#)

Following TRND came the [Cures Acceleration Network](#) (CAN), which was authored by Senator Arlen Specter (D-PA) and passed in the Health Insurance Reform package earlier this year. CAN creates an additional grant program within the NIH that is focused on the translation of science into medicine. The Administration requested a \$1 billion increase for the NIH in FY 2011 and both the House and Senate Appropriation bills fully fund the \$1 billion dollar increase. We have been told this includes \$50 million for TRND's second year and \$50 million for CAN's first year.

Children's' Rare Disease Network – Provides Slate Card for Pepsi Refresh Projects

Pepsi is accepting 1,000 ideas every month for its Pepsi Refresh grant competition. The top ideas, voted on by the public each month, will receive grants. [Children's Rare Disease Network](#) has compiled a list of all the rare disease organizations that are competing for a Pepsi Refresh grant on their Facebook page. Please [click here](#) and use your vote to support organizations that help children with rare diseases. You can add your project to the list by contacting catherinec@rareproject.org.

FASTER TRACK to CURETHEPROCESS

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

