FOR IMMEDIATE RELEASE

A Great Win for Rare Diseases in U.S. Senate Appropriation Bill

New FDA funding and requirements for guidances will help give rare diseases access to the accelerated approval process

July 15, 2010 (Novato, California) — Just 16 months after the Kakkis EveryLife Foundation kicked off the CURETHEPROCESS Campaign, the Foundation, in association with the National Organization of Rare Diseases (NORD) and numerous other patient and physician organizations, have increased the support and commitment to improving rare disease regulatory policies.

A US Senate Appropriation bill has been submitted including language supporting two of the Campaign's goals. Specifically, the Bill supports the creation of new guidances which could improve the scientifically sound use of surrogate endpoints and new clinical study designs and analysis. The Senate Bill also includes an appropriation for the Food and Drug Administration to hire new staff to fulfill these requirements.

"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," said Emil Kakkis, M.D. Ph.D., President of the Kakkis EveryLife Foundation. "We are very pleased to see so much progress made, in such a relatively short time."

The Senate Appropriations Committee will now review the FY 2011 Agriculture, FDA, and Rural Development Appropriations Bill. The bill 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. The Bill also 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 manager's package that should be adopted at Committee includes language, cleared by the FDA that builds on the Brownback/Brown Amendment language that was included in the FY 2010 Appropriations Bill (Section 740). The language requires the FDA Commissioner to *"…develop updated guidance documents and review standards for the development of safe and effective products to treat rare diseases and neglected tropical diseases…"*

Specifically, the Bill spells out requirements to:

- Maximize the use of accelerated approval where feasible and appropriate, including guidances on the use of surrogate endpoints that are reasonably likely to predict clinical benefit of drugs and biological products under the regulations under Subpart H
- Work with drug company sponsors to facilitate expanded access to investigational therapies
- Develop guidance on clinical development programs for rare diseases
- Increase coordination among individual drug, biological product, and device review divisions across FDA centers to support the development of safe and effective medical products for rare and neglected diseases

The FDA is required to implement these as a part of the FY 2010 Brownback/Brown Amendment reforms and report back to the Appropriation Committee on implementation of these items.

"The Senate Bill is a good step forward in improving the regulatory process for rare diseases. By creating a more predictable pathway for orphan treatments, we will shorten development timelines and reduce the financial risk associated with the development of rare disease therapeutics. The result will be a surge in development activity for even the most rare

disorders, giving more patients with rare biochemical and genetic disorders earlier access to effective treatments." said Dr. Kakkis.

The Foundation initiated the **CURETHEPROCESS** Campaign to give even the rarest diseases access to the accelerated approval process and put treatments on the fast track. There are more than 7,000 rare disorders that together affect over 25 million Americans and their families; however less than 5% have treatments as few drug companies conduct research on rare diseases since it is currently difficult to recoup the costs of developing treatments for such small populations. More than 130 patient and physician organizations have endorsed the Campaign goals to:

1. Establish a new Office of Drug Evaluation for Genetic and Biochemical Diseases at the FDA, consolidating and expanding expertise to ensure safe, effective and timely patient access to needed treatment.
2. Create a new standard to qualify biomarker or surrogate measures of the effect for treatments of rare disorders, and allow these treatments full access to the accelerated approval pathway for life threatening diseases.
3. Devise new clinical study designs for rare diseases that account for disease complexity and patient variability to properly capture treatment effects on all aspects of the disease.

To learn more about the Kakkis EveryLife Foundation, please go to www.Kakkis.org.

Contact: Julia Jenkins, Director of Public & Government Relations
415.254.5758 jjenkins@kakkis.org

###