



Liddy Shriver Sarcoma Initiative

Helping those dealing with sarcoma

December 3, 2009

Emil Kakkis M.D. Ph.D.  
President  
Kakkis EveryLife Foundation  
77 Digital Dr, Suite 210  
Novato, CA 94949  
Dear Dr. Kakkis,

We would like to take this opportunity to express our support for your *CureTheProcess Campaign* for rare disease treatment development. We definitely agree with you that we could be doing more with the science we already have.

We acknowledge that the *CureTheProcess Campaign* strives to inspire science-driven public policy to increase the predictability of the regulatory process for rare disease treatments. Further, we support your goal to give even the most rare diseases access to the accelerated approval process and fulfill more completely the original intentions of the Orphan Drug Act.

We endorse your efforts to:

- Establish a new Office of Drug Evaluation for Genetic and Biochemical Diseases, consolidating expertise to ensure safe, effective and timely patient access to needed treatment.
- 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.
- Devise new clinical study design paradigms for rare diseases that properly account for clinical heterogeneity and disease complexity to properly capture treatment effects.

To show our support, we will encourage the visitors to our web site to advocate for the goals of the campaign. We will publish portions of the "*Goals of the CureTheProcess Campaign*" in the upcoming issue of our electronic publication, the Electronic Sarcoma Update Newsletter, and include a link to the *CureTheProcess Campaign* web site and logo.

Sincerely yours,

Bruce D. Shriver, PhD  
Beverly R. Shriver, RN  
Co-Founders  
Liddy Shriver Sarcoma Initiative