



June 1, 2010

Emil Kakkis, MD, PhD  
President  
Kakkis EveryLife Foundation  
77 Digital Drive, Suite 210  
Novato, CA 94949

Dear Dr. Kakkis:

Sarcoidosis is a potentially fatal inflammatory disease that can appear in almost any organ in the body. Although little known, it is one of the most common causes of fibrotic lung disease in the U.S. and often results in chronic debilitating or life threatening heart, neurological and internal organ disease. To date, the cause remains unknown and patients remain without safe, effective treatments.

On behalf of the Foundation for Sarcoidosis Research, I would like to take this opportunity to express our support for your campaign to **CURETHEPROCESS** for rare disease treatment development.

Sarcoidosis can affect people of any age, race and gender it. However, it most often strikes adults in the prime of their life (ages 20 to 40). According to a recent FSR Survey, roughly 1/3 of patients are unable to work due to their illness. **Accelerating the approval process for rare disease treatments is a critical step to improving the lives of those living with sarcoidosis.**

We applaud 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.

Please continue to keep us informed about the ways that we can encourage our constituents to advocate for the goals of the campaign. For additional information about sarcoidosis or the education, awareness and research activities of FSR, please visit [www.stopsarcoidosis.org](http://www.stopsarcoidosis.org).

Regards,

Debbie Durrer  
Executive Director