

# Alpha-1 Association



Support, Education and Advocacy since 1991

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Dear Dr. Kakkis,

I would like to take this opportunity to express our support for your campaign to CURETHEPROCESS for rare disease treatment development. We agree that with your statement 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 are committed to encouraging our members to advocate for the goals of the campaign. Furthermore, if approved by the Board, will share information with them via website links & email and support participation in other campaign initiatives and outreach programs.

Sincerely yours,

Marlene S. Erven  
Executive Director