National MPS Society
Support for Families. Research for a Cure.

September 27, 2010

Fax: 202-690-6166

The Honorable Kathleen Sebelius
U.S. Department of Health & Human Services
200 Independence Avenue, S.W.
Washington, DC 20201

Dear Secretary Sebelius:

I am writing on behalf of children and young adults affected with the terminal illness MPS (Mucopolysaccharidoses). The National MPS Society is writing to gain your support for bold change at the FDA this coming year. As you work to develop the President's budget for Fiscal Year 2012, we respectfully urge you to make the Food and Drug Administration (FDA) a major priority. We, along with 156 other patient organizations and physician societies, are partners in the CURETHEPROCESS Campaign spearheaded by the Kakkis EveryLife Foundation. The Campaign advocates for establishing a new specialized Office of Drug Evaluation for Rare Biochemical and Genetic Diseases and new policies and guidance to support rare disease treatment development. *M*ore than 30 million Americans and their families are affected by over 7,000 rare diseases, yet 95% of these diseases are without an approved treatment. A great deal of science exists that could be translated to human patients but will not because of the current challenges in development and regulation.

The government continues to make substantial investment into basic science at the NIH, but relatively few treatments that are developed are subsequently translated to human use. This is happening because the investment in basic science is not matched with a proportional investment in the FDA's review of these treatments. Partly due to underfunding, the FDA has lost some of its best people. This loss of top talent and insufficient resources has left the Agency struggling to maintain its mission to protect and improve human health.

The CURETHEPROCESS campaign is asking for a relatively small but smart increase and reallocation of resources in specialized review groups, in the FDA's Office of New Drugs would dramatically improve the FDA's recruitment, training and retention. The right people with sufficient time and focus to specialize will sharply improve the review of treatments. These changes would be acclaimed and applauded by the millions of Americans suffering with a rare disease and would send a loud signal to the biotech industry and venture capital to invest in the development of new life saving treatments for these rare disorders, creating thousands of high paying US biotech jobs. **We support The CURETHEPROCESS campaign one hundred percent** and we feel the time is **NOW** to do something bold and meaningful at the FDA.

We urge you to meet with Dr. Emil Kakkis, the founder of the Kakkis EveryLife Foundation, to learn how the FDA can help accelerate biotechnology for rare diseases. Contact Dr. Kakkis at ekakkis@kakkis.org or 415-884-0223. Our families with children and young adults affected with this terminal disease need cures now.

Thank you for your leadership on behalf of our families.

Best Regards,

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