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

September 27, 2010

The Honorable Margaret Hamburg, M.D.
Food and Drug Administration
10903 New Hampshire Ave
Silver Spring, MD 20993-0002

Fax: 301-847-3531

Dear Commissioner Hamburg:

As you work to finalize the FDA's budget for Fiscal Year 2012, we respectfully urge you to make an improved regulatory process for rare diseases a major priority. There are over 7,000 rare diseases that affect more 30 million Americans and their families. Yet 95% of these diseases are without an approved treatment. As you know, the agency's funding levels inadequately reflect the expansive nature of the agency's tasks at hand.

The National MPS Society, *along* with 156 other patient organizations and physician societies, are partners in the CURETHEPROCESS Campaign. The Campaign, spearheaded by the Kakkis EveryLife Foundation, is advocating for a new Office of Drug Evaluation for Rare Biochemical and Genetic Diseases and **we support this effort one hundred percent**.

The government continues to make substantial investment into the development of treatments at the NIH, but relatively few treatments are translated to human use because we do not match that investment in the FDA's review of these treatments. For many rare diseases, the unpredictable regulatory process and the difficulties in navigating the typical development pathway means that the conversion of science to medicine of many potentially effective treatments never begins. The new regulatory science initiatives may be helpful, but will take years to provide improvements. We need action now.

With the majority of its budget going to staff and operational costs, at the current rate of growth the agency will be unable to sustain, much less expand, its current scope. FDA needs excellent staff with scientific expertise and specialized focus to review complex rare disease drugs. While many at FDA understand the problem, there has been a reluctance to take action on the issue of review group specialization that will move the FDA forward despite agreement with the goals and numerous discussions at many levels within FDA.

The CURETHEPROCESS campaign is asking for a relatively small but smart increase of resources, along with a bold personnel plan for the FDA that would dramatically improve the FDA's recruitment, training and retention of top personnel by increasing the academic stature and value of working at FDA. By enhancing the focus on reviewers only on their area of expertise in specialized review divisions, and then requiring and rewarding their work in academics, the best quality people can be recruited and retained to supplement those already at FDA. These organizational changes to improve the review focus at FDA would be acclaimed and applauded by the millions of Americans suffering with a rare disease and give you an early measurable success as FDA commissioner. These changes would also provide a loud signal to the biotech industry and venture capital to invest in the development of new life saving treatments for these rare disorders and creating thousands of high paying US biotech jobs. We must do something bold now at FDA.

We urge you to contact Dr. Emil Kakkis, the founder of the Kakkis EveryLife Foundation to discuss these changes that could have a profound benefit on FDA's effectiveness. Please contact him at ekakkis@kakkis.org or 415-884-0223 to learn how the FDA can help accelerate biotechnology for rare diseases. Our families' terminally ill children need cures now!

Thank you for your leadership on this important issue.

Respectfully Yours,

Ernest Dummann, President
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