

## Kakkis EveryLife Foundation

Julia Jenkins, director of Public and Government Relations with the Kakkis EveryLife Foundation, hosted a brunch in Washington, DC, in September 2009 to help build a platform with this group to form a lasting coalition on long-term policy goals for the rare disease community. The goal is to share information and to support fellow patient organizations in their legislative efforts. While each organization may have many different projects, this forum is specifically for legislative, policy and regulatory initiatives meant to open dialogue, share information, work toward common goals and build a lasting coalition for the rare disease community. Representatives from the CF Foundation, Genetic Alliance, National PKU Foundation, NSGC, NORD, Hemophilia Federation and National MPS Society attended the meeting.

Information about legislation followed by the groups was shared, and there were common bills, such as H.R. 2866, Improving Access to Clinical Trials Act, H.R. 1441, The Ryan Dant Healthcare Opportunity Act of 2009, and Social Security Compassionate Allowances. All groups are taking a wait-and-see approach to healthcare reform.

Information to be used for consistency:

- There are 7,000 rare diseases
- 10 percent of the population has a rare disease
- 30,000,000 people in the United States have a rare disease

Major goals:

- Rare Disease Congressional Hearing for 2010
- Rare Disease Congressional Caucus
- To support legislation that increases access to treatment for patients with rare diseases
- To prevent legislation that hinders access to treatments
- To support funding increases for the U.S. Food and Drug Administration and National Institutes of Health (NIH)

The attendees all agreed on the importance of speaking with one voice for issues pertinent to each of us. Plans were tentatively made for face-to-face meetings in DC every two months, with the host organization rotating.

The National MPS Society was invited to a meeting with NIH Director Francis S. Collins, MD, PhD, where he discussed his plans for the future of the NIH, and took questions and comments from leaders of scientific organizations and nonprofit voluntary and advocacy organizations. The event was held on Sept. 9, 2009, on the NIH campus in Bethesda, MD. The Constituents' Meeting was the first of its kind, and held just three weeks into Dr. Collins' tenure as director of the NIH. He emphasized points in the NIH mission statement, science in pursuit of fundamentals of knowledge and the application of that knowledge. To that effect he will focus on investigator-initiated research, pushing for innovation while overhauling the peer review process and also supporting big science. He encouraged organizations to follow funded research through the Research Portfolio Online Reporting Tool, <http://report.nih.gov/>. Dr. Collins presented five opportunities or areas of focus for the NIH:

1. Applying unprecedented opportunities in genomics and other high-input technologies to understand fundamental biology and uncover causes of diseases.
2. Translate basic science discoveries (e.g., imaging, nanotech) into new and better treatment (e.g., gene or stem cell therapy). Utilize public/private partnerships, gave example of TREND program.
3. Put science to work for the benefit of healthcare reform (e.g., areas of comparative effectiveness research, pharmacogenomics, social and behavioral research, healthcare research economics [such as payment incentive models]).
4. Encouraging a greater focus on global health (e.g., World Health Organization and Gates Foundation).
5. Reinvigorating and empowering the biomedical research community to increase their ability to be competitive globally (e.g., case for value in medical research, encourage innovative research, and support training programs to bring people to research).

### Legislative Committee:

Ernie Dummann, chair  
 Steve Chesser  
 Debbie Dummann  
 Stephen Frye  
 Tom Gniazdowski  
 Steve Holland  
 Terri Klein  
 Austin Noll  
 MaryEllen Pendleton  
 Laurie Turner  
 Barbara Wedehase  
 Kim Whitecotton

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