National MPS Society

Support for Families. Research for a Cure.

Advocating with FDA

Austin Noll

MPS Society Annual Meeting

September 30, 2010

FDA Advocacy Opportunity

FDA held first public hearing on rare diseases in June

Purpose: Gain perspectives on various aspects of the development of medical products for the diagnosis, treatment or management of rare diseases

Meeting open to everyone

I attended and testified on behalf of the MPS Society

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Meeting Participants

- **Patients / family members**
- **National Organization for Rare Disorders (NORD)**
- **Industry**
- **Dr. Kakkis' EveryLife Foundation**
- **Other Patient Societies**

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Our Message to FDA

- **Need group dedicated to rare disease review**
  - Reviewers that understand genetics
- **Need ability to use reasonable biomarkers in studies**
  - In many situations, such as when there is CNS and bone involvement, clinical outcomes are very difficult to measure
- **Need more appropriate study designs**
  - No double-blinded, randomized studies with placebo controls
  - Sample sizes that reflect the rarity of these conditions
  - Inclusion criteria reflecting heterogeneity of population

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Takeaways from Meeting

- **Requests coming from the patient societies and from industry were all very similar**
- **Glad to see FDA formally recognizing that issues exist with the process**
- **Concerned with how long it may take FDA to act**

Must Continue to Push for Reform

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How can you help?

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Policy with Partners – PwP

The Path to the Future

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Ask Me About

PwP

Policy with Partners

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PwP – What is It?

- A program designed to allow society members and others to ***easily advocate for* MPS**
- Contact your elected officials via pre-made faxes, emails
- Potential chance to visit D.C. personally to share your views

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Help us get the word out

PwP Wants You

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Thank You