legislative update

DEAR FRIENDS,

I have exciting news to share with you. Your board of directors will be conducting their February quarterly board meeting in Washington, DC, to incorporate a couple of days of legislative advocacy into the agenda. It is a fantastic opportunity for the Society's board and Legislative Committee to visit with senators and representatives to further our mission. Our goal is to spread awareness about MPS and help them understand how their decisions on healthcare law, appropriations, disability laws, respite, etc. have a direct impact on families and individuals living with MPS. We hope to build our relationships with the federal legislators so that when important issues come up we have already laid the groundwork.

A specific item which we will be advocating for is inclusion of MPS language in the FY2014 Appropriations Bill. Each year, the Senate and House direct government programs, such as the National Institutes of Health (NIH), on priorities for government spending. During the last several years we have seen increases in NIH grant funding which we attribute to the inclusion of MPS language in the appropriations bill each year. Below is a summary from the NIH website on MPS funding:

Estimates of Funding for Various Research, Condition and Disease Categories (RCDC)

Research/Disease Areas (Dollars in millions and rounded)	FY 2008 Actual	FY 2009 Actual (Non-ARRA)	FY 2010 Actual (Non-ARRA)	FY 2011 Actual	FY 2012 Estimated	FY 2013 Estimated
Mucopolysaccharidoses (MPS)	$7	$7	$8	$10	$10	$10

We have not seen an increase in MPS funding during the last couple of years, but more importantly, we have NOT seen a decrease. With the current fiscal cliff concerns, we are at great risk for having a funding decrease. Therefore, it is as important as ever that we continue to advocate for this crucial funding source for MPS research.

During the next few months, please look for our advocacy action alerts as we call upon Society members to contact their representatives whom we will be visiting on your behalf. When a constituent (YOU) makes a point of contacting your representative in advance, they do take notice! Last year, we were thrilled to hear from senators' offices that they had heard from constituents in their state about MPS already. It gave us a beneficial position to further advocate on behalf of the constituents in their states or district. YOU do matter and your voice is heard!

If you are interested in serving on the Legislative Committee or would like to share your advocacy story, please contact me directly—I would love to hear from you.

Stephanie Bozarth
Chair, Committee of Federal Legislation
Stephanie.Bozarth@mpssociety.org
703.256.1980

Policy with Partners: Time to Take Action!

Current Legislative Priorities and Action Items:

• Ask your congressman to join the Rare Disease Caucus

With the Caucus now introduced in the 112th Congress, we need your help to drive membership. The caucus will help to further educate our legislators about the special needs of our MPS community and other rare disease communities with similar issues. This is where we start our search for advocacy champions who can greatly influence legislation important to us. You will be able to determine if your congressman is in the caucus at **www.rarediseaseadvocates.org**.

• Develop a relationship with your representative and let your voice be heard!

We asked our consultants at M+R Strategic Services to conduct an online training session for our members. The goal was to help our members learn how to meet with their members of Congress to advocate for research funding and other important policies related to MPS diseases. There's no better way for us to fight for funding and policies to advance new treatments and ultimately a cure for MPS than meeting with lawmakers and telling our stories.

A previously recorded training session is posted on the Society's website; it takes less than an hour to view. In addition, there are lots of great handouts posted on the website that provide a step-by-step guide to meeting with your lawmakers in your home state. Go to our website, click on "Members Only - Legislative Toolkit." Under "How to Webinar," click on "Guide to in-district Legislative Visits."

Rare Disease Legislative Conference & Lobby Day, Feb. 26–27, 2013

The conference and Lobby Day are free and open to the public. Travel scholarships in the amount of $500 are available through an application process. To register and apply for scholarships go to **www.rareadvocates.org**.

Legislative Conference—Tuesday, Feb. 26
National Press Club, ballroom
529 14th Street Northwest, Washington, DC 20045
(Near the White House & Metro Center)
Registration and continental breakfast from 8:30–9:15 a.m.

Lobby Day—Wednesday, Feb. 27

Once you register, we will contact your members of Congress on your behalf to request meetings for the Lobby Day on Wednesday, Feb. 27. You will have between three and five meetings with members of Congress or their staff.

continued >>

Legislative Committee:

Stephanie Bozarth, *chair*
Amy Barkley
Jeff Bardsley
Austin Bozarth
Dawn Checrallah
Ernie Dummann
Steve Holland
Terri Klein
MaryEllen Pendleton
Kelly Rose
Laurie Turner
Kim Whitecotton
Roy Zeighami
Barbara Wedehase

>> Palomar Hotel, by Kimpton, located in Dupont Circle, is offering a discounted room rate of $183/night for Monday, Tuesday and Wednesday. There are a limited number of rooms available at the discounted rate.

The Rare Disease Legislative Conference and Lobby Day have five main goals:

- educate patient advocates about the legislative and appropriations process
- inform Congress about rare diseases and the many different needs of the patient community
- build lasting relationships with members of Congress and their staff
- ensure that the needs of rare disease patients are considered in future legislation and policy
- empower patient advocates to take an active role in the democratic process

Patient-Centered Outcomes Research Institute

One of the most promising new initiatives to come about from health reform was the consensus that public health is improved by knowing definitively what kind of care works and doesn't work in a given situation. The Patient-Centered Outcomes Research Institute (PCORI) will begin to systematically tackle questions related to standards of clinical care and will have an important impact on healthcare delivery in the United States, including rare diseases.

PCORI is an independent organization created to help patients, clinicians, purchasers and policy makers make better informed health decisions. PCORI will commission research that is responsive to the values and interests of patients and will provide patients and their caregivers with reliable, evidence-based information for the healthcare choices they face.

PCORI is committed to transparency and a rigorous stakeholder-driven process that emphasizes patient engagement. PCORI will use a series of forums and formal public comment periods to increase awareness of its work and obtain public input and feedback prior to adoption of priorities, agendas, methodological standards, peer review processes or dissemination strategies.

Rare disease advocacy groups worked hard during health reform to ensure that any rare disease research that is conducted by PCORI will be a special advisory committee on rare diseases. The rare disease advisory committees will aide in the design of the research study and must include scientific and health services experts, clinicians, patients and others with expertise in rare diseases.

Advocacy Personal Stories

A Parents Perspective on Advocacy

by *Roy Zeighami*, father of Reed (MPS III)

The National MPS Society is dedicated to advancing the values of patients with MPS and related diseases through legislative advocacy. Interaction with our government ensures that:

- patients get the support they need to care for their loves ones;
- MPS gets its fair share of government research dollars;
- patients have access to treatments; and
- regulators make appropriate risk vs. reward trade-offs in the approval of treatments for severe unmet needs.

As the parent of a 5-year-old child with MPS III, it sometimes feels like research moves at a snail's pace. Along with our partner organizations, the Society has been involved in some key successes in speeding up access to new treatments.

You may be aware of the fact that the Society was a key enabler of the Faster Access to Specialized Treatments (FAST) act which was ultimately absorbed into a bill called the FDA Safety and Innovation Act, passed in 2012. FAST encourages the FDA to use surrogate endpoints to approve new drugs for rare diseases. Surrogate endpoints are quantitative biological measures of drug efficacy (also known as biomarkers). Examples of biomarkers are GAG levels in urine or cerebral spinal fluid, brain volume, etc., depending on the disease and its pathology. Proving efficacy of a drug against a biomarker can be done quickly, objectively and in a very cost-effective way. Once a drug is granted accelerated approval based on a surrogate endpoint, it can be placed on the market, giving immediate access to all patients. Patients can then be monitored to ensure they receive clinical benefit.

In contrast, traditional drug development requires regulators to wait until patients actually enrolled in trials show a clinical benefit before approving a drug for the general population. For a slow-moving, progressive disease, proving clinical benefit may take years. This approach burdens drug development cost, narrows the set of patients whom drug companies can consider for trials, and makes the development of treatment for some diseases economically unviable.

Though completely understanding how these new laws will play out will take years, there is reason to be optimistic that FAST will encourage the development of new drugs for MPS and related diseases.

It is time to build on our legislative success and continue to advocate for the needs of our patients. Our work is not done. In particular, there has been an alarming trend of off-shoring of clinical trials for rare disease. This has happened with MPS IV, MPS III A and other diseases like Duchenne Muscular Dystrophy. I personally believe that American patients should be among the first to benefit from basic research done here in the United States. I have the opportunity to speak at the Rare Disease Congressional Caucus briefing in Washington, DC, on Nov. 14. At this meeting, I will share the impact that off-shoring of the Shire MPS III A trial has had on our family. We know we are not alone and that many of you share our frustration. The hope is that by sharing our story, we can remove whatever roadblocks exist to enabling these trials here in United States in the future.

Congratulations to board member Roy Zeighami who received the Rare Disease Legislative Advocates (RDLA) Patient Advocate award Nov. 14, 2012, at the RDLA Rare Voice Awards Gala in Washington, DC. This award recognizes a patient, parent or volunteer who took a leadership role in successfully advocating for rare disease legislation that was included in The Food and Drug Administration Safety and Innovation Act. Earlier that day Roy spoke at the Rare Disease Congressional Caucus Briefing on "The patient impact of regulatory challenges and roadblocks to participation in clinical trials only conducted outside the United States."