

legislative update

Did You Know?

- 2013 Society advocacy goals are to avoid across-the-board budget cuts due to sequestration which will dramatically reduce National Institutes of Health (NIH) funding for MPS research, ensure insertion of MPS-related language into the 2014 Appropriations Bill, passage of a Senate resolution to create National MPS Awareness Day on May 15, 2013, and spread MPS awareness and build relationships with all of our key decision makers.
- **The Society's board of directors and Legislative Committee advocated in the United States Senate and House of Representatives on behalf of all individuals affected by MPS and their families. We had appointments with 22 senators' and 10 House of Representatives' offices over two days.**
- **11 board of directors, six Society members, and four staff members were in Washington, DC, representing perspectives as the individual with MPS, parents, aunt, sister, along with parents and relatives who have lost their loved ones with MPS. This was the largest, most diverse representative effort the Society has ever coordinated for one unified advocacy visit to DC.**
- The Society awarded Sen. Lindsay Graham (R-SC) for his advocacy achievements on behalf of MPS Awareness Day and NIH funding for grants directed at MPS research. The senator has been a loyal advocate to the MPS and related diseases community.
- Sissi Langford, former Legislative Committee member and SC constituent, was the founder of MPS Awareness day in 2006. The goal of this day is to honor those living with MPS, spread awareness, and raise funds and interest for research. Sissi, along with the board and committee, presented the advocacy achievement award to Sen. Graham. Since 2006, MPS Awareness Day has become an international event celebrated around the world.
- **As a result of the Society advocating for MPS language inclusion in the Appropriations Bill each year, NIH funding for MPS-related research grants has increased to \$10 million a year.**
- Each office the Society visited stressed the importance of communication from their constituents. Many stated they would sign on to support awareness day, if their constituents asked them directly.
- Thanks to BioMarin Pharmaceuticals, Inc. and Shire HGT that provided grants to cover costs of the Society's advocacy efforts.

*May 15 is International
MPS Awareness Day*

>> As you can see by the bullet points, we have raised the bar with our advocacy efforts. This year we included the entire board of directors, Legislative Committee, and Society members to hit the hill with a unified advocacy purpose. One thing is clear—senators and representatives want to hear directly from constituents. Therefore, we utilized our strengths of representation across the states and came with a goal to fulfill that challenge. Our next steps moving forward include follow-up with all our senators and representatives to ensure all our advocacy goals are successfully fulfilled in 2013. We will be calling upon Society members to take part as constituents and ask your senators to sign the MPS resolution for awareness day on May 15, 2013, and remind all representatives about the importance of MPS language in the Appropriations Bill. Overall, each representative's office firmly stated that hearing from their constituents will guide their decisions to support our advocacy goals. **YOUR VOICE NEEDS TO BE HEARD!** ❁

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A Parent's Perspective

by *Kim Whitecotton*

When I held my new son in my arms for the very first time, I never imagined how our lives were going to change and the momma bear I would become. After we received our son's MPS II diagnosis when he was 18 months old, I immediately began advocating for his medical needs. When Scotty was 3 I began advocating for his schooling needs. I learned that you must educate yourself before becoming an effective advocate. I had done so much advocating for our son locally and on a state level that I wanted to help on a national level and ran for the Society's board of directors. I was elected and chose to serve on the Legislative Committee. Our committee advocates on significant legislation pertaining to MPS-related medical research, health services, special education and disabilities policies. The first year I was on this committee I made my first advocating trip to Washington, DC, and it was one of the greatest experiences of my life. To meet with our very important legislators on behalf of all our families, to share our story and ask for their help for rare diseases was very rewarding. I was very impressed with the young health legislative aides who listened to all our stories.

Edwardsville Woman Continues Battle Against Disease That Took Her Son

MPS Society member Kris Klenke recently was featured in an article by the *Belleville News-Democrat*, Belleville, IL. The article discusses Kris' involvement with the Society and her recent trip to Washington, DC. To read the article, go to www.bnd.com/2013/03/04/2519349/edwardsville-woman-continues-battle.html.

Fiscal Cliff Debates May Impact Orphan Drug Prices

The National Organization for Rare Disorders (NORD) predicts that the struggle to avoid the next fiscal cliff may impact escalating prices for orphan drugs. Rare disease patients have for years voiced concerns regarding increases for the cost of orphan drugs, however the issue may face even more scrutiny as legislators put spending and budgets under the microscope.

Although legislators were able to reach a solution to deal with tax increases, the budget sequestration was only delayed and the debt ceiling was not changed, leading to further debate during early 2013. To reach an agreement, drug costs may become part of the debate, with orphan drugs a focus because of their high cost.

NORD considered suggesting changes be made to the Orphan Drug Act, a law designed to facilitate the development and commercialization of drugs to treat rare diseases by providing sponsors of therapies with certain benefits, such as reduced taxes. However NORD decided that revising the law could ultimately create more problems than benefits, and is not expected to take sides in the orphan drug pricing debate.

NORD Celebrates 30 Years of Progress that Started with the Orphan Drug Act

Patient advocates played a key role in Congressional approval of the Orphan Drug Act. In the decade before 1983, only 10 new products had been developed by the pharmaceutical industry for rare diseases.

The Orphan Drug Act would provide financial incentives to encourage companies to develop treatments for small patient populations. However, it was stalled in Congress until Abbey Meyers and other representatives of patient organizations formed a coalition to get the legislation approved.

It was not an easy task, and it involved learning to work with Congress, the media and—most important—each other. But the patient advocates did their job well and the legislation was approved by Congress and signed by President Ronald Reagan on Jan. 4, 1983.

Shortly afterward, the patient leaders held a meeting at which they decided to continue their partnership through an organization to represent all Americans affected by rare diseases. That organization was the National Organization for Rare Disorders (NORD). Learn more about NORD's timeline of rare disease advocacy challenges and triumphs at www.rarediseases.org/nord-30th-anniversary.

Policy with Partners: Time to Take Action!

- **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 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. There also are lots of great handouts on the website that provide a step-by-step guide to meeting with your lawmakers in your home state. Go to our website (Members Only—Legislative Toolkit). Under "How to Webinar," click on "Guide to In-District Legislative Visits."

Legislative Committee:

Stephanie Bozarth, *chair*

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

MPS Society Makes Its Presence Known on Capitol Hill

“I absolutely loved the trip. It was a privilege and an honor to speak on behalf of Allison and the many others who can’t speak for themselves.”

— **MaryEllen Pendleton**,
aunt of Allison Kirch (MPS III)

“It was exciting, encouraging and inspiring visiting and speaking with our senator and Congressman’s offices. They really listened to what we had to say.”

— **Kris Klenke**



Sissi Langford presents Sen. Lindsay Graham (R-SC) with the advocacy achievement award



Roy Zeighami and Gordon Wingate



Deb Dumann, MaryEllen Pendleton, Stephanie Bozarth and Jerry Todd



Lisa Todd, Stephanie and Austin Bozarth and MaryEllen Pendleton



MaryEllen Pendleton



Gordon Wingate, Steve Holland, Jeff Bardsley, Angela Guajardo and Wes Hambrick from Sen. John Cornyn’s (R-TX) office.



MPS Society advocates 2013



MPS Society advocates meeting in Sen. Lindsay Graham's (R-SC) office.



Angela Guajardo, Gordon Wingate, Anne and Tom Gniazdowski and David Rardin from Rep. Bill Johnson's (R-OH) office.



Carrie Dunn, Kim Whitecotton, Kelly King, Kris Klenke, MaryEllen Pendleton and Laurie Turner

"I am impressed by the passion and determination of this board. Stephanie Bozarth coordinated more than 20 meetings and made sure every member was prepared for every meeting they attended. Based on the feedback we received, I believe the advocacy efforts of the MPS Society continue to be a valuable and important method of awareness."

— Sissi Langford,
former chair of Committee
on Federal Legislation