legislative update

Thanks to the support and dedication from longtime friend and Society supporter Sen. Lindsey Graham (R-SC) and his great staff, **Senate Resolution 450** passed, **designating May 15, 2012, as National MPS Awareness Day**! We thank Society members who wrote letters and sent e-mails to generate this success.

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

THE PAST SEVERAL MONTHS have been a whirlwind of advocacy opportunities for the National MPS Society and all our members. I hope you have had a chance to jump in and make that leap into becoming an advocate for yourself, your loved one, and/or for the Society in general. I also hope you have caught the advocacy bug! Warning: It can be contagious. Once you start communicating with your representatives and building relationships, you soon realize that YOU can make a difference, they will listen, and it can be incredibly empowering. As we move our advocacy efforts forward, it continues to be critically important to build and cultivate relationships with our decision makers in both the House and Senate. We must grow our list of Senate and House champions to move our mission forward of funding research, spreading awareness, and creating an environment where regulations aren't deterring research interest. Your House representative or senator could be one of our greatest champions who makes all the difference. From a legislator's point of view, the most influential advocates are their very own constituents (voters). I can advocate on your behalf but what they really want is to hear from someone living in their state or district. It all starts with telling your story and building that relationship so that you can bring more attention to the needs of our children and adults living and suffering with MPS and related diseases. I hope I can guide you forward into becoming an advocate and provide you with the information that is relevant to our Society.

Two ways to get a running start is to join the Society's Policy with Partners to be included in all e-mails and action alerts, and secondly to take our NEW online advocacy Webinar (available in the Members Only section of our Web site) to learn how to influence your lawmakers by visiting them in your home state. We will guide you and provide all the information you need to be a powerful voice!

Sincerely,

Stephanie Bozarth
Chair, Committee of Federal Legislation

Policy with Partners: Five Minutes of Your Time to Take Action!

The Legislative Committee reviews and tracks legislation important to our community which we then forward to Policy with Partners (PwP). When you sign up for PwP, you will be included in all action alerts and e-mails. On occasion, depending on importance, some legislative action alerts will go to all members. PwP is a program set up to pinpoint a core of Society members who we can count on to advocate when called upon. If you want to be a PwP member, please sign up on our Web site or call the main office.

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**.

• H.R. 4132, Faster Access to Specialized Treatments Act (FAST Act)

The FAST Act will modernize and expand the U.S. Food and Drug Administration's accelerated approval pathway to encompass a broader range of diseases and leverage 21st century drug development tools and strategies. This reform will speed the approval of much-needed therapies and cures to patients who are facing serious and life-threatening conditions, including Alzheimer's disease, autoimmune diseases, multiple sclerosis, Parkinson's disease and hundreds of rare diseases that remain untreated. Take action at **www.rarediseaseadvocates.org**.

• H.R. 3059, Support the Creating Hope Act S.606

The Creating Hope Act provides private market incentives for development of drugs for pediatric rare diseases through the extension of the priority review voucher program to children with rare diseases. The Creating Hope Act is cost neutral—it does not require any appropriation. Take action at **www.rarediseaseadvocates.org**.

Current News:

MPS Society Advocates on the Hill

March 12–14: MPS Society members Steve Holland (president/MPS I parent), Stephanie Bozarth (vice president/MPS IV parent), Jeff Bardsley (board member/MPS II affected), Dawn Checrallah (board member/MPS I parent), Nick Boyce (MPS I affected) and Amy Bardsley (MPS II parent) were at the Capitol advocating for the Society's legislative priorities on behalf of all members. In three days, these Society members had 21 appointments representing 18 states. They were well received and found the legislative staff very attentive and supportive of the Society's priorities. The following requests were made of the senators:

- insertion of the MPS-related language into the 2013 Appropriations Bill;
- signing on as a co-sponsor of the National MPS Awareness Day Resolution; and
- support for TREAT and FAST

Lifespan Respite Coalition Update

Thank you to everyone who helped secure Senate signors to the FY 2013 Senate appropriations request for Lifespan Respite funding. Seven senators signed on in a three-day turnaround! The final letter can be found on the National Respite Coalition Web site.

National Center for Advancing Translational Sciences (NCATS) Is Focus of Congressional Hearing

"Despite phenomenal progress in basic science, we still lack effective treatments for far too many diseases, and this translational pipeline to get there is long, 14 years on the average, and it's leaky," said National Institutes of Health (NIH) Director Dr. Francis Collins during congressional testimony on March 20. Both Collins and NCATS Acting Director Dr. Thomas Insel testified on the president's FY 2013 budget request before the U.S. House of Representatives Appropriations Subcommittee on Labor, Health and Human Services, Education, and Related Agencies, chaired by Rep. Denny Rehberg (R-MT).

The hearing focused on both the NIH and NCATS budgets, and featured panel discussions about the many opportunities that exist for NCATS to work in synergy with private industry to improve the translation process. Collins also restated NIH's commitment to fund basic biomedical research as its highest priority. Insel showcased

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>> NIH's Clinical and Translational Science Awards program, saying "80 percent of [the NCATS] budget is the CTSA program" and that in the next five years, the goal is to increase "engagement of communities, not only as a source of patient volunteers, or research volunteers, but increasingly to get them in at the front end to help define what the research problems need to be, and to bring them in as a full partner." Insel emphasized, too, that fixing the pipeline is "what NCATS is all about…figuring new ways to develop compounds and new ways to develop diagnostics."

Visit the NCATS new Web site at **www.ncats.nih.gov** to learn about the center's latest news and events, innovative research initiatives and programs, areas of focus, and current opportunities for collaboration.

Did You Know?

Michael J. Astrue, commissioner of Social Security, added 52 new Compassionate Allowance conditions to the list, one of which is I-Cell (ML II). MPS I, MPS II and MPS III had previously been added to the list of Compassionate Allowance conditions. The Compassionate Allowance program allows patients with certain diseases to receive disability decisions within days rather than months or years. The new conditions will become effective August 2012.

Advocacy Personal Stories

Rare Disease Day 2012

by *Roy Zeighami*

On Feb. 29, International Rare Disease Day, I participated in the first lobby day in Washington, DC, coordinated by the Rare Disease Legislative Advocates (RDLA). Parents of children with MPS diseases and other rare diseases were represented. The EveryLife foundation offered $500 travel grants to help defray the cost of travel. Short meetings were scheduled by the EveryLife foundation throughout the day with the Congressional senior staff and sometimes with a member of Congress themselves. The families spent a few short minutes in the meetings sharing their story and then made their "ask," support for a specific bill. Beginner advocates were paired with seasoned advocates and were informed about bills that were in consideration prior to their meetings. It was amazing to watch the transformation that happened as newcomers gained confidence and an understanding of the importance of what they are accomplishing.

Only we, as patient advocates, can share OUR story and describe what WE want—from NIH funding for research, to statues that affect how drugs are approved and clinical trials are run, to direct support and services for families. Without your voice, your elected representative can only guess what is important to you. While there is much, perhaps justified, cynicism around politics, one thing holds true—politicians want to be re-elected. By showing that we are motivated to meet with our representatives, we send a strong message: We are not going away and will be heard.

Over this next year, I encourage you to build a relationship with your representative. Find out who your representative is and visit their district office near your home. The National MPS Society has information available at **www.mpssociety.org/news/legislative** to help you set up appointments and craft your talking points. Remember, you are extremely powerful. Your story is compelling and your child's story deserves to be told.

Hopefully, there will be many more RDLA lobby days. Even if you can't make it to Washington, you can have an impact. Visit your representative's district office, sign up on their Web page for alerts, and go to their town hall meetings. There is so much policy work to be done and it all starts with your relationship with your representatives.

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>> My Visit to Capitol Hill

by *Nick Boyce* (MPS I)

Nick Boyce (MPS I)

In March of 2012, I had the opportunity to visit Capitol Hill. Part of my visit consisted of meeting with the offices of the U.S. Senate. In the past, my mom has always been an advocate for me, so it was a very rewarding experience as an adult to speak on a political level on my own behalf and the behalf of MPS children who are unable to speak for themselves.

It was nice to be able to share my personal experiences with those on the Hill. Most of whom I met with were not aware of what MPS was and how it affects everyday life.

During my visit, I also had the opportunity to meet in person my senators from Rhode Island, Sen. Sheldon Whitehouse and Sen. Jack Reed. I attribute this opportunity to my mom who had previously attended a town hall meeting in my hometown. Meeting them in Washington, essentially serving as a follow-up meeting after having already heard my story and the challenges I'm faced with every day, allowed them to put a face with my name and MPS.

If given the opportunity to participate in another visit, I would definitely take the trip. Every voice heard only moves us closer to making a better future for all children and adults living with MPS.

The Lewis family, whose daughter, Madison, has MPS III, was invited to visit with Sen. Kay Hagan (D-NC) at UNC Health on May 1 in support of her efforts of the TREAT Act, which accelerates the review and approval process for medicines that treat an unmet medical need or significantly advance the standard of care for people suffering from deadly diseases.

"It was a wonderful experience for our family and one that will hopefully have a positive impact," said the Lewises. "Our objective was to impress that while the rare diseases our MPS families deal with are far less common than some of the more prevalent overall societal diseases, they are no less devastating to our families and children who have to endure them. Streamlining the FDA approval process to allow faster access to beneficial treatments will provide additional hope and support to all our loved ones."

(l. to r.) Wayne Lewis, Dr. Art Aylsworth, Sen. Kay Hagan, Dr. Joseph Muenzer, Morgan Lewis, Paige Lewis and Madison Lewis (MPS III)