legislative update

Advocacy. Research. Hope.

During the last several months, I have been to several networking and educational conferences around rare disease advocacy and orphan drug development. This fall, I attended the Drug Information Association/National Organization for Rare Disorders Conference on Orphan Drugs, the FDA Rare Disease Patient Meeting on Demystifying the FDA, Rare Disease Legislative Advocates Meeting, and the Rare Voice Gala. I have been inspired by how much advocacy and research energy is swelling around rare diseases. In fact, MPS and related diseases have been at the forefront of benefiting from this new age of disease etiology knowledge, flexibility changes in regulatory framework and our improving economy. It is an exciting time full of hope. A time that I am hopeful will sustain and bring to light new and innovative approaches to treating MPS and all related diseases.

Personally, my family has had our own hope renewed by the MPS IV A (Morquio A) clinical trial in which my daughter Annabelle has been participating for the last two years. We enrolled her in the phase III pivotal study trial in 2011; currently the study drug is moving through the process of FDA review. I testified to the FDA in an advisory committee meeting on Nov. 19 about our experience with Morquio A and what have been the benefits of receiving enzyme replacement therapy. As a parent, I am extremely hopeful that the drug Annabelle has been receiving will be approved by the FDA because we have personally witnessed our daughter's quality of life improve significantly since she started the trial at age 5. She is a happy, thriving child. I attribute her overall happiness to her increased stamina and endurance to fully participate in life, school, friendships, family outings, geocaching, music classes, Girl Scouts, swimming, biking and everything in between. She has the energy to engage in life more than she had prior to being in the enzyme replacement therapy trial. It is a far cry from what we were told to expect at the time of her diagnosis when she was only 6 months old. Hope renewed!

But my work does not end here. There is still a long and bumpy road ahead toward more research, along with protecting and advocating for initiatives that give rare disease drug development a much-needed boost. I am ready to navigate that road because I am not doing it alone.

Our advocacy efforts around regulations and initiatives are one of the three key factors that have advanced the interest in orphan drug development. We can all celebrate in knowing that our rare disease voice is being heard in legislation such as the Orphan Drug Act, the Transforming the Regulatory Environment to Accelerate Access to Treatments Act, FDA accelerated approval guidelines, and National Institutes of Health and FDA funding to implement programs that provide incentives and understanding for rare disease research and drug development. We must continue to call to action Society members and other MPS friends to rally around important legislation that will continue to sustain orphan drug research and development interest along with protecting the rights of all rare disease patients. In this moment, take some time to realize: hope for a brighter future is stronger than ever. Advocating is critical to sustain our momentum.

Stephanie Bozarth
Chair, Committee of Federal Legislation
Vice President, Board of Directors
Stephanie.Bozarth@mpssociety.org
703.256.1980

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**.

CALL TO ACTION!

New—Website section with action alert links

Get updates on MPS-supported initiatives and priorities along with catching up on action alerts through the MPS Society legislative section on the website.

Nomination of MPS I to the Secretary's Advisory Committee on Heritable Disorders in Newborns and Children (SACHDNC)

In January 2012 the National MPS Society submitted the nomination package for MPS I to be considered for the Recommended Uniform Screening Panel (RUSP). These are diseases screened at birth and recommended by the secretary of the Department of Health and Human Services (HHS) for states to screen as part of their universal newborn screening programs (NSB). Diseases on the RUSP are chosen based on evidence that supports the potential net benefit of screening, the ability of states to screen for the disease, and the availability of effective treatments. It is recommended that every newborn be screened for all diseases on the RUSP. Most states screen for the majority of diseases on the RUSP; newer conditions are still in process of adoption. Some states also screen for additional diseases. Although states ultimately determine what diseases their NBS program will screen, the RUSP establishes a standardized list of diseases that have been supported by the SACHDNC and the Secretary of HHS.

An expert committee, comprised of clinicians and newborn screening experts and the Society's Executive Director Barbara Wedehase, provided the detailed information required for the nomination package:
Lorne Clarke, MD
Paul Harmatz, MD
Joan Keutzer, PhD
Paul Orchard, MD
C. Ronald Scott, MD
Barbara Wedehase, MSW, CGC

During the May 17, 2012, meeting of the SACHDNC, the committee accepted the nomination and requested the Condition Review Workgroup review the evidence regarding the harms and benefits related to newborn screening for MPS I. The review was delayed until July 2013, and at that time Barbara Wedehase was asked to participate as an expert advisor on the Technical Expert Panel. The review process is expected to continue through May 2014.

Demystifying the FDA Meeting: Sept. 10, 2013

by Stephanie Bozarth

Recently I attended an FDA-sponsored meeting that invited patients, advocates, and patient advocacy groups to participate in a meeting around the drug development process and emphasizing the importance of the patient's voice. As a parent advocate (like myself) or patient, we are best suited to understand risk vs. benefit ratio in the context of our disease. Risk vs. benefit has been a very important topic in the FDA approval process of rare disease drugs. In the last several years, the FDA has taken real steps toward including the patient's voice. This is especially important when the disease is rare and with limited experts. Basically, it is considering what level of risk a patient would reasonably take in order to get the benefits of a drug, even when the benefits may be limited. In the rare disease community, we are often faced with this very real dilemma on a regular basis. In the absence of any or few treatment options, we have to make the best choice possible to improve the quality of life for our loved ones. We must quickly become the experts in understanding our thresholds for risk.

At this meeting, the FDA and several speakers from industry and patient advocacy groups spoke about the drug approval process and the steps taken by the FDA to include the patient's voice at several steps during the review process. The FDA has been hiring patient representatives from the rare disease community as Special Government Employees (SGE) to be at the table during the drug review conversation among the doctors and regulators. Most recently, the FDA hired an MPS IV parent as a patient representative, which I can reasonable assume was in preparation for the MPS IV enzyme replacement therapy (ERT) drug review process. In addition, the FDA commonly holds advisory committee meetings, which allows for patients and patient advocates to testify to FDA regulators and reviews regarding the drug in question.

As mother of Annabelle (MPS IV A), I have been very involved and anxiously intrigued about this process as it may benefit or hurt my daughter's outcome living with MPS IV. I look forward to more FDA patient meetings to hear about the work the FDA is doing, and how we can have a successful partnership in bringing more treatments and cures to the MPS community.

You can learn about FDA upcoming patient meetings by signing up for e-mail alerts at **www.FDA.gov**.

Rare Disease Legislative Advocates Update

On Sept. 11, 2013, Rare Disease Legislative Advocates (RDLA) hosted a luncheon and conference call to discuss new legislation and advocacy efforts. Following is a summary of the speakers and the topics covered. Many of the bills and letters discussed below can be found at the RDLA website at **www.rareadvocates.org**, as well at the participating organizations' websites.

Modernizing Our Drug and Diagnostics Evaluation and Regulatory Network (MODDERN) Cures Act of 2013 (H.R. 3091)

Eric Giascho of the National Health Council briefly talked about the MODDERN Cures Act which is designed to "modernize our drug and diagnostics evaluation and regulatory network." The MODDERN Cures Act hopes to advance personalized medicine by getting the right medicine to the right people and advance the discovery of therapies for unmet medical needs.

The Patient Choice Act of 2013 (H.R. 2090), Fight to Live

Adam Harbison, from Rep. Morgan Griffith's (R-VA) office, discussed H.R. 2090, which is a bill that provides patients with a means to be involved in investigational medicine (i.e., not approved) if they are willing to pay for their own treatment. Mr Harbison noted that such a bill will empower patients to seek treatment the patients deem appropriate and/or worth the risk/investment.

Patients' Access to Treatments Act (PATA) (H.R. 460)

There is an increasing trend to move expensive medications to specialty tiers where the patient has to pay a percentage of the costs rather than a fixed copay. Some patients may have to pay 20–50 percent of the drug costs under the current system. Maria Spencer of the Arthritis Foundation stated that H.R. 460 is a bill designed to limit cost share payments of specialty tier medications at 10 percent. Ms. Spenser noted that a study conducted by the Arthritis Foundation found that such changes would only increase premiums by $3.

U.S. Ratification of the Convention on the Rights of Persons with Disabilities (CRPD)

Esme Grant of the United States International Council on Disabilities noted that CRPD or the "Disabilities Treaty" is an international treaty developed by the United Nations. The treaty has been the framework for countries to create their own national disability laws. At present, 134 countries have ratified the CRPD; the United States has not. While the president has signed the treaty, ratification requires a Senate majority (two-thirds approval). During the spring of 2013, a Senate vote fell short of the two-thirds needed to pass the bill (by five votes). According to Ms. Grant, they hope to return the bill to the Senate floor shortly for another vote.

Newborn Screening Saves Lives Act—Senate Changes

Emil Wigode from the March of Dimes noted that there currently is no date for when this bill will be introduced, but they are moving forward to raise awareness of the need for federal programs to provide assistance to states in order to improve and expand their newborn screening programs; support parent and provider education; and ensure laboratory quality and surveillance for newborn screening.

Congressional Letter Seeks Accelerated Approval

A letter was circulated in Congress calling on the FDA to fulfill the mandate to improve access to the Accelerated Approval pathway for rare diseases as required by the Food and Drug Administration Safety and Innovation Act. The rare disease community jumped into action, contacting their members of Congress and urging them to sign. On Sept. 20, 2013, the final letter was sent to the FDA with 109 Congressional signatures.

FDA Safety over Sequestration Act (H.R. 2725)

Jenny Carey of California Healthcare Institute commented on their progress with H.R. 2725. The bill is in response to the pharmaceutical companies paid "user fees" being denied to the FDA due to sequestration. According to Ms. Carey, that is $85 million that the FDA now does not have. The main argument for exempting this practice is that user fees are private—not public—monies and they should not be withheld from the FDA. Ms. Carey stated the bill is being designed so that future fiscal years will not have this situation and allow the FDA to have the private monies they need to conduct their activities.

Advocating: A Guide to Sharing Your Personal Experience with MPS and Related Diseases

Sharing your personal experience with MPS is a very powerful way to engage legislators and secure their support. In fact, it will be the most important part of your meeting. Your story will make MPS real for legislators in a way that facts and data simply can't. Your personal experience will be the aspect of the meeting that legislators are most likely to remember and discuss with colleagues. The power of your personal story cannot be overstated!

To shape your personal story in a way that best supports the meeting goals, it can help to think through the following questions:

- How has MPS affected the life of your loved one?
- How has MPS affected your family?
- What are the day-to-day impacts?
- How has it impacted you emotionally? Socially? Economically?
- What specific examples can you provide that illustrate the depth and breadth of the impacts?
- Number of days missed from work or overall impact on your ability to work.
- Number of doctor appointments over the course of a month or the number of hours spent in doctor appointments.
- Medical costs.
- What does your child have to deal with that other children do not? Are there things your child cannot do because of MPS?
- How do you want this legislator to feel? What can you tell them about your experience with MPS that will make them feel this way?
- What do you want this legislator to do? What can you tell them about your experience with MPS that will motivate them to act?
- What challenges do you face that would be helped by the legislative actions you are requesting?

Also, it is important to:

- Keep it brief. Getting the legislator's attention is important, and a story that is too long or unstructured can lose meaning. A story that is three to five minutes will allow you to share the most important aspects of your experience with MPS. Less is actually more—give them a glimpse into your life and experience and it will have a BIG impact.
- Bring a picture. A picture of your child is important as it gives your lawmaker a visual connection to your story and makes your meeting even more memorable.
- Don't get bogged down in medical details or too many facts. The legislator will get all the facts he or she needs from the information you leave at the end of the meeting. Tell your story and talk about what your life is like because of MPS. Focus on making the lawmaker FEEL something, not on having them understand all the facts.
- Remember that you are the expert in this story. While legislators are experts in what they do, they are not experts on MPS. The information and experience you have is valuable and will help them better understand MPS and the challenges for individuals and families.

Patient and Parent Perspectives

A Young Generation of Advocates

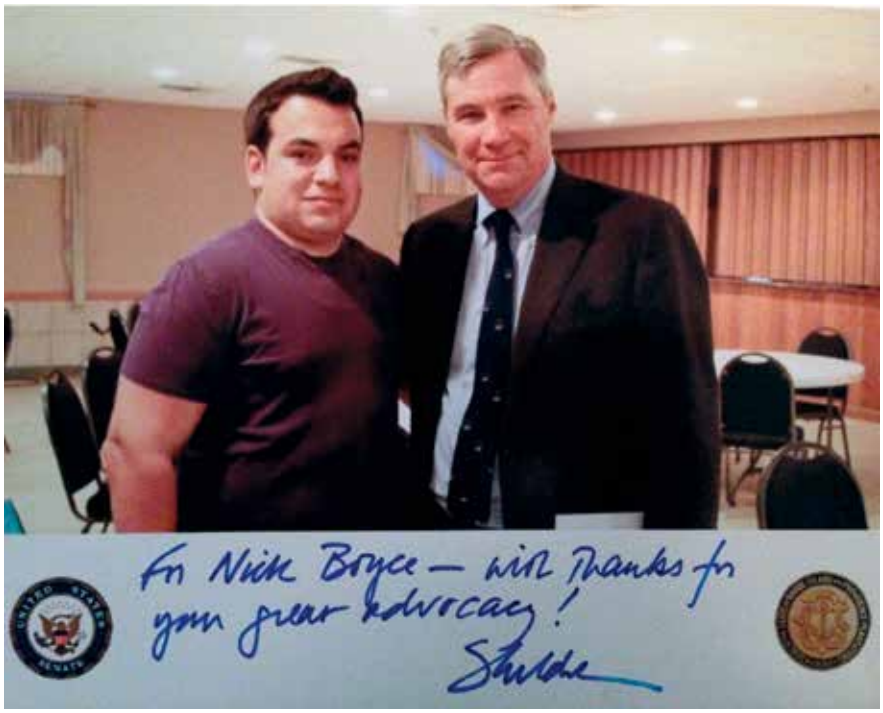
As a parent of an MPS I young adult, I've spent many years advocating. Through the years, I always hoped that at some point my son Nick would be able to advocate for himself. I always knew there would come a time when I wouldn't be there in the background adding my comments or suggestions.

This day eventually came three years ago when Nick started his freshman year at Providence College. Right from the start he worked through the Office of Academic Services requesting his required accommodations. He is now in his senior year and is a member of their Disability Focus Group.

In 2012, my son had the opportunity to come to Capitol Hill as part of the Society's yearly visit and advocate with me on behalf of the MPS Society. During this visit he was fortunate to have the opportunity to meet with one of our own state senators. It was a great experience for him to be able to share his story and speak on behalf of MPS. Since that visit Nick has continued his advocacy work within our own state. This past year we were extremely happy to find out that our senator, Sheldon Whitehouse, had signed the resolution making May 15 National MPS Day.

It's possible and it's important for our young adults to become advocates for themselves on all levels, especially on legislative causes. I encourage all young adults to reach out to their individual state legislators, introduce themselves and share their stories. If you'd like additional information from Nick, please e-mail me at dawn.checrallah@mpssociety.org. They have such great stories to tell and hearing from them directly can have such an impact. They have such powerful voices and deserve to be heard!

Dawn Checrallah
Committee on Federal Legislation



Nick Boyce (MPS I) with Sen. Whitehouse (D-RI) at a town hall meeting in Rhode Island.