legislative update

Policy with Partners: Time to Take Action!

The Legislative Committee reviews and tracks legislation important to our community, which we then forward to the Society's Policy with Partners (PwP) group members. Previously you would need to sign up for PwP e-mails, however, due to the impressive number of members participating in action alerts and the improved ease of advocating, the Legislative Committee will be including all Society members in alerts and updates in the future. We will continue to track legislation and only ask for member advocacy participation when it would greatly benefit or significantly affect the MPS community.

THIS SUMMER THE LEGISLATIVE COMMITTEE has continued to gain speed in our advocacy efforts with great promise for the future. We were successful with advocating for the MPS Awareness Day Senate Resolution, changes in legislation that will allow for more innovation and opportunities in drug development for the rare disease community, inclusion of MPS language in the Senate version of the Appropriations Bill, providing a toolkit and webinar for our members to engage in advocacy within their own district, and providing such ease in advocating with the "click and send" action alerts that anyone can do it in just a few spare minutes. The "click and send" action items have been extraordinarily valuable to our Society due to the fact that our families our juggling so much every day. Simply, we needed to make it easier so more people could participate. Additionally, I have been so grateful for engaging M+R Consulting because it has given our committee the tools to be successful, passing that along to the entire MPS community. As a mom of three young children and one having MPS IV, I know the value of keeping it succinct and with a clear purpose. If any members would like to join the Legislative Committee, please do not hesitate to contact me to see if it would be a good fit for you.

Sincerely,

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

After The Ruling: A Consumer's Guide

by Mary Agnes Carey, Kaiser Health News

On June 28, 2012, the Supreme Court upheld the 2010 federal healthcare law, dismissing the challenge by states to the law's requirement that individuals get insurance. The justices, however, did give states the right to opt out of a critical provision requiring them to expand Medicaid programs for the poor and disabled. The decision will have sweeping ramifications for consumers, state officials, employers and healthcare providers, including hospitals and doctors.

While some of the key features don't kick in until 2014, the still-controversial law has already altered the healthcare industry and established a number of consumer benefits.

A Q&A about some of the law's provisions that are already up and running, as well as major features of what's to come, can be found at **www.kaiser-healthnews.org/Stories/2012/March/22/consumer-guide-health-law.aspx**.

Current Legisltaive 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 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 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."

We aren't a large organization, but we are powerful—because of YOU! You are all fantastic advocates. Our strength as an organization isn't defined by our size but by the involvement of our members.

Obama Signs FDA User Fee Legislation Bringing Hope to Rare Disease Patients

On July 9, 2012, President Obama signed into law *The Food and Drug Administration Safety and Innovation Act (FDASIA), S. 3187*, bipartisan legislation that will spur the development of lifesaving treatments for 30 million Americans suffering from rare diseases.

"We are thrilled the language to improve access to the FDA's Accelerated Approval pathway for rare diseases has been included in *FDASIA*," said Emil Kakkis, MD, president, EveryLife Foundation for Rare Diseases. "We thank Representatives Cliff Stearns (R-FL) and Ed Towns (D-NY) for being champions for the rare disease community."

Stearns and Towns first introduced the *Unlocking Lifesaving Treatments for Rare Diseases Act* to empower the FDA to use all the science available for allowing surrogate endpoints in clinical trials for rare diseases to determine whether a drug is working, significantly decreasing the development time and cost. Stearns and Towns later introduced the *Faster Access to Specialized Treatments Act* that improved Accelerated Approval for life-threatening diseases while maintaining high safety and efficacy standards.

Legislative Committee:

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

FDASIA is the culmination of more than a year of negotiations between industry and FDA and includes the reauthorization of the drug and device user fees.

The FDA's Accelerated Approval has been successful in getting treatments approved for cancer and AIDS patients, but has been essentially unavailable for rare disease treatments. There currently are fewer than 400 FDA-approved treatments for nearly 7,000 rare diseases. Investment and interest in development will surge for rare diseases if there is access to the Accelerated Approval pathway.

"We would not have been successful if it were not for the great work of Energy and Commerce Chairman Fred Upton (R-MI), Biotechnology Industry Organization, and more than 300 patient organizations that advocated for improving the FDA's regulatory process," said Kakkis.

Congress Recognizes National MPS Awareness Day

"I would like to recognize the National MPS Society for their 37 years of supporting families while searching for cures for this genetic disease," said Rep. Kenny Marchant (R-TX) while addressing Congress on May 16, 2012.

"Mucopolysaccharidosis or MPS is a group of genetically determined lysosomal storage diseases that render the human body incapable of producing certain enzymes needed to break down complex carbohydrates. The damage caused by MPS on a cellular level adversely affects the body and damages the heart, respiratory system, bones, internal organs and central nervous system. MPS often results in intellectual disabilities, short stature, corneal damage, joint stiffness, loss of mobility, speech and hearing impairment, heart disease, hyperactivity, chronic respiratory problems and, most importantly, a drastically shortened life span. Symptoms of MPS are usually not apparent at birth and without treatment the life expectancy of an individual affected begins to decrease at a very early stage in their life. Research toward combating MPS has resulted in the development of limited treatments for some of the MPS diseases."

"I ask my colleagues and their staff to join me in recognizing May 15, 2012, as National MPS Awareness Day. This is an important time during which the MPS disease community will help increase the awareness of this devastating disease, as well as supporting research to improve treatments, find cures and receive early diagnosis. MPS families are encouraged to reflect and support each other and to reach out to those families who have lost loved ones to MPS. By wearing their purple ribbons and sharing these ribbons within their community, they are increasing public awareness about this disease. This date is also the start of the National MPS walk/run season along with other local community activities to raise awareness, along with money for research and family assistance programs. I commend the National MPS Society and their many volunteers for an unwavering commitment to bring about awareness of this disease, and to continue to advocate for federal legislation to streamline the regulatory processes and to speed effective treatments and cures for their loved ones. More must be done to find cures and effective treatments, but let us reflect on the importance of this day. I ask that all of my colleagues join me in commemorating National MPS Awareness Day."