# legislative update

**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

## Dear Society Members,

Senators Graham (R-SC), Hoeven (R-ND), Begich (D-AK), Whitehouse (D-RI), Heitkamp (D-ND), Murkowski (R-AK) and Boxer (D-CA) cosigned the Senate Resolution 142 to designate May 15, 2013, as National MPS Awareness Day. We did it, again! As always, it was very exciting leading up to our MPS Awareness Day as many Society members are reaching out to their senators, sharing their personal story and requesting that they consider cosigning this resolution. Many times we have no confirmation on who is going to cosign until the actual day. Thank you to all our members who participated in the advocacy alert only days prior. If your senator did not cosign, it is still a success because it gave you an opportunity to share a special story with your representative which may shape decisions around rare disease legislation, healthcare, National Institutes of Health funding, government oversight, special accommodation programs, etc. Your story is critical because it provides your representative with the rare opportunity to hear a personal testimony about what it is like to have a rare disease, like MPS, or to care for and love someone who has a rare, degenerative disease. Thank you for participating and please continue to do so. Your voice is important.

In addition to MPS Awareness Day, other legislative news includes the National Organization of Rare Diseases (NORD) celebrating the 30th anniversary of the Orphan Drug Act. I was able to attend the NORD gala in DC, which was inspiring as always. I heard from many individuals who have played key roles in bringing attention to rare diseases and shaping legislation. I especially loved the personal stories from the rare disease community and how they have overcome and met challenges head-on to get to a brighter future. ❁

*Stephanie Bozarth*
*Chair, Committee of Federal Legislation*
*Vice President, Board of Directors*
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## Brain Research Through Advancing Innovative Neurotechnologies Initiative

The NIH Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative is part of a new presidential focus aimed at revolutionizing our understanding of the human brain. By accelerating the development and application of innovative technologies, researchers will be able to produce a revolutionary new dynamic picture of the brain that, for the first time, shows how individual cells and complex neural circuits interact in both time and space. Long desired by researchers seeking new ways to treat, cure and even prevent brain disorders, this picture will fill major gaps in our current knowledge and provide unprecedented opportunities for exploring exactly how the brain enables the human body to record, process, utilize, store and retrieve vast quantities of information, all at the speed of thought. For more information, visit **www.nih.gov/science/brain/index.htm**.

# 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. There also 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 under Members Only—Legislative Toolkit. Under "How to Webinar," click on "Guide to in-district Legislative Visits."

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# NIH 2013 Budget Cuts Announced

## Agency releases details of the sequester's effects

by *Kate Yandell*, May 10, 2013

The numbers are in on the extent of the National Institutes of Health (NIH) budget cuts for 2013, ScienceInsider reported. As expected, due to the sequester the agency's budget fell by $1.55 billion to a total of $29.15 billion for the year, a 5 percent cut.

The agency would have awarded 34,902 grants but is eliminating 1,357 of those, including 703 that would have been for new competing grants. The rest of the grant cuts would have been extensions of previously funded research.

The NIH had already reduced some continuing awards by 10 percent, and the agency said that while some of those funds could possibly be restored, the grants would likely continue to be funded below their originally intended levels. Furthermore, the NIH has promised to increase ongoing grants each year with inflation in the past, but starting with grants awarded in 2012 it will no longer do so.

NIH Director Francis Collins took to Twitter to express his frustration with the cuts, soliciting researchers to tell their tales of the sequester's impact under the hashtag #NIHSequesterimpact. "I'll be sharing some of your stories to try to turn this mess around," he tweeted.

"Despite the reduced funding, the NIH remains committed to the mission of seeking fundamental knowledge about the nature and behavior of living systems and the application of that knowledge to enhance health, lengthen life, and reduce the burden of illness and disability," the agency said in a statement.

April 16, 2013

The Honorable Fred Upton
Chairman
Committee on Energy and Commerce
U.S. House of Representatives
Washington, DC 20515

The Honorable Joe Pitts
Chairman
Subcommittee on Health
Committee on Energy and Commerce
U.S. House of Representatives
Washington, DC 20515

The Honorable Michael Burgess
Vice Chairman
Subcommittee on Health
Committee on Energy and Commerce
U.S. House of Representatives
Washington, DC 20515

Dear Chairman Upton, Chairman Pitts, and Vice Chairman Burgess:

The rare disease patient community greatly appreciates your leadership in offering a solution to the funding issues associated with the Preexisting Condition Insurance Plan (PCIP) Program and strongly supports swift passage of H.R. 1549, the Helping Sick Americans Now Act. The prohibition against preexisting condition exclusions that the Affordable Care Act (ACA) established is one of several vital insurance market reforms that will ensure patients with life threatening and debilitating, chronic rare diseases will have access to their treatment regimen. The PCIP program is a critically important component of the ACA market reforms because it affords those patients that currently lack insurance coverage due to these discriminatory practices an opportunity to enjoy temporary coverage until they can transition to meaningful coverage on Jan. 1, 2014.

The decision in February to suspend PCIP enrollment could have catastrophic consequences for the rare disease patient community because of treatment delays. Impediments to accessing rare disease therapies not only lead to poor health outcomes, but also drive up health care costs due to increased physician office visits, emergency room interventions, hospital admissions, and surgical interventions. In order to ensure patient access, we strongly support your efforts to:

- **Protect program solvency for existing PCIP beneficiaries:** The Department of Health and Human Services (HHS) has not disclosed how much of the $5 billion in funding remains for more than 100,000 currently enrolled PCIP beneficiaries. The anxiety resulting from this uncertainty is troubling for a subset of the population who has already had to overcome significant challenges in obtaining proper diagnosis and finding a viable therapeutic option. Additional program funding will remove any uncertainty that the program remains solvent for the remainder of 2013.
- **Lift the enrollment suspension:** Due to the eligibility requirement of being uninsured for six consecutive months prior to PCIP enrollment, there are thousands of patients who would have been eligible to enroll in PCIP after the suspension dates of February 16th (Federal PCIP) and March 2nd (state-operated PCIP). Removing this condition and providing more funding would save and improve the lives of thousands in the rare disease community by affording them access to immediate coverage until they can transition to new coverage in January.
- **Provide safeguards to ensure a smooth transition to coverage:** The statute requires HHS to develop procedures to transition PCIP beneficiaries into the qualified health plans offered in the Affordable Insurance Exchange. The rare disease patient community is very concerned that HHS has not taken such action, which is especially unsettling in light of the fiscal health of the PCIP program.

Due to our relatively low number of patients per condition, the rare disease community is often overlooked when policies are being negotiated on Capitol Hill or implemented by HHS. Providing additional funding not only to sustain the PCIP program for the remainder of 2013, but also to give more uninsured patients with preexisting conditions the opportunity for immediate coverage will demonstrate a strong commitment to the rare disease patient community. Thank you for your support.

Sincerely,

Adult Congenital Heart Association
Alpha-1 Association
Alpha-1 Foundation
CADASIL Association
COPD Foundation
GBS/CIDP Foundation International
Global Genes
Hemophilia Federation of America
Hereditary Angioedema Association
Immune Deficiency Foundation
Lipodystrophy United
Little Miss Hannah Foundation
The Mastocytosis Society
MLD Foundation
National Gaucher Foundation, Inc.
National MPS Society
National PKU Alliance
Noah's Hope for LINCL-Batten Disease

cc: The Honorable Henry Waxman
The Honorable Frank Pallone

113TH CONGRESS 1ST SESSION

# S. RES. 142

Designating May 15, 2013, as "National MPS Awareness Day."

IN THE SENATE OF THE UNITED STATES

MAY 15, 2013

Mr. GRAHAM (for himself, Mr. HOEVEN, Mr. WHITEHOUSE, Mr. BEGICH, Ms. HEITKAMP, Ms. MURKOWSKI, and Mrs. BOXER) submitted the following resolution; which was considered and agreed to

## RESOLUTION

Designating May 15, 2013, as "National MPS Awareness Day."

Whereas mucopolysaccharidosis (referred to in this resolution as "MPS") are a group of genetically determined lysosomal storage diseases that render the human body incapable of producing certain enzymes needed to break down complex carbohydrates;

Whereas MPS diseases cause complex carbohydrates to be stored in almost every cell in the body and progressively cause cellular damage;

Whereas the cellular damage caused by MPS—

(1) adversely affects the human body by damaging the heart, respiratory system, bones, internal organs, and central nervous system; and

(2) 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;

Whereas symptoms of MPS are usually not apparent at birth;

Whereas, without treatment, the life expectancy of an individual afflicted with MPS begins to decrease at a very early stage in the life of the individual;

Whereas research has resulted in the development of limited treatments for some MPS diseases;

Whereas promising advancements in the pursuit of treatments for additional MPS diseases are underway as of the date of agreement to this resolution;

Whereas, despite the creation of new remedies, the bloodbrain barrier continues to be a significant impediment to effectively treating the brain, which prevents the treatment of many of the symptoms of MPS;

Whereas the quality of life of the individuals afflicted with MPS, and the treatments available to those individuals, will be enhanced through the development of early detection techniques and early intervention;

Whereas treatments and research advancements for MPS are limited by a lack of awareness about MPS diseases;

Whereas the lack of awareness about MPS diseases extends to individuals within the medical community;

Whereas the cellular damage that is caused by MPS makes MPS a model for the study of many other degenerative genetic diseases; and

Whereas the development of effective therapies and a potential cure for MPS diseases can be accomplished by increased awareness, research, data collection, and information distribution: Now, therefore, be it

*Resolved*, That the Senate—

(1) designates May 15, 2013, as "National MPS Awareness Day"; and

(2) supports the goals and ideals of "National MPS Awareness Day"