

New Division for Biochemical and Genetic Diseases

The National MPS Society, along with 111 patient and professional organization partners, is advocating for resources to provide a new, more specialized FDA drug review office focused on streamlining the development path for rare biochemical and genetic diseases. We have requested a \$10 million appropriation to build the human, scientific and organizational capacity necessary to evaluate products targeted to rare diseases at the FDA to establish a new division for biochemical and genetic diseases within the Office of New Drugs. There are more than 7,000 rare disorders that together affect more than 25 million Americans and their families. In the last 25 years, less than 5 percent of these disorders have a treatment approved for marketing by the FDA. Treatments for many of these diseases may never be developed because of roadblocks in the development process, such as a lack of investment and a challenging regulatory environment. The FDA will hold hearings on June 29 and June 30; the Society has sent written testimony of its support.

Rare and Neglected Disease Caucus

While in Washington, we encouraged members of Congress to join the formation of the Rare and Neglected Disease Caucus to help address specific issues related to developing treatments for rare diseases. The papers were filed on May 18, 2010, and two members of Congress have signed on: Rep. Joseph Crowley (D-NY) and Rep. Fred Upton (R-MI). We are still looking for two senators to sign on. The Society investigated whether to have an MPS Congressional caucus, but felt that our Society would benefit from joining with other disease organizations. Thanks to the Kakkis EverydayLife Foundation for moving this forward.

Screening Your Candidates for Fall 2010 Election

Mid-term elections will be Nov. 2, 2010, with at least 36 out of 100 seats in the Senate, and all House seats up for election. Many of these senators, representatives and other candidates will be in their own states during the month of August and on weekends throughout the summer visiting their constituents. There will be a number of forums: town hall meetings, debates, picnics, or call to meet and have a one-on-one conversation. This is a perfect opportunity for you and your family to be active in deciding who is the best candidate on issues that are important to our MPS families. Whenever possible, take your MPS child and thoughtfully tell your candidates what is important to your family, whether it be special education, increased funding for the NIH, funding for respite care, Medicaid funding, and/or streamlining the protocols with the FDA to expedite effective treatments. Or all of the above. If you are able to meet with your candidates, be sure and do some follow-up correspondence to reiterate issues of importance. Opening the lines of communication can prove to be very effecting when your Legislative Committee calls on members of Congress. If you have questions, please contact any of the Legislative Committee members or call the MPS office.

Legislative Committee:

Ernie Dummann, chair
 Steve Chesser
 Jennifer Clarke
 Debbie Dummann
 Steve Holland
 Terri Klein
 Dave Madsen
 Austin Noll
 MaryEllen Pendleton
 Laurie Turner
 Barbara Wedehase
 Kim Whitecotton

NIH Receives \$1 Billion Boost in 2011 Budget

A \$1 billion boost for the NIH announced in the 2011 budget quelled fears that President Obama's proposed non-security discretionary spending freeze would decrease budgets at federal science agencies. Numbers released from the White House's Office of Management and Budget tells of slight increases in the budgets for the NIH. To see President Obama's FY2011 budget request in its entirety go to www.Whitehouse.gov/omb/budget/fy2011/assets/budget.pdf.

Senate Resolution 528 — National MPS Awareness Day

Thanks to Sen. Lindsey Graham (R-SC), Colin Allen and Leigh Ellen Lybrand for their successful passage of S.R. 528 recognizing May 15, 2010, as National MPS Awareness Day. Although originally reported as S.R. 530, when recorded, the actual resolution is 528. This day was envisioned to honor and remember those with MPS and related disease while conveying knowledge and understanding to members of our local community, state and our elected officials in Congress. Thank you notes were hand-delivered to the following senator's who co-sponsored this resolution: Sen. Mark Begich (D-AK), Sen. Kent Conrad (D-ND), Sen. Daniel Inouye (D-HI), Sen. Lisa Murkowski (R-AK), Sen. Richard Burr (R-NC), Sen. Byron Dorgan (D-ND), Sen. John Kerry (D-MA), Sen. Bill Nelson (D-FL) and Sen. Arlen Specter (D-PA). Sincere thanks to all who contacted their senators to co-sponsor.



Ernie and Debbie Dummann with Colin Allen and Leigh Ellen Lybrand, staff from Sen. Lindsey Graham's office (R-SC)

New National Health Reform Law Helps MPS Patients Keep Insurance

The historic healthcare reform laws of 2010 (Accountable Care Act or ACA) will provide many health insurance protections for MPS patients. Implementing the provisions included in both The Patient Protection and Affordable Care Act (March 23, 2010), along with improvements from the Health Care and Education Affordability Reconciliation Act of 2010 (March 30, 2010), represent a huge undertaking for the government. The National MPS Society is committed to ensuring that MPS patients take advantage of the full benefits of this important law.

The law will be implemented over several years, but some important provisions that help MPS patients keep their insurance become effective later this year. These include:

- **Elimination of lifetime caps:** By October 2010 insurance companies may not establish lifetime dollar benefits for essential health benefits.
- **Elimination of retroactive insurance cancellation (a.k.a. “recissions”):** By October 2010, insurance companies are not permitted to retroactively cancel insurance.
- **Dependent coverage of children to age 26:** Starting in September 2010, insurance plans that offer dependent coverage must extend that coverage to dependants up to age 26. In some cases, the effective date is the date of the first open enrollment period after September 2010. Many insurance companies have already adopted this requirement. Check with your insurance company for more details.

Other important provisions of the new law are focused on creating new insurance opportunities for patients and making health insurance more affordable. The MPS Society will keep you informed of key changes as details emerge.