Please Vote on Tuesday, Nov. 2, 2010

When voting this fall, it is important to remember the issues that are significant to those and their families affected with MPS disease. Some key issues which affect us on a daily basis are: special education, healthcare, Medicaid, funding for research, etc.

MPS Mom Meets with President Obama

MPS mother and advocate Jennifer Restemayer meets with President Obama for the 90-day anniversary of the signing of the Affordable Care Act and the New Patients' Bill of Rights.

MPS mom and advocate Jennifer Restemayer from North Dakota was asked to attend and met with President Obama for the 90-day anniversary of the signing of the Affordable Care Act and the New Patients' Bill of Rights. At this meeting Jennifer expressed concerns regarding her daughter, Allison, who was diagnosed seven years ago with MPS I. Jennifer relayed her concerns about insurance issues as her daughter is on enzyme replacement therapy, which is very expensive, but slows the natural progression of her disorder. Allison's father's health insurance policy has a $2 million lifetime maximum. After a major spinal surgery and an increasing dosage requirement for her daughter, Jennifer fears their family's insurance maximum will soon be reached. Under the Affordable Care Act, Allison and her family will no longer need to worry about hitting a lifetime benefit limit because all insurance companies are prohibited from imposing lifetime benefit limits, effective for health plan years beginning on or after Sept. 23, 2010. The new law announced the release of new regulations implementing the patients' bill of rights protections included in the Affordable Care Act. Jennifer's remarks took place immediately after a meeting with Secretary of Health and Human Services Kathleen Sebelius, Secretary of Labor Hilda Solis, state insurance commissioners and insurance company CEOs in the Roosevelt Room. We send Jennifer our thanks for all her advocacy efforts in Washington as well as in her home state.

A fact sheet of the Affordable Care Act and the New Patients' Bill of Rights is available at **www.whitehouse.gov**.

MPS Dad Gives Public Testimony at FDA Hearing

The U.S. Food and Drug Administration (FDA) Rare Disease Committee, created by the Brownback/Brown Amendment, held its first public meeting in June of this year. National MPS Society board member Austin Noll volunteered to go at his own expense to present "Why Clinical Study Designs and Surrogate Endpoints are Needed for Treatment and Development: Mucolipidosis and Mucopolysaccharide Diseases" at the meeting. This presentation voiced the Society's concerns on how and why change is needed at the FDA to help speed up the delivery of new treatments and reduce the cost to consumers for therapies.

Dr. Emil Kakkis from the CureTheProcess campaign also presented on campaign goals, including the need for guidance on surrogate biomarker endpoints and the use of small clinical trial designs, as well as the specialized drug review office for rare diseases. Although they both gave separate testimony, both Austin Noll and Dr. Kakkis voiced concerns on improving the drug review process by flexibility, sensitivity, collaboration on the FDA's use and interpretation of the laws and regulations.

The Rare Disease Committee must submit a report to Congress in March of 2011 on the recommended changes for rare disease drug development, and review and then provide guidance to address these problems by September 2011. The next public meeting will be held in January 2011.

Lifespan Respite Update

The FY 2011 Senate Labor/Health and Human Services/Education bill includes $7 million for Lifespan Respite—this is $2 million more than the president's request and $4.5 million more than last year's funding. The House Appropriations Subcommittee on Labor/Health and Human Services/Education has approved its funding bill for FY 2011 but has not yet released any detailed information other than an overall increase for Administration on Aging programs of $135 million more than FY 2010 funding and $26 million more than the president's request. Thank you to everyone for working so hard to move respite and family caregiver issues forward this year. This is great news.

Senate OKs $1 Billion Boost for NIH

A U.S. Senate panel gave its seal of approval to a $1 billion bump in the National Institutes of Health's (NIH) 2011 budget on July 27, 2010. The Senate's Subcommittee on Labor, Health and Human Services, Education and Related Agencies echoed the sentiment of its House of Representatives counterpart by approving the billion dollar increase, which would bring the NIH's 2011 budget to about $32 billion—a 3.2 percent raise over the agency's 2010 Congressional allotment. President Barack Obama originally requested the amount this February, at the same time calling for increased drug development efforts in 2011. This increase for the NIH includes $50 million for first-time funding of the Cures Acceleration Network and second-year funding of $50 million for therapeutics for rare and neglected diseases.

The House and Senate versions of the bill containing the NIH boost now move on to the full appropriations committees in both bodies, to be considered by the full legislature. A vote on the final bill is not expected until after this November's midterm elections.

Improving Access to Clinical Trials Act—Update

The U.S. Senate passed the I-ACT, a bipartisan piece of legislation, on Aug. 5, 2010. This legislation enables patients with rare diseases to participate in clinical trials without losing eligibility for public healthcare benefits. The legislation was introduced by Sen. Ron Wyden (D-OR), with Sens. Chris Dodd (D-CT), James Inhofe (R-OK), Richard Shelby (R-AL) and Dick Durbin (D-IL) as original co-sponsors; an additional 14 co-sponsors also signed on. Current law prevents many people who receive Supplemental Security Income from accepting research compensation because it makes them ineligible to receive government medical benefits. This penalty has stopped significant numbers of people with rare diseases from participating in clinical studies. Following Senate approval, the bill now awaits consideration by the U.S. House of Representatives.

Rare and Neglected Disease Congressional Caucus Formed

The bipartisan and bicameral caucus was formed in May 2010 and was announced by Rep. Joe Crowley (D-NY) at the National Organization for Rare Disorders gala held in Washington, DC. Caucus co-chairs are Congressmen Crowley and Fred Upton (R-MI), with Sens. Sherrod Brown (D-OH) and John Barrasso (R-WY) recently signing on. These co-chairs will focus on:

- bringing congressional attention to the approximately 6,800 known rare diseases that exist and have no approved therapies;
- ensuring sufficient funding is available for research and orphan product development;
- offering incentives for companies to create new drug biologics and humanitarian use devices; and
- providing an opportunity for families, advocacy groups and members of congress to exchange ideas and concerns on policies.

Cures Acceleration Network—Update

On July 27, 2010, the Senate Labor, Health and Human Services Appropriations Subcommittee included $50 million for the Cures Acceleration Network (CAN) in the FY2011 appropriations bill. CAN creates an additional grant program within the NIH that is focused on the translation of science into medicine. CAN was the main topic of discussion during the short subcommittee mark-up, with Chairman Tom Harkin (D-IA) and Sen. Arlen Specter (D-PA), who authored this legislation, both expressing support for CAN.

On July 15, 2010, the House Labor, Health and Human Services Appropriations Subcommittee included "up to" $50 million for CAN. Currently there is $50 million marked on both the House and Senate side—good news! Your Society appreciates the calls, e-mails and faxes sent by members to ensure our voices are heard on this important topic.

MPS Mom Represents National MPS Society at 60th Anniversary of NIDDK Event

The Juvenile Diabetes Research Foundation held a Congressional breakfast in honor of the 60th Anniversary of the National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK), which funds research vital to and shares goals of preventing, treating and curing devastating diseases. The National MPS Society and several other advocacy and professional groups participated and thanked the NIDDK for their dedication to improve the health and lives of people who have or are at risk for diseases within the NIDDK's mission.

A congressional breakfast on Capitol Hill recognized the achievements of biomedical research conducted and supported by the NIDDK during the past six decades, and the institute's commitment to continued research progress. The program featured remarks from NIDDK Director Dr. Griffin Rodgers, along with the presentation of awards to a few highly distinguished investigators from fields across the NIDDK. We thank MPS mom and board member Jennifer Clarke for attending and representing the National MPS Society. The NIDDK has granted approximately $5 million in the last three years to fund MPS and related disease research.

Ways to GIVE

- Renew your membership or sponsor another family
- Gifts in honor of a special person
- Gifts in memory of a special person
- Matching gifts through your employer (check with your human resource office)
  1. Request a matching gift form from your employer
  2. Complete the employee section of the form
  3. Mail to the Society and we'll do the rest
- Contribute through the Combined Federal Campaign if you are employed by the federal government — CFC #10943
- Designate the Society as a member of your local United Way. You will need to supply them with the Society's name, address and Federal ID number (FEIN #11-2734849)
- Annual Fund donation
- Major gift (usually 10 times that of your Annual Fund gift)
- Planned gift
  1. Bequest in your will
  2. Charitable remainder trust or charitable gift annuity
  3. Charitable lead trust
  4. Life insurance policy
  5. Gift of appreciated assets (stocks, mutual funds and bonds)
- Gifts may be applied to the Society's general operating purposes or restricted to one of our designated programs.

Contact: terri@mpssociety.org or 877.**MPS**.1001