



Community Action Alerts:

Caucus Briefing Alert: Call Your Member of Congress

Call your Members of Congress and ask that they send a staffer to our upcoming Rare Disease Congressional Caucus Briefing addressing the Urgent Healthcare Policy Needs of the Rare Disease Community. The event is taking place in the Cannon Caucus Room (Room 345), on Thursday February 26th at Noon. The briefing will highlight challenges related to coverage, access to specialists, step therapies, and medically necessary foods. [Click here to view our action alert.](#)

Sign-on to Thank Reps. Upton & DeGette for their Leadership

Several rare disease groups are circulating an organizational sign-on letter to thank Chairman Upton (R-MI) & Representative DeGette (D-CO) for leading the 21st Century Cures Initiative, and for incorporating patient voices and perspectives into the effort. For those who might not be familiar with 21st Century Cures, [click here to read a blog post](#) on the Initiative, which provides a brief history and overview of the effort. To date, the EveryLife Foundation, Global Genes, Parent Project Muscular Dystrophy, and the Sarcoma Foundation of America have endorsed the letter. View the letter [here](#) and email mbronstein@everylifefoundation.org to get your organization signed-on or if you have questions. Please share with your networks so we can send a strong message to Congress and thank them for making rare diseases a priority.

Sign-on to Protect Patient Access to Rare Disease Therapies

Many rare disease patients enrolled in Medicaid cannot access lifesaving and life improving Food and Drug Administration ("FDA") approved therapies as a result of barriers designed to make it impossible for rare disease patients to benefit from medical innovation. The National MSP Society, is asking patient organizations to join them in urging Congress to prohibit Medicaid plans from rationing access to rare disease therapies. Without access, innovation means nothing for patients. Click [here to view the letter](#) - you may email Stephanie Bozarth stephbozarth@yahoo.com to sign-on.

Sign-On to Support the Dormant Therapies Act to Create New Treatments for Unmet Medical Needs

The Dormant Therapies Act creates a vital pathway for the development of cures and new treatments that have the potential to improve the lives of millions of Americans living with chronic diseases and disabilities. With this legislation, Congress can provide the incentive for researchers to pursue promising therapies that would otherwise be abandoned due to a lack of patent protection. The dormant therapies provisions will provide companies who develop a product that treats an unmet medical need with a fixed period of protection from generic competition, which begins when a medicine is approved by the Food and Drug Administration (FDA). Click here to [view the sign-on letter](#). To sign-on email Sara Reid by Wednesday, March

4th sreid@nhcouncil.org.

Join Over 135 Patient Organizations in Supporting the OPEN ACT

In a major achievement on behalf of the rare disease community, support for the OPEN ACT (Orphan Product Extensions Now, Accelerating Cures & Treatments) on Capitol Hill is growing. The bill provides incentives for companies to repurpose or “rarepurpose” existing treatments for rare disease indications. The legislation was included in the draft 21st Century Cures discussion bill released by the Energy & Commerce Committee - a huge success made possible by the patient community. In addition, Reps. Bilirakis (R-FL), Butterfield (D-NC), and McCaul (R-TX) have co-sponsored the stand-alone version of the OPEN ACT, which was officially introduced on Friday, February 13th as HR 971. The bipartisan bill introduction was bolstered by strong support from the patient community, with more than 135 organizations signing-on in support of the bill. The EveryLife Foundation is still looking for supporting patient organizations to sign-on. Please contact Max Bronstein mbronstein@everylifefoundation.org to get signed-on today.

Rare Disease Legislation in the News:

21st Century Cures Has Landed

In 2014, Reps. Upton (R-MI) and DeGette (D-CO) began a bipartisan effort to create legislation to foster biomedical research and innovation in the U.S. After months of hearings, public feedback, white papers, and roundtables, the Energy & Commerce Committee released a sprawling 400-page draft discussion bill. The draft includes several standalone bills including the OPEN ACT (HR 971), CURE Act (Expanded Access – HR 909), and the Advancing Neurological Diseases Act (HR 292) among other provisions. Chairman Upton has indicated that he would like a final bill to be voted on by the full House by Memorial Day, and is actively urging the Senate to assemble companion legislation. On January 29th, Senators Lamar Alexander (R-TN) and Richard Burr (R-NC) released a white paper report, which mirrors many of the topics addressed in the 21st Century Cures draft discussion bill. For more info and history on the 21st Century Cures Initiative, check out this [blog post](#) from the EveryLife Foundation. FasterCures has also [responded to the discussion draft](#).

NIH Funding Bills Galore

Since the start of the new Congress, a wide variety of proposals have been put forth to boost funding for the National Institutes of Health (NIH). One proposal, the Accelerating Biomedical Research Act, would exempt the agency from budget caps that were put in place to help control the deficit, which has stymied funding increases in recent years. Another legislative proposal would remove NIH from the annual funding process in Congress, enabling its budget to grow in proportion to the US GDP. While these are welcome legislative proposals, and it is encouraging to see Members making research a priority, all proposed bills lack bipartisan support at the time of this writing.

NDD United Letter Goes to Capitol Hill

As you may know, recent across-the-board budget cuts, commonly called sequestration, have harmed our nation's capacity to fund medical research at the National Institutes of Health (NIH) and vital services at the Food and Drug Administration (FDA). It gets worse; sequestration will take effect again this year unless Congress can pass legislation to reduce the deficit. A broad coalition of groups has formed to protect the ‘discretionary’ side of the federal budget – this is the portion that funds programs critical to the rare disease community at the NIH, FDA, CDC

and other agencies. To date, nearly 2,100 organizations have signed-on to the [letter](#) calling for a balanced approach to deficit-reduction and to finally end the threat of sequestration. This week, the coalition is sending their letter to Hill leadership.

Rare Disease Advocacy Events:

Hill Briefing: Pulmonary Fibrosis

Wednesday, February 25: Join speakers from the NIH, FDA, Boehringer-Ingelheim, the Pulmonary Fibrosis community, and NORD to learn about the scope and impact of pulmonary fibrosis, a deadly rare disease. The briefing will be held at Noon in 2103 Rayburn House Office Building. RSVP to Matt Gallivan matt.gallivan@mail.house.gov.

Hill Briefing: Urgent Healthcare Policy Needs of the Rare Disease Community

Thursday, February 26: Join patient advocates and congressional staffers for a lunch briefing addressing healthcare challenges in the rare disease community. The event is taking place at Noon in the Cannon Caucus Room (#345). Advocates and congressional staff are welcome to attend. [RSVP here](#).

Hill Briefing: The Future of Rare Bone Disease Research

Wednesday, March 18: Join the Rare Bone Disease Advocacy Alliance for a Capitol Hill briefing held in 121 Cannon House Office Building. The event will feature distinguished speakers from Johns Hopkins, the NIH, the Lymphangiomatosis & Gorham's Disease Alliance, Osteogenesis Imperfecta Foundation, and the OsteoPETrosis Society. For more information or to RSVP, contact kmulroy@wscdc.com.

Rare Disease Week is On Capitol Hill!

The RDLA team is in Washington DC this week as part of Rare Disease Week. The week will include several events including a film screening, legislative conference, lobby day, congressional briefing, and a visit to the National Institutes of Health. For a full schedule of Rare Disease Week events, see the [RDLA website](#).