



Breaking Legislative News

21st Century Cures Act (HR 6)

The vote on the historic 21st Century Cures Act (HR 6) has been delayed until after the July 4th holiday. Reports indicate that there remains some controversy over how to pay for some of the provisions in the legislation. Those involved have indicated that they are optimistic that those issues will be resolved. At the time of this writing, the bill has 180 bipartisan co-sponsors! Much of this is thanks to your advocacy efforts, but we need more co-sponsors if we want to ensure that this historic legislation passes the House. The Energy & Commerce Committee has setup a [clearinghouse for all information related to the 21st Century Cures Act](#).

Patient-Focused Impact Assessment Act Introduced in Senate (S. 1597)

Last week, Sens. Wicker (R-MS) and Klobuchar (D-MN) introduced the Patient-Focused Impact Assessment Act in the Senate. The bill requires the FDA to develop a patient engagement tool and also requires the FDA to disclose the resulting safety and effectiveness data of approved drugs. This bill builds on requirements from FDASIA and was championed by Parent Project Muscular Dystrophy (PPMD). For more information and to view the bill's press release, [click here](#).

Action Alerts

21st Century Cures Action Alert

From the EveryLife Foundation for Rare Diseases: Congress is on the verge of voting on a historic bill called the 21st Century Cures Act (HR 6) – a piece of legislation that could be a game changer for the rare disease community. The bill contains several provisions to incentivize the development of treatments for rare diseases, and holds the potential to double the number of treatments available to rare disease patients. In addition, the bill includes billions in new funding for critical research at the National Institutes of Health and new funding to accelerate drug approval at the Food and Drug Administration. But in order for this bill to pass the House, we must unite and make sure Congress gets our message loud and clear. [Contact your Representatives TODAY and ask them to co-sponsor the 21st Century Cures Act!](#)

FDA Funding Sign-On Letter

From the California Life Sciences Association: We must protect critical funding for the drug review divisions at FDA, which are under threat of budget cuts (sequestration) that could greatly hamper the drug approval process. Organizations are being asked to endorse legislation, FDA SOS Act HR 1078, to protect these vital funds. [Read the full letter here](#) – organizations that wish to sign-on may email Jenny Carey, jcarey@califesciences.org.

Events

Briefing on the Prescription Drug User Fee Act

On Tuesday, June 30th, BIO and PhRMA are holding an introductory briefing on the Prescription Drug User Fee Act (PDUFA) at 12:00-1:30pm at their offices located at 1201 Maryland Ave, SW Suite 900, Washington DC. This briefing will review progress made over the past 20 years in terms of greater predictability and

transparency in the drug and biologic review process and discuss the PDUFA VI reauthorization process and opportunities for stakeholders to provide input on FDA's drug review program. Please join leaders from the patient community and the biopharmaceutical industry to discuss the PDUFA reauthorization process and how patient and provider organizations can engage. Please RSVP to Gautami Inamdar at ginamdar@bio.org.

Precision Medicine Event at the NIH

On Wednesday, July 1st, the NIH is holding a workshop on the Precision Medicine Initiative from 8:00am - 5:00 pm at the John Porter Neuroscience Center in Bethesda, Maryland. The workshop will focus on the design of an inclusive cohort, building and sustaining public trust, direct-from-participant data provision, and effective and active participant engagement characteristics of a national research cohort of one million or more volunteers. For more information and to register, visit the [NIH's website](#).

RDLA's In-District Lobby Days Webinars

On Wednesday, July 8 and Wednesday, August 5th, RDLA is holding two webinars in preparation for our annual In-District Lobby Day. This is a prime opportunity to meet with your representatives in your own backyard and talk about policy issues that are critical to the rare disease community. We are offering two webinars to help advocates prepare for the event - we recommend tuning in for both. [Click here for register for the In-District Lobby Day event and webinars](#).

RDLA's Northeast Legislative Conference: New Jersey

On Monday, July 20th, join us in Newark, NJ at Rutgers for our first Regional Legislative Conference. The event will kick-off at 11:00am and feature Reps. Lance and Pallone, as well as panels on top policy issues for the rare disease community. We will also provide opportunities for advocates to engage in practice meetings to help them prepare for In-District Lobby Days in August. [Click here for more information and to register](#).

RDLA's West Coast Legislative Conference: California

On Friday, July 31st, join us in San Francisco, CA at UCSF for our Regional Legislative Conference. Registration will be held at noon and feature panels addressing top policy issues in the rare disease community. This is a perfect opportunity for advocates to learn more about policy and to engage in practice meetings to help them prepare for In-District Lobby Days. [Click here for more information and to register](#).

Now Seeking Nominations for RareVoice Awards Gala

On Wednesday, November 4th, RDLA will gather the rare disease community to recognize those who have made difference over the past year. We are seeking nominations for congressional staff, patient advocates and/or organizations, as well as government agency leaders for the prestigious awards. The deadline for nominations is Friday, July 31st. [For more information and full nomination details, visit this web page](#).

***This E-Blast shares action alerts, legislative, and policy news and events from the patient advocacy community. RDLA does not take positions on the issues herein, but serves as a supportive clearinghouse for the rare disease community. Send us an email if you'd like your alerts and/or events included! Email mbronstein@everylifefoundation.org