



## Breaking Legislative News

### 21st Century Cures Heading to House Floor, Major Progress for Rare Disease Patients

In the past two weeks, the momentous 21st Century Cures legislation has been enhanced and passed out of subcommittee and full committee (by a unanimous vote of 51-0)! This bipartisan legislation is a major accomplishment for Chairman Upton (R-MI) and Representative DeGette (D-CO), both of whom led the initiative. The bill (HR 6) includes a variety of provisions of importance to the rare diseases community. The inclusion of these provisions is a direct result of your hard work and concerted advocacy:

- OPEN ACT (repurposing existing drugs for rare indications)
- Expanding Hope Act (Priority Review Vouchers)
- CURE Act (Expanded Access)
- Neurological Disease Surveillance Network
- Patient Focused Drug Development
- New funding for the NIH & FDA

Looking forward, the 21st Century Cures legislation is expected to be brought to the House floor and voted on prior to the July 4th recess. For the full bill and summary, please visit the [E&C committee website](#). Please stay tuned for additional action alerts; we may need to call on the advocacy community once again to ensure that the bill clears the final hurdle in the House. Meanwhile, the Senate continues its work on the companion legislative effort, now known as Innovation for Healthier Americans. This effort is not as developed as the House legislation, but we are hopeful that the Senate will take swift action if the bill clears the House. [Click here to view the PowerPoint from the recent RDLA meeting](#), which has more information provisions of interest to the rare disease community.



By special invitation, rare disease advocates Lisa and Max Schill (age 6) of New Jersey were invited to the markup and had the chance to meet with several of the E&C Members (see photos below). Max has Noonan syndrome, a rare genetic disorder that is one of [several types of RASopathies](#).

### Sens. Hatch (R-UT) & Klobuchar (D-MN) Introduce the OPEN ACT in the Senate

On Thursday, May 21, Sens. Hatch and Klobuchar took a bold stance on behalf of rare disease patients by [introducing](#) the OPEN ACT - S. 1421 (Orphan Products Extension Now, Accelerating Cures & Treatments). The introduction of the bill came on the same day that the House Energy & Commerce Committee unanimously approved the 21st Century Cures legislation. The

timing bodes well for the OPEN ACT to be included in the Senates' initiative, Innovation for Healthier Americans, a companion effort to the bill in the House. [Ask your Senators to support the OPEN ACT.](#)

### **Sens. Graham (R-SC) & Durbin (D-IL) Found NIH Caucus**

Sens. Graham and Durbin have launched a caucus for the National Institutes of Health (NIH). The bipartisan caucus will serve as a platform to raise awareness about a variety of the challenges facing the agency. In particular, funding constraints resulting from a decade of near flat-funding and the budget cuts imposed by sequestration. In addition, the Caucus may also do more to highlight the challenges facing young and emerging researchers, many of whom struggle to obtain funding.

### **Members of Congress Speak at RDLA Hill Briefing on 21st Century Cures**

On Thursday, May 21st, three Members of Congress spoke at the Rare Disease Congressional Caucus briefing: 21st Century Cures - Priorities for the Rare Disease Community. We are so grateful to Chairman Upton (R-MI) as well as Reps. Bilirakis (R-FL), and Lance (R-NJ) for joining the briefing and speaking about the 21st Century Cures Initiative and its importance to rare disease patients. [Click here to see video of their remarks.](#) Panelists included representatives from NORD, Research!America, FasterCures, Kids v. Cancer, BioCentury TV, and the EveryLife Foundation for Rare Diseases. Stay tuned for a full video of the briefing, which we will share on the [RDLA website](#) and on [Facebook](#).

### **Action Alerts:**

#### **Ask Your Senators to Cosponsor the OPEN ACT S. 1421**

From the EveryLife Foundation for Rare Diseases: Now that the OPEN ACT has hit the ground running in the Senate, we need additional Senators to sign-on in support of the legislation. A strong base of bipartisan support will be crucial as the Senate advances their Innovation for Healthier Americans legislation. Remember, the OPEN ACT has the potential to double the number of rare disease treatments available to patients, and has been endorsed by 155 patient organizations. [Take action now and ask your Senators to cosponsor the OPEN ACT.](#) You may find [more information about the OPEN ACT here.](#)

### **Policy Events:**

On Monday, June 1st, **Research!America is convening a conference call** to discuss various patent reform bills which could impact drug development for rare diseases. For additional background, see this [sign-on letter from the patient community](#). To join the call, RSVP to Ellie Dehoney [edehoney@researchamerica.org](mailto:edehoney@researchamerica.org). The call in number is (877)875-7554 and the code is 6168580.

On Monday, June 1st, **the American Cancer Society Cancer Action Network** will host a precision medicine event in Washington, DC at the Hyatt Regency. The event will include a panel of distinguished speakers who will address some of the tools needed for precision medicine. [Learn more and register here.](#)

On Wednesday, June 3rd, **Eli Lilly's Digital Advocacy Institute is holding a webinar** - Being Effective At-Home: Virtual Lobby Day and In-District Meetings. In this webinar, learn about how to reach your representative in their home district. [Click here for more information and to sign-up.](#)

On Thursday, June 11, **FasterCures is convening a meeting:** Partnering with Patients on Value, Coverage, & Reimbursement. The meeting is being held at 8:30am at the Milken Institute School of Public Health at George Washington University in Washington, DC. [Click here for more information and to register.](#)

On Wednesday, June 17th, **RDLA will hold its monthly webinar**, which will focus on the 21st Century Cures legislation. The agenda is open so if there are topics you'd like to see covered please email

[arussell@everylifefoundation.org](mailto:arussell@everylifefoundation.org). [Click here to RSVP for the meeting](#).

Mark your calendars! On Monday, July 20th, **RDLA will hold its first Regional Legislative Conference at Rutgers** in New Jersey. On Thursday, July 31st, RDLA will convene another Regional Legislative Conference at UCSF in San Francisco. These events will serve as an opportunity for advocates to learn about the top policy issues in the rare disease community and to help prepare advocates to participate in our upcoming In-District Lobby Day event as well. During the month of August, Members of Congress will be back home in their districts. This is a perfect opportunity to get to know your representative and educate them about the various policy issues in the rare disease community. **RDLA will be sponsoring in-district meetings** during this period to give you a chance to meet with your Members right in your district! [Registration will open June 15th](#).

On Wednesday, November 4th, **RDLA will hold its annual Rare Voice Awards Gala** in Washington D.C. We are currently seeking nominations in three categories: Congressional Staff, Patient Advocates or Organization, and Government Agency Leaders. Please take a moment to [visit our nominations page and nominate](#) those who have worked so hard on behalf of the rare disease community.

\*\*\*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](mailto:mbronstein@everylifefoundation.org)