



## Headline News

### [21<sup>st</sup> Century Cures Draft Legislation Released](#)

The powerful Energy & Commerce Committee has released a draft of the much-anticipated 21<sup>st</sup> Century Cures legislation. The draft addresses a wide-variety of issues of interest to the rare disease community, including expanded access policies, drug repurposing and exclusivity extensions (OPEN ACT), patient focused drug development, and a host of other medical issues. Already, some dissent has emerged from the Democrats who claim their issues were not included in the bill. We will continue to closely track and monitor changes to the legislation and alert advocates for action opportunities. You may view the full bill and summary [here](#). FaegreBD consulting has also released an [analysis](#) of what the path forward might look like.

### [Cystic Fibrosis in the State of the Union](#)

As many of you no doubt heard, the President's address highlighted the work of one outstanding advocate, Bill Elder Jr., on behalf of the cystic fibrosis community. During the speech, Bill was invited to sit next to First Lady Michelle Obama in recognition of his efforts. In collaboration with Vertex Pharmaceuticals Inc., the Cystic Fibrosis Foundation was able to play a major role in financing the research that enabled a new treatment for the deadly disease.

## Community Action Alerts

### [Deadline Approaching, Sign-On to Support the OPEN ACT](#)

The EveryLife Foundation for Rare Diseases is calling for organizations to sign-on support the [OPEN ACT](#), legislation to incentivize repurposing of existing drugs for rare disease indications. To date, over 100 organizations have signed-on! Support in Congress is growing as well - the Energy & Commerce Committee has included this

legislation as part of the broader, 21<sup>st</sup> Century Cures package. Read the [letter to Congress](#) and view the action alert [here](#). To sign-on, send an email to Max Bronstein [mbronstein@everylifefoundation.org](mailto:mbronstein@everylifefoundation.org). Signing deadline is Friday, January 30th.

### [Sign-on to support the Advancing Research for Neurological Diseases Act HR 292](#)

Reps. Burgess (R-TX) and Van Hollen (D-MD) have introduced HR 292, the Advancing Research for Neurological Diseases Act of 2015. The bill would create a national data collection system at the Centers for Disease Control (CDC) focused on neurological diseases, including Parkinson's disease. It is estimated that between 500,000 to 1.5 million Americans are living with Parkinson's, but this lack of understanding inhibits Parkinson's research, programs, and services. The Parkinson's Action Network (PAN) strongly supports this legislation. Similar legislation passed unanimously in the House of Representatives in 2010 but stalled in the Senate – PAN need's your help! Learn more [here](#) and click [here](#) to take action.

### [Congress Needs to Hear from You: Submit Your Patient Stories](#)

Rare Disease Week is just a few weeks away! For those who are unable to attend this year, please send us your stories so we can hand deliver them to your Members of Congress on your behalf. Click [here](#) to complete the form, and feel free to email Andy Russell [arussell@everylifefoundation.org](mailto:arussell@everylifefoundation.org) should you have any questions.

### [Sign-on to Protect Incentives for Medical Innovation](#)

The Global Colon Cancer Association and the Information Technology and Innovation Foundation are leading an effort on a national medical innovation declaration to be delivered to U.S. government leaders, highlighting the importance of robust incentives to encourage investment in R&D for life-saving biopharmaceuticals. Click [here](#) to sign-on.

### Upcoming Events

#### February 23-27: Rare Disease Week is right around the corner!

We are less than one month away from this year's Rare Disease Week on Capitol Hill. Have you [registered](#) yet? Rare Disease Week (Feb 23-27) is a five day event aimed at empowering the rare disease community. If you would like more information, or have any questions about all the events, we have scheduled an

informational webinar for Jan. 28th.

Additionally, there are still travel scholarships available to advocates from the following states: Alaska, Arizona, Arkansas, Hawaii, Idaho, Iowa, Kentucky, Maine, Mississippi, Missouri, Montana, Nebraska, New Hampshire, New Mexico, North Dakota, South Carolina, South Dakota, Utah, Vermont, West Virginia, and Wyoming. Please click [here](#) to apply.

#### [February 26: Rare Disease Congressional Caucus Briefing](#)

Mark your calendars for the next Rare Disease Congressional Caucus briefing, being held on Capitol Hill from noon-1:30pm on Thursday, February 26 (room TBA). The briefing will address the urgent health care policy needs of the rare disease community - click [here](#) to register.

#### [February 18: Rare Disease Week Webinar](#)

The next Rare Disease Week Webinar will be held on Wednesday, February 18 at 2pm (Eastern time). The webinar will provide an overview of Rare Disease Week and provide participants the opportunity to ask any questions they may have. Click [here](#) to register.

#### [March 3rd: Blue Skies & Brickwork Health Policy Summit](#)

Novartis is holding its 10th annual Blue Skies & Brickwork Health Policy Summit on Tuesday, March 3rd, in Washington D.C. from 8:30am-2:00pm. The event will take place at the Newseum, located at 555 Pennsylvania Ave. NW, DC. You may RSVP and view the event summary [here](#).

#### [January 29: Scoring Progress Against Cancer](#)

A new tool called PACE Continuous Innovation Indicators will help inform policymakers about the impact of cancer innovation. Keynote will feature John C. Lechleiter, CEO of Eli Lilly and Company. The event will take place at the Knight Conference Center at the Newseum, 555 Pennsylvania Ave, NW DC, on Thursday, January 29 from noon-2:45pm. RSVP to [rsvptocll@togorun.com](mailto:rsvptocll@togorun.com).