



## RDLA February 2020 Newsletter

**Reminder: Registration for Rare Disease Week on Capitol Hill is OPEN!** Register for Rare Disease Week [here](#).



## CAPITOL HILL UPDATES

**International Pricing Index:** The Trump Administration proposed the International Pricing Index for drugs in 2018 and continues to be promoted by the Administration and Congressional proposals. The International Pricing Index proposal is intended to lower drug prices in the United States by setting certain drug prices on an international benchmark. The International Pricing Index would be based on the prices of drugs in 14 other countries. Many in the rare disease community are concerned that an International Pricing Index would limit access to existing and future therapies especially therapies for rare diseases. Additional information can be found on the EveryLife Foundation's [website](#).

**Medicaid Block Grants Announcement:** On Thursday, January 30<sup>th</sup>, the Trump Administration announced a plan to allow states the option to convert Medicaid funding into block grants. With block grants, states would have the ability to limit health benefits and drugs and set premiums.

Currently, Medicaid programs must cover all Food and Drug Administration (FDA)-approved drugs. Under the new guidance, state Medicaid programs can establish their own formularies (a list of covered drugs). The states with block grants would be able to make new cuts to benefits, including determinations around which prescription drugs are covered, and implement new out-of-pocket costs to beneficiaries while still receiving the Medicaid program's guaranteed drug rebates. Under the Medicaid block grants, states would be required to cover one drug in each category or class (or more) as well as "substantially all" antipsychotics and antidepressants, HIV drugs, and opioid treatment medications. Medicaid advocates are concerned that block grants

would limit states' abilities to respond to economic downturns and expensive new drugs, leading to cuts in Medicaid.

**Orphan Drug Act:** Recently, the House Energy and Commerce Committee held a hearing which opened discussion on the Orphan Drug Act (ODA). The hearing highlighted that education on the value of ODA to Members of Congress and decision-makers is still very much needed. The EveryLife Foundation for Rare Diseases created an ODA fact sheet that you can find [here](#).



## COMMUNITY ACTION ALERTS & POLICY RESOURCES

EveryLife Foundation for Rare Diseases is asking advocates to urge their Members of Congress to reject dangerous **International Pricing Index proposals** that could impact the availability of already scarce treatments and quality of care. To take action, click [here](#).

Kids v. Cancer is asking organizations to sign on in support of the **Creating Hope Reauthorization Act**. The Creating Hope Reauthorization Act will permanently reauthorize FDA priority review vouchers (PRVs) for rare pediatric diseases. The PRV is a pediatric rare disease therapy development incentive program in which companies developing products for children with cancer and other life-threatening diseases may be eligible to receive a voucher from FDA that can be applied to the review for a future product that does not meet this same criteria. If your organization would like to officially support the Creating Hope Act, please sign on [here](#).

National Foundation for Ectodermal Dysplasias is asking patient advocacy organizations to sign a letter in support of the **Ensuring Lasting Smiles Act**. To sign on, visit the website [here](#). NFED is also asking advocates to send emails to their legislators to ask them to co-sponsor the Ensuring Lasting Smiles Act. Please visit [here](#).

National PKU Alliance is asking advocates to contact their Members of Congress in support of the **Medical Nutrition Equity Act, H.R. 2501**. H.R. 2501 would provide coverage of medically necessary foods and vitamins for digestive and inherited metabolic disorders under federal health programs and private health insurance. To contact your Representative to cosponsor H.R. 2501, click [here](#).

National Society of Genetic Counselors is asking advocates to contact their Members of Congress in support of the **Access to Genetic Counselors Act, H.R. 3235**. H.R. 3235 would expand coverage of services provided by genetic counselors under the Medicare program. To contact your Representatives to cosponsor H.R. 3235, click [here](#).

Aidan Jack Seeger Foundation is asking organizations to sign onto a letter in support of **Aidan's Law, H.R. 534**, to make newborn screenings of MPS1, Pompe, ALD, and SMA available nationwide. To view and sign on to the letter, click [here](#).

EveryLife Foundation for Rare Diseases is asking advocates to contact their Members of Congress in support of the **Advancing Access to Precision Medicine Act**. This legislation would ensure that many children and young adults living with an undiagnosed condition will have access to DNA sequencing clinical services beyond Whole Genome Sequencing that are currently out of reach. To contact your Representatives to cosponsor H.R. 4393, click [here](#).



## COMMUNITY EVENTS

*Below are upcoming policy and advocacy events of interest to the rare disease community. To view more policy and advocacy events for this year and beyond, please visit the [RDLA events calendar](#).*

**Two Upcoming Rare Disease Week Webinars:** The Deep Dive Webinar on Rare Disease Week will take place on February 13, 2020. The webinar will cover an overview of the events, travel stipends, and the Legislative Conference and include time for questions. Register to join the Deep Dive Webinar [here](#). The First Time Attendees Orientation Webinar will be held on Thursday, February 20, 2020 at 1 pm EST. This webinar will review important information and answer last minute questions. Register to join the First Time Attendees Orientation Webinar [here](#).

**Rare Disease Week on Capitol Hill, February 25-28, 2020:** RDLA will bring hundreds of rare disease patient advocates to Washington, DC for a week of events dedicated to empowering patients, families, and friends to become legislative advocates. Advocates will have an opportunity to meet with Members of Congress and learn best practices for successful advocacy. Register for the week's events [here](#). Find more information on Rare Disease week at [www.rareadvocates.org/rdw](http://www.rareadvocates.org/rdw).

The full list of events during Rare Disease Week can be found [here](#).

On February 24<sup>th</sup>, the Food and Drug Administration (FDA) is holding a Rare Disease Day 2020 public meeting. Register to attend in-person or livestream [here](#). On February 28<sup>th</sup>, as part of Rare Disease Week on Capitol Hill, the NIH will host Rare Disease Day at NIH. This event aims to raise awareness about rare diseases, the people they affect and NIH research collaborations to advance new treatments. Register for this event [here](#).

**FDA Public Workshop:** The Center for Biologics Evaluation and Research (CBER) at the Food and Drug Administration (FDA) announced a public workshop entitled "Facilitating End-to-End Development of Individualized Therapeutics" on March 3, 2020. The purpose of the public

workshop is to foster development of individualized therapeutic products for the treatment of one individual or a very small number of patients, based on engineering a product aimed at the specific molecular mechanism underlying a patient's (or small group of patients') illness. For more information and to register, [click here](#). For information on available travel stipends provided by the EveryLife Foundation, contact [svonfelden@everylifefoundation.org](mailto:svonfelden@everylifefoundation.org).

**Capitol Crawl Commemorative Rally:** The Pain Advocate Warriors is organizing a rally for Medical Freedom and Quality of Life on May 15, 2020 at the Capitol. For more information, visit [here](#).

**Rally for Medical Research Hill Day:** Save the date for the Rally for Medical Research Hill day on Thursday, September 17, 2020, with the "Rally Hill Day" reception taking place during the evening of Wednesday, September 16, 2020. To learn more about the Rally for Medical Research, [click here](#).

### **Stay Connected**

Stay up to date on breaking rare disease legislative news by following @RareAdvocates on [Twitter](#) and [Facebook](#) and rare\_advocates on Instagram.

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\*\*\*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 [svonfelden@everylifefoundation.org](mailto:svonfelden@everylifefoundation.org).