



RDLA January 2020 Newsletter

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



CAPITOL HILL UPDATES

Appropriators Reach Deal on Funding Bill: On December 16, 2019, House Appropriations Committee Chairwoman Nita Lowey (NY) released the text of two legislative packages that included all 12 fiscal year 2020 funding bills and extensions of numerous healthcare and other programs. This legislation, H.R. 1865, includes \$41.7 billion for the National Institutes of Health (\$2.6 billion increase), \$8 billion for the Centers of Disease Control and Prevention (CDC) (\$636 million increase), \$3.1 billion in discretionary funding for the Food and Drug Administration (\$91 million increase), \$17 million for the CDC’s Newborn Screening Quality Assurance Program (\$1 million increase), and \$17.8 million for Health Resources and Services Administration’s Heritable Disorders program (\$1.5 million increase). This bill passed the House and Senate and was signed into law by the President on December 20, 2019.

GAO Study on Economic Burden of Rare Diseases: Language was included in H.R. 1865, and passed into law on December 20, 2019, to require the Government Accountability Office (GAO) to conduct a study on the economic costs of untreated and undiagnosed rare diseases. The study will be provided no later than two years from the date of enactment. The inclusion of this language was led by the EveryLife Foundation for Rare Diseases.

Hearing on the Ensuring Lasting Smiles Act: The House Energy and Commerce Committee held a hearing titled “Legislation to Improve Americans’ Health Care Coverage and Outcomes” on January 8, 2019. The hearing included testimony on the Ensuring Lasting Smiles Act. To learn more, read the testimony, and watch a recording, visit [here](#).



COMMUNITY ACTION ALERTS & POLICY RESOURCES

The EveryLife Foundation and other patient community organizations are seeking the support of fellow patient advocacy community organizations wishing to join in offering support for existing Hill proposals that include **provisions for best price/AMP exemptions**. In recent months, there has been significant Hill activity to create legislative fixes to existing barriers in implementing innovative payment solutions for the emerging transformative & gene therapies. While many coalition groups have been active on these issues, they have not included strong patient community voices and thus the Hill has asked that we specifically weigh in on the need for legislation to allow for outcomes-based agreements for innovative and gene therapy. This proposed legislative solution establishes a flexible framework that would enable biopharmaceutical companies and payers to develop customized approaches that ultimately foster patient access. To add your organization to those supporting this Hill outreach, please indicate your interest in [signing the letter](#) by emailing Steve Silvestri at the EveryLife Foundation by Friday, January 31st at ssilvestri@everylifefoundation.org.

The Friends of PCORI Reauthorization is asking advocates to contact their Members of Congress in support of the **Patient Centered Outcomes Research Institute (PCORI)** reauthorization. PCORI supports research to help patients and providers make evidence-based healthcare decisions. To find sample letters, 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](#).

The 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](#).

The National PKU Alliance is asking advocates to contact their Members of Congress in support of the **Medical Nutrition Equity Act, H.R. 2105**. H.R. 2105 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. 2105, 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](#).

The 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](#).

The EveryLife Foundation for Rare Diseases is circulating a sign on letter to support 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. Please contact Steve Silvestri (ssilvestri@everylifefoundation.org) if your patient organization would like to sign.



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](#).

Informational Webinar on Rare Disease Week will take place on January 16, 2020. The webinar will cover an overview of the events, travel stipends, and the Legislative Conference and include time for questions. Register to join on the [RDLA website](#).

Expanded Access Summit: Wide Trial invites patient advocates, industry, academia and nonprofits to the Third Annual Expanded Access Summit at the National Press Club in Washington, DC on January 27-29th. This Summit is free to patients. Learn more [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](#).

On February 24th, the Food and Drug Administration (FDA) is planning a Rare Disease Day public meeting. Please keep an eye out for communications from FDA on this event. On February 28th, 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](#).

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](#).

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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.