RDLA November 2019 Newsletter

Capitol Hill Updates

FY2020 Appropriations: On October 31st, the Senate passed a package of four appropriations bills including Agriculture which contains funding for the Food and Drug Administration. The Senate also took a procedural vote (which was rejected) on a second appropriations package that included Labor, Health and Human Services, and Education Appropriations.

On September 27, 2019 just prior to the end of the fiscal year, the President signed a continuing resolution (CR) into law to extend government funding and avoid a government shutdown. The CR extends the FY2019 funding through November 21st. The House and Senate will need to either pass the FY 2020 appropriations bills before November 21st or another continuing resolution to avoid a government shutdown again. Under the continuing resolution, government agencies will carry out their program using the funding levels from last year. For example, this will limit FDA's ability to start new initiatives until the increased funding levels for FY2020 are enacted.

Drug Pricing Legislation Moves Forward in the House: On October 17, 2019, the full House Energy and Commerce Committee voted to advance H.R. 3, the Lower Drug Costs Now Act. This proposal, led by House Speaker Nancy Pelosi, includes provisions that would allow the federal government to negotiate drug pricing and tie prices more closely to those set internationally. You can learn more about the bill's intentions here and watch the full markup here. The California Life Sciences Association (CLSA) also examined how this proposal could impact drug development here.

Pediatricians Accelerate Childhood Therapies Act, H.R 4519: Representatives Eliot Engel (NY-16) and Cathy McMorris Rodgers (WA-5) introduced H.R. 4519 on September 26, 2019. H.R. 4519 would authorize the National Institutes of Health (NIH) to award grants to scientists to support researchers focusing on pediatric research. In addition, H.R. 4519 would urge NIH to consider opportunities to align pediatric research topic areas including childhood cancer, precision medicine, rare diseases, and others.

Biomedical Innovation Expansion Act, H.R. 4667: Representative Mikie Sherrill (NJ-11) introduced H.R. 4667 on October 11, 2019. H.R. 4667 would reauthorize NIH innovation projects with funding of $10 billion over 10 years. The funding levels would include $3 billion

for a Precision Medicine Initiative, $1.5 billion for the Biden Cancer Moonshot, and $758 million to support research on rare diseases.

Community Action Alerts & Policy Resources

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.

Genetic Alliance is asking organizations to write letters in support of the **Ending the Diagnostic Odyssey Act, H.R. 4144**. H.R. 4144 would allow states to conduct a three-year pilot program to increase the Federal Medical Assistance Percentage (FMAP) rate to provide Whole Genome Sequencing clinical services for children on Medicaid with a disease that is suspected to have a genetic cause. If your organization would like to send a letter in support of H.R. 4144, please email Vilma Whittier at vwhittier@geneticalliance.org.

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.

To learn more about the bills, please watch the recording of the September RDLA Webinar.

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.

Alliance for a Stronger FDA Webinar: The Alliance for a Stronger FDA is hosting a webinar on the FDA Appropriations on November 6th, 1-2 pm. Register here.

FDA Public Workshop: The FDA is hosting a public workshop, "Advancing the Development of Pediatric Therapeutics (ADEPT 6): Pediatric Clinical Trial Endpoints for Rare Diseases with a Focus on Pediatric Patient Perspectives" on November 12th from 8 am to 4:30 pm. For more information and to register, click here.

Fall 2019 Policy Consortium: National Patient Advocate Foundation is hosting the Fall 2019 Policy Consortium, *Trust Your Patient. Trust Your Provider. Building Trust to Reduce Health Disparities* on Wednesday, November 13, 2019 from 8:30 am-2:30 pm at the Newseum Knight Conference Center. Register here.

Thyroid Eye Disease Lunch Briefing: The Alliance for Eye and Vision Research is hosting a lunch briefing on Capitol Hill on November 14th at 12 pm in Rayburn 2075. To register, email dinaabeau@aol.com.

RDLA November Webinar and In-Person Meeting: The next RDLA Monthly Webinar and In-Person Meeting will take place on November 21, 2019. The RDLA meetings are attended either through a webinar or in person in Washington, DC. Advocates, staffers, and industry are welcome to join. Register to join on the RDLA website. If you would like to present at the meeting about a current policy issue, please email Shannon von Felden at svonfelden@everylifefoundation.org.

The Next Generation of Value Assessment: Including the Patient Voice: PhRMA Foundation and National Health Council is hosting an event to inform stakeholders on useful tools and new approaches on value assessments on Tuesday, November 12 from 10:00 am - 4:00 pm ET in Washington, DC. Register here.

Lunch and Learn Series: A Primer on CBD: American Brain Coalition and Bridge the Gap Syngap are inviting patients and patient organizations to attend a lunch and learn about the

emerging legislative and regulatory environment for cannabidiol-derived products and the potential impact on patients, clinicians, and researchers. The lunch and learn will take place on November 14, 2019 at noon in Washington, DC. Learn more here.

Rare Disease Caucus Briefing: Rare Disease Legislative Advocates in coordination with the Rare Disease Congressional Caucus is hosting a rare disease lunch briefing on the Economic Burden of Rare Diseases on December 4, 2019 at 11:30 am. Register to attend here.

RareVoice Awards: Please register for the RareVoice Awards on December 4, 2019. The RareVoice Awards is an event to educate Congress on rare disease issues and celebrate advocates who give rare disease patients a voice on Capitol Hill and state legislatures. Learn more at www.rareadvocates.org/rarevoice-awards. To register, click here.

Expanded Access Summit January 27-29, 2020 easummit.net 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.

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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.