



EMPOWERING THE RARE DISEASE COMMUNITY

A PROGRAM OF THE EVERYLIFE FOUNDATION FOR RARE DISEASES

RDLA March 2019 Newsletter

Capitol Hill Updates

ALD Newborn Screening Bill Introduced: H.R. 534, also known as Aidan's Law, would provide funding for states to screen for ALD in newborns. This bill was named for Aidan Jack Seeger whose life was taken by adrenoleukodystrophy (ALD) at age 7. Unfortunately, those affected by this fatal brain disease are often left undiagnosed for years. Early diagnosis through newborn screening allows those affected to access life-saving treatment before it is too late.

Ensuring Lasting Smiles Act: Senators Baldwin and Ernst and Representatives Peterson and Riggleman reintroduced the Ensuring Lasting Smiles Act in February. ELSA would assure that individuals born with congenital anomalies receive health benefits for the medically necessary treatments they need. ELSA would require all groups and individual health care plans to cover procedures to restore or repair normal body function for any missing or abnormal body part, including teeth. It closes a loophole that allows health care plans to deny such procedures as “cosmetic”.

Lymphedema Treatment Act: Senator Cantwell reintroduced the Lymphedema Treatment Act, S. 518, in the Senate with 41 cosponsors in February. The Lymphedema Treatment Act would provide Medicare coverage for compression items used to treat lymphedemas as durable medical equipment.

Rare Disease Congressional Caucus Update: We are excited to share the Rare Disease Congressional Caucus has two new co-chairs in the 116th Congress. Senator Roger Wicker of Mississippi joined Senator Amy Klobuchar of Minnesota as the Senate co-chairs and Representative Gus Bilirakis joined Representative G.K. Butterfield as the House co-chairs. We look forward to continuing to work with the Rare Disease Congressional Caucus and the co-chairs to give the rare disease community a voice on Capitol Hill.

Rare Disease Week on Capitol Hill 2019: In February, over 800 rare disease patients, caregivers, family members, and industry representatives attended events as part of the Rare Disease Week on Capitol Hill. Rare Disease Legislative Advocates is proud of the 435 rare disease advocates who met with 298 Members of Congress and their staff on Capitol Hill! Learn more about this exciting week [here](#) and save the date for 2020: February 24-28, 2020!

Community Events

Public Workshop on Enhancing the Incorporation of Patient Perspectives in Clinical Trials: Clinical Trials Transformation Initiative will convene a public workshop, "Enhancing the Incorporation of Patient Perspectives in Clinical Trials" in collaboration with the FDA on Monday, March 18, from 9:00 a.m. to 5:00 p.m. in Silver Spring, MD.

The workshop will seek ideas for best practices and key considerations for enhancing the incorporation of patient perspectives on clinical trial access, design, conduct, and post-trial follow-up. It will also gather input from patients, caregivers, industry, academic researchers, and expert practitioners on the challenges and barriers to patient participation in clinical trials. Register for the workshop by March 11th [here](#).

RDLA Monthly Webinar and In-Person Meeting, March 21, 2019: The next RDLA Monthly Webinar and In-Person Meeting will take place on Thursday, March 21st. The RDLA Monthly Meetings are an opportunity to educate patient advocates about pressing health policy topics so that they can be successful legislative advocates. The 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.

FDA Meeting on Patient Perspectives on the Impact of Rare Diseases: Bridging the Commonalities: The Food and Drug Administration announced a public meeting on April 29, 2019 at the FDA White Oak Campus and an opportunity for public comment on "Patient Perspectives on the Impact of Rare Diseases: Bridging the Commonalities." This public meeting is intended to obtain patients' and caregivers' perspectives on impacts of rare diseases on daily life and to assess commonalities that may help the Agency and medical product developers further understand and advance the development of treatments for rare diseases. The goal of this meeting is to identify common issues and symptoms in rare diseases to help advance medical product development.

Register to attend the event in-person or via webcast at [Eventbrite](#). If you need special accommodations, please contact Eleanor Dixon-Terry at 301-796-7634 or Eleanor.Dixon-Terry@fda.hhs.gov by April 15, 2019. Comments can be submitted to the public docket by May 30, 2019. Submit electronic comments to [Regulations.gov](#). Submit written comments to the Division of Docket's Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. All comments must be identified with the docket number FDA-2019-N-0077.

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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.