WHAT’S HAPPENING

RARE DISEASE COMMUNITY CALLS ON CONGRESS & FDA TO ENACT LIFE-SAVING PUBLIC POLICY SOLUTIONS

Hundreds of rare disease advocates from around the country were recently brought to Washington, D.C. by the Rare Disease Legislative Advocates (RDLA), a program of the EveryLife Foundation for Rare Diseases (ELF). The group gathered in the nation’s capital to implore Congress and the U.S. Food & Drug Administration (FDA) to take actions that will change the face of rare disease diagnosis and treatment forever.

“More than 30 million Americans are diagnosed with a rare disease, yet 95% of these rare diseases have no FDA approved therapies. This is unacceptable. The enactment of common-sense legislation is the only way to address this public health crisis and ensure that all rare disease patients have access to early diagnosis and lifesaving treatments,” said Julia Jenkins, Executive Director of ELF.

RDLA presented the RareVoice Awards to honor individuals who have demonstrated leadership in advocating for federal and state public policy solutions that benefit rare disease patients. Among those honored: United States Senator Roger Wicker (R-MS) and Representative Lucille Roybal-Allard of California (D-40).

“Every life matters and every voice deserves to be heard. We are here to ensure Congress and the federal agencies understand it is within their power to put into place life-saving solutions, giving every person with a rare disease the opportunity for safe, effective and affordable treatments,” concluded Ms. Jenkins.

Rare Disease Week on Capitol Hill brings rare disease community members from across the country together to be educated on federal legislative issues, meet other advocates, and share their unique stories with legislators.

In 2020, the week of events will start with the Rare Disease Congressional Caucus briefing, which will convene policy experts and rare disease stakeholders to educate Congressional staff and the public on issues of importance to the rare disease community.

All events are free for patients, caregivers and other advocates but advance registration is required for each event. Please sign up for our email list at https://everylifefoundation.org/newsletter-signup or check RDLA on Twitter, Facebook, and Instagram for updates.

SHOW OF STRENGTH: Rare disease community members from across the country gather at the Ronald Reagan Building during Rare Disease Week on Capitol Hill to be educated on federal legislative issues, meet other advocates, and share their unique stories with legislators.

ABOUT THE EVERYLIFE FOUNDATION FOR RARE DISEASES:

The EveryLife Foundation for Rare Diseases is a 501(c)(3) nonprofit, nonpartisan organization dedicated to advancing the development of treatment and diagnostic opportunities for rare disease patients through science-driven public policy. The Foundation does not speak for patients, but instead provides the training, education, resources and opportunities to make patient voices heard. By activating the patient advocate, the Foundation believes it can change public policy and save lives.
WHAT’S HAPPENING
EVERYLIFE FOUNDATION IDENTIFIES PRIORITIES IN POLICY AGENDA

300+ rare disease patients and advocates were in Washington, D.C. to advocate for policies that will ease the physical, emotional and financial toll taken on patients by more than 7,000 rare diseases. At the recent Rare Disease Congressional Caucus Briefing, ELF outlined its public policy agenda, including several key priorities:

• Calling on Congress to pass the Newborn Screening Saves Lives Reauthorization Act (H.R. 2507 & S. 2158) (https://everylifefoundation.org/newborn-screening), that supports the screening of newborn babies in all 50 states for rare diseases. Currently, this legislation has expired, putting the health of newborn babies through America’s most successful public health program at risk. The need for quick passage of the Newborn Screening Saves Lives Reauthorization Act, is epitomized by the story of Erica and Chloe Barnes. Erica lost her daughter, Chloe, to a rare disease. Had newborn screening been available to Chloe, her rare disease would have been detected at birth and treatment made available immediately. Instead, her mom faced heartbreak, a delayed diagnosis, and medical system unequipped for her family. She has since become a tireless advocate for newborn screening (www.minnesotaalumni.org/stories/university-to-form-rare-disease-advisory-council) and efforts to remove other barriers to proper healthcare through her work at the University of Minnesota.

• Urging the FDA to establish an FDA Rare Disease Center of Excellence (https://everylifefoundation.org/fda-center-excellence) as authorized by Congress’ passage of the 21st Century Cures Act in 2016. The Center will help advance the pharmaceutical treatment pipeline for rare diseases, especially those (93%) that currently have no FDA approved treatment options.

• The launch of a first-of-its-kind project to measure the real economic impact that living with a rare disease has on patients, caregivers, healthcare providers, insurers, employers, schools, and other stakeholders. Results will be published in 2020, then shared with Members of Congress to help ensure that all cost considerations are factored into critical evidence determinations and public policy decisions.

Two other patient stories highlight the need for common-sense legislation:

• Isabel Bueso, a native of Guatemala, came to the U.S. to participate in a clinical trial for mucopolysaccharidosis type VI (MPS VI), and for six years has become an advocate for rare diseases during treatment. Due to a change in federal immigration policy that eliminates the ability of patients from outside the U.S. to remain in America during clinical treatment, she is currently facing deportation. Courageous and undaunt-

ed, Isabel is working with members of Congress to seek a permanent solution for her and all rare disease patients affected by the new policy. Read more about Isabel’s journey at https://everylifefoundation.org/everylife-foundation-statement-on-uscis-action-on-deportation-requests-impacting-rare-disease-patients

• Aidan Abbott, a 14-year-old teen from Slinger, Wisconsin, is working with Senator Tammy Baldwin (D-WI) to pass the Ensuring Lasting Smiles Act (www.baldwin.senate.gov/press-releases/ensuring-lasting-smiles-act-2019). Aidan was born with ectodermal dysplasia (ED), a rare congenital disease that affects hair, skin and sweat glands. Some patients with ED are born without many or all of their teeth and suffer collapsed jaws and facial deformation. The Ensuring Lasting Smiles Act would ensure health plans cover medically necessary services related to a patient’s anomaly or birth defect, including any serious dental and oral-related procedures that are necessary to maintain health and overall function.

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