

2024

Rare Disease Week on Capitol Hill



Kelly Lee SUMMARY REPORT

A Newly Diagnosed, Mom's Perspective: Highly Recommend.

Hosted by the EveryLife Foundation for Rare Diseases and supported by Rare Disease Legislative Advocates (RDLA), this event marked its 13th year in Washington, D.C. with over 600 hundred people. The EveryLife Foundation is “dedicated to empowering the rare disease patient community to advocate for impactful, science-driven legislation and policy that advances the equitable development of and access to lifesaving diagnoses, treatments and cures.” Vicki Seyfert-Margolis, EveryLife Foundation Chair, Founder and CEO of Respond Health Family Advocate said during the kickoff, “As you know, our rare disease mascot is the zebra. Zebras may be rare, but when they band together, they are HEARD.”



Attending this well-planned, well-organized, well-versed, diverse event improved my understanding of the rare disease landscape. The prep webinars, the panel presentations with discussions, the one-pager legislative “asks”, advocacy tools, the RDLA app, and opportunities to network were incredibly helpful. I was surprised to learn there are more than 30 million Americans, that’s 1 in 10, living with one or more rare diseases. 10,000+ known rare diseases, each one unique and different, yet our challenges and barriers to care are very much the same... the diagnostic journey and limited access to care and treatment. Delays in receiving a diagnosis result in an average of \$220,000 of avoidable costs per person. 95% of these 10,000+ known diseases have NO FDA approved treatment... this is a public health crisis. For

me, this really hits home, “There comes a moment when you realize that what you’re really advocating for is quality of life. That’s the moment you realize you won’t give up” ~ Adapted from InYardProducts.com. I am grateful for the experience, the NNPDF travel stipend, and the people I met. I have follow-up to do.

The Key Takeaways:

- Your story is important.
- Your lived experience is impactful.
- Your lessons learned are reasons for change.
- You truly can make a difference.



Talking with U.S. Senator Ted Cruz about what NPC is and what it does as it progresses and giving him a picture of Grace to him to keep as a reminder.

Pending the logistics of life, I encourage you to join me next February for #RDW2025. Continual effort is needed, year-round, to move the needle for patient centric healthcare policy. If you are interested or have questions, I am happy to share more. In the meantime, watch *Bombardier Blood* (bombardierblood.com), a documentary about a man's journey with hemophilia as he conquers the Seven Summits. It is his literal and figuratively inspiring climb for access to care and treatment.

Please know and remind yourself often, "You can do something big, or you can do something small. Because whatever you do...it's better than nothing at all."

