2024
Rare Disease Week on Capitol Hill

Garland Alvey SUMMARY REPORT

EveryLife Foundation for Rare Diseases hosts a yearly Rare Disease Week on Capitol Hill. It was four days filled with incredibly useful topics from information on how to advocate, critical legislative asks that need support now, and what challenges to anticipate post regulatory approval. Our small Niemann-Pick Disease Type C (NPC) advocacy group joined with over 800 fellow advocates of the rare disease community to champion critical and lifesaving legislation. The delay in diagnosis and lack of approved treatments has a devastating impact not only in lives lost, but with economic burden to society. A most recent study found that this crisis in public health has cost the government over $1 trillion in 2019. Over the 4 days a lot was covered and we were grateful to attend on behalf of all NPC families.

Day 1: A welcome reception with a screening of a documentary film called Special Blood.

Day 2: We heard from EveryLife organizers, Amgen Pharmaceuticals Ltd, Faegre Drinker, Mehlman Consulting, other advocates, industry partners, and policy experts. We covered topics on Legislative Outlook and deep dives into specific asks. For example, one of the asks was to urge lawmakers to create an Interagency Coordinating Committee. This is a tried and tested model for over 20 years in muscular dystrophy where all relevant federal agencies, industry partners, and patient family groups come together to work more efficiently and quickly through challenges and hurdles. We then took a massive group photo with the over 700 folks who attended. Then we all took time to meet with our state delegations to go over meeting specifics. Everyone was encouraged to participate in a variety of ways and then to "practice our pitch."

NPC families were then invited to discuss legislative language for the EPICrd Act that is coming together. The draft bill would comprehensively address disparities in Medicaid access to medicines, out-of-state providers, and other items and services for rare conditions. Thank you BioMarin Pharmaceutical Inc.!

Day 3: All of our meetings with various members of the house and senate were prearranged and booked in an easy to follow app. Meetings were a great chance to tell Abby's story and discuss the urgency of action on what is needed to

get her a treatment across the finish line. It's so amazing to me how shocked people are to hear about these types of diseases that affect children as well as adults and they all want to know more and ask what they do to help. We left behind our asks, flyers, and of course our teddy bears from Rich's Stitches.

Hearing such moving stories from all the attendees just added fuel to my desire to do more. It's sad to know so many are going through this, but good to know we aren't alone and can join forces to make real change.

After a fun dinner with some amazing new NPC family members we ended the day strong. We were amped to work closer together to help Abby, Ryan and Gracie.

Day 4: We all reconvened on the final day for topics in DEIA as well as an opportunity to join the Rare Disease Caucus Briefing. Thank to Senator Amy Klobuchar and Rep. Rob Wittman for your efforts with this caucus. I can only hope Senator Mark Warner and U.S. Senator Tim Kaine will spare their legislative assistants to attend this quarterly and remote meeting. We heard from FARA about what obstacles they are facing with a recently approved first treatment. We learned from Sick Cells and the long and difficult journey sickle cell disease has been on to get a gene therapy approved.

Last, but certainly not least was the Rare Artist reception. We took time to honor the finalists in the art competition. Their pieces were incredible testimony to the emotions felt by all of patients and raregivers. Thank you National Niemann-Pick Disease Foundation, Inc. for the logistical support and to all of you who donate to allow us to fight these fights.