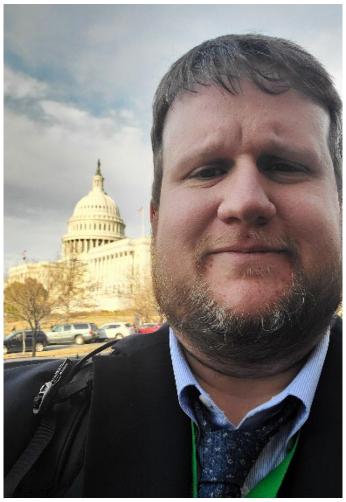
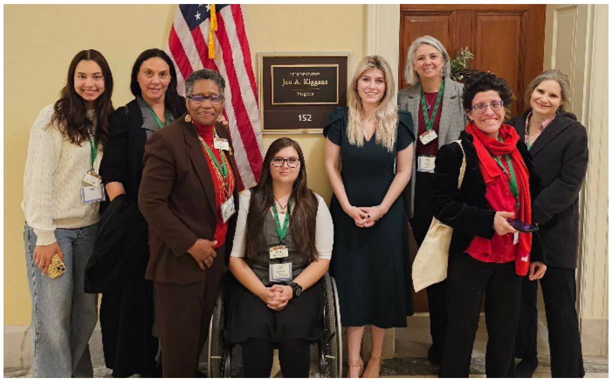
2025
Rare Disease Week on Capitol Hill

Garland Alvey SUMMARY REPORT



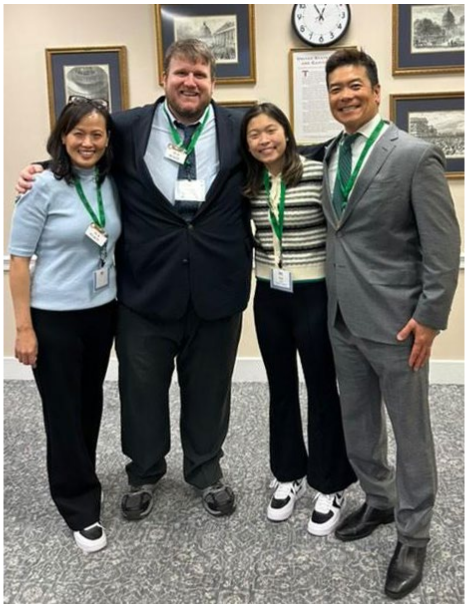
Rare Disease Week on Capitol Hill is always a powerful experience. It's a chance for our community to come together, speak directly with policymakers, and ensure that rare diseases - especially Niemann-Pick disease - stay on the radar of those making decisions that impact research, treatment access, and patient care.

This year, I had the opportunity to attend and meet with congressional staffers from Virginia's delegation, including Senator Mark Warner and Representatives Bobby Scott and Rob Wittman. The key theme in our discussions was the need to protect critical funding for agencies like the NIH, FDA, and Medicaid, which play essential roles in supporting rare disease research and healthcare.



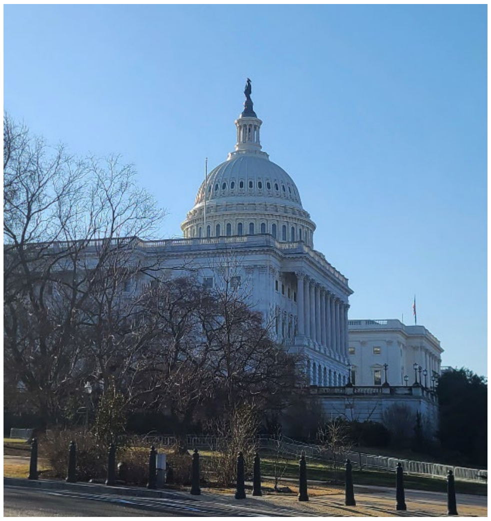
A common concern raised in many meetings was the risk of broad, sweeping budget cuts to these programs. While we understand the need for fiscal responsibility, we emphasized that cutting research and healthcare funding will ultimately hurt families like ours. Families navigating life with rare diseases that already struggle to get the support and treatments they need. Our message was clear: Congress must take a more thoughtful, strategic approach when considering budget reductions, one that prioritizes the well-being of rare disease patients.

The Lasting Impact of Niemann-Pick Families



One thing that became clear throughout Rare Disease Week is the deep and lasting impact that Niemann-Pick families have on everyone we meet... including lawmakers. When we share our stories about childhood dementia, progressive disease, and the devastating reality of losing our children too soon, people listen.

There's something profoundly moving about hearing firsthand from parents who are desperately fighting for treatments and from children who are losing abilities before their families' eyes. Legislators and staffers may meet with hundreds of advocates, but the stories of Niemann-Pick disease stay with them. They ask follow-up questions, they get emotional, and they tell us they can't imagine what we're going through. It's this kind of connection that helps turn passive listeners into active supporters... people who will carry our message forward when they return to the decision-making table. One legislative aid recalled my name and was looking forward to updating me on what his Congressman has cosponsored since our last meeting.



Advocacy Doesn't Start or End in DC

One of the biggest takeaways from this year's Rare Disease Week is just how important it is to advocate year-round, not just during one week in Washington, D.C. While going to Capitol Hill is a great opportunity, you don't have to wait for Rare Disease Week to get involved. Advocacy can start at home, in your own community.

It can be as simple as:

- Reaching out to your local representatives to share your family's story and let them know how policies impact rare disease patients.
- Connecting with state-level agencies to push for newborn screening or better access to Medicaid waivers.
- Talking with your child's school about accommodations and educating teachers about Niemann-Pick disease.
- Raising awareness in your own networks - because the more people who understand the challenges of rare diseases, the stronger our advocacy becomes.

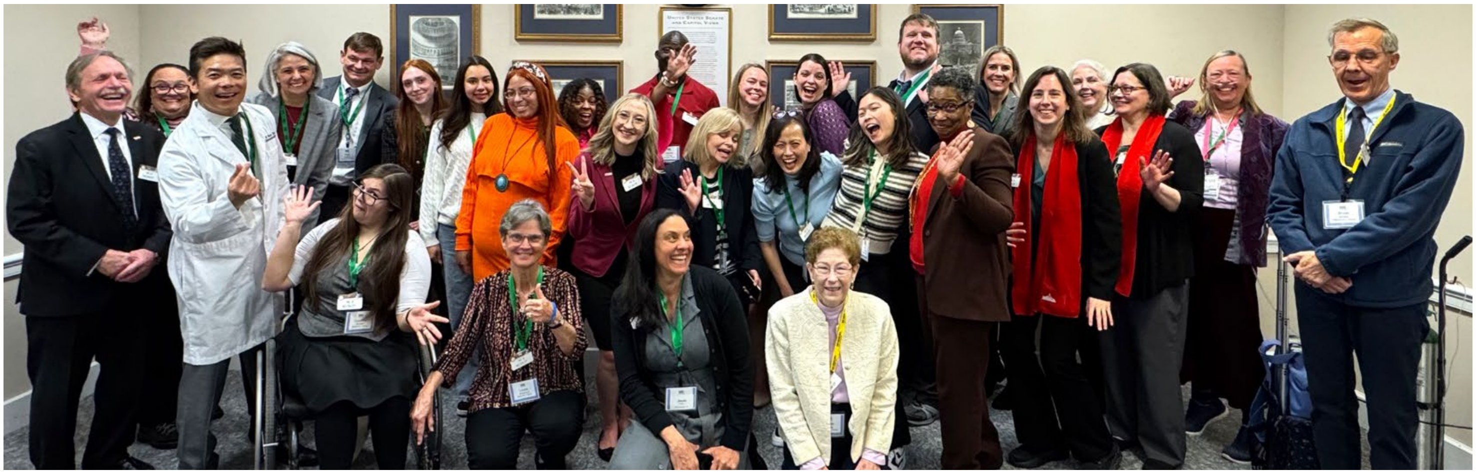
Every conversation matters. Every story shared makes an impact. The more we speak up, the harder we are to ignore.

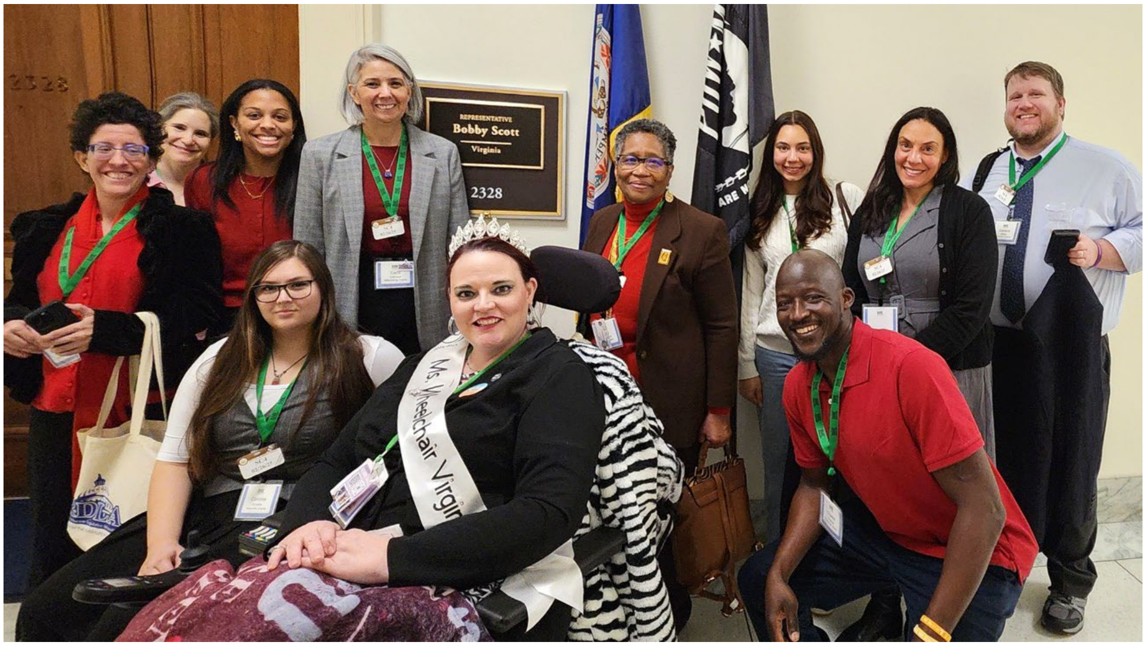
The Power of Our Community

Rare Disease Week reminds us that we are not alone in this fight. When we come together as a community, our voices carry weight. Policymakers are listening, and it's up to us to keep the pressure on to ensure that rare disease research, treatments, and patient support remain a priority.

I'm grateful to NNPDF for providing the Advocacy Stipend, which helped make this opportunity possible. If you've ever thought about getting involved but weren't sure where to start, know that your voice is valuable. Start small, start local - but start. Together, we can create real change.

Garland Alvey
Board of Directors, NNPDF

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