GFPD

ADVOCACY INITIATIVES

Our mission is to improve the lives of individuals with peroxisomal disorders by funding research, championing scientific collaboration, and empowering families and professionals through educational programs and support services.

thegfpd.org

1 Credit for Caring Act

H.R. 2036/S. 925

Family caregivers are the foundation of America's care system, with one in five adults providing care for a loved one. They contribute $600 billion in unpaid labor annually, saving taxpayers billions, yet nearly 8 in 10 (78%) spend over $7,200 out-of-pocket on care-related expenses. Too often, family caregivers already facing financial strain must leave the workforce or reduce hours due to caregiving.

This bill would help working caregivers offset the cost of some caregiving expenses (e.g., adult day services, home care aides, and other supports) by providing up to a $5,000 tax credit for 30% of qualified total expenses above $2,000. Eligibility would be certified by licensed healthcare practitioners. It includes provisions to prevent double-dipping and, unlike existing child and dependent care credits, also supports caregivers of non-dependents or loved ones living apart.

3 PROTECT for Rare Act

S. 3551

Aligns the statutory definition of "medically accepted use" for low-prevalence conditions with sources that reflect the standard of care (i.e., not just FDA label and compendia, but peer-reviewed literature, and clinical guidelines).

This bill expands off-label access and removes the barrier of medical necessity if the treatment is supported in clinical guidelines or two peer-reviewed journal articles. Similar to legislation that addressed issues cancer patients faced over a decade ago, this bill expands the definition of "medical necessity" so that rare and ultra-rare disease patients are relieved of additional financial burden while pursuing off-label treatments.

5 Ally's Act

H.R. 4606/S. 3400

Ensures private insurance covers bone anchored hearing aids (BAHA), a type of osseointegrated device (OID), and cochlear implants—the only effective treatments for certain hearing losses when traditional hearing aids aren't sufficient.

6 Medical Foods and Formulas Access Act

H.R. 5684/S. 3304

Ensures individuals with rare digestive or metabolic disorders have access to covered medically necessary nutrition prescribed by healthcare practitioners (e.g., nutritional formulas), preventing serious health risks and potentially fatal outcomes. The bill builds on prior TRICARE legislation that improved coverage for military families, extending protections to all who rely on medically necessary nutrition.

7 Join the Rare Disease Congressional Caucus

Bipartisan leaders join together to advance policies that support patients, caregivers, and research across the rare disease community.

ACTS ADVANCING EARLIER DIAGNOSIS

2 The Genomic Answers for Children's Health Act

H.R. 7118

Supports faster, more accurate diagnoses for children with suspected rare or genetic conditions by clarifying Medicaid coverage for whole genome and whole exome sequencing (genomic sequencing) under the Early and Periodic Screening, Diagnostic, and Treatment (EPSDT) benefit. Early access to genomic diagnostics can dramatically shorten the lengthy, costly, and stressful journey to a correct diagnosis, which often involves years of unnecessary tests, misdiagnoses, and delayed treatment that can cause irreversible harm or even death. Genomic sequencing has a higher diagnostic yield than other tests and is a cost-effective way to identify rare conditions while enabling timely, often life-saving care.

4 Federal Newborn Screening System

Newborn screening is one of the most powerful and cost-effective public health tools we have. It saves lives and improves quality of life by identifying serious conditions before symptoms appear, enabling timely monitoring, early intervention, and treatment while reducing overall costs.

Without early and accurate diagnosis, families often endure a lengthy and expensive diagnostic odyssey filled with delays and avoidable suffering. These delays are not without human cost, as three in ten children with a rare disease will not live to see their fifth birthday. A strong newborn screening system ensures babies have early access to the care they need and deserve.

Learn more about the GFPD's advocacy efforts, please visit: **thegfpd.org/advocate**

WHY I AM A RARE DISEASE ADVOCATE

MADELEINE BRYCE | Tulsa, OK

I advocate because investing in rare disease healthcare is not a niche investment, it's a national investment. The treatments pioneered for small patient populations often become the breakthroughs that transform millions of lives. What we build for the most vulnerable among us becomes a pathway to hope for all of us.

EDDIE DAY | Spring Hill, TN

I am a rare disease advocate because my daughter lived and passed away from Zellweger syndrome. Her diagnosis showed me how isolating and underfunded rare conditions can be. I advocate to honor her life, raise awareness, support families, and push for research and earlier diagnoses so no one faces a rare disease alone.

RYAN MAPLE | Knoxville, TN

Rare disease advocacy is crucial in driving changes to legislation and funding research. Rare does not mean insignificant. Many individuals fighting a peroxisomal disorder, my son included, cannot have their own voice in the fight. I will always be that voice.

SUZANNE COLLINS | Laurinburg, NC

I am a rare disease advocate because 3 of my 5 children were diagnosed with a rare, life-limiting peroxisomal disorder. A nation is judged by how it protects those who cannot protect themselves. Giving a voice to the voiceless and standing up for families like mine is how I honor Elaysha, Jeremiah, and Lizzie (#Forever15).

ABBY DENIS | Hatboro, PA

I advocate for rare disease because my son lived it every day. Through him, I've seen the gaps in awareness, research, and support. I advocate to give his life meaning beyond his diagnosis and to ensure that rare families are seen, heard, and valued.

KATIE & TED SACRA | Myrtle Beach, SC

We advocate so a rare diagnosis meets action—not isolation. Our son, and the 1 in 10 Americans with a rare disease, deserve care, research, and policies that deliver real, meaningful progress. We amplify our community's voice to help every person with a rare disease not just survive, but truly thrive.

"So I'd love for him to be able to move his hands, even just a few inches would be great. Just so he could play with toys or interact with things."
— Mother of child with Zellweger Spectrum Disorder (ZSD)

"After over a decade of stable vision, [my adult child] woke up one morning and told me 'It's very dark and I cannot see.'"
— Mother of child with ZSD

"With no sound coming to [our child's] ears, she stopped talking, stopped laughing, and stopped communicating with us. Instead, she gave us silence."
— Mother of deceased child with ZSD

"Basically, dealing with the deafblindness, we realized that we had to be their eyes, their ears."
— Father of adult with ZSD

"Very soon his seizures began to worsen, no longer a small tremor, but a large quake that ravaged his entire body."
— Father of deceased child with ZSD

"The discomfort from the bleeding on her GI tract made it difficult for her to tolerate her tube feedings, often causing her to frequently vomit after she ate and making it more difficult for her to gain weight, which was already a struggle since birth."
— Mother of child with ZSD

GLOBAL FOUNDATION for PEROXISOMAL DISORDERS
GFPD

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