PROJECT ALIVE

www.projectalive.org | give@projectalive.org | (352) 664-2031 | 501(c)(3) Tax ID: 46-17970

Hunter Syndrome: A Call for Action on Capitol Hill

Overview of Hunter Syndrome

Hunter syndrome (Mucopolysaccharidosis II, MPS II) is a rare, progressive, and terminal genetic disorder that primarily affects boys. As a lysosomal storage disorder, it leads to the buildup of harmful substances in cells, causing physical and cognitive deterioration. Without treatment, individuals with Hunter syndrome often face a life expectancy of just 10-20 years. Despite the urgency, treatments remain limited, and many families are left with few options.

Project Alive: Empowering Families, Advancing Research, and Advocating for Change

Project Alive is committed to improving the lives of individuals with Hunter syndrome through research, advocacy, and community support. Our efforts are focused on accelerating the development of life-saving therapies and ensuring that all children with Hunter syndrome have access to appropriate care.

Key Policy Areas:

1. Accelerating Kids Access to Care Act

We urge lawmakers to support the **Accelerating Kids Access to Care Act** to facilitate quicker access to clinical trials, ensuring children with rare diseases like Hunter syndrome can participate in treatments that are critical to their survival. This legislation will help remove barriers to access and expedite treatment options, particularly for children who are often excluded from early-stage research.

2. Rare Pediatric Disease Priority Review Voucher

We call for the extension and expansion of the **Rare Pediatric Disease Priority Review Voucher Program** to incentivize the development of treatments for rare pediatric diseases, including Hunter syndrome. This program has already demonstrated success in speeding up the availability of treatments for other rare conditions and could significantly impact the speed at which Hunter syndrome therapies reach the market.

3. Ensure Federal Biomedical Research Funding and Public Health Agency Support

Recent terminations and funding/communication freezes have jeopardized critical research programs, particularly at the **FDA** and **NIH**. We need consistent, robust federal funding and leadership at these agencies to ensure that life-saving treatments for progressive, terminal diseases like Hunter syndrome can make it out of clinical trials and into the hands of families who desperately need them. Without proper funding and agency support, these treatments will remain out of reach for our children, and the advancements we've made toward clinical solutions could be lost.

Why This Matters

The lack of accessible treatment options for progressive, terminal diseases like Hunter syndrome leaves families in a state of constant uncertainty. We cannot afford to wait any longer for the breakthrough therapies that could save the lives of our children. Federal support in these key areas will help accelerate clinical trial development, improve access to care, and ensure that future treatments are made available to the children who need them most.

Call to Action

We urge you to stand with families affected by Hunter syndrome and other rare diseases. Please support legislation that prioritizes access to care, encourages research, and ensures that federal agencies continue to drive the progress necessary for life-saving therapies. Our children's futures depend on the actions taken today.

Give our kids a chance to grow up

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Key Facts & Stats

- Hunter syndrome is an ultra-rare disorder impacting **roughly 500 children in the US**.
- **30 million, or 1 in 10** people have a rare disease in the US.
- Most children with Hunter syndrome **do not live past adolescence**, many passing before their 13th birthday.
- Hunter syndrome impacts people all around the country, **regardless of race, ethnicity, or background**.
- There are **3 ground-breaking clinical trials** happening for Hunter syndrome in our country, two of which have already began filing for approval or will this year. A cure is within reach.
- Families face **hundreds of thousands of dollars per year** in medical expenses, adaptive equipment, and specialized care.
- At least **half of families rely on Medicaid** programs.

Supporting the Rare Disease Community Alongside

EVERYLIFE FOUNDATION FOR RARE DISEASES

RDLA Rare Disease Legislative Advocates
POWERED BY THE EVERYLIFE FOUNDATION

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