The EveryLife Foundation for Rare Diseases
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February 16, 2024

Representative Nanette Barragán
US House of Representatives
2312 Rayburn House Office Building
Washington, DC 20515

Representative Anna Eshoo
US House of Representatives
272 Cannon House Office Building
Washington, DC 20515

Representative Gus Bilirakis
US House of Representatives
2227 Rayburn House Office Building
Washington, DC 20515

Representative Michael McCaul
US House of Representatives
2300 Rayburn House Office Building
Washington, DC 20515

Representative Michael Burgess
US House of Representatives
2161 Rayburn House Office Building
Washington, DC 20515

Representative Lori Trahan
US House of Representatives
2439 Rayburn House Office Building
Washington, DC 20515

Dear Representatives McCaul, Bilirakis, Burgess, Barragán, Eshoo, and Trahan,

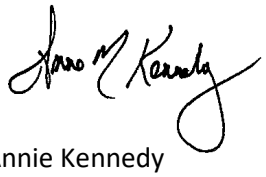
On behalf of the EveryLife Foundation for Rare Diseases, I am writing to express our enthusiastic support and appreciation for your leadership of the Creating Hope Reauthorization Act of 2024. The introduction and passage of this bill will reauthorize the Rare Pediatric Priority Review Voucher Program for a four-year period. This vital incentive comes at no cost to taxpayers yet has proven to spur innovation in rare diseases that disproportionately affect children.

Without Congressional action, the Rare Pediatric PRV Program will expire on September 30, 2024, leaving one less tool to bring treatments to a rare disease community in which only five percent of diseases have an FDA-approved treatment. It is imperative that we continue to advance solutions to treat these devastating diseases. Allowing the Rare Pediatric PRV program to expire would eliminate a powerful incentive for the development of treatments for the 70 percent of rare diseases that start in childhood. Developing treatments for rare pediatric diseases is even more challenging due to very small populations, complexities involved in conducting clinical trials in children, the nature of many pediatric genetic diseases, and delays in diagnosis among other factors.

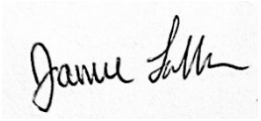
The PRV Program has enabled life-saving treatments to reach children with rare diseases faster without adding new costs to taxpayers. Since 2012, 49 rare PRVs have been issued, bringing treatments to about 40 distinct rare disease communities. PRVs have been associated with an increased rate of progress through the clinical trial process, leading to approvals for rare diseases such as spinal muscular atrophy, sickle cell disease, Rett syndrome and other diseases that previously lacked any treatment options. In at least one example, the existence of the Rare Pediatric PRV can be credited for ensuring the treatment was available in the pediatric population immediately upon first approval, rather than having the treatment be first approved for an older subset of the population and then having to wait years for additional pediatric research to be conducted; years that many kids with rare diseases do not have.

Time is the most precious commodity for the rare disease community, even more so for the children facing these devastating diagnoses. Each time a scientific discovery fails to make it to clinical trials, or a promising early therapeutic target faces delays or demise, lives are lost, further investment in the disease is stymied, and the kids and families who give so much to the research in their disease area are left with less hope and fewer options.

An extension of this program with bi-partisan support will help rare disease families with life-threatening conditions throughout the US. The EveryLife Foundation is proud to support the Creating Hope Reauthorization Act. We are grateful for your leadership and ongoing commitment to the health and wellbeing of the 30 million Americans with a rare disease. We look forward to working with you and your colleagues to ensure this bill becomes law.



Annie Kennedy
Chief of Policy, Advocacy, & Patient Engagement



Jamie Sullivan, MPH
Vice President of Policy