Support the Reauthorization of the Rare Pediatric Disease Priority Review Voucher Program

The Rare Pediatric Disease PRV Program expired on December 20, 2024, despite broad bipartisan support for its reauthorization. To restore this critical incentive, the *Give Kids a Chance Act* (H.R. 1262/ S.932), which reauthorizes the PRV Program for five years, was reintroduced. Congress must ensure this vital incentive, which has no cost to taxpayers, continues to drive innovation in rare pediatric diseases, offering hope to children and families.

Background

- The Creating Hope Act expanded the PRV Program to include drugs that treat rare pediatric diseases as part of the Food and Drug Administration Safety and Innovation Act (FDASIA) in 2012[1].
- To be eligible for a PRV, the treatment must obtain a Rare Pediatric Designation from the FDA, be eligible for priority review, and it must be the first approval for the drug's active ingredient.
- After the FDA approves an eligible treatment, the company is issued a PRV. The opportunity to obtain a PRV is an important incentive in pediatric rare disease therapy development because:
    - Earlier Review: A company that gets a PRV can use it on a future treatment that wouldn't otherwise qualify for priority review, leading to a 4-month reduction in review time.
    - Revenue Generation: Some companies choose to sell the PRV to another company, generating revenue for the seller that is often used to continue and expand their rare disease research and development programs.
- The Rare Pediatric Disease Priority Review Voucher Program expired on December 20, 2024. As a result, companies must have received a Rare Pediatric Designation by December 20, 2024, to be eligible. After September 30, 2026, FDA will no longer be able to award Rare Pediatric PRVs[2].

Why is the Rare PRV Program Critical to Rare Disease Therapy Development?

- Developing drugs for rare pediatric diseases is challenging due to the small populations affected, difficulties associated with conducting clinical trials for children, delays in diagnosis, and more.
- About 70% of rare diseases are pediatric-onset, and 95% of rare diseases have no approved treatments[3].
- 62 Rare Pediatric PRVs have been issued since 2012 for innovative treatments in over 40 diseases like spinal muscular atrophy, Duchenne muscular dystrophy, and progeria syndrome[4].
- The impact of the PRV program is growing. Over half of all PRVs awarded since 2012 have been in the last five years. A new incentive takes time to impact decision-making fully. It takes an average of 15 years to develop a drug.

Contact Information for Bill Leads

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[1] Federal Food, Drug, and Cosmetic Act (FD&C Act), 21 U.S.C. § 36 (2012

[2] https://www.fda.gov/industry/medical-products-rare-diseases-and-conditions/rare-pediatric-disease-rpd-designation-and-voucher-programs

[3] Nguengang Wakap S, Lambert DM, Olry A, Rodwell C, Gueydan C, Lanneau V, et al. Estimating cumulative point prevalence of rare diseases: analysis of the Orphanet database. Eur J Hum Genet. 2020;28(2):165–73.

[4] https://www.federalregister.gov/