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

The Give Kids a Chance Act (H.R. 3433), passed in the house in September 2024, reauthorizes the Rare Pediatric Priority Review Voucher (PRV) Program for 5 years, but the extension of the program rests on the passage of the Creating Hope Reauthorization Act (S. 4583) in the Senate. Congress must act to ensure this vital incentive that has no cost to taxpayers can continue to spur innovation in rare diseases that disproportionately affect children.

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 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 an eligible treatment is approved by the FDA, 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 who gets a PRV can use it on a future treatment that wouldn't otherwise qualify for priority review, leading to about a 4-month reduction in review time.
    - Revenue Generation: some companies chose 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 program was last reauthorized in 2020 for a 4-year period. Without Congressional action, companies will have to receive 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 exclusively pediatric onset and overall, 95% of rare diseases have no approved treatments[3].
- The impact of the PRV program is growing. More than half of PRVs were awarded since 2019. A new incentive takes time to fully impact decision making since it takes an average of 15 years for a drug to be developed and approved by the FDA.
- 56 Rare Pediatric PRVs have been issued since 2012 for innovative treatments in 40 diseases like spinal muscular atrophy, Duchenne muscular dystrophy, and progeria syndrome[4].
- 7 out of 7 developers interviewed by the GAO reported that PRVs were a factor in their decisions[5].

Contact Information

For more information on the Creating Hope Reauthorization Act of 2024, please contact Senator Casey - sara_maskornick@help.senate.gov, or Senator Mullin - jake_johnson@mullin.senate.gov

[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/
[5] Government Accountability Office. (2020). Drug Development: FDA's Priority Review Voucher Programs. (GAO Publication No. 20-251). Washington, D.C.: U.S. Government Printing Office. Retrieved from https://www.gao.gov/assets/gao-20-251.pdf.