The Cost of PRV Inaction

Failing to Reauthorize the Rare Pediatric Disease Priority Review Voucher Program (PRV) Will Stall Lifesaving Innovation and Undercut U.S. Biotech Competitiveness

What is the RPD PRV Program?

Created by Congress in 2007 to spur treatments for neglected diseases, the Rare Pediatric Disease Priority Review Voucher (PRV) program was expanded in 2012 to accelerate the development of therapies for rare pediatric conditions. Administered by the U.S. Food and Drug Administration (FDA), the program has been reauthorized with bipartisan support every four years (2016 and 2020), providing vital certainty to innovators and delivering lifesaving treatments and hope to families across the country.

Despite strong bipartisan backing, the **program's authorization lapsed in December 2024**, halting a vital incentive for drug developers who have historically relied on it to advance novel treatments for rare pediatric diseases.

Barriers to RPD Drug Development:

- **scientific complexity**
- **small patient populations**
- **economic barriers**
- **logistical and technical issues**

53

To date, **53 PRVs have been awarded across 39 rare pediatric diseases.** Prior to the creation of the RPD PRV, only 3 of those 39 diseases had any FDA approved treatment.[i]

74%

Notably, **74% of qualifying drugs who were awarded a RPD PRV are first in disease**,[ii] indicating that trailblazing innovation is underway and has already improved treatment options for children.

Why is the PRV Program Critical to Rare Pediatric Drug Development?

Rare disease drug development faces many barriers. **Just 5% of the 10,000 known rare diseases have an approved treatment on the market. Yet 95% of rare diseases don't have an FDA-approved treatment option.**

Holding or selling a PRV can be transformative for small and mid-size biotechs, attracting investment or providing critical capital to fund additional R&D. At the same time, the program helps expedite broader patient access to urgently needed treatments.

The PRV program offers hope to children with few or no treatment options. It serves as a powerful incentive to stimulate the development of therapies for diseases that are not economically viable to pursue - and does so at no direct cost to taxpayers.

- **The impact is real:** Children with neglected diseases have benefited from real therapeutic advances because of the PRV. The program has directly supported the development of treatments for conditions such as pediatric high-risk neuroblastoma - a devastating childhood cancer - and cystic fibrosis, among many others.

i - Impact of the Rare Pediatric Disease Priority Review Voucher Program on Drug Development 2012-2024. NORD. July 2024. *https://rarediseases.org/wp-content/uploads/2024/07/NORD-PRV-Policy-Report.pdf*

ii - Impact of the Rare Pediatric Disease Priority Review Voucher Program on Drug Development 2012-2024. NORD. July 2024. Page 10-15. *https://rarediseases.org/wp-content/uploads/2024/07/NORD-PRV-Policy-Report.pdf*

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How Does the PRV Program Work?

Priority review vouchers can only be awarded to a qualifying drug sponsor whose novel treatment addresses a qualifying area of unmet need, including for rare pediatric disease. The voucher can be redeemed to shorten the FDA's review time of a different single human drug application (6 months v. 10 months), including drugs that would not otherwise qualify for expedited review. Vouchers can also be sold to other drug developers.

Process

1. **The PRV application process is integrated into the FDA's standard review processes. When a voucher is redeemed, the FDA works within their existing resources to expedite the applicable drug's final months of review.**
2. **When submitting a marketing application for an applicable drug or biologic to treat a rare pediatric disease, a sponsor will include a request for a RPD PRV.**
3. **If the FDA approves the application, they will award a PRV to the sponsor.**
4. **The sponsor can keep the voucher to redeem for a different drug or transfer it to another sponsor.**
5. **To redeem a PRV, a sponsor must notify FDA of its intent to redeem the voucher and pay the priority review user fee. The drug for which the voucher is redeemed does not need to be targeted to a rare pediatric or orphan indication.**

When a sponsor presents a voucher to FDA for priority review, the Agency aims to review the application within **6 months, versus 10 months** for a standard review.

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