RDLA Policy Primer:
Rare Pediatric Disease Priority Review Voucher (PRV) Program

Background

The Rare Pediatric Disease Priority Review Voucher (PRV) Program was expanded to include drugs that treat rare pediatric diseases in the Food and Drug Administration Safety and Innovation Act (FDASIA) in 2012. The purpose of the PRV is to expedite the development and promotion of drugs in the rare pediatric disease, tropical disease, and medical countermeasure fields.

Developing drugs for rare pediatric diseases is particularly challenging due to the small populations affected, difficulties associated with conducting clinical trials for children, delays in diagnosis, and more. The impact of the PRV program is growing. Over 55 PRVs have been awarded since 2012, more than half of which were awarded in the last five years. 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.

About the PRV Program

The PRV Program incentivizes pharmaceutical companies to develop rare pediatric disease treatments at no cost to taxpayers. Before a treatment is approved, the company can obtain a Rare Pediatric Designation from the FDA. To be eligible, the drug must 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 that can be used to obtain priority review for a treatment that wouldn't otherwise qualify. Priority review means the FDA generally reviews a treatment within 6 months rather than the standard 10-month period.

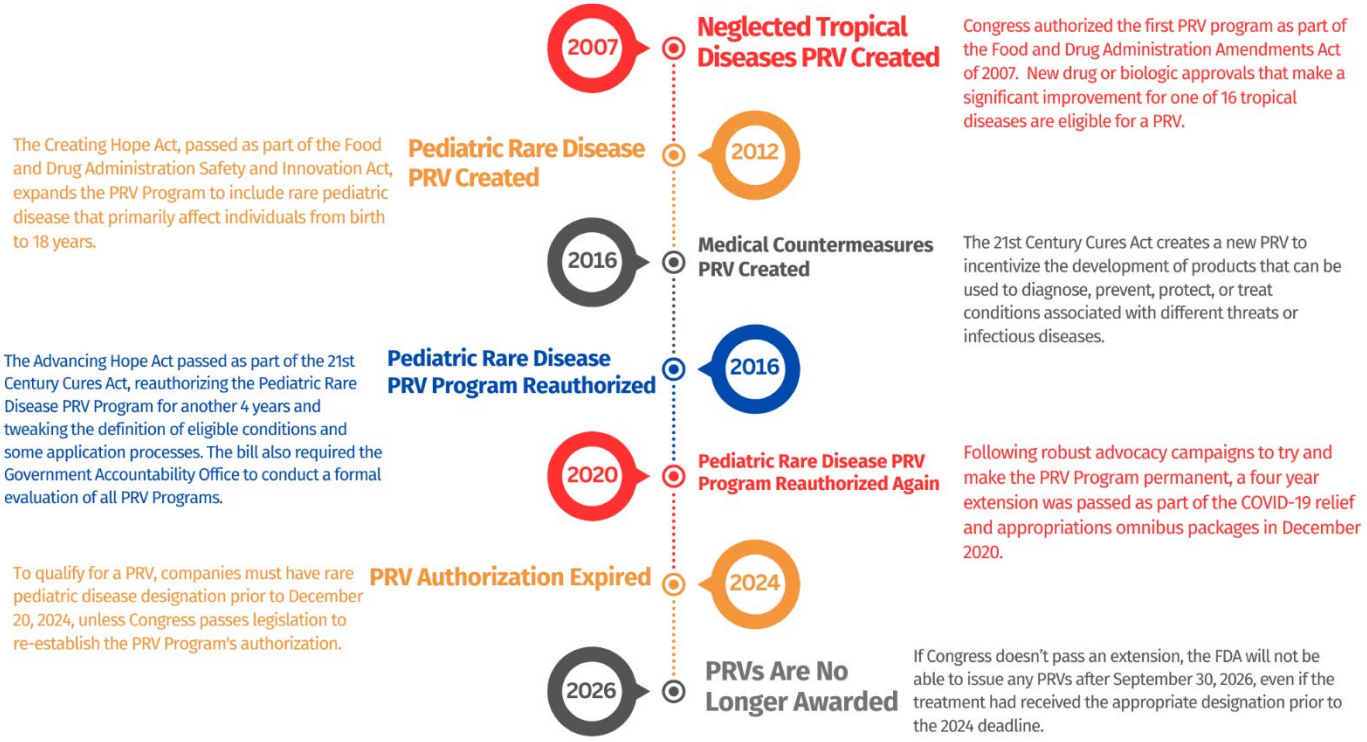
Alternatively, the PRV can be sold to another company, generating revenue for the seller. The revenue generated from selling a PRV is part of the incentive for companies to invest in developing treatments for rare pediatric conditions. Companies purchase PRVs to get treatments on the market faster than the typical 10-month review period.

Policy Considerations

In late December 2024, a bipartisan healthcare policy package, including the Give Kids a Chance Act, was released and set to be included in the end-of-year government funding extension. Due to forces unrelated to the healthcare policy package or the rare disease community, most health provisions, including the PRV Program, were excluded from the final bill. Unfortunately, the PRV Program expired on December 20, 2024.

The House has re-introduced the Give Kids a Chance Act (H.R. 1262), which includes provisions to reauthorize the PRV Program as previously proposed in the Creating Hope Reauthorization Act of 2024 (H.R. 7384/ S. 4583). The Senate has yet to introduce a bill in the 119th Congress.

Timeline



Resources

[EveryLife PRV Support Page]
[Impact of the Priority Review Voucher Program on Rare Pediatric Disease Drug Development]
[Impact of the Rare Pediatric Disease Priority Review Voucher Program on Drug Development 2012-2024]
[GAO: Drug Development- FDA's Priority Review Voucher Programs]
[FDA: Rare Pediatric Disease Designation Information]