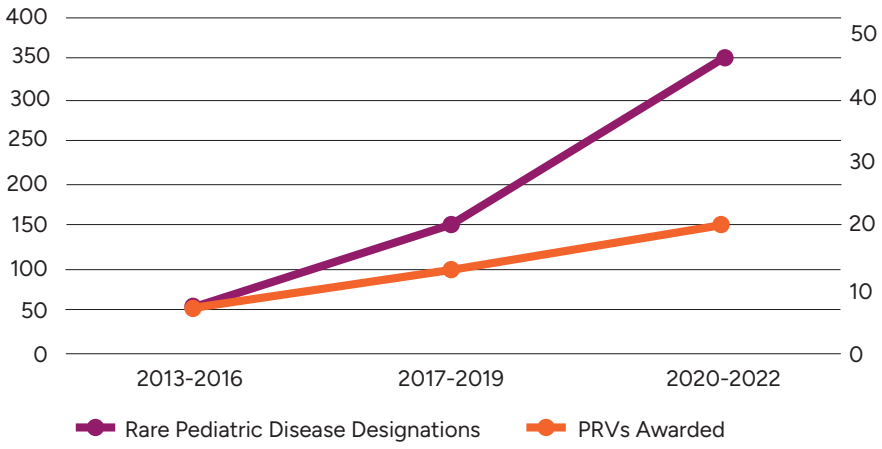
Hope for Millions of Children Living With Rare Diseases

Impact of the Rare Pediatric Disease Priority Review Voucher Program on Drug Development 2012-2024

Half of the estimated 30 million Americans living with rare diseases are children. Many of the more than 10,000 known rare diseases are life-threatening, but 95% of rare diseases do not have an FDA approved treatment. The Rare Pediatric Disease Priority Review Voucher (RPD PRV) program has proven to be effective in stimulating drug development to help address the tremendous unmet medical needs that exist in the pediatric rare disease community.

Without reauthorization of the RPD PRV program by Congress, this critical tool for encouraging drug development in these particularly challenging to study patient populations will come to an end. **NORD's report** provides new, in-depth analysis of the impact of the RPD PRV program, how it helps children with pediatric rare diseases and why it must be reauthorized.

Trends in RPD PRVs and Rare Pediatric Disease Designations



The RPD PRV program has been successful in helping to spur new and innovative treatment options for children living with rare disease. For the children who still lack treatment options for their rare condition, letting this program lapse would be a devastating step backward. Given the vast unmet medical needs that remain, Congress should act swiftly to reauthorize it to help resolve the treatment challenges faced by millions of children living with rare diseases.

Support H.R.1262/ S.932, the Give Kids A Chance Act of 2025 to maintain the Rare Pediatric Disease Priority Review Voucher program for at least another five years.

Read NORD's RPD PRV analysis:
rarediseases.org/rare-pediatric-disease-prv-program
Learn more about NORD's rare disease policy work:
rarediseases.org/driving-policy

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KEY FINDINGS

Since the Rare Pediatric Disease Priority Review Voucher program started in 2012:

More than 560 rare pediatric disease designations[1] have been made and **53 RPD PRVs** have been awarded[2] showing the positive impact RPD PRVs have had in driving commitments to study and develop rare pediatric disease treatments.

39 rare diseases have gained treatment options. Remarkably, **36 of these 39 rare diseases had no FDA-approved treatments** prior to the creation of the program.

Only 23 of the 53 RPD PRVs awarded have been redeemed, equal to two additional drugs receiving priority reviews per year.

Vouchers are not being redeemed to extend monopolies for high-spend drugs; **only three drugs ranked among the top 50 by Medicare drug spend in 2022** received FDA priority review due to this program.

1. As of the end of 2022
2. As of April 30, 2024