HEALTHCARE HIGHLIGHT
DECEMBER 2025

Rare Pediatric Disease Priority Review Vouchers

For children living with rare and life-threatening diseases, access to innovative and effective therapies is essential to their survival. However, developing treatments for rare pediatric conditions poses significant challenges: high costs of research, significant complexities in pediatric studies, and the limited number of patients make investing in these therapies less attractive to pharmaceutical companies than in therapies for more prevalent adult conditions.

If you are interested in learning more about the rare disease ecosystem, including regulatory and policy trends, please contact Jennifer Bernstein at jbernstein@monumentadvocacy.com.

The Food and Drug Administration's (FDA) **Pediatric Priority Review Voucher Program (PPRV)** was designed to incentivize the development of treatments for rare pediatric diseases, as most drug development has traditionally focused on adult conditions with large markets. It is based on the FDA's priority review voucher program, which was designed to incentivize the development of treatments for neglected tropical diseases.

PROGRAM HISTORY

- Program created in 2012
- Reauthorized in 2016 & 2020
- **Program lapsed on Dec. 20, 2024**

On December 20, 2024, Congress failed to include PPRV reauthorization in the end-of-year spending package. Since then, FDA has been unable to award new vouchers, though companies that were issued a voucher prior to December 20, 2024, may still redeem or transfer them.

PROGRAM OVERVIEW

A company **must develop** a new drug or biologic that treats or prevents a serious or life-threatening rare disease primarily affecting individuals **under 18** years of age.

- The disease must meet the FDA's **definition of "rare"** (fewer than 200,000 people in the U.S.).
- The therapy must offer meaningful **new clinical benefit**, not just a reformulation or minor change.

A company then obtains **FDA designation** as a "rare pediatric disease product." Before approval, the company applies to the FDA for Rare Pediatric Disease Designation.

- **FDA reviews** the request to confirm that the disease qualifies and that the product targets the pediatric population.
- This designation must be **granted <u>before</u> submission** of the marketing application (NDA or BLA).

After obtaining a designation, the company must submit a **New Drug Application (NDA)** or **Biologics License Application (BLA)** that includes data demonstrating safety and efficacy for the rare pediatric indication.

If the FDA **approves** the therapy and **confirms** it meets all statutory criteria, the agency will award a Rare Pediatric Disease Priority Review Voucher to the company. The voucher is issued as a reward for successfully developing the qualifying therapy.

As of April 30, 2024, 63 vouchers have been awarded across 47 different rare pediatric diseases.

Vouchers can be sold from anywhere from $67M to $350M

CURRENT STATUS

The Give Kids A Chance Act (H.R. 1262 / S.932)

The Give Kids A Chance Act, which includes a five-year reauthorization of the PPRV, was passed in the House on December 1, 2025. Senate action is still pending. Support for the program's reauthorization has been bipartisan and bicameral, with 303 cosponsors in the House and 16 in the Senate. Additionally, more than 200 patient organizations have endorsed the bill.

The rare disease patient community remains hopeful that the program will be reauthorized by the end of 2025 or in early 2026.

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