

THE FDA'S PEDIATRIC RARE DISEASE PRIORITY REVIEW VOUCHER PROGRAM

MAINTAINING PROGRESS FOR CALIFORNIANS WITH RARE DISEASE



Congress must prevent uncertainty and pass a five-year reauthorization of the PRV.

CREATING HOPE FOR CHILDREN WITH RARE DISEASE

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 **Rare Pediatric Disease (RPD) Priority Review Voucher (PRV) Program** was established more than a decade ago to provide crucial incentives for pharmaceutical and biotech companies to develop new therapies for rare conditions that affect the pediatric community.

As of 2024, 53 RPD PRVs have been awarded for 39 different rare pediatric diseases. Prior to the creation of the RPD PRV program, only three of these 39 rare pediatric diseases had any FDA-approved treatments.

RARE DISEASE PREVALENCE



More than 95% of rare diseases lack an FDA approved therapy and about 70% of rare diseases are exclusively pediatric onset

NO COST, HIGH REWARD

RPD PRVs have enabled the development of new rare disease treatments at no cost to taxpayers



THE BENEFITS OF A PRIORITY REVIEW VOUCHER

To be eligible for an RPD PRV, the treatment must obtain Rare Pediatric Designation from the FDA, be eligible for priority review, and be the first approval for the drug's active ingredient. A company is then issued an RPD PRV after an eligible treatment is approved by the FDA, which provides an opportunity for two beneficial uses:



As the title suggests, a company may use an RPD PRV to receive an earlier review on a future treatment they develop that wouldn't otherwise qualify, leading to about a 4-month reduction in review time



A company may also choose to sell their RPD PRV to another company, generating revenue for the seller that is often used to continue and expand their rare disease research and development programs. This is a helpful mechanism particularly for small and mid-size companies with fewer products on the market.

PROVIDING CERTAINTY THROUGH REAUTHORIZATION

The RPD PRV program was last authorized for four years in 2020. Following several stopgap extensions, the continuing resolution that Congress passed to keep the government funded at the end of 2024 included an extension of the RPD PRV through March 14, 2025. Without a timely reauthorization, the FDA will no longer be allowed to initiate the process necessary to issue new RPD PRVs and the pipeline of potential cures could languish.

Congress must provide certainty by reauthorizing the critical RPD PRV program for at least five more years to create hope for children with rare diseases.

CALIFORNIA CREATES FOR KIDS



Of the 53 total RPD PRVs awarded between 2012-2024, **23 have been awarded to CA** companies