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FDA program aims to spur development of drugs for rare pediatric diseases

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Approximately 15 million U.S. children have a rare disease, according to the U.S. government accountability office (GAO).

One way the Food and Drug Administration (FDA) supports drug development for children with rare diseases is through implementation of the Rare Pediatric Disease (RPD) Priority Review Voucher (PRV) program.

The RPD PRV program was established in 2012 to encourage development of new drug and biological products for the prevention and treatment of rare pediatric diseases. It was reauthorized in 2016 and 2020. Unless the program is reauthorized by Congress, the FDA will no longer be able to award RPD PRVs after Sept. 30, 2026.

“While some products receiving an RPD PRV treat a serious or life-threatening manifestation of a disease, others treat the underlying disease, representing significant progress in developing the first disease-modifying therapies for many rare pediatric diseases,” said Erika Torjusen, M.D., M.H.S., director of the FDA’s RPD designation program and a pediatrician subspecialized in allergy immunology.

A rare disease is statutorily defined as a disease or condition that affects fewer than 200,000 individuals. Collectively, rare diseases are a leading cause of childhood mortality in the U.S., according to the Centers for Disease Control and Prevention’s National Center for Health Statistics.

Under the program, a sponsor who receives an approval for a drug or biological product for a rare pediatric disease may qualify for a voucher that can be redeemed to receive priority FDA review for a different product. The sponsor also can transfer or sell the voucher to a third party, with reported purchase prices averaging $100 million (range $67.5 million to $350 million), according to the GAO.

To date, 61 RPD PRVs have been awarded since 2014.

Examples of approved products that earned an RPD PRV include:

- Concizumab-mtci (Alhemo) approved for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in patients 12 years and older with hemophilia A with FVIII inhibitors or hemophilia B with FIX inhibitors. Many treatments for hemophilia are administered via intravenous infusions. Concizumab-mtci is the first approval of a subcutaneous injection for this population.
- Elexacaftor, tezacaftor and ivacaftor combination treatment (Trikafta) approved for treatment of cystic fibrosis (CF) in patients 12 years and older who have at least one F508del mutation in the *CFTR* gene (estimated to represent 90% of the CF population). Previously, many of these patients had no approved therapeutic options.
- Exagamglogene autotemcel (Casgevy) approved for treatment of sickle cell disease in patients 12 years and older with recurrent vaso-occlusive crises. This represents the first U.S. approval of a CRISPR/Cas9-based gene-editing therapy.

The high level of interest in this program has been demonstrated by significant surges in the number of requests for RPD designation received by the FDA in the years of the scheduled sunsets.

The FDA's Office of Pediatric Therapeutics and Office of Orphan Products Development contributed to this article.

Resources

- FDA's RPD PRV Program
- FDA's Rare Disease Innovation Hub
- Novel drug approvals at FDA