Biotechnology Innovation Organization

THE ORPHAN DRUG ACT AND RARE PEDIATRIC DISEASE VOUCHER PROGRAM PROMOTE INVESTMENT IN RARE DISEASES

The **Orphan Drug Act** and the **Rare Pediatric Disease Priority Review Voucher Program** are two powerful incentives to stimulate drug development in underserved diseases or conditions. Rare conditions often lack the market opportunity to attract significant investment or may present other substantial development obstacles and costs that may deter investment from biopharmaceutical companies. Rare pediatric diseases are faced with additional development challenges due to the added complexity of enrolling and studying pediatric patients, which results in the need for incentives in addition to the Orphan Drug Act. One such example is the Rare Pediatric Disease Priority Review Voucher Program designed to encourage the development of therapies specifically for rare pediatric diseases.

While the Orphan Drug Act provides incentives for companies when they develop a therapy intended to treat a rare disease, in order to receive a **Rare Pediatric Disease Priority Review Voucher**, a company must be developing a therapy that is intended to treat **a rare disease with symptoms that primarily impact children.**[1]

- Rare pediatric diseases face challenges due to the added complexity of enrolling and studying pediatric patients. The Orphan Drug Act and the Rare Pediatric Disease Priority Review Voucher Program work together to spur development of innovative therapies for rare pediatric diseases.
- In fact, since the Rare Pediatric Priority Review Voucher Program was implemented, a significant increase in the rate of progress from early to mid-phase clinical programs has been observed.[2]

At this time, the Orphan Drug Act is a permanent program but unless Congress makes the priority review voucher program permanent or extends the sunset, FDA may not award any Rare Pediatric Disease Priority Review Voucher after September 2020, unless the drug **is designated** as a drug for a rare pediatric disease and receives marketing approval by September 2022.

ORPHAN DRUG ACT

Exclusivity

The FDA grants **market exclusivity for 7 years to the first sponsor** of an orphan drug that receives FDA approval for a designated rare disease or condition.

Tax Credit

A sponsor may claim **tax credits of up to 25%** for expenditures incurred during the clinical trials. The tax credit allows companies to save money on qualifying clinical trial costs that they can then use for further development of their therapy.

Waiver of Prescription Drug User Fees

The sponsor's fee as outlined in the prescription drug user fee agreement at the time of submitting a marketing application to FDA may be **waived** for a designated rare disease product, **reducing costs** associated with the FDA approval process for a new orphan drug. Waivers of **PDUFA fees**, like the tax credit, allow companies to put the money they save towards **developing** new therapies.

Substantial unmet need remains for children with rare diseases. Policy makers should ensure that the Rare Pediatric Disease Priority Review Voucher program is made permanent to ensure that these needs are met.[3]

RARE PEDIATRIC DISEASE PRIORITY REVIEW VOUCHER PROGRAM

Priority Review

FDA can award **Priority Review Vouchers** to sponsors of rare pediatric disease product applications. Under this program, a sponsor who receives an approval for a drug or biologic to treat a "rare pediatric disease" may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product.

Once redeemed, FDA aims to speed the length of time that it takes to review an application by **4 months.** When a priority review voucher is issued, the drug developer pays a priority review voucher fee in addition to a user fee to FDA in order to support resources needed for review of the product.

Quick Facts

Rare diseases are defined as those which afflict **200,000** or fewer patients in the U.S.

The National Institutes of Health recognizes more than 7,000 rare diseases and medical disorders. **30 million** Americans (one in 10) suffer from rare diseases and disorders.

Only 4% of patients suffering from rare diseases have available treatments.

It is estimated that approximately **50%** of rare diseases impact **children — 30% will die before the age of 5.**

1. Section 529 describes the pediatric population as from birth through 18 years
2. Hwang et al, Health Affairs; Vol. 38, No. 2, 2019
3. Kimmel L, Conti RM, Volerman A, et al. Pediatrics. 2020;145(4)