Biotechnology Innovation Organization

RARE PEDIATRIC DISEASE PRIORITY REVIEW VOUCHER PROGRAM: INCENTIVIZING THE DEVELOPMENT OF THERAPIES FOR RARE PEDIATRIC DISEASES

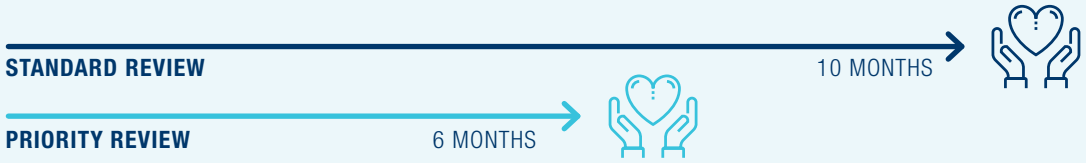
The Rare Pediatric Disease Priority Review Program (RPD PRV) was established to incentivize the development of therapies to treat rare pediatric diseases. **The RPD PRV achieves this goal by granting a priority review voucher to a drug developer who develops and gains approval for a therapy that treats a rare pediatric disease.**

PROCESS FOR PRIORITY REVIEW VOUCHERS

1	**Obtaining Rare Pediatric Disease Priority Review Designation**	A company may request pediatric rare disease designation before the submission of the product application	FDA assesses if the product is intended to treat a rare, serious or life-threatening disease in which the serious or life-threatening manifestation(s) primarily affect individuals aged from birth to 18 years, among other criteria	FDA awards rare pediatric disease designation	Upon approval of the therapy for the rare pediatric disease, a voucher is issued
2	**Using Rare Pediatric Disease Priority Review Voucher**	A company has a PRV from a previous drug approval or purchases a PRV from another company	Company informs FDA of intent to use the voucher on an upcoming drug submission and pays both standard FDA user fees and a special voucher fee to review the product under priority review	FDA accepts voucher and aims to review the drug within six months	

Source: https://www.raps.org/regulatory-focus/news-articles/2017/12/regulatory-explainer-everything-you-need-to-know-about-fdas-priority-review-vouchers

Standard drug review takes approximately **10 months** whereas a priority review accelerates the FDA's review to **6 months**, without altering standards for approval.



> "The PRV transformed us from a clinical stage company to a commercial organization, allowing us to reinvest in resources to advance our other rare disease drugs in the pipeline."
>
> **RARE PEDIATRIC DISEASE DRUG DEVELOPER**

> "The availability of the PRV program for our product supported the decision to move the product to commercialization."
>
> **RARE PEDIATRIC DISEASE DRUG DEVELOPER**

Since the establishment of the RPD PRV in 2012, 21 therapies have been approved for the treatment of 18 rare pediatric diseases, including:

- Morquio A syndrome
- Hypophosphatasia
- Duchenne muscular dystrophy
- Biallelic RPE65 mutation-associated retinal dystrophy
- Primary haemophagocytic lymphohistiocytosis (HLH)
- Heriditary orotic aciduria
- High-risk neuroblastoma
- LAL deficiency
- Batten disease
- Cystic fibrosis
- X-linked hypophosphatemia (XLH)
- Spinal muscular atrophy (SMA)
- Rare bile acid synthesis disorder
- B-cell acute lymphoblastic leukemia
- Lennox-Gastaut syndrome or Dravet syndrome
- Adenosine deaminase-severe combined immunodeficiency (ADA-SCID)
- Mucopolysaccharidosis type VII (MPS VII, Sly Syndrome)

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THE RARE PEDIATRIC DISEASE PRIORITY REVIEW VOUCHER ENCOURAGES DEVELOPMENT OF INNOVATIVE THERAPIES FOR RARE PEDIATRIC PATIENTS

The Rare Pediatric Disease Priority Review Voucher Program is Important for Pediatric Patients.
The number of treatments for rare pediatric diseases has increased over the past decade, however, substantial unmet need for treatment options remains for most children with rare diseases.

Today, half of rare diseases affect children, and it is estimated that **95% of the ~7000 identified are diseases have no treatment options.**

There were **402 FDA-approved orphan drug indications** between 2010 and 2018, of which **42 (10.4%)** were approved for children only, **247 (61.4%)** for adults only, and **113 (28.1%)** for both children and adults. Thus, there is a clear need to continue to encourage drug development for rare pediatric diseases.

Source: Kimmel, L., Conti, Rena., Volerman, A., Chua, KP. (2020). Pediatric Prahn Drug Indications: 210-2018.

> "We and other innovative drug manufacturers are continuously evaluating our product pipeline. The potential to obtain a voucher can help attract institutional investors, especially important for pre-market companies, because it can be transferred and the proceeds can be used to help speed product commercialization, which drives the potential return on investment."
>
> **RARE PEDIATRIC DISEASE DRUG DEVELOPER**

Legislative Action Required!

FDA may not award any rare pediatric disease priority review vouchers after September 2020, unless the drug that **is designated** as a drug for a rare pediatric disease and receives marketing approval by September 2022. **BIO calls on Members of Congress to make permanent this important incentive so that the most vulnerable among us are given a fighting chance.**

Therapies that have been awarded an RPD PRV have been among the most innovative. These novel technologies include **gene therapy, enzyme replacing therapies, and monoclonal antibodies, among others.** The PRV has been a powerful incentive in moving companies toward innovating in these novel and unique ways that will have far reaching implications for drug development long after their initial use.

> "The sale of the PRV provided us with an important source of nondilutive capital to help advance our pipeline of rare and ultra-rare therapies, accelerating the availability of these potential therapies to patients. The PRV transformed us from a clinical stage company to a commercial organization, allowing us to reinvest in resources to advance our other rare disease drugs in the pipeline."
>
> **RARE PEDIATRIC DISEASE DRUG DEVELOPER**