FDA Priority Review Voucher (PRV) Program: What PWS Advocates Need to Know

Overview

The FDA Priority Review Voucher (PRV) Program incentivizes the development of treatments for serious and underserved diseases. It grants a transferable voucher for expedited FDA review to drug sponsors who develop treatments for specific conditions, including rare pediatric diseases like Prader-Willi syndrome (PWS).

Timeline & Legislative Milestones

- **2007:** PRV Program launched via FDA Amendments Act for neglected tropical diseases (NTDs).
- **2012:** Expanded under FDA Safety and Innovation Act (FDASIA) to include rare pediatric diseases.
- **2014:** Extended to medical countermeasures (e.g., biodefense).
- **2020:** Consolidated Appropriations Act reauthorized pediatric PRV program
- **Expired December 2024 when it was cut from the Big CR**

How the PRV Program Works

- Companies that develop approved therapies for qualifying rare pediatric diseases receive a **voucher**.
- This voucher allows **priority review** (6 months instead of 10) for another drug application.
- Vouchers can be **sold or transferred**—some have been sold for over $150 million.
- There is ZERO cost to taxpayers

Why It Matters for PWS

- PWS is a complex rare pediatric disorder with **limited approved treatments for its core symptoms.**
- The PRV program is a **critical incentive** for biotech firms to invest in drug development for PWS.
- Currently, there are **two Phase 3 clinical trials** underway to treat PWS—led by **Harmony Biosciences** and **Acadia Pharmaceuticals**. Both companies entered the PWS R&D space using PRVs, demonstrating the program's direct impact on treatment development.
- Without this program, **limited market size** and high R&D costs discourage innovation.

Call to Action

- **Preserve and strengthen** the PRV program to ensure continued drug development in rare pediatric diseases.
- **Eliminate sunset clauses** that create uncertainty and stall long-term investment.
- **Support legislation** that promotes permanent authorization of the PRV program.

Key Takeaway: The PRV program is one of the few proven incentives to drive rare disease innovation. For the PWS community, its continuation is not optional—it's essential.