The U.S. FDA Rare Pediatric Disease Priority Review Voucher Program

A Brief Overview

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FDA Priority Review Voucher (PRV) Program

Goal: Incentivize drug development

- Food and Drug Administration Amendments Act (FDAAA) of 2007
- Builds upon the success of the Orphan Drug Act (1980)
- Originally targeted Neglected Tropical Diseases
- Since expanded to include rare pediatric disease (2012) *Created under the Food and Drug Administration Safety and Innovation Act (FDASIA) and specifically targets the need for additional therapies for rare pediatric diseases. FDASIA defines a "rare pediatric disease" as one which "primarily affects individuals aged from birth to 18 years, including age groups often called neonates, infants, children and adolescents," and is a rare disease according to federal statute (200,000 persons in the US or fewer).*

Rare Pediatric Disease Designation (RPDD)

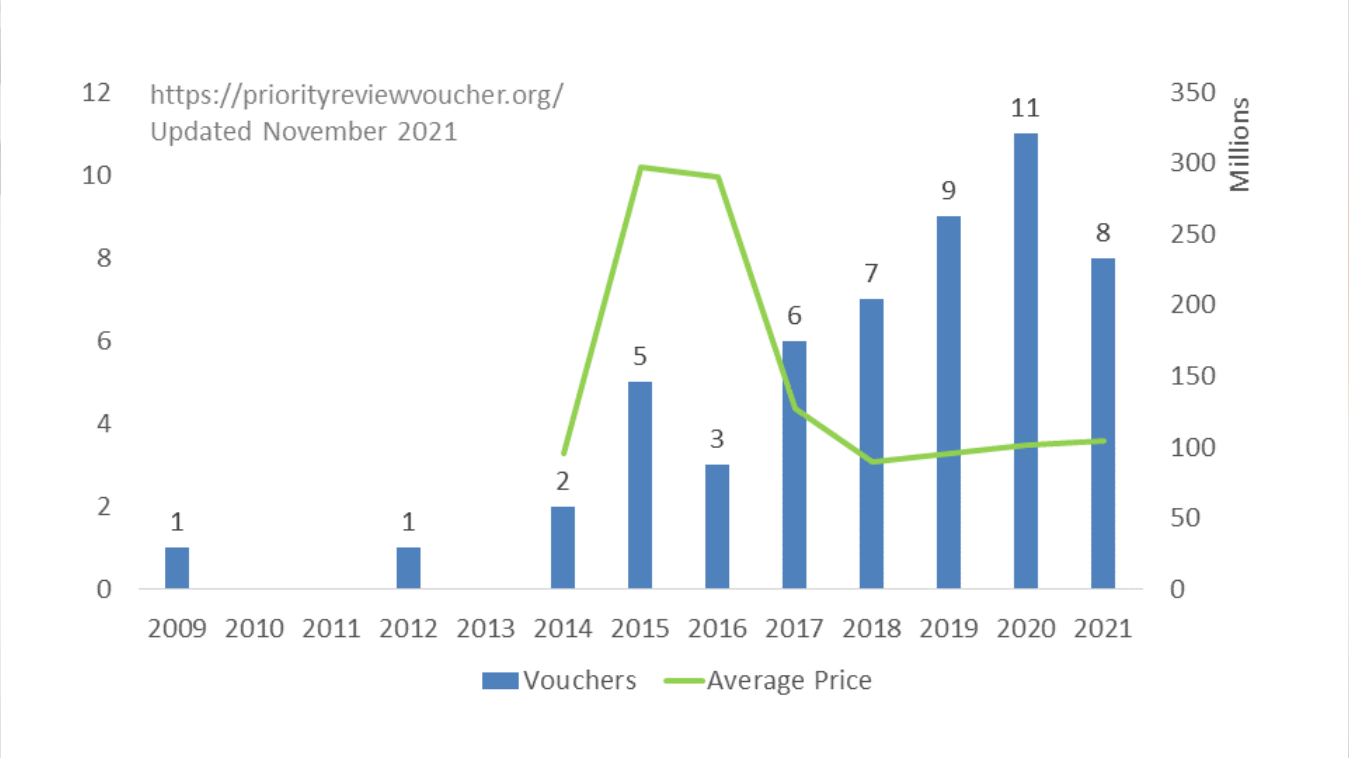
- Criteria for RPDD: Similar to Orphan Drugs but 18 years and younger (neonates, infants, children, and adolescents)
- RPDD Application submitted to FDA Office of Orphan Products Development (OOPD)
- RPDD request must be submitted within 2 weeks of submitting an Orphan Drug Designation (ODD) Application (Sponsor receives benefits from both RPDD & ODD programs)
- FDA makes decision on request no later than 60 days after submission.

Characteristics of PRVs

- May be redeemed to expedite review of subsequent New Drug Application (NDA) or a Biologics License Application (BLA) for another product
- May be sold to another sponsor for use in the same manner.
- Because PRVs may be sold, a secondary market for the vouchers has emerged

Priority Review Voucher (PRV) Market

Price: Depends on supply & demand
Value: Potential sales, patent life, and competitive benefits
Expiration: PRVs do not expire



Some Recent Pediatric Voucher Sales

- BioMarin Announces Sale of Priority Review Voucher.
  Undisclosed purchaser will pay BioMarin **$110 million**.
  Feb 9, 2022 | Source: BioMarin Pharmaceutical Inc.
- Mirum Announces Sale of Priority Review Voucher.
  Undisclosed purchaser will pay Mirum **$110 million**.
  Nov 17, 2021 | Source: Mirum Pharmaceuticals, Inc.
- Rhythm Pharma Announces Sale of Priority Review Voucher
  Alexion (AstraZeneca) will pay Rhythm **$100 million**.
  January 05, 2021 | Source: Rhythm Pharmaceuticals, Inc.
- Sarepta Therapeutics Announces Sale of Priority Review Voucher
  Gilead will pay Sarepta **$125 million**.
  Sep 21, 2020 | Source: Informa Pharma Intelligence
- Lumos Pharma Announces Sale of Priority Review Voucher.
  Merck will pay Lumos **$60 million**.
  July 27, 2020 | Source: Lumos Pharma, Inc.

Some Limitations on PRVs

- PRV can only be sold once
- There is an FDA fee required to redeem the voucher*

  Range: $4.6MM (2011) - $1.3MM (2022)
- Does not alter FDA standards of drug approval
- Awardee must market the drug within 365 days of approval
- Contain no API that has been approved in any other FDA application
  - Approved/Used in other countries okay, as long as not FDA approved for US.
  - Combination products okay as long as contains an API not previously FDA approved.

*These fees are in addition to the required Prescription Drug User Fee Act (PDUFA) fees.

References

- Priority Review Vouchers

https://sites.fuqua.duke.edu/priorityreviewvoucher/

- Rare Pediatric Disease Priority Review Vouchers

https://www.fda.gov/regulatory-information/search-fda-guidance-documents/rare-pediatric-disease-priority-review-vouchers

- Tropical Disease Priority Review Voucher Program

https://www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/tropical-disease-priority-review-voucher-program

- Regulatory Explainer: Priority Review Vouchers

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

- Wikipedia: Priority Review Voucher

https://en.wikipedia.org/wiki/Priority_review