

PDUFA Needs to be More Orphan-Friendly

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Statutory Provisions...

- Companies do not pay one-time application user fee for orphan drugs
- Orphan drugs qualify for waivers of annual product and facility user fees if:
 - Public health need or bar to innovation, or
 - Company qualifies as a small business
- Waivers may be partial or complete

1992 Interim Guidance...

- NDA holder is the “entity subject to user fee”
- Defines “small business” to be \$10M annual gross revenue
- Reflects pre-implementation concerns of FDA that waivers would be abused

Some Orphans Pay User Fees...

- No waiver is granted if NDA holder is a company with more than \$10 million in revenue
- US licensee is evaluated based on its own revenue, the revenue of the NDA holder and all affiliates of the NDA holder

Congressional Intent...

- “The bill’s sponsors do not intend for the fees authorized under this act to serve as a disincentive to the research and development of important prescription drug products, nor should the fees impose an undue financial burden on any company.”

Joint Statement of Senators Orrin Hatch and Edward Kennedy
Prescription Drug User Fee Act of 1992
October 7, 1992

Dr. David Kessler...

- “There is no way we would expect them to pay these fees.”

Statement to the Senate Labor and Human Resources Committee
in 1992 in response to a question about developers working on
orphan drugs for a disease that affects only 60 children

Case Study: Tyrosinemia Type I...

- Rare genetic metabolic disorder
- More than 100 cases documented
- Failure to thrive, fever, vomiting, bruising, enlarged liver
- Can lead to acute life-threatening liver failure
- Orphan drug, along with dietary restrictions, offsets deficiency

Impact on Rare Disease Patients...

- Daypro®

- 989,453 patients
- \$335,000 user fees
- **33¢ per patient per year**

- Orfadin®

- 65 patients
- \$335,000 user fees
- **\$5153.85 per patient per year**

Scott Levin Prescription Drug & Diagnostic Audit and
2005 Redbook

Impact on Orphan Drug Development...

- Reduces the incentive to develop orphan drugs for very small patient populations
- Reduces funds available for post-market studies
- Reduces funds available for other clinical research into rare diseases

NORD Solution...

- Any orphan designated product that does not qualify for a complete waiver of product and facility user fees under the current user fee guidance of the FDA, shall nonetheless be granted a complete waiver of such fees by the FDA, IF it meets all of the following conditions:

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1. Must be an FDA designated orphan drug approved by the FDA for the designated indication
 2. Orphan drug had US sales in the previous year of less than \$25 million for the active moiety, for all indications, dosage forms, and strengths for which the drug is approved as well as for any off-label uses.

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3. Meets the current public health requirement of PDUFA waiver standards
 4. The company responsible for the user fee for the product applies for the waiver in the manner and timeframe specified by the FDA

And finally...

- The FDA shall accept and act upon such application without regard to whether the company responsible for the payment of the user fee is also the NDA holder

Summary...

- Problem
 - Current 1992 Guidance is a barrier to innovation and disincentive to conduct clinical research on rare diseases
- Solution
 - Remove barriers to innovation and support discovery research that is so critical to NORD and the rare disease community