Office of Orphan Products Development
Food and Drug Administration

Uncommon Incentives to fight Uncommon Diseases

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The Orphan Drug Act Signed in 1983

Established the public policy that the Federal Government could/would assist in the development of treatment for rare diseases

What is an "Orphan Drug"?

- **Regulation 21 CFR 316.20:**
  - **A drug to treat a rare disease/condition affecting < 200,000 people in the US**
  - **A vaccine, a preventive drug, or a diagnostic drug to be administered to < 200,000 persons per year**
  - **A drug with no expected profitability**

The Worldwide Orphan Disease Problem

- "Between 10 and 20 million Americans suffer from one of the approximately 5,000 identified rare diseases.

  (National Commission on Orphan Diseases - 1989)

- In Europe, there may be 25 to 30 million citizens affected by rare diseases.

Characteristics of an Orphan Disease (U.S. Experience)

- Rare
- 50% Pediatric
- 85% Serious/life threatening
- No effective treatment available
- Heterogeneous

MISSION

FDA Office of Orphan Products Development (OOPD)

To assist and encourage the identification, development, and availability of safe and effective products for people with rare diseases/disorders.

FDA Office of Orphan Product Development Responsibilities Include

- Providing orphan product designations/incentives
- Administering orphan products grant program
- Providing Humanitarian Use Device Designations (HUDs)

What Are U.S. Orphan-Drug Designation Incentives?

- Seven-year marketing exclusivity for the first FDA approval of a designated drug
- Tax credits = 50% of clinical expenses
- Exemption of User Fees
- Assistance in drug regulatory process
- Orphan products grant funding

Market Impact of Orphan Drug Act Incentives

- Reduced fixed development costs.
- Increased motivation for firms to develop drugs for small populations.
  - *NBER Working Paper No. 9750; June 2003*

Orphan products must be just as safe and effective as other drugs approved by FDA

- Undergo same review standards as non-orphans

Since 1983

- > 1414 active designated orphan products
- 268 Approved orphan products
  - 212 drugs
  - 57 biologics

Unanticipated Benefits

- Building Companies/Orphan Industry
- Growing biotech science
- Increasing number of small-to-medium-sized entities (SMEs)

Orphan Products Research Grants

- **Basic Research**

To support CLINICAL trials

- Includes studies of drugs, biologics, medical foods, and medical devices.
- Determine safety and effectiveness.

Grant Support for Investigation of Rare Disease Treatment

- Supports mainly Phase 1 and 2 trials
- FDA funds approximately 15-20 new grants per year
- Provides $200,000 to $350,000 in total costs per year -- up to 3 years

39 Products Approved Through Research Funded By Orphan Grants Program

Next Application Deadline: March 28, 2006

OOPD Web Site

http://www.fda.gov/orphan

- Office of Orphan Products Development Overview
- Guidelines for designation application
- List of designated and approved orphan products
- Grant application information
- List of ongoing orphan grant studies

The Office of Orphan Products Development, FDA

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