



The Orphan Drug Act and the Development of Products for Rare Diseases

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The Office of Orphan Drug Development
at the FDA works closely and in collaboration
with the Office of Rare Disease Research



Presentation Outline

- Orphan Drug Act (1983)
- “Orphan” drugs and diseases
- Functions of the Office of Orphan Product Development (OOPD)
- Financial incentives of orphan drug status
- OOPD grant program
- Incentive for tropical disease products
- OOPD device regulation



The U.S. Orphan Drug Act (ODA) Signed in 1983

US Congress established the public policy that the Federal Government could/would assist in the development of products for the diagnosis, prevention or treatment of rare diseases or conditions.

What is an Orphan Drug?

- A drug (or biologic) intended to treat a rare disease or condition affecting fewer than 200,000 persons in the United States

or

- A drug (or biologic) which will not be profitable within 7 years following approval by the U.S. Food & Drug Administration

What is an Orphan Disease?

- Affects <200,000 persons in the US
- Affects >200,000 in US, no expectation that therapeutic development costs will be recovered from sales in the US
- 6,000 rare diseases
- Affects 25 million Americans



Principle functions of the FDA Office of Orphan Product Development.

1. Designate drugs as having “orphan status”
2. Award grants for clinical development
3. Regulate orphan devices through the Humanitarian Use Device (HUD) program
4. Serve as FDA’s rare disease focal point with outreach to patient groups and industry

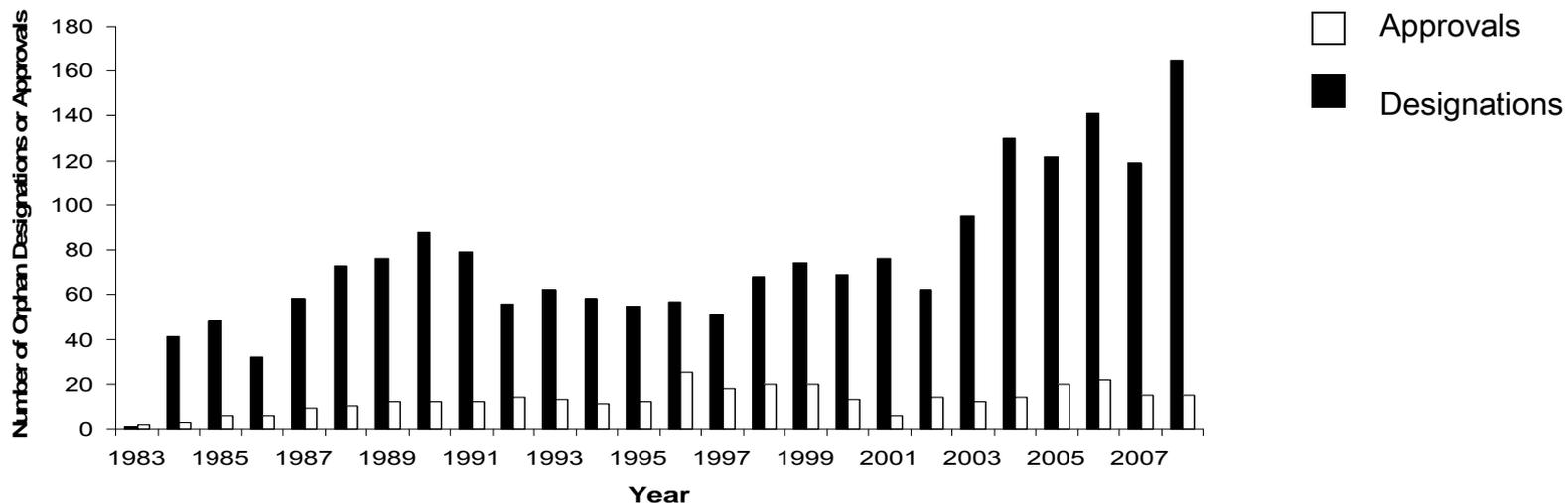


What OOPD Does NOT do...

- Pricing
- Access/Insurance
- Conduct of intramural research
(preclinical or clinical)

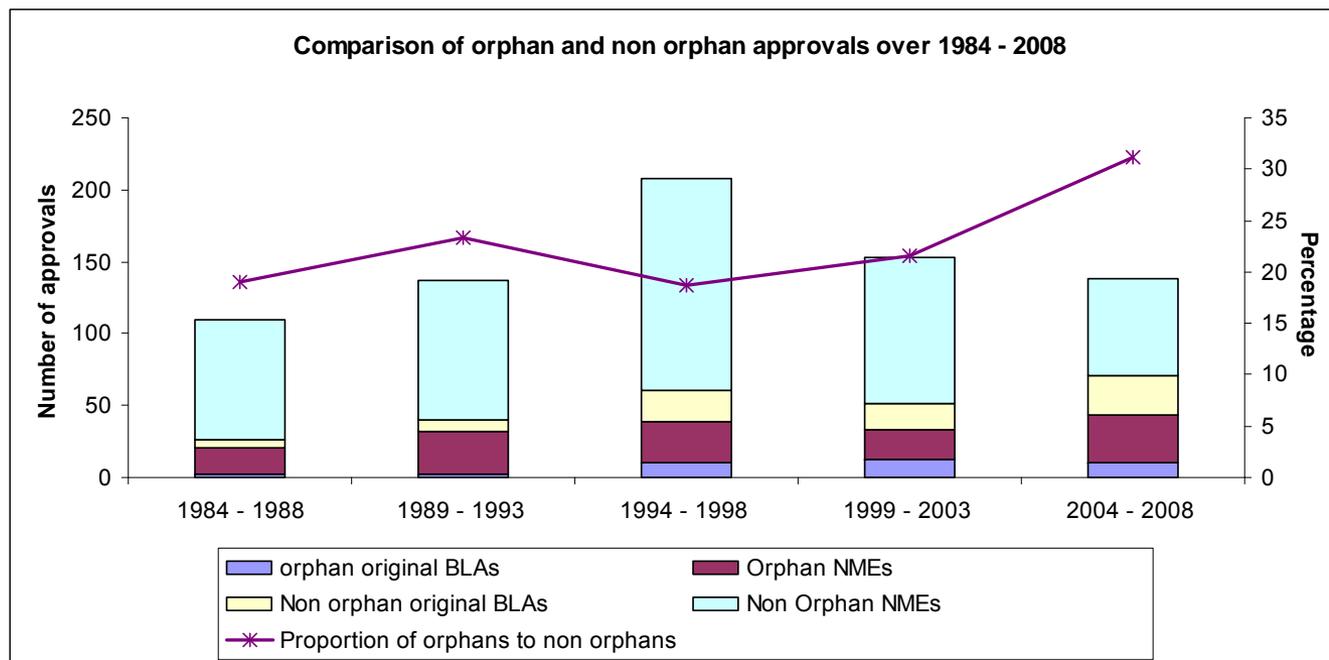
Impact of the ODA on number of orphan designations

- As of 2009, 3030 designation applications
- 2122 orphan product (drugs and biologics) designations

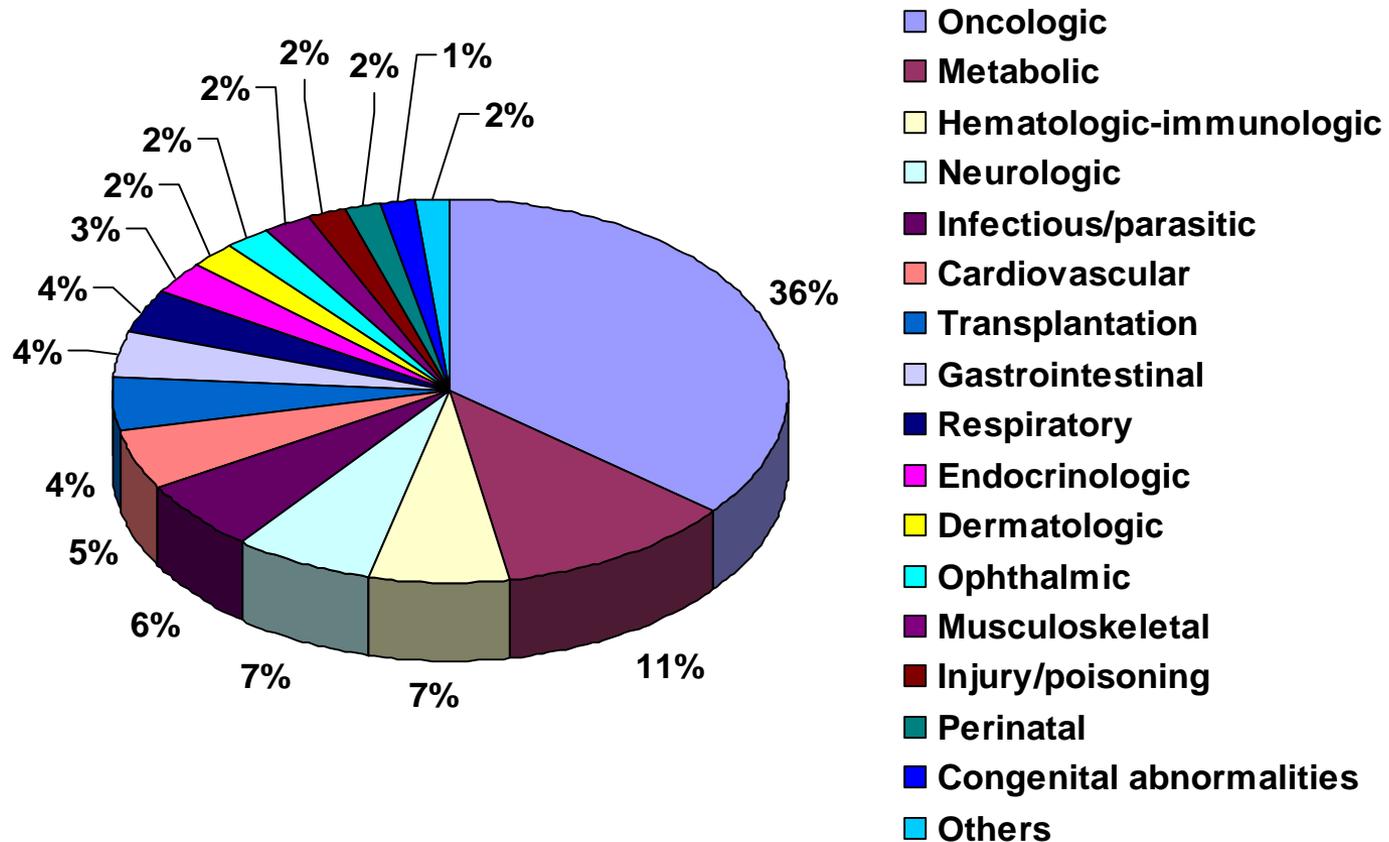


Impact of the ODA on orphan approvals

- 2009: 353 Approved Orphan Products!
- About 1/3rd of all FDA Approvals



Diseases/Conditions Targeted by Designated Orphan Drugs*





What are the Incentives for Orphan-Drug Designation?

- 7-year marketing exclusivity
- Tax credits (up to 50% of clinical development costs)
- Exemption/Waiver of application (filing) fees
- OOPD assistance during the development process



Orphan Products Grants Program

- 90-100 applications per year from domestic or foreign, public or private, for-profit or nonprofit entities
- Fund about 10-20 new grants per year
- Supports academic and industry sponsored research
- Orphan designation is not a grant requirement

Composition of Orphan Grants

- 25% have a Phase 1 component
- 55% have a Phase 2 component
- 20% have a Phase 3 component
- About 25% of funding goes to companies
- About 50% are company/academic institution collaboration



Grants Statistics

- 1983: \$500,000
- To date: \$261 million for 508 grants
- Current annual budget \approx \$14 million

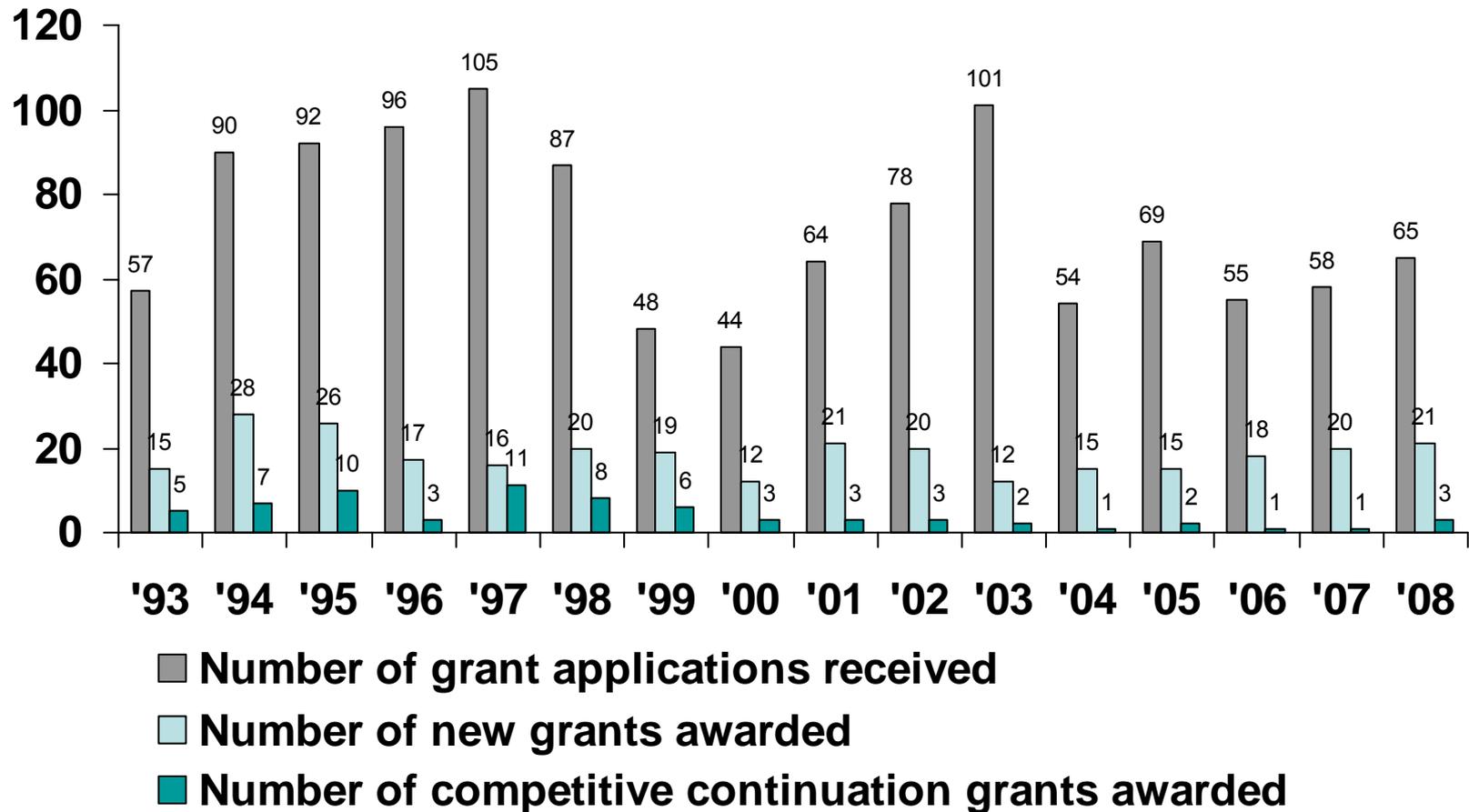


Is the OPD Grant Program Successful?

- YES!
- 44 FDA approved products funded through the OOPD Grants Program
- Generation primary research articles



Annual Number of OPD Grant Applications

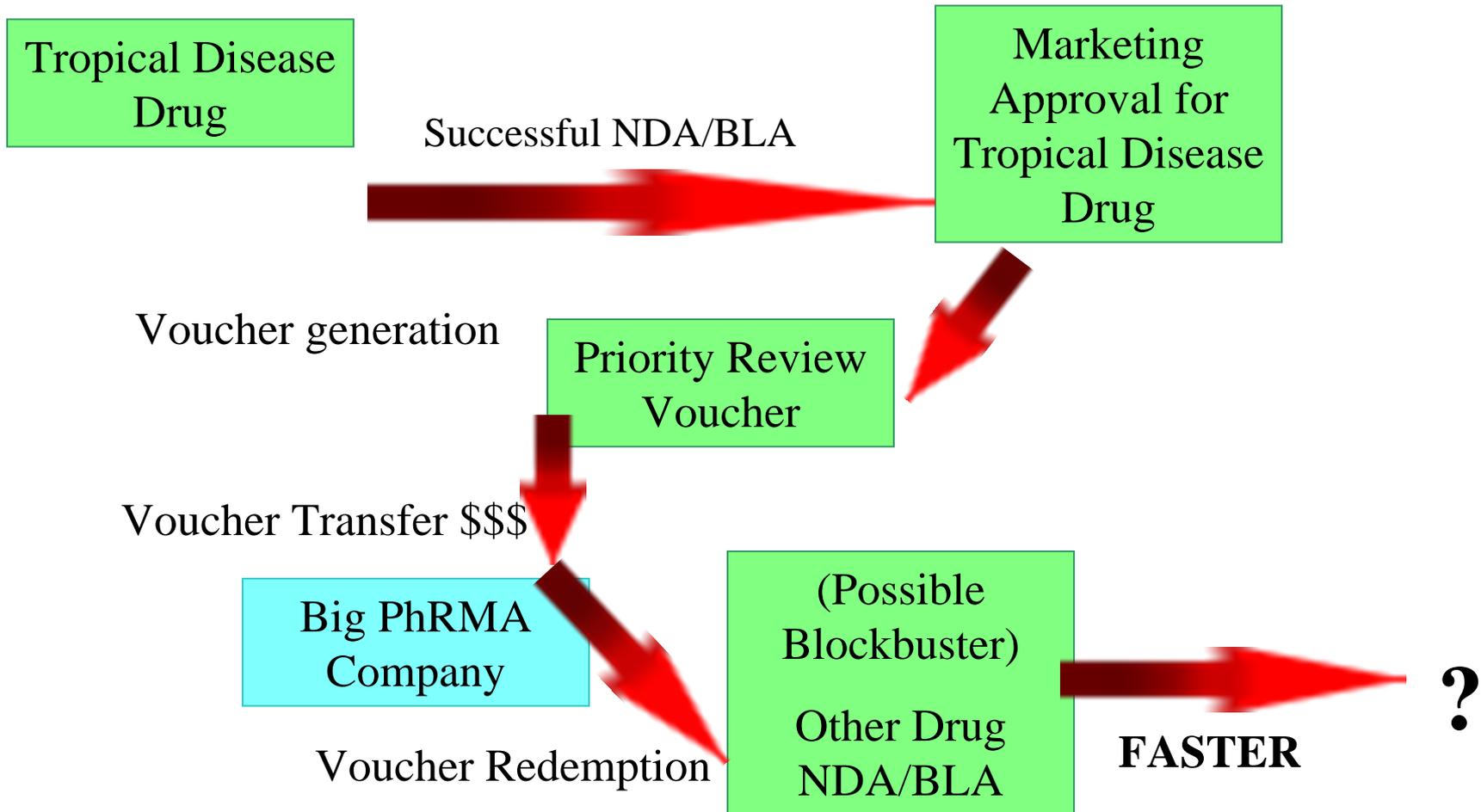




FDAAA 2007 incentive for tropical disease drugs

- Drugs for diseases that are common elsewhere but rare here qualify for orphan status
- Little to no development for past 50+ years
- Priority Review Vouchers (PRVs)

Priority Review Voucher Mechanics





What about Medical Devices?

Humanitarian Use Device (HUD)

- HUD = Device treating a disease affecting <4,000 in the US per year (incidence)
- A HUD then undergoes further FDA/CDRH review to determine if it qualifies for a Humanitarian Device Exemption (HDE)

Humanitarian Device Exemptions (HDE)

- HDE similar to a pre-market approval (PMA) but is exempt from the effectiveness requirement
 - Not for profit (unless pediatric device)
 - Device has to be used with IRB approval
 - No comparable device marketed
- FDA/CDRH approval of a HDE authorizes marketing of the HUD



Accomplishments:

Is the Orphan Drug Act a Success?

- 2100+ designations
- 353 drug and biologic approvals
- 44 product approvals from grants
- ~ 132 HUD requests granted
- 44 HDE approved via CDRH



Questions

