The Evolving Regulatory Landscape for Orphan Drugs: FDA Perspectives

Debra Lewis, O.D., M.B.A.
Acting Director, Office of Orphan Products Development
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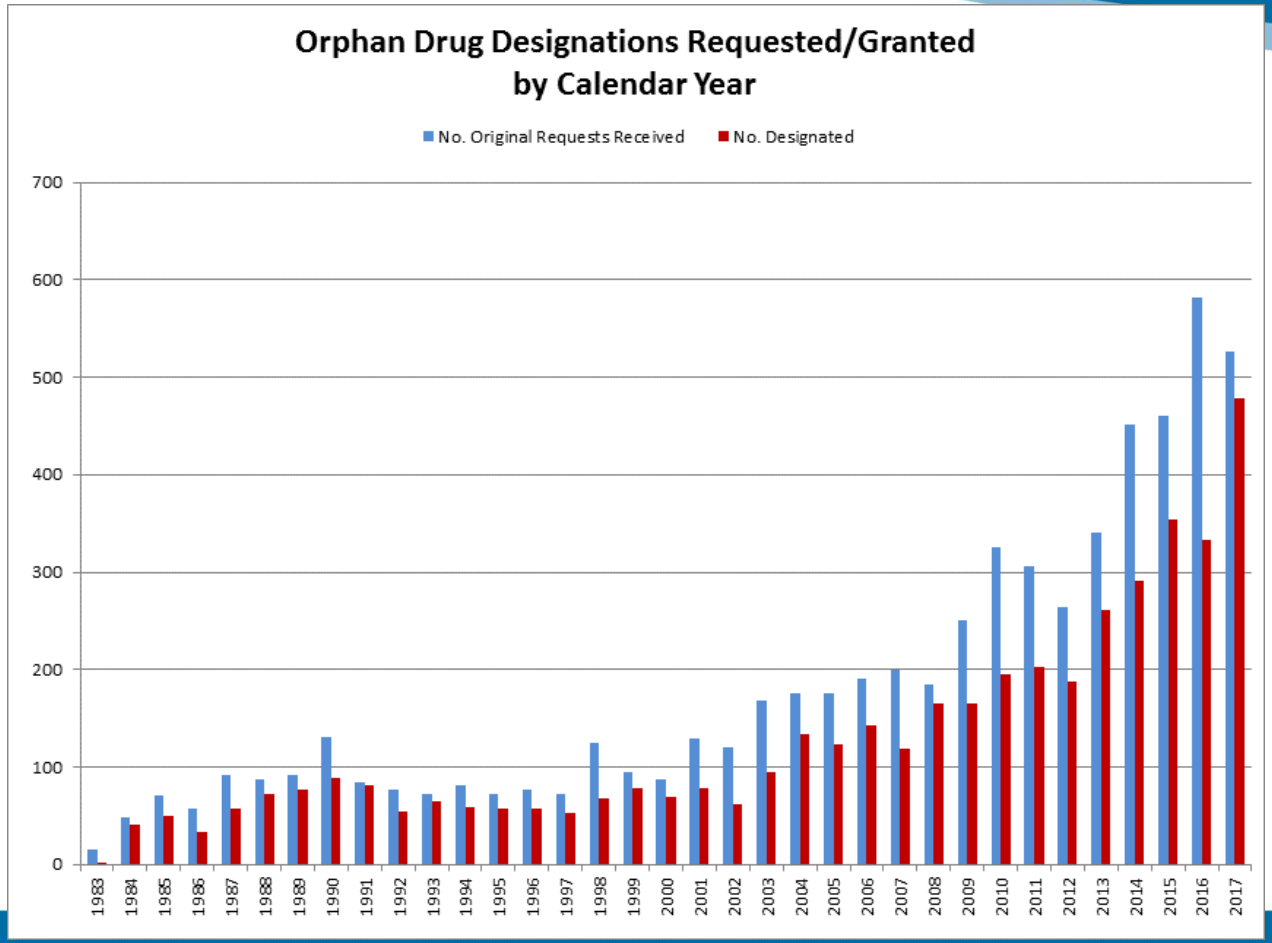
OOPD Core Programs

Mission: To promote the development of drugs, devices, biologics, and medical foods for patients with rare diseases and special populations

DESIGNATION PROGRAMS	
1	**Orphan Drug Designation & Exclusivity**
2	**Rare Pediatric Disease (RPD) Designation** • *New definition- disease or condition must be rare and its serious or life-threatening manifestations must occur in individuals 18 years and younger* • *Co-administer with Office of Pediatric Therapeutics as of May 15, 2017* • *Part of the RPD Priority Review Voucher Program*
3	**Humanitarian Use Device Designation (HUD)** • *Part of the HUD/HDE pathway* • *Disease or condition is not more than 8,000 individuals in the US per year*

GRANT PROGRAMS	
1	**$15M Orphan Products Clinical Trials Grant Program** • *Funding and monitoring 85 rare disease clinical trials*
2	**$6M Pediatric Device Consortia Grant Program** • *Appropriations increased from $3M to $6M in FY2017* • *Funding and monitoring 7 different consortia* • *New funding cycle will be awarded in late 2018*
3	**$2M Orphan Products Natural History Grant Program** • *NIH providing additional $3.5M to fund total of 6 studies*


Orphan Drug Designations Requested/Granted
by Calendar Year
No. Original Requests Received
No. Designated
700
600
500
400
300
200
100
0
1983
1984
1985
1986
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Orphan Drug Modernization Plan Highlights

- Commissioner's June 29, 2017 Blog laid out elements of the Plan
- OOPD elimination of backlog goal accomplished ahead of schedule
- 100% of all new orphan drug designation requests will receive a response by 90 days
- Leveraged inter-center consult process developed to standardize the process for orphan consults; also established the Orphan Council
- Launched the Orphan Drug Designation Request Form and Webinar Tutorial
- Modernization Plan Pilot launched Feb 28, 2018 – Rare Disease Day

21st Century Cures Act

- Rare Pediatric Disease (RPD) Priority Review Voucher Program
  - Extends program: Drugs designated by 2020 may receive voucher if approved by 2022
  - New definition of RPD: (1) rare; and (2) “a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect [children]”
  - GAO study: Results by January 2020
- Amends ODA to clarify that FDA may fund natural history study grants
  - As part of Orphan Products Grant program
- Humanitarian Device Exemption
  - Changes HUD limit from < 4,000 to no more than 8,000 individuals/year in U.S.
  - Requires FDA to issue draft guidance that defines the criteria on establishing “probable benefit” for Humanitarian Device Exemptions by June 2018.

FDARA

- Sec. 502. Pediatric Devices
  - Allows “an appropriate local committee” to approve emergency use HUD.
  - Reauthorizes the PDC Grants program through 2022
- Sec. 504. Development of Drugs & Biologics for Pediatric Cancers (aka RACE Act)
  - Extends PREA to require pediatric studies of certain adult oncology drugs that are directed at certain molecular targets that are relevant to pediatric cancers
  - Updates the PREA orphan exemption so PREA applies to a drug if it is intended to treat an adult cancer with a molecular target that is relevant to pediatric cancer
  - Calls for a public meeting, a 5-year report to Congress and a GAO study, each with questions about the impact of the amendments to PREA on pediatric cancer research and the orphan drug program

FDARA (continued)

- Sec. 505. A Provision on Drug Development for Pediatric Use
  Requires a report within 2 years regarding approved orphan drugs that are lacking important pediatric information related to safety, dosing, and effectiveness.
  - To help improve orphan drug labeling for pediatric populations.
- Sec. 603. Reauthorization: Orphan Grants Program through 2022
- Sec. 607. Orphan Drugs: Clinical Superiority
  - Old: FDA's existing clinical superiority framework for designation and exclusivity.
  - New: Upon designation, requires FDA to notify the sponsor of the accepted plausible hypothesis of clinical superiority.
  - New: Upon orphan exclusive approval requires FDA to publish a summary of the basis of clinical superiority findings.

Patient Engagement Highlights

- Patient Affairs Staff (PAS) created in late 2017: cross-cutting
  - Patient Engagement Collaborative – FRN Dec 2017
  - Cross-Center Patient Experience Listening Sessions - Rare Diseases Pilot NORD MOU
- Center Processes continue to advance
  - Patient Focused Drug Development and PASE
  - Devices Patient Engagement Advisory Committee

Guidance and Policy Highlights

- Orphan Drug Related Guidances:
  - Pediatric subpopulations
  - RPD priority review voucher
  - Orphan Designation Considerations for Tissue Agnostic Oncology

- Tissue Agnostic Therapies in Oncology: Regulatory Considerations for Orphan Drug Designation Public Meeting, May 9, 2018 at White Oak or via WebEx

OOPD Contact Information

For more information on Orphan Drug Designation and other OOPD programs go to:

www.fda.gov/orphan

Still have questions?

Email us at orphan@fda.hhs.gov

Call us at 301-796-8660

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