



U.S. Food and Drug Administration
Protecting and Promoting Public Health



Office of Orphan Products Development

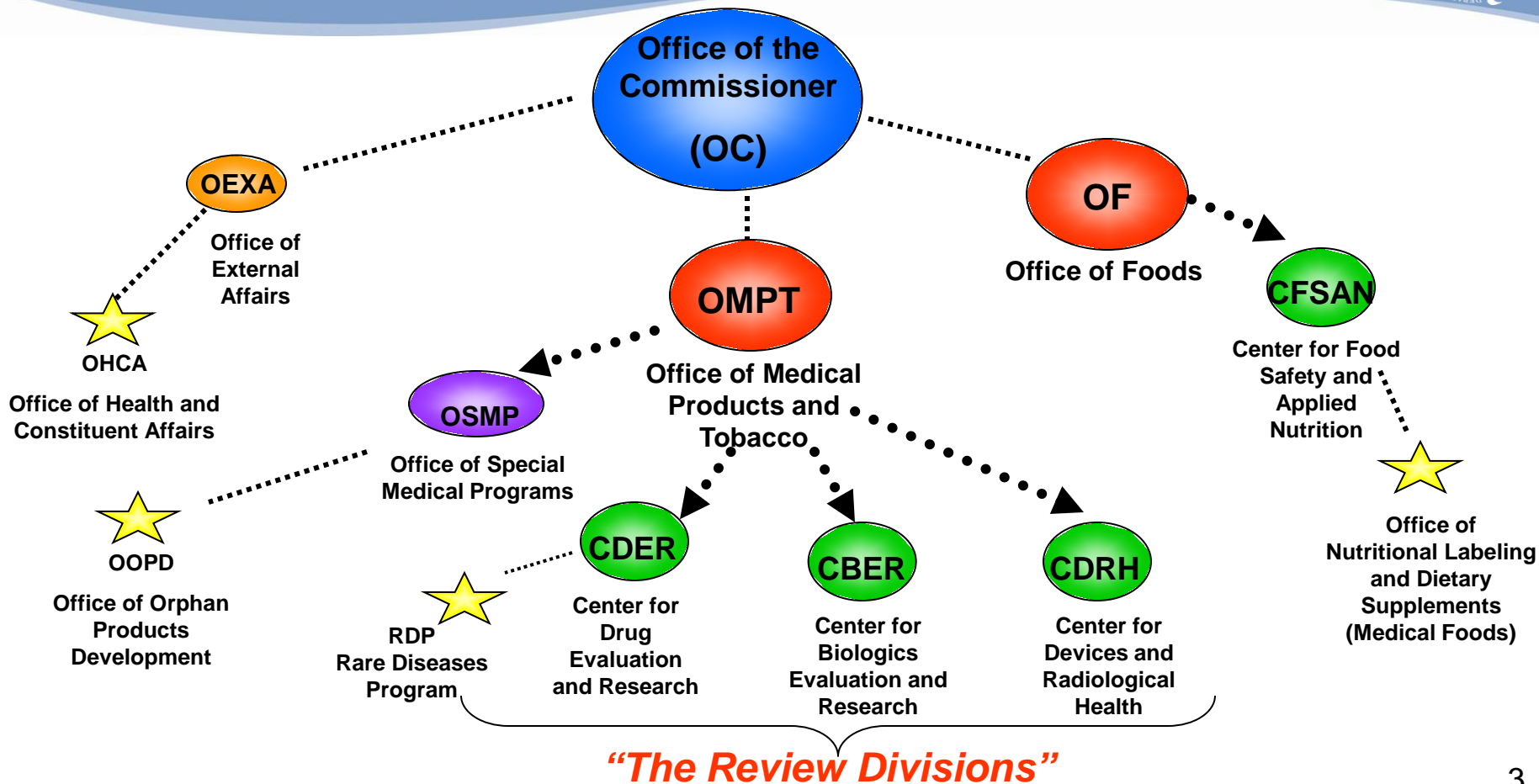
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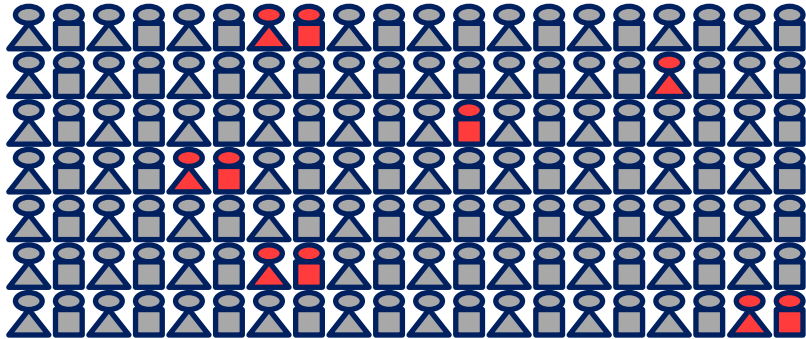


Learning Objectives

- To understand the mission of Office of Orphan Products Development (OOPD)
- To review the programs managed by OOPD
- To understand the legislative basis and evolution of the Humanitarian Use Device (HUD)/Humanitarian Device Exemption (HDE) program



Rare Diseases and Challenges



- ~7,000 known rare diseases
- Only a portion have approved treatments
 - **>500 orphan drug approvals (drugs & biologics)**
 - **69 Humanitarian Use Device approvals**

OOPD Mission Statement

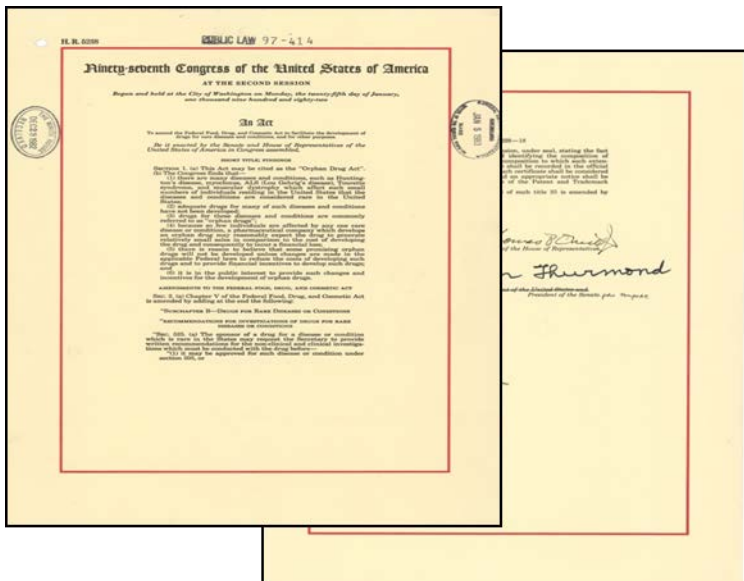
*To promote the development of products, including drugs, devices, biologics, and medical foods, for the treatment, diagnosis, and prevention of **rare diseases and conditions**.*

DESIGNATION PROGRAMS	GRANT PROGRAMS
Orphan Drug Designation	Orphan Products Grants
Rare Pediatric Disease Designation	Orphan Products Natural History Grants
Humanitarian Use Device Designation	Pediatric Device Consortia Grants

OOPD Programs

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Orphan Drug Designation

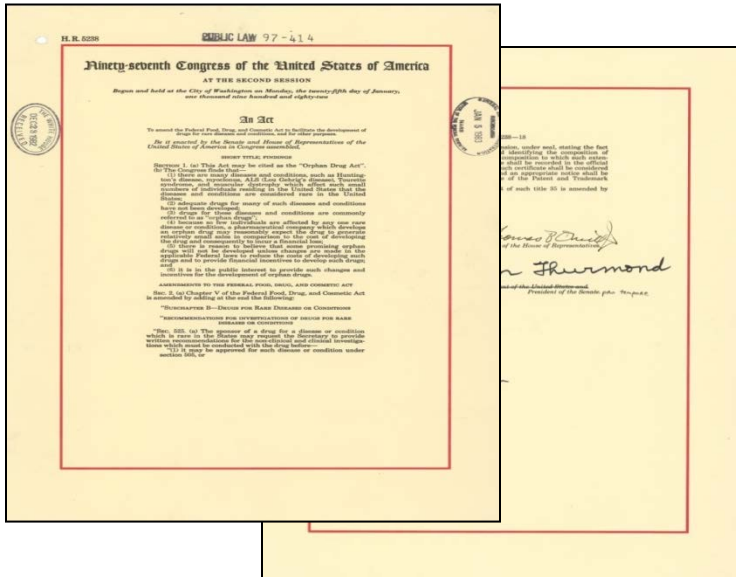


The Orphan Drug Act (ODA) in 1983 was created to motivate industry to develop drugs and biologics for rare diseases by providing financial incentives:

- Tax Credits for 50% of Clinical Trial Costs
- Waiver of Marketing Application User Fee
- Eligibility for 7-Year Marketing Exclusivity

Orphan Drug Act

- Defined “rare disease” for drugs and biologics
 - Disease/condition that affects < 200K people in the U.S.; or
 - Drug that will not be profitable



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Rare Pediatric Disease Designation

- Created under Food and Drug Administration Safety and Innovation Act (FDASIA) of 2012 to encourage development of drugs and biologics for “rare pediatric diseases (RPD)”
- Basic Idea:
 - If a sponsor receives approval of a “rare pediatric disease product application” for a “rare pediatric disease,” the sponsor is eligible to receive a *Priority Review Voucher (RPV)* which can be redeemed, or transferred to another sponsor, to obtain priority review of another application that would otherwise be ineligible for priority review
- Website:
www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/RarePediatricDiseasePriorityVoucherProgram/default.htm

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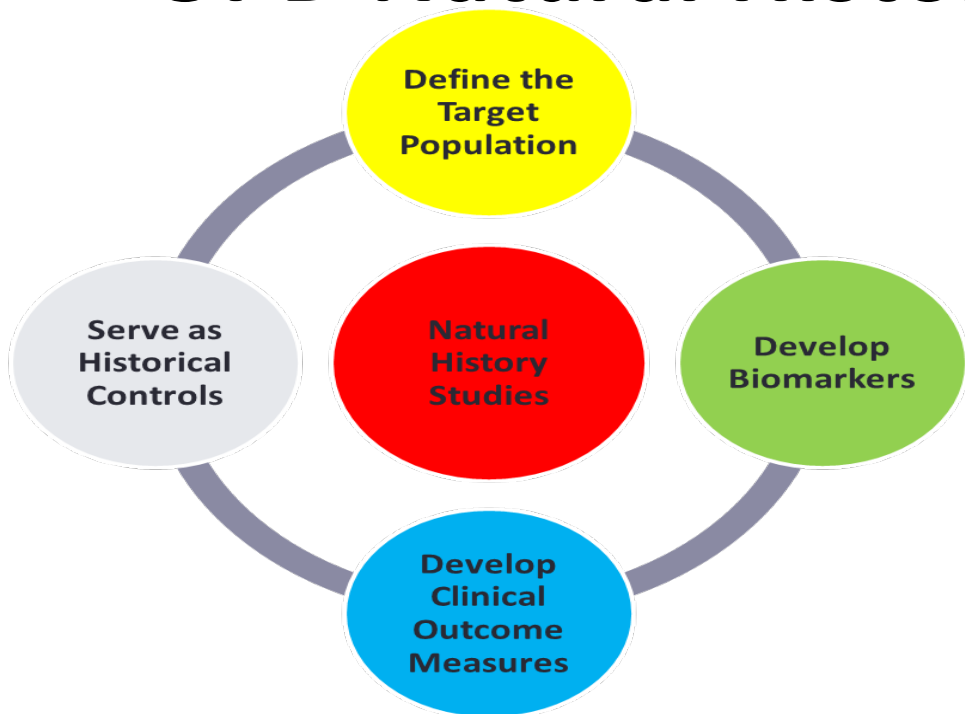
Orphan Products (OPD) Grants

- Encourage clinical development of products for use in rare diseases or conditions
- Applies to drugs, biologics, devices or medical foods
- Grants funding for clinical trials
 - Usually 3 to 4 years
 - 60 to 85 ongoing grant-funded projects
 - Fund 10 to 15 new grants per fiscal year
- Website:
www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/WhomtoContactaboutOrphanProductDevelopment/ucm2005538.htm

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OPD Natural History Grants Program



The ultimate goal is to support marketing approvals

- Help characterize the natural history of a rare disease or condition
- Identify subpopulations based on clinical signs and symptoms or biomarkers
- Develop and/or validate clinical outcome measures, biomarkers, and diagnostics

www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesCondition/OrphanProductsNaturalHistoryGrantsProgram/default.htm

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Pediatric Device Consortia (PDC) Grants

- Goal is to support the development of nonprofit consortia designed to stimulate projects for pediatric devices
- Types of Services and Referrals Provided:
 - Intellectual Property / Legal; Business Planning; Funding Advice; Regulatory Consulting; Preclinical and Clinical Study Planning; Prototyping and Design Services
- Consortia websites:
[www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/
PediatricDeviceConsortiaGrantsProgram/default.htm](http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/PediatricDeviceConsortiaGrantsProgram/default.htm)

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HUD/HDE Program

- **Humanitarian Use Device (HUD)**
 - a medical device intended to benefit patients in the treatment or diagnosis of a disease or condition that affects or is manifested in not more than 8,000 individuals in the United states per year.
- **Humanitarian Device Exemption (HDE)**
 - the premarket submission that allows a HUD to be marketed once a demonstration of safety and probable benefit has been satisfied by FDA.

Section 520(m) of the FD&C Act Humanitarian Device Exemption

“To the extent consistent with the protection of the public health and safety and with ethical standards, it is the purpose of this subsection **to encourage the discovery and use of devices intended to benefit patients in the treatment and diagnosis of diseases or conditions that affect not more than 8,000 individuals in the United States.**”

Law (FD&C Act) Regulation

- 21 CFR 814.100(a):

“...to encourage the discovery and use of devices intended to benefit patients in the treatment or diagnosis of diseases or conditions that affect or are manifested in **fewer than 4,000 individuals in the United States per year.**”

HUD/HDE Program: Legislative History

- **Safe Medical Device Act (SMDA) [1990]**
 - HDE program was created
- **Food and Drug Administration Modernization Act (FDAMA) [1997]**
 - HDE program was revised

HUD/HDE Program: Legislative History

- **Food and Drug Administration Amendments Act (FDAAA) [2007]**
 - intended to motivate development of pediatric devices
 - removed profit restriction if labeled for pediatric use
 - annual distribution number (ADN)
 - requires annual device review by Pediatric Advisory Committee

HUD/HDE Program: Legislative History

- **Food and Drug Administration Safety and Innovation Act (FDASIA) [2012]**
 - expanded exemption from profit prohibition
 - modified ADN definition
- **21st Century Cures Act [2016]**
 - increased population threshold from 4,000 to 8,000
 - modified IRB oversight

21 CFR 814.100(c): Two Step Process

- **Step 1:** Obtain designation of the device as a HUD from the FDA's Office of Orphan Products Development
- **Step 2:** After HUD designation is granted, submit an HDE application to the Center for Devices and Radiological Health (CDRH) or Center for Biologics Evaluation and Research (CBER)

Summary

- OOPD's mission is ***'to promote the development of products, including drugs, devices, biologics, and medical foods, for the treatment, diagnosis, and prevention of rare diseases and conditions'***
- OOPD oversees three designation and three grant programs
- The HUD/HDE program, created in 1990, established a pathway to market for medical devices intended to treat or diagnose diseases that occur in small (rare) populations

How to Reach OOPD

Email: orphan@fda.hhs.gov

Phone Number: 301-796-8660

OOPD Website:

[www.fda.gov/forindustry/
developingproductsforrareconditions/default.htm](http://www.fda.gov/forindustry/developingproductsforrareconditions/default.htm)

Providing Industry Education

1. CDRH Learn – Multi-Media Industry Education

- over 80 modules - videos, audio recordings, power point presentations, software-based “how to” modules
- accessible on your portable devices: www.fda.gov/Training/CDRHLearn

2. Device Advice – Text-Based Education

- comprehensive regulatory information on premarket and postmarket topics: www.fda.gov/MedicalDevices/DeviceRegulationandGuidance

3. Division of Industry and Consumer Education (DICE)

- If you have a question - Email: DICE@fda.hhs.gov
- Phone: 1(800) 638-2041 or (301) 796-7100 (Live Agents 9am – 12:30 pm; 1-4:30 pm EST)
- **Web Homepage:**
www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/ContactUs--DivisionofIndustryandConsumerEducation/default.htm