FDA Rare Disease Updates: Incentives and Resources

Sandra Retzky, DO, JD, MPH
Director, Office of Orphan Product Development
Office of the Commissioner | US FDA

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Learning Objectives

- Describe the **incentive programs** to aid development of drugs targeting rare diseases
- Become aware of **FDA resources** available to help with your programs

What is a “Rare Disease”?

- Defined by law and is different for drugs and biologics versus devices
- Drugs/Biologics:
  - Diseases that affect < 200,000 persons in the US
- Devices:
  - Diseases with an incidence of ≤ 8,000/year in the US

What is an “Orphan Product”?

A product that demonstrates promise for the diagnosis, treatment, or prevention of a rare disease or condition and includes:

Drugs

Biologics

Medical Devices

Medical Foods

Special Challenges

- Genotypic/phenotypic heterogeneity
- Very small, widely-dispersed patient population
- Natural history of the disease is often not well understood
- Complexity in identifying the appropriate endpoints

Incentives

Incentives: Orphan Drug Designation

1. Tax credits to defray costs of qualified clinical studies
2. Waiver of marketing application fees
   - *For FY22, user fees are $3,117,218*
3. Potential eligibility for 7-year marketing exclusivity ("*orphan exclusivity*") upon marketing approval

7-Year Market Exclusivity

- FDA will not approve another “same drug” (i.e., active moiety or molecular structure) for that same use/indication by a different sponsor for 7 years
- Example: if drug A has orphan exclusivity for treatment of cystic fibrosis, FDA could not approve another same drug A for **that disease**. However, FDA could approve other drugs for cystic fibrosis and could approve drug A for other diseases, including other rare diseases

Making a designation request

- Submit prior to marketing application submission
- Describe the **disease/condition**
- **Provide population estimate**—evidence disease is rare
- Support with **sufficient scientific rationale** demonstrating promise or “medical plausibility”

Sufficient Scientific Rationale

- Evidence that the drug holds promise for being effective
- Provide data from:
  - Clinical data, case study reports;
  - Animal models; **or**
  - *In vitro* data (with proposed MOA and pathogenesis of disease when no adequate animal model is available)
- Data from adequate and well-controlled studies are **not** required

After A Designation Request Is Submitted…

- Typical review cycle: **90 days**
- Will either receive:
  - Designation Letter

    OR

  - Deficiency Letter
- Once designated, sponsor is required to submit annual reports until drug is approved

Challenge Question #1

Regarding orphan drug designation, which of the following is NOT true:

A. Must have human data

B. May be an acute or chronic condition

C. Population estimates can come from literature

D. May qualify for tax credits during development

Rare Pediatric Disease PRV Program

- Incentivizes development of therapies for rare pediatric diseases
- Approval of a **"rare pediatric disease product application,"** the sponsor is eligible to receive a PRV which can be redeemed, or transferred, to obtain priority review of another application that would otherwise be ineligible for priority review
- RPD designation does not guarantee voucher award upon approval

Humanitarian Use Device (HUD) Designation

- Treating or diagnosing a disease or condition affecting ≤ 8,000 individuals in the US/year
- Eligible to submit an HDE application
  - Allows a product to be approved based on a showing of safety and probable benefit

Challenge Question #2

Which of the following is true:

A. Incentive programs exist for drugs but not devices

B. The typical review cycle for ODD is 90 days

C. In vitro data is unacceptable for an RPDD request

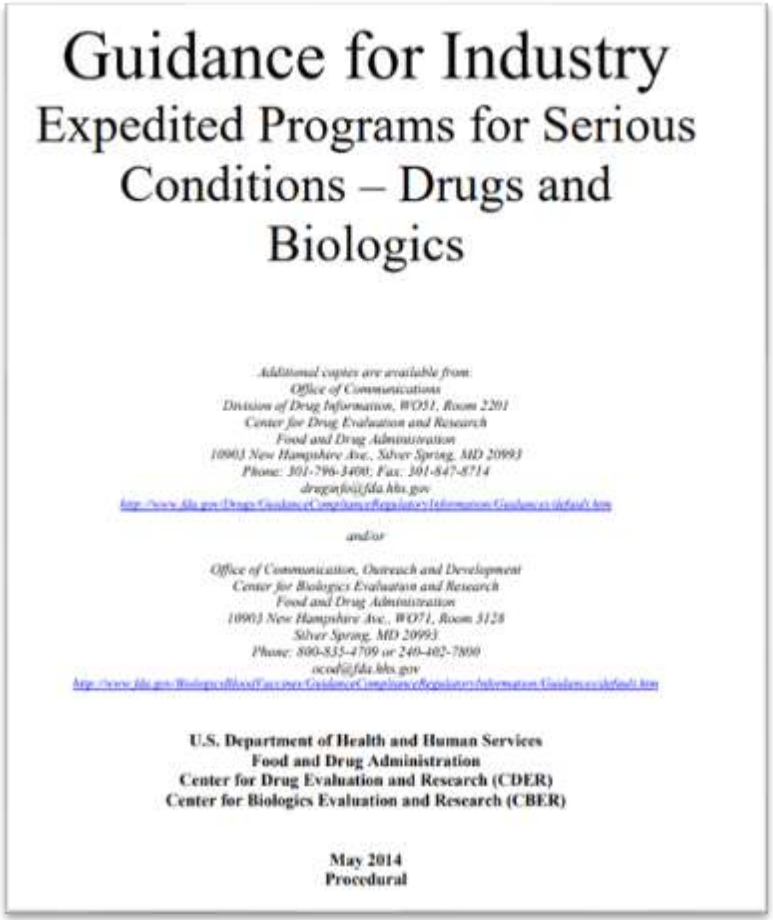
D. Rare pediatric disease vouchers cannot be sold to others

Resources

OOPD Grants Programs

- Clinical Trial Grants Program
- Natural History Grants Program
- Rare Neurodegenerative Disease Grant Program

Expedited Programs



Guidance for Industry

Expedited Programs for Serious Conditions – Drugs and Biologics

Additional copies are available from:
Office of Communications
Division of Drug Information, WO51, Room 2201
Center for Drug Evaluation and Research
Food and Drug Administration
10903 New Hampshire Ave., Silver Spring, MD 20993
Phone: 301-796-3400; Fax: 301-847-8714
druginfo@fda.hhs.gov
http://www.fda.gov/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/default.htm

and/or

Office of Communication, Outreach and Development
Center for Biologics Evaluation and Research
Food and Drug Administration
10903 New Hampshire Ave., WO71, Room 3128
Silver Spring, MD 20993
Phone: 800-835-4709 or 240-402-7800
ocod@fda.hhs.gov
http://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Guidances/default.htm

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

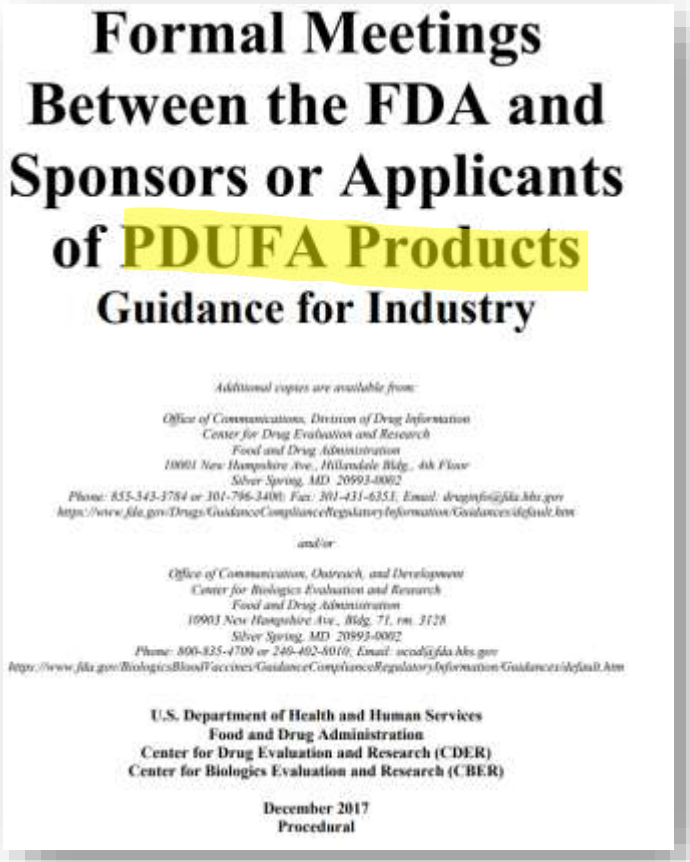
May 2014
Procedural

1. Fast Track
2. Breakthrough Designation
3. Accelerated Approval
4. Priority Review
5. Regenerative Medicine Advanced Therapy

Guidances

- Guidance documents represent FDA's current thinking on a topic.
- They do not bind FDA or the public.
- You can use an alternative approach if the approach satisfies the requirements of the applicable statutes and regulations.
- To find them, best to use a google search.

Meetings with FDA



Formal Meetings Between the FDA and Sponsors or Applicants of PDUFA Products

Guidance for Industry

Additional copies are available from:

Office of Communications, Division of Drug Information
Center for Drug Evaluation and Research
Food and Drug Administration
10001 New Hampshire Ave., Hillandale Bldg., 4th Floor
Silver Spring, MD 20993-0002
Phone: 855-543-3784 or 301-796-3400; Fax: 301-431-6353; Email: druginfo@fda.hhs.gov
https://www.fda.gov/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/default.htm

and/or

Office of Communication, Outreach, and Development
Center for Biologics Evaluation and Research
Food and Drug Administration
10903 New Hampshire Ave., Bldg. 71, rm. 3128
Silver Spring, MD 20993-0002
Phone: 800-835-4709 or 240-402-8010; Email: ocod@fda.hhs.gov
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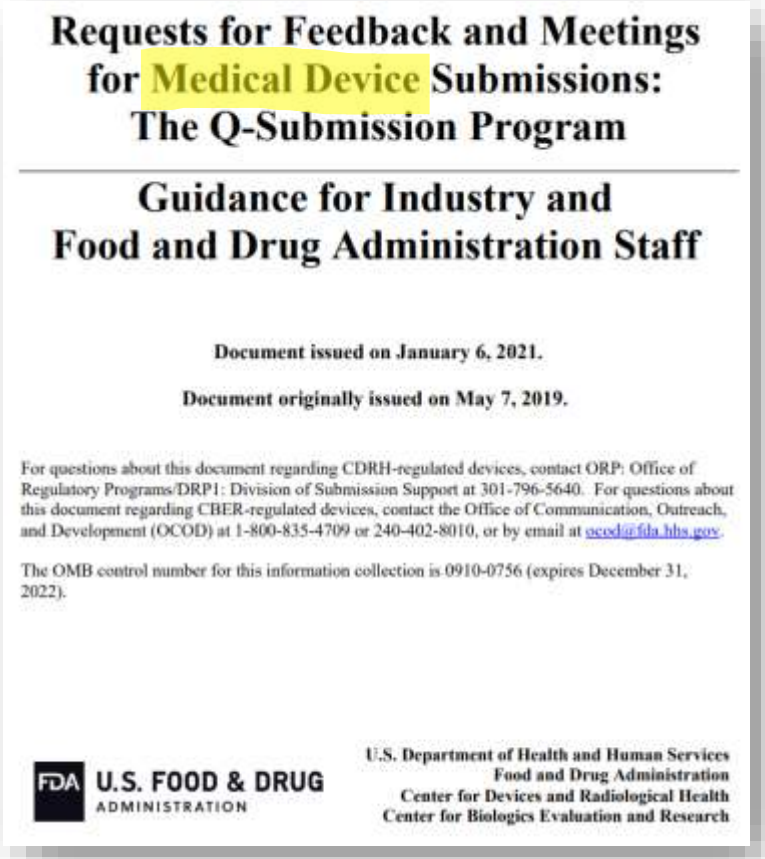
U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

December 2017
Procedural



Requests for Feedback and Meetings for Medical Device Submissions: The Q-Submission Program

Guidance for Industry and Food and Drug Administration Staff

Document issued on January 6, 2021.

Document originally issued on May 7, 2019.

For questions about this document regarding CDRH-regulated devices, contact ORP: Office of Regulatory Programs/DRP1: Division of Submission Support at 301-796-5640. For questions about this document regarding CBER-regulated devices, contact the Office of Communication, Outreach, and Development (OCOD) at 1-800-835-4709 or 240-402-8010, or by email at ocod@fda.hhs.gov.

The OMB control number for this information collection is 0910-0756 (expires December 31, 2022).

FDA U.S. FOOD & DRUG ADMINISTRATION

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Devices and Radiological Health
Center for Biologics Evaluation and Research

Challenge Question #3

Which of the following is true?

A. FDA's guidance documents are law

B. Breakthrough designation is an incentive program

C. FDA grants are only available to academics

D. Orphan drug designations remain active until they are withdrawn or revoked

Save the Date!

FDA's Annual Rare Disease Day Event!

February 27, 2023

Registration opens in mid-January 2023

Closing Thoughts

Incentives and resources are available from FDA—especially for rare disease drug development.

Use them!

Resources

Prescription Drug User Fee Amendments

Orphan Products Grants Program

Expedited programs for serious conditions

Formal Meetings Between the FDA and Sponsors or Applicants of PDUFA Products

Requests for Feedback and Meetings for Medical Device Submissions