

The Role of the Office of Orphan Products Development in the Pediatric Device Initiative



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Orphan Products Pediatrics Overview

FDA Orphan Programs:

1. Orphan Drug Designations
2. **Orphan Products Grants for Devices**
3. **Humanitarian Use Devices**
4. **Pediatric Device Consortia Grant Program**
5. Outreach and Regulatory Facilitation

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OOPD GRANTS PROGRAM (R01)

Orphan Products Grants Program

GOAL of the Grants Program: to encourage clinical development of products, including drugs, biologics, medical devices, or medical foods, for use in rare diseases or conditions affecting < 200,000 individuals in the United States.

Orphan Products Grant Program

- Also, a practical program for advancing marketing approvals and relevant publications that impact on rare diseases

Orphan Products Grants Program

- Approximately 80 applications per year
- Competitive grant program – ~30% success
- Fund about 15-20 new grants per year
- Supports academic and industry sponsored research
- Domestic or foreign, public or private, for-profit or nonprofit entities

OOPD Grant Program

- Request for Application (RFA) available at www.fda.gov/orphan and www.grants.gov
- Next Receipt Date, February 2, 2011
- IND/IDE must be in effect at the time of the grant application submission. This must be active and include the protocol for which funding is being requested.

OOPD Grant Review

- Application, review, and scoring is much like NIH grant application
- Primary review: Grants scored by independent ad hoc expert panels for technical merit
- Second Level review by a National Council (Process Approval)

OPD Grants Program: Budget

- The current annual budget for grant funding is approximately \$14 million
- Clinical trials may be awarded:
 - Phase I trials, up to \$200,000/year for up to 3 years
 - Phase II & III trials, up to \$400,000/year for up to 4 years

Grants Statistics

- To date, since 1983, FDA has provided more than \$260 million for more than 500 grants for studies on rare diseases.
- Current annual budget \approx \$14 million
- 45 FDA approved products were at least partially funded through the OOPD Grants Program.

Approved Products Supported by Grants

- 45 products partially funded by OOPD grants have been approved for marketing
 - Examples:

- **NeuRx RA/4 Intramuscular Diaphragm Pacing Stimulation System:** diaphragmatic control for high level spinal cord injuries.
- **Pulmonary Angioscope:** visualization of pulmonary emboli
- **VEPTR (Vertical Expandable Prosthetic Titanium Rib):** thoracic insufficiency syndrome





**HUMANITARIAN USE DEVICE
(HUD)
DESIGNATION**

Why Humanitarian Device Exemptions?

- Ordinarily, Premarket approval applications for new medical devices must show that products are **safe** and **effective**
- For very rare diseases, FDA will approve such devices if manufacturers demonstrate the **safety** and **probable benefit** to patients.
- This exemption from the effectiveness standard is the HDE provision

HUDs and HDEs

- The Safe Medical Devices Act 1990 authorized FDA to exempt a device from requirements to show effectiveness if:
 - Used in <4,000 people in the U.S. per year
 - Device sold “at cost”
 - PEDIATRIC DEVICES are exempt from this restriction on making a profit
 - Device to be used with IRB approval

HUDS

- Obtaining a HUD designation is the first step for a device to be approved via the HDE pathway.
- HUD designation requests are processed by the OOPD staff

HDE Application – 2 parts

- Request for HUD Designation
 - Office of Orphan Products Development (contact: Eric Chen, 301-796-8660)
- HDE Application Submission
 - CDRH/ ODE (contact: Sheila Brown, 301-796-6563)

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PEDIATRIC DEVICE CONSORTIA

GRANT PROGRAM

Pediatric Device Consortia Grant Program

- FDAAA-Pediatric Medical Device Safety and Improvement Act 2007
 - Establish nonprofit consortia to stimulate pediatric device development
- Not a direct research grant
 - fund nonprofit consortia that **support** pediatric device developers
- Results-driven
 - aimed at moving products forward along the development continuum.
- OOPD is responsible for carrying out the program but:
 - encompasses devices used in all pediatric diseases, not just rare diseases.

Pediatric Medical Device Safety and Improvement Act of 2007

– What is a consortium?

....a network: business; clinicians; manufacturers; engineers

....an information source

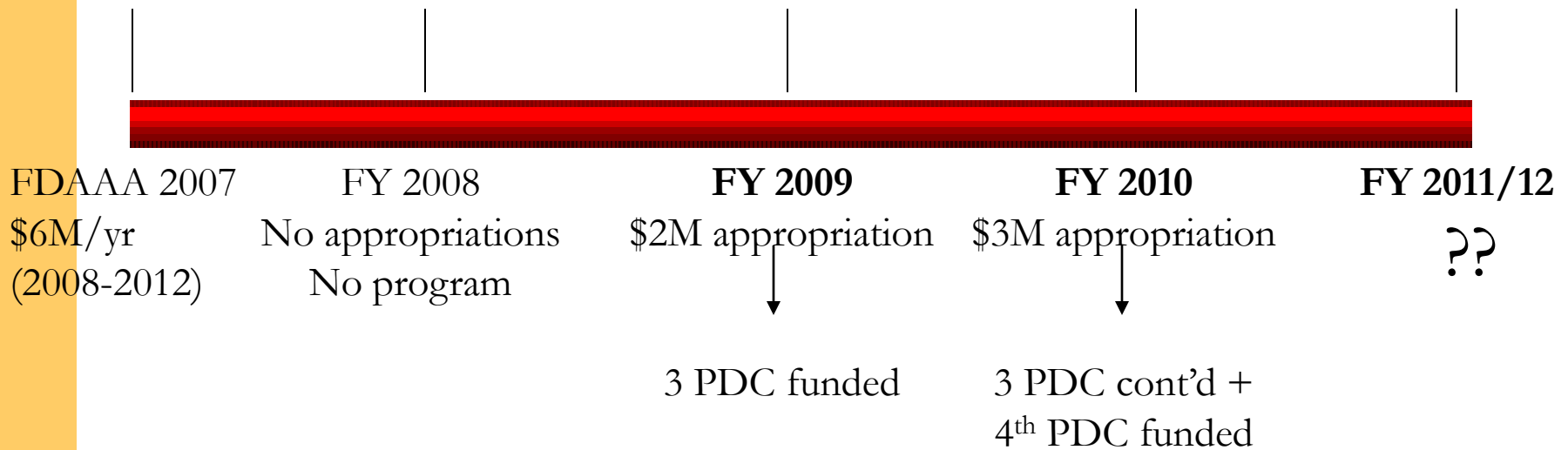


“A nonprofit consortium that receives a grant or contract under this section shall facilitate the development, production, and distribution of medical devices by--

1. Encouraging innovation and connecting qualified individuals with pediatric device ideas with potential manufacturers
2. Mentoring and managing pediatric device projects through the development process, including product identification, prototype design, device development, and marketing.
3. Connecting innovators and physicians to existing Federal Sources and non-Federal Resources.
4. Assessing the scientific and medical merit of proposed pediatric device projects
5. Providing assistance as needed on business development, personnel training, prototype development, post-market needs and other activities consistent with the purpose of this section

Pediatric Device Consortia Grant Program

TIMELINE



WHO?

- James Geiger, MD and the Michigan Pediatric Device Consortium, \$1,000,000/ year.
- Michael Harrison, MD and the UCSF Pediatric Device Consortium, \$500,000/ year.
- Pedro del Nido, MD and the Pediatric Cardiovascular Device Consortium, \$500,000/year.
- Pablo Garcia and the MISTRAL Pediatric Device Consortia, \$500,000/year



Partnerships Fostered

- Pediatric Medical Device Institute and University of Michigan
- MISTRAL Consortium and Institute for Pediatric Innovation

Progress So Far.....



Over **80** pediatric device projects have been evaluated by the pediatric device consortia since October of 2009.