



Humanitarian Use Device (HUD) Program Overview

Katie Chowdhury, MS

Biomedical Engineer/Health Science Administrator
Office of Orphan Products Development
Office of Special Medical Programs
U.S. Food and Drug Administration



Learning Objectives

- To understand why Congress established the Humanitarian Use Device (HUD) designation and Humanitarian Device Exemption (HDE) programs
- To define the term “Population Estimate” and how it applies to different categories of devices
- To understand how HUDs apply to pediatric patients
- To know where to submit an HUD application

21 CFR 814.100(c): Two-Step Process

- **Step 1:** Submit a HUD designation request and receive approval 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)



Humanitarian Use Device (HUD) Program

Why Humanitarian Use Devices?

- In 1990, Congress established the HUD designation and HDE marketing pathway program
 - encourage development of devices intended for rare diseases
- General Requirement for a new device to enter the market:
 - reasonable assurance that the device is **safe** and **effective**
- Under the HUD/HDE pathway:
 - device is **safe** and provides a **probable benefit**

HUD Definition

A Humanitarian Use Device (HUD) is a medical device intended to benefit patients in treatment or diagnosis of a disease or condition that affects or is manifested in not more than 8,000 individuals per year in the United States.

21 CFR 814.3(n)

Guidance Document

- “Guidance for Industry and FDA Staff: Humanitarian Use Device (HUD) Designations”
 - Issued on January 24, 2013

www.fda.gov/downloads/RegulatoryInformation/Guidances/UCM336515.pdf

HUD Designation by OOPD

- Sponsor submits a HUD Designation Request to FDA's Office of Orphan Products Development (OOPD)
 - Two copies
- FDA sends the Sponsor an acknowledgement letter with an assigned HUD number for each application

Content of HUD Request

- **Cover Letter**
 - requesting OOPD consider the device for HUD designation for a rare disease or condition or an ‘orphan subset’ of a disease or condition
- **Description of the Disease or Condition**
 - device intends to treat or diagnose
- **Population Estimate**
 - Calculation of the number of patients affected or manifested with the disease/condition in the United States per year
- **Description of the Device and Scientific Rationale for its Proposed Use**
 - written description and engineering diagrams
 - supporting use of the device; preclinical, clinical, and/or proof-of-principle data pertaining to the device
- **Supporting Documentation with Appended References**
 - demonstrate the device is designed to treat/diagnose a rare disease or condition (or orphan subset) that affects or is manifested in not more than 8,000 individuals per year in the United States

How OOPD Reviews a HUD Request

1. Evaluate disease or condition based upon device functionality
2. Evaluate target population that may benefit from device
 - May broaden (or reduce) scope of patient population as appropriate
 - Target population must be not more than 8,000 individuals/per year

45 day review → FDA issues Decision letter to applicant

HUD Decisions

- **Approval:** Designate the device for the disease or condition
 - Population estimate must be not more than 8,000/year in the U.S.
 - Disease or condition may not always be identical to what was requested by the applicant
- **Disapproval**
- **Request Additional Information**

Disease/Condition

- Evaluate how device works to treat/diagnose the disease or condition
- Evaluate how it performs in the patient population
- Factors examined such as:
 - disease/condition pathogenesis, course, or prognosis; or
 - resistance to treatment
- Designate device for a disease/condition, and not intended use
- May designate the disease/condition to be broader than eventual HDE approved indications for use/label

Prevalence vs. Incidence

- **Prevalence:**
 - Total number of patients with a disease/condition in the population at a given time
 - **example:** number of patients with Cystic Fibrosis on April 1, 2015 in the United States
- **Incidence:**
 - Number of new patients diagnosed with a disease/condition during a particular time period
 - **example:** number of new patients diagnosed with Cystic Fibrosis from January 1 – December 31, 2015 in the United States
- **Generally, FDA relies on Incidence for HUD designation**

Population Estimate: Therapeutic Devices

Number of:

- new patients per year; and
- diagnosed with the relevant disease or condition; and
- potentially eligible for treatment with the device.

Population Estimate: Diagnostic Devices

Number of:

- new patients per year; and
- subjected to diagnosis with device, regardless of test result (e.g., positive or negative).
- **example:** screening of all newborns for a rare disease/condition – does not qualify

Population Estimate: Devices for Repeat or Multiple Use

Number of:

- new patients who would be eligible for, or subjected to, the device in a given year
- not on the total number of expected uses of a device in a given year by all patients.
 - For example, a device to treat both eyes in a given year would count as one patient instead of two.

“Orphan Subset” - Definition

- Is a subset of individuals with a non-rare disease or condition on whom use of device is appropriate
 - where use of the device on the remaining individuals with that disease or condition would be inappropriate
 - For example, some intrinsic feature of the device prevents it from being used in some patients who have a disease or condition and as such those patients are not excluded in the population estimate

“Orphan Subset” Factors - Considered

- **Adverse Event Profile**

- Device is shown to have a serious adverse event profile and should be restricted only to certain patients

- **Mechanism of Action**

- Device characteristics limit its use
 - For example, a blood pump that is too large to fit in the chest cavity of a small person

- **Prior Clinical Experience**

- May show that the device has no significant activity in some patients
- Should only be used in the remaining population with the disease

“Orphan Subset” Factors - Not Considered

- Eligibility of Patients in Proposed Clinical Trial
 - Inclusion/exclusion criteria of a study does not qualify patients as an “orphan subset”
- Sponsor’s Choice to Narrow Indication for Use
 - May not exist just because of a specifically chosen patient population
- Unmet Medical Need
- Standard of Care
- Price

Device Description/Scientific Rationale

- Adequate information to describe the function of the device
- Adequate information to support the use of the device or justify use of the device for an “orphan subset”
 - e.g., preclinical, clinical, and/or proof-of-principle data

Pediatrics

- Younger than 22 years of age
- Device may be eligible if the pediatric population affected by the disease or condition is not more than 8,000 individuals per year
- OOPD will designate a device solely for pediatric use if it qualifies based upon the population estimate

Where to Submit HUD Designation Request

Send two signed and dated submissions. One hardcopy (original) and copy (paper or eCopy):

Office of Orphan Products Development
Food and Drug Administration
10903 New Hampshire Ave.
WO32, Room 5295
Silver Spring, MD 20993

How to Reach OOPD

Email: orphan@fda.hhs.gov

Phone Number: 301-796-8660

Resources

For additional assistance regarding the HUD Program:

- **OOPD website**

www.fda.gov/orphan

- **HUD guidance**

[www.fda.gov/downloads/RegulatoryInformation/
Guidances/UCM336515.pdf](http://www.fda.gov/downloads/RegulatoryInformation/Guidances/UCM336515.pdf)

Summary

- A HUD designation allows for a device intended for use in not more than 8,000 patients per year with a rare disease or condition to be eligible for an alternative pathway to market
- The HUD/HDE Marketing Pathway is a Two-Step Process
- The Sponsor should submit specific documentation to support the HUD request

Summary

- OOPD designates devices for a disease or condition and not for a specific indication or label
- Disease Incidence and Population Estimates are important factors in determining HUD eligibility
- Where appropriate, a device for a non-rare disease or condition may qualify for an orphan subset

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