Humanitarian Device Exemptions

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Objectives

- Overview of HDEs
- Statutory changes re: profit
- 2 Step Submission Process
- HDE Submission Content
- HDE Facts

HDE Overview

- HDE approval authorizes **marketing** of a humanitarian use device (HUD)
- IRB approval required before the device is used (except in emergency situations)
- Labeling must clearly identify device as a HUD, and that effectiveness for that indication has not been demonstrated

HDE Overview

- Device not otherwise available (through a 510(k) or PMA)
- No comparable **device** currently available (through a 510(k) or PMA)
- Device:
  - Does not pose unreasonable risk of illness or injury (i.e., **safety** is demonstrated), **AND**
  - **Probable benefit** outweighs the risk (i.e., exempt from effectiveness requirements of a PMA)

Food and Drug Administration Amendments Act 2007 (FDAAA)

A HUD is eligible to be sold for profit if the device meets the following criteria:

- The device is intended for the treatment or diagnosis of a disease or condition that occurs in pediatric patients or in a pediatric subpopulation, and such device is labeled for use in pediatric patients or in a pediatric subpopulation in which the disease or condition occurs ***and*** was not approved prior to 2007

- Annual Distribution Number (ADN) is based on the number of individuals likely to use the device, and the number of devices reasonably necessary to treat such individuals.

Food and Drug Administration Safety and Innovation Act 2012 (FDASIA)

A HUD is eligible to be sold for profit if the device meets the following criteria:

- The device is intended for the treatment or diagnosis of a disease or condition that occurs in pediatric patients or in a pediatric subpopulation, and such device is labeled for use in pediatric patients or in a pediatric subpopulation in which the disease or condition occurs; ***or***
- The device is intended for the treatment or diagnosis of a disease or condition that does not occur in pediatric patients or that occurs in pediatric patients in such numbers that the development of the device for such patients is impossible, highly impracticable, or unsafe

Also, previously approved HDE are now eligible for ADN

HUD/HDE Submission Process

- Part 1
  - Submit HUD Designation Request to FDA's Office of Orphan Products Development (not CDRH)
- Part 2
  - Submit HDE to CDRH

HDE Submission Content

- Reference to HUD designation letter (granted by Office of Orphan Products)
- Explanation why device would not otherwise be available
- Statement that no comparable device is legally marketed (approved or cleared)
- Device description
- Bench and animal testing

HDE Submission Content

- Clinical experience including
  - data, literature, investigation(s), marketing experience (OUS)
  - clinical trials to support an HDE are usually not randomized or controlled due to small sample size and lack of a comparable marketed device

HDE Submission Content

- Manufacturing information
  - Quality System Regulation (QSR) applies
- Labeling (physician and patient)
  - Must including HUD statement - no effectiveness demonstrated
- Request for ADN, if eligible
- HDE Filing checklist: http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/HowtoMarketYourDevice/PremarketSubmissions/HumanitarianDeviceExemption/ucm049704.htm

ADN Calculation

The ADN is calculated by multiplying the number of devices reasonably needed to treat, diagnose, or cure an individual by 4,000.

Example:

Annual incidence for a specific HUD is 3,000 and the number of devices reasonably needed per patient is 2.

2 x 4,000 = ADN 8,000

Approval Threshold

Safety - Device does not expose patients to unreasonable risk of illness or injury

AND

Probable benefit outweighs the risks of using the device, taking into account the probable risks and benefits of alternative therapies

HDE Approvals

58 approved HDEs since 1996

List of approved HDEs and their Summaries of Safety and Probable Benefit (SSPB) available at:

http://www.fda.gov/MedicalDevices/ProductsandMedicalProcedures/DeviceApprovalsandClearances/HDEApprovals/ucm161827.htm

Key Points

- HDEs are exempt from effectiveness requirements
- HDE approval
  - is marketing approval
  - IRB approval required
  - Informed Consent not required by FDA
- No requirement to submit PMA/510(k)
- Can have multiple HDEs for same indication from different sponsors

Thank you!

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