Humanitarian Use Device Humanitarian Device Exemption

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HUD and HDE Topics

- Regulations/Federal Register/Guidance
- Distinguish between HUD and HDE
- IRB responsibilities
- Physician and HDE-holder responsibilities
- FDA concerns
- Revised HDE guidance

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Humanitarian Use Device and Humanitarian Device Exemption

- Regulation
  - 21 CFR 814 Subpart H
  - 21 CFR 56 Institutional Review Boards
  - 21 CFR 803 Medical Device Reporting
- Guidance document
  - List of all HUDs
  - Click on HUD number to access labeling
  - Frequently asked questions and answers

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Humanitarian Use Device

- Humanitarian Use Device (HUD)
  - Office of Orphan Products Development designates a device as an HUD
  - Serious disease or condition affecting fewer than 4,000 individuals in the US per year
  - No other comparable device available

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Humanitarian Device Exemption

The HDE application is reviewed by CDRH (75 days)

- Bench and animal testing
- Summary of clinical experience: could be data, literature, investigation(s), marketing experience **(but not valid scientific evidence as in the case of a PMA)**
- Approval based on probable benefit outweighs risk of injury from its use **(but not reasonable assurance of safety and effectiveness – as in PMA approval)**
- HDE label states "the effectiveness of this device for this use has not been demonstrated"
- The HDE allows marketing distribution for the HUD

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Humanitarian Device Exemption (HDE)

- The HDE-holder must
  - Have both HUD and HDE before device is shipped to institutions with an Institutional Review Board
  - Report clinical experience (safety info) to FDA, annual reports
  - Maintain IRB correspondence
  - Ensure IRB initial and continuing review

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Humanitarian Device Exemption Federal Register

- The HDE holder is responsible for ensuring that the HUD is not administered to or implanted in a patient prior to obtaining IRB approval at the health care facility.
- An HDE holder may wish to enforce this requirement by not shipping the HUD to the facility until it has received confirmation of IRB approval.

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IRB initial review of an HUD Federal Register

- FDA believes that the approval criteria set forth in the IRB regulations at 21 CFR 56.111, can and should be interpreted to include consideration of the patient's need for the HUD and the likelihood that the device is appropriate for the patient's condition or disease state.

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IRB initial review of an HUD Federal Register

- An IRB may approve the use of the HUD
  - in general, for HUD patients that qualify
  - for groups of HUD patients that meet certain criteria
  - under a HUD treatment protocol
  - on a case by case HUD basis

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IRB initial review of an HUD Federal Register

- An IRB may specify limitations on the use of the device based upon
  - one or more measures of disease progression
  - prior use and failure of any alternative treatment modalities
  - reporting requirements to IRB
  - appropriate follow up precautions and evaluations
  - any other criteria it determines appropriate

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IRB Continuing Review

- Follow written procedures for continuing review of devices
  - Convened meeting
  - Expedited review
  - Ask HDE-specific questions
    - Are patients receiving information packets? Has the HDE been used outside of approved labeling and were reports made?

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IRB withdrawal of HDE approval

- **IRB requirement to withdraw approval for**
  - Failure to follow IRB or FDA requirements
  - Unexpected serious harm to patients

An IRB will have to ask specific questions at continuing review to elicit the above information.

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Humanitarian Device Exemption
Human subject protections

- Physician responsibilities
  - Patient consent per **IRB policy**
    - "the effectiveness of this device for this use has not been demonstrated"
  - Give patients HDE patient-information packet
  - Medical Device Reporting (MDR) to FDA (see next slide) and IRB

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Humanitarian Device Exemption
Adverse events reported via MDR

- Physicians and Institutions must submit MDRs to FDA and IRB
  - Reports of death – within 10 working days
  - Reports of serious injury – within 10 working days
  - MDR Report: approximately 38 fields to complete

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Humanitarian Device Exemption

- Areas of concern and acceptable actions
  - Off label use (guidance)
    - Physician should check with IRB to ensure facility has no restrictions
    - Summary report to IRB and HDE-holder following the use
  - Research of off label use
    - Requires an Investigational Device Exemption (IDE)

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Recent HUD Compliance Concerns

- HUD used in research for new use without IDE
  - CDRH required the investigator to obtain IDE
- HUD used without HDE
  - CDRH/FDA met with the Firm. Recalled the HUDs
- Flagrant off-label use of HUDs
  - CDRH informed IRBs, physicians, and firms that this is not the intent of an HUD

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HDE Guidance
Pediatric Medical Device Safety and Improvement Act of 2007

Device firms can make a profit on HUDs, if. . . . . .

- It is intended for the treatment or diagnosis of a disease that occurs in **pediatric** patients (may also include adult use)
- The distribution of the HDE does not exceed the annual distribution number, (ADN) which is specified by FDA
- The firm notifies FDA if the ADN is exceeded

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Section on Role of IRBs

- New section with 29 Questions and Answers
- Examples:
  - Differences between HDE, IDE, 510(k), and PMA?
  - How to distinguish between HUD use and HUD research?
  - How to evaluate request for HUD approval?
  - IRB's oversight of physician's use of an HUD?
  - What to consider when physicians request to use a HUD?
  - IRB's concerns with HDE holder charging for HUD?
  - What information do patients receive?
  - HIPAA questions

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Summary

- Description of HUD and HDE
- Regulations/Federal Register/Guidance
- IRB responsibilities
- Physician & HDE-holder responsibilities
- FDA concerns
- Revised HDE Guidance

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Reference

- http://www.fda.gov/cdrh/ode/hdeinfo.html

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Questions

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