Office of Human Research Ethics Training Tips

Expanded Access IND/IDE and HUD IRB Review

What is expanded access?

Expanded access, also called "compassionate use," provides a pathway for patients to gain access to investigational drugs, biologics and medical devices for serious diseases or conditions. The purpose is not research but treatment for a patient or patients with an unapproved drug or device. Although it is not research the FDA requires review and approval by a convened IRB.

(A10: Under current FDA regulations, for all expanded access uses including individual patient access uses, investigators are required to ensure that IRB review and approval is obtained consistent with 21 CFR part 56 (21 CFR 312.305(c) (4)). 21 CFR part 56 requires, among other things, that the IRB review the expanded access use at a convened meeting at which a majority of the IRB members are present ("full IRB review") (21 CFR 56.108(c)).)
http://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm351261.pdf
http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/HowtoMarketYourDevice/InvestigationalDeviceExemptionIDE/ucm051345.htm

What does the FDA consider when granting an expanded access IND?

(1) The patient or patients to be treated have a serious or immediately life-threatening disease or condition, and there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition;

(2) The potential patient benefit justifies the potential risks of the treatment use and those potential risks are not unreasonable in the context of the disease or condition to be treated; and

(3) Providing the investigational drug for the requested use will not interfere with the initiation, conduct, or completion of clinical investigations that could support marketing approval of the expanded access use or otherwise compromise the potential development of the expanded access use.

What does the FDA consider when granting an expanded access IDE?

(1) The patient is (was) confronted by a life-threatening or serious disease or condition that is not an emergency.

Life threatening: Diseases or conditions where:

(1) the likelihood of death is high unless the course of the disease is interrupted or
(2) there is a potentially fatal outcome. The condition need not be immediately life-threatening or to immediately result in death.

***Serious diseases or conditions*:** Diseases or conditions involving risk or serious irreversible morbidity, such as sight-threatening or limb-threatening conditions, paralysis, stroke.

(2) There is no generally acceptable alternative for treating the patient.

(3) This particular use of the device is not already approved under an existing IDE.

(4) The patient does not qualify for the device through an ongoing clinical investigation.

What is a Humanitarian Use Device?

A Humanitarian Use Device (HUD) is a device that is intended to benefit patients by treating or diagnosing a disease or condition that affects or is manifested in fewer than 4,000 individuals in the United States per year. A device manufacturer`s research and development costs could exceed its market returns for diseases or conditions affecting small patient populations. The HUD provision of the regulation provides an incentive for the development of devices for use in the treatment or diagnosis of diseases affecting these populations. HUDs are granted Humanitarian Device Exemptions (HDE).

IRB Review

Submissions will be received as truncated NHSR applications with the Expanded Access Drugs, Expanded Access Devices, or Humanitarian Use Device addendum attached as well as the required documents listed in the addendum.

The Board should:

- Weigh risks and benefits (listed in addendum)
- Review informed consent process (listed in addendum, ICF will be attached).
- Ensure safety monitoring is in place (listed in addendum)
- For devices, if the physician is or will be properly trained to use and/or monitor the device

What will be different?

- The application should reflect that results are not meant to be generalizable (not research).
- There will be no data analyses.
- As this is not research, HIPAA will be addressed under clinical care so a HIPAA authorization form will not be attached for review.
- As this is not considered research, CITI training in NOT required.
- For HUDs, continuing review may be conducted using the expedited review procedures (see 21 CFR 56.110) unless the IRB determines that full board review should be performed. **The agency believes that the expedited review procedures are appropriate for continuing review since the initial review would have been performed by the full board and use of the HUD within its approved labeling does not constitute research**. You may consider HUDs for expedited category 9.