

## 8.7 HUMANITARIAN USE DEVICES

A humanitarian use device (HUD) is a device intended to benefit patients by treating or diagnosing a disease or condition that affects or is manifested in not more than 8,000 individuals in the United States per year. A HUD must receive approval for use through the receipt of a humanitarian device exemption (HDE), which exempts it from the standard requirements for clinical investigation of effectiveness.

A HUD may be administered only in facilities having oversight by an IRB, including continuing review of use of the device. In addition, a HUD may be administered only if such use has been approved by an IRB. The Lurie Children's IRB will review the use of a HUD at the Institution or it may agree to rely on an external IRB to oversee such use and an Institutional Authorization Agreement will be executed.

When applying for IRB approval to use an HUD, an investigator must submit an Initial application in Cayuse IRB. The IRB will only consider use of an HUD for the indication identified in the HDE.

While administration of an HUD in accordance with its indication as approved by the FDA does not constitute research and may not meet the FDA requirements for informed consent, the Lurie Children's IRB requires informed consent to protect the rights and welfare of the patients. If the holder of the HDE wishes to collect safety and effectiveness data in a clinical investigation for the HDE-approved indication(s) the HUD is not subject to IDE requirements. However, other clinical investigation requirements still apply, including IRB approval.

Clinical investigations of a HUD for a different indication other than the HDE-approved indication must be conducted in compliance with the IDE regulations at 21 CFR Part 812, subject to IRB approval, and in compliance with protection of human subjects, including informed consent and, if applicable, additional safeguards for children

Investigators will be asked to provide periodic reviews to the IRB no less than once year, and may be required to implement special precautions or follow-up evaluations. The FDA considers expedited review procedures to be appropriate for continuing review when the use of a HUD within its approved labeling does not constitute research.

- i. Determination of a significant risk (SR) or non-significant risk (NSR) device for HUD

When the IRB is deciding whether to approve the use of a HUD according to its approved indications, its review does not include an SR/NSR determination. In addition, the IRB does not have to make a SR/NSR determination when it receives a request to review a clinical investigation of a HUD (e.g., collection of safety and effectiveness data) when that clinical investigation concerns the HDE-approved indication(s) only. The FDA considers such investigations exempt from the IDE requirements in 21 CFR Part 812.

If the IRB receives a request to review an application for an investigational study of the HUD for a different indication than the HDE-approved indication, then the clinical investigation is subject to the IDE regulations at 21 CFR Part 812 and a SR/NSR determination must be made.

- ii. Emergency Use of an HUD without Prior IRB Approval

An HUD may be administered a single time without prior approval by the IRB in an emergent situation in which a physician determines that approval from the IRB cannot be obtained in time to prevent serious harm or patient death.

The FDA recommends that the physician obtain informed consent from the patient and ensure that reasonable patient protection measures are followed, such as devising schedules to monitor the patient, taking into consideration the patient's specific needs and the limited information available about the risks and benefits of the device.

The FDA further recommends that the physician submit a follow-up report on the patient's condition to the HDE holder and first check with the IRB before such use to review any Institutional policy.

In such an emergency situation, the physician shall, within 5 days after the use of the device, provide written notification to the IRB of such use.

Note: user facilities must submit reports to FDA, the IRB of record, and the manufacturer whenever a HUD may have caused or contributed to a death, and must submit reports to the manufacturer (or to FDA and the IRB of record if the manufacturer is unknown) whenever a HUD may have caused or contributed to a serious injury.

## **8.8 EMERGENCY USE AND EXPANDED ACCESS FOR MEDICAL DEVICES**

An unapproved device usually may only be used on human subjects through an approved clinical study in which the subjects meet certain criteria and the device is only used in accordance with the approved protocol. Circumstances under which a health care provider may wish to use an unapproved device to save the life of a patient or to help a patient suffering from a serious disease or condition for which there no other alternative therapy exists. The use of an investigational device outside of a clinical trial for treatment of a patient is called "expanded access."

If enrollment in an existing clinical trial protocol is not possible (e.g., a patient is not eligible for any ongoing clinical trials, or there are no ongoing clinical trials to address the patient's condition), patients/physicians have the potential to receive expanded access to investigational devices under one of three alternative mechanisms:

- Emergency Use
- Compassionate Use (or Single Patient/Small Group Access)
- Treatment Use

### **A. Emergency Use**

Emergency use is the use of an investigational device in an emergency situation. It is intended to provide patients and physicians with access to devices intended to treat life-threatening or serious diseases or conditions when there is no available alternative and no time to obtain FDA approval. Emergency use may apply even if the investigational device is being studied in a clinical trial under an IDE: if a physician needs to use the device in a manner inconsistent with the approved investigational plan; or a physician who is not part of the clinical study, wishes to use the device to treat a patient with a life-threatening or serious disease or condition. Emergency use of an investigational device may occur before an IDE is approved and when a device is not being studied under an IDE.