Penn
University of Pennsylvania
Human Research Protections Program

Compassionate Use of a Humanitarian Use Device (HUD) in a Single Patient

Introduction

A Humanitarian Use Device (HUD) is defined as a medical device intended to benefit patients in the treatment or diagnosis of a disease or condition that affects or is manifested in not more than 8,000 individuals in the United States per year (Orphan Diseases). An HUD is marketed under an HDE and is exempt from the requirement of establishing a reasonable assurance of effectiveness.

Although the use of a HUD within its approved labeling does not constitute research, the FDA requires IRB approval be obtained before a HUD can be used in a facility.

Compassionate Use in a Single Patient

If a physician in a non-emergent situation determines that there is no generally acceptable alternative treatment or device for the patient's condition, the HUD may be used off-label in a compassionate use situation. However, IRB review is required prior to treatment with the device.

Requirements for IRB Submission

The following documents should be submitted via an initial submission in HSERA when requesting a compassionate use of an HUD in a single patient.

1. Documentation from the HDE holder allowing the off-label clinical use
2. An independent assessment by an uninvolved physician (i.e., not the referring physician)
3. A description of the patient(s)' condition and the circumstances necessitating use of the device; an assessment of why alternative therapies or diagnostics are unsatisfactory; a description of schedules to monitor the patient(s) based on the patient(s)' specific needs; an assessment of information about the risks and probable benefits of the device for the proposed patient(s)
4. Documentation of FDA approval for compassionate use of the HUD (see HDE holder responsibilities), *or notification that this is pending*
5. The HUD manufacturer's product labeling, clinical brochure, and/or other pertinent manufacturer informational materials
6. The FDA HDE approval letter
7. Humanitarian Use Device Consent Form OR the patient information brochure prepared by the manufacturer
8. Cover letter with additional information that may help in the review and document list identifying each attachment (name, version, date)
9. Obtain any institutional clearances for the device prior to compassionate use.

What Level of Review is Required?

- If the HUD is already approved for on-label use at the institution, chair concurrence may be utilized for IRB review.

- If the HUD is not already approved for on-label use at the institution, full convened board review will be required.

Physician responsibilities following compassionate HUD use

1. Submit a follow-up report on the patient's condition and information regarding the patient protection measures to the HDE holder, who would then submit this information as a HDE report to the FDA.
2. Report to the HDE holder/FDA and to the IRB whenever a HUD may have caused or contributed to a death or serious injury, or has malfunctioned and would be likely to cause or contribute to a death or serious injury if the malfunction were to recur (21 CFR 814.126(a))