Expanded Access to Investigational Drugs and Devices (HRPP 6.5)

Description:

This policy describes treatment use of an investigational drug or device (test articles) for individuals or groups of patients with serious or immediately life-threatening diseases or conditions who lack therapeutic alternatives (expanded access).

Definitions:

Expanded Access (Compassionate Use): treatment use of an investigational drug or device for patients with serious or immediately life-threatening diseases who lack therapeutic alternatives.

Expanded Access **may also refer to:**

- A drug withdrawn for safety reasons, but there exists a patient population for whom the benefits outweigh the risks
- Substituting a similar, but unapproved drug during a shortage of the approved drug
- Use of an approved drug where availability is restricted by safety programs (called REMS) if patients cannot otherwise get it

NOTE: the purpose of expanded access is to TREAT, not to collect research data.

General Requirements:

- **In most cases, expanded access must be reviewed and approved by the full IRB before treatment begins**
  - **Exception**: for individual patients, the doctor may request a waiver of full IRB review using FDA Form 3926 (the IRB Chair of another reviewer must still give prior approval).
- Informed consent from the patient is required (unless specific exceptions apply under FDA rules)

- Generally limited to a single course of therapy for a single patient for a specified duration, but longer treatment may be approved by the FDA for chronic conditions
- If the patient's situation is a true emergency, treatment may proceed under emergency use procedures (HRPP Policy 6.4)

Investigator Procedures:

For a **single patient**, the physician must:

- Assess the risk to the patient and ensure it is less than the risk from the disease itself
- Submit a Single Patient Expanded Access application through RSS
- Submit a copy of the completed FDA Form 3926 to the IRB
- Submit written permission from the holder of the IND/IDE to use the drug/device
- Submit a consent form for use by a single patient

NOTE: the treating physician may request review and concurrence by the IRB Chair, instead of a convened IRB, by checking box 10b on the FDA form 3296.

For **intermediate-size populations and widespread treatment use** through a treatment IND or treatment protocol:

- The protocol must be submitted on Biomedical Application through RSS
- Convened IRB review is required

Informed Consent:

- **Written informed consent** from the patient or the patient's LAR is **required before treatment,** unless there is an emergency situation that qualifies for a waiver
- **The consent form must:**
    - Use clear, plain language specifically aimed at "patients" who expect direct benefit instead of "subjects" who may not expect direct benefit
    - Include HIPAA required information
    - Include a clear disclosure of the financial obligations of the patient