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: treatment use of an investigational drug or device for patients with serious or immediately life-threatening diseases who lack therapeutic alternatives.

May also refer to:

- Use in situations when a drug has been withdrawn for safety reasons, but there exists a patient population for whom the benefits outweigh the risks.
- Use of a similar, but unapproved drug to provide treatment during a drug shortage of the approved drug.
- Use of an approved drug where availability is limited by a risk evaluation and mitigation strategy (REMS) for diagnostic, monitoring, or treatment purposes, by patients who cannot obtain the drug under the REMS.

General Considerations:

Expanded access Protocols (EAPS), sometimes referred to as “treatment use” or “compassionate use” protocols, have the PRIMARY PURPOSE of diagnosing, monitoring, or treating a patient’s disease or condition rather than collecting the kind of information about the drug that is generally derived from clinical trials.

Individual patient expanded access is generally limited to a single course of therapy for a special duration. However, the FDA may authorize multiple courses of therapy or chronic therapy for individual patient expanded access, including treating a chronic disease or condition requiring extended treatment.

Expanded Access Protocols are clinical investigations and require IRB review and approval (except as described below), and informed consent.

Expanded Access is also different than Emergency Use of an Unapproved Product (see HRPP Policy 6.4). Emergency Use is NOT research and does not require IRB review or approval. Emergency Use under FDA regulations requires that the patient be in a life-threatening situation, with no effective alternative therapy, and insufficient time to obtain IRB approval.

Investigator Procedures:

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

- Determine that the probable risk is not greater than the probable risk from the disease or condition.
- Complete and submit a Single Patient Expanded Access application thru 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 investigational drug or device.
- Submit a consent form for use by a single patient.

The treating physician may request review and concurrence by the IRB Chair, instead of convened IRB review, 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 a Biomedical Application thru RSS.
- Convened IRB review is required.

Informed Consent:

- Must be obtained from the patient or the patient's LAR.
- Documents should use plain language specifically aimed at "patients" who expect direct benefit, as opposed to "subjects" who may or may not expect direct benefit.