Expanded Access to Investigational Drugs or Devices
(Compassionate Use)

OVERVIEW
There are circumstances in which investigational medical products such as an Investigational New Drug (IND) or Investigational Device (IDE) is the only option available for patients with a serious, life-threatening, disease or condition.

In some cases, patients can receive Expanded Access to unapproved investigational drugs and devices. In these circumstances, the Food and Drug Administration (FDA) and the sponsor must determine whether such use of an IND or IDE should occur.

The Expanded Access Use provision allows access to unapproved drugs or devices when patients do not meet the requirements for inclusion in the clinical trial and the treating physician believes the following criteria to be true:

• The patients have a serious or life-threatening disease or condition, and there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition;
• 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
• Providing the test article 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.

Expanded Access Use for single patients or small or intermediate-size patient populations is sometimes described as Compassionate Use. When a physician wishes to use an unapproved test article under this Compassionate Use provision, the physician must adhere to the procedures outlined below.

PROCEDURES
Expanded Access to Investigational Drugs or Biologics
Physician Responsibilities: The physician must obtain authorization from the sponsor. If the sponsor disagrees with the use, then the physician cannot use the test article.

The sponsor or physician should submit a request to the FDA. If the FDA disagrees with the use, then the physician cannot use the test article.

The physician should then submit his or her request to the HRPP Office in order to gain IRB approval. This request must include the following:

1. Confirmation that the patient does not meet requirements for inclusion in a clinical trial studying the test article in question;
2. Treatment Plan including patient protection measures that will be followed (schedule for monitoring the patient, taking into consideration the investigational nature of the drug or device and the specific needs of the patient);
3. Investigator’s Brochure;
4. Draft consent form for the patient to sign;
5. Sponsor and FDA approval letters and/or documents for the requested expanded access; and
6. Confirmation of clearance from the Central Office of Research Administration (CORA).

A follow-up report should be submitted to the FDA, sponsor, and IRB summarizing the patient’s outcome, which should include any problems that may have occurred as a result of the use. This summary report must be submitted to the reviewing IRB as soon as possible.

**IRB Responsibilities:** The IRB will review the submission for compliance with the requirements above and review the consent form. The decision will be communicated in writing to the physician.

**Expanded Access to Investigational Devices**

**Physician Responsibilities:** The physician must obtain authorization from the sponsor. *If the sponsor disagrees with the use, then the physician cannot use the test article.*

The sponsor or physician should submit a request to the FDA. *If the FDA disagrees with the use, then the physician cannot use the test article.*

The physician should then submit his or her request to the HRPP Office in order to gain IRB Concurrence. This request must include the following:

1. A description of the patient’s condition and the circumstances necessitating treatment;
2. A discussion of why alternatives therapies are unsatisfactory and why the probable risk of using the investigational device is no greater than the probable risk from the disease or condition;
3. An independent assessment from an uninvolved physician;
4. An identification of any deviations in the approved clinical protocol that may be needed in order to treat the patient;
5. Treatment Plan including patient protection measures that will be followed (schedule for monitoring the patient, taking into consideration the investigational nature of the drug or device and the specific needs of the patient);
6. Draft consent form for the patient to sign;
7. Sponsor and FDA approval letters and/or documents for the requested expanded access; and
8. Confirmation of clearance from the Central Office of Research Administration (CORA).

A follow-up report should be submitted to the FDA, sponsor, and IRB summarizing the patient’s outcome, which should include any problems that may have occurred as a result of the use. This summary report must be submitted to the reviewing IRB as soon as possible.
HRPP Office and IRB Responsibilities:
The HRPP Office staff will forward the submission to the IRB Chairperson for review. Additional information may be requested from the physician, if necessary. The staff will then communicate in writing to the physician the decision of the IRB Chairperson.

FDA approval and IRB concurrence or approval is required before the use of the device. The submissions to the FDA and IRB can occur simultaneously, but both are needed before use.

REFERENCES
FDA’s Guidance on IDE Early/Expanded Access
FDA’s Guidance on Expanded Access to Investigational Drugs for Treatment Use
FDA’s Guidance on Expanded Access (Compassionate Use)