Policies and Procedures

SECTION: Human Research Protection Program	NUMBER: 129	
CHAPTER: Institutional Review Board	ISSUED: 7/2010	REVISED:
POLICY: Expanded Access to Investigational New Drugs	PAGE 1 OF 3	

1. PURPOSE

Investigational products are sometimes used for treatment of serious or life-threatening conditions either for a single subject or for a group of subjects. The procedures that have evolved for an investigational new drug (IND) used for these purposes reflect the recognition by the FDA that, when no satisfactory alternative treatment exists, subjects are generally willing to accept greater risks from test articles that may treat life-threatening and debilitating illnesses. The following mechanisms expand access to promising therapeutic agents without compromising the protection afforded to human subjects or the thoroughness and scientific integrity of product development and marketing approval.

2. OPEN LABEL PROTOCOL OR OPEN PROTOCOL IND

2.1. Open label protocols or open protocol INDs are usually uncontrolled studies carried out to obtain additional safety data (Phase 3 studies). Open protocol INDs are typically used when the controlled trial has ended and treatment is continued so that the subjects and the controls may continue to receive the benefits of the investigational drug until marketing approval is obtained. These studies require prospective IRB review and informed consent.

3. TREATMENT IND

3.1. Treatment Investigational New Drugs (*Federal Register*, May 22, 1987) are used to make promising new drugs available to desperately ill patients as early in the drug development process as possible. FDA will permit an investigational drug to be used under a treatment IND if there is preliminary evidence of drug efficacy and the drug is intended to treat a serious or life-threatening disease, or if there is no comparable alternative drug or therapy available to treat that stage of the disease in the intended patient population. In addition, these patients are not eligible to be in the definitive clinical trials, which must be well underway, if not almost finished.

3.2. An immediately life-threatening disease means a stage of a disease in which there is a reasonable likelihood that death will occur within a matter of months or in which premature death is likely without early treatment. For example, advanced cases of AIDS, herpes simplex encephalitis, and subarachnoid hemorrhage are all considered to be immediately life-threatening diseases. Treatment INDs are made available to patients before general marketing begins, typically during Phase 3 studies. Treatment INDs also allow FDA to obtain additional data on the drug's safety and effectiveness.

3.3. Treatment IND studies require prospective IRB review and informed consent. A sponsor may apply for a waiver of local IRB review under a treatment IND if it can be shown to be in the best interest of the subjects, and if a satisfactory alternative mechanism for assuring the protection of human subjects is available (e.g., review by a central IRB). Such a waiver does not apply to the informed consent requirement. An IRB may still opt to review a study even if the FDA has granted a waiver. The Creighton University IRB must be notified and shall, on a case-by-case basis, consider whether local review is required.

4. GROUP C TREATMENT IND

4.1. The "Group C" treatment IND was established by agreement between the FDA and the National Cancer Institute. The Group C program is a means for the expanded access distribution of investigational agents to oncologists for the treatment of cancer under protocols outside the controlled clinical trial. Group C drugs are generally Phase 3 study drugs that have shown evidence of relative and reproducible efficacy in a specific tumor type. They can generally be administered by properly trained physicians without the need for specialized supportive care facilities. Group C drugs are distributed only by the National Institutes of Health under National Cancer Institute protocols. Although treatment is the primary objective and patients treated under Group C guidelines are not part of a clinical trial, safety and effectiveness data are collected.

4.2. Because administration of Group C drugs is not done with research intent, the FDA has generally granted a waiver from IRB review requirements. However, the Creighton IRB still chooses to review all Group C treatment IND studies conducted under the jurisdiction of Creighton University. Therefore, Principal Investigators are required to submit all Group C treatment IND studies to the IRB for review, as appropriate.

5. PARALLEL TRACK

5.1. The FDA's Parallel Track policy (57 FR 13250, April 15, 1992) permits wider access to promising new drugs for AIDS and HIV-related diseases under a separate expanded access protocol that "parallels" the controlled clinical trials that are essential to establish the safety and effectiveness of new drugs. It provides an administrative system that expands the availability of drugs for

treating AIDS and other HIV-related diseases. These studies require prospective IRB review and informed consent.

6. EMERGENCY USE IND

6.1. The need for an investigational drug may arise in an emergency situation that does not allow time for submission of an IND in the usual manner. In such cases, the FDA may authorize shipment of the drug for a specified use. Such authorization is usually conditioned upon the sponsor filing an appropriate application as soon as practicable.

6.2. Prospective IRB review is required unless the conditions for exemption are met. Informed consent is required unless the conditions for exception are met (see IRB Policy 128, "Emergency Use of Unapproved Drugs/Devices/Biologics").