
Yale University Institutional Review Boards

600 PR.2 Compassionate Use of an Investigational or Unapproved Device

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Overview

As discussed in Policy 600, Use of Investigational New Drugs and Devices in Human Research, the FDA allows for those situations in which an investigational or unapproved device is needed to save the life of a patient or to prevent irreversible morbidity (see Procedure 600 PR.1). The FDA recognizes, however, that there are circumstances in which an investigational device is the only option available for a patient faced with a serious, albeit not life-threatening condition (referred to as "compassionate use" of the device)*. In these circumstances, FDA uses its regulatory discretion in determining whether such use of an investigational device should occur. Accordingly, a clinician who wishes to use an investigational device for compassionate use in treating a patient will need to request the device sponsor to prepare a supplement to the IDE for submission to the FDA for review and approval. **Once FDA approves the use, the clinician must seek concurrence from the IRB Chair to proceed with the use of the device for patient treatment.**

IDE Supplement for FDA Approval and IRB Chair Review

Unlike emergency use of an unapproved device, prior FDA approval is needed before compassionate use occurs. In order to obtain FDA approval, the sponsor should submit an IDE supplement requesting approval for a protocol deviation under section 812.35(a) in order to treat the patient. The IDE supplement should include the following information, provided by the clinician to the sponsor:

- A description of the patient's condition and the circumstances necessitating treatment;
- A discussion of why alternative 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;
- An identification of any deviations in the approved clinical protocol that may be needed in order to treat the patient; and
- The patient protection measures that will be followed. These measures include:
 - Informed consent from the patient or a legal representative/surrogate;
 - Clearance from the institution (Yale-New Haven Hospital). Note, in the case of protocols to be carried out at YNHH, once approved by the IRB, the clinician must bring evidence of IDE approval and accompanying information or protocol to the relevant Vice President or Service Line Director at YNHH for approval insofar as inventory and costs may affect Hospital finances and procedures;
 - Concurrence of the IRB chairperson, based upon the chairperson's review of the supplemental information prepared for the FDA's approval, including the patient informed consent form;
 - An independent assessment from an uninvolved physician; and
 - Authorization from the IDE sponsor, if an approved IDE exists for the device.

* Note on the term, "Compassionate Use" -- Emergency and Treatment INDs are sometimes referred to as "Compassionate Use" INDs, but the term "Compassionate Use" is not in the IND regulations; it is only in the IDE regulations.

The clinician should not treat the patient identified in the supplement until FDA approves use of the device under the proposed circumstances. In reviewing this type of request, FDA will consider the above information as well as whether the preliminary evidence of safety and effectiveness justifies such use and whether such use would interfere with the conduct of a clinical trial to support marketing approval. FDA approval should be provided to the IRB Chair for review along with the aforementioned supplemental information.

Patient Monitoring and Reporting Requirements

If the request is approved by the FDA, and the requisite institutional clearance and IRB Chair concurrence are obtained, the clinician should devise an appropriate schedule for monitoring the patient, taking into consideration the investigational nature of the device and the specific needs of the patient. The patient should be monitored to detect any possible problems arising from the use of the device. Following the compassionate use of the device, a follow-up report should be submitted to FDA as an IDE supplement in which summary information regarding patient outcome is presented. If any problems occurred as a result of device use, these should be discussed in the supplement **and reported to the IRB as soon as possible**.

Compassionate Use for Multiple Patients

The above compassionate use criteria and procedures can also be applied when a clinician wishes to treat a few patients rather than an individual patient suffering from a serious disease or condition for which no alternative therapy adequately meets the medical need. In this case, the clinician should request access to the investigational device through the IDE sponsor. The sponsor should submit an IDE supplement that includes the information identified above and indicates the number of patients to be treated. Such a supplement should include the protocol to be followed or identify deviations from the approved clinical protocol. As with single patient compassionate use, a monitoring schedule should be designed to meet the needs of the patients while recognizing the investigational nature of the device. Follow-up information on the use of the device should be submitted in an IDE supplement after all compassionate use patients have been treated.