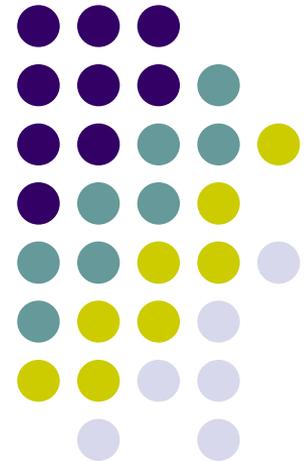


Single Patient Use of Investigational Drugs

A Tutorial for UH Physicians and Staff

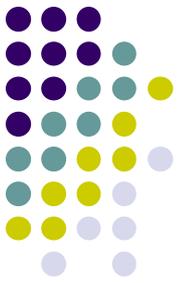
June 17, 2010



About this Tutorial

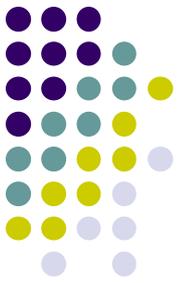


- This tutorial has been developed to explain UH IRB policies and procedures for using investigational drugs for treating individuals with serious or life-threatening medical conditions.
- These policies reflect FDA regulations on “Single Patient Use” of investigational drugs.



Definitions of Key Terms

- An **Investigational Drug (ID)** is a drug that is being evaluated for safety and efficacy under the authority of the FDA, usually in anticipation of FDA approval for its commercial use. In this tutorial, investigational drug is referred to as ID.
- **IND** (Investigational New Drug) is a designation issued by the FDA, providing a company or an individual physician with the authority to use an investigational drug in humans for research or treatment purposes according to FDA regulations.

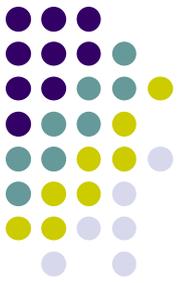


Group C Guideline Protocol

- A **Group C Guideline Protocol** is a treatment protocol sponsored by the National Cancer Institute (NCI) for certain investigational oncology drugs. The FDA authorizes the NCI to distribute Group C drugs directly to individual physicians to treat patients under the approved NCI protocol. Although treatment is the primary objective of these protocols, safety and efficacy data are collected.

Prospective IRB approval is not required for ID available under a Group C protocol. Treating physicians should use the consent form provided in the Group C protocol.

Off-Label Use of an FDA-Approved Drug



- **Off-Label Use** refers to a treatment use of an FDA-approved drug for an indication other than that for which the drug was approved.

A physician can administer a drug off-label under his or her medical license without an IND or prospective IRB approval. The physician must use his or her best medical judgment, base such uses on scientific rationale, and document the use and its effects.

If the off-label use is being conducted under a research protocol or data are being collected for research purposes, prospective IRB approval and documented informed consent are required.

Required Approvals for a Single Patient Use

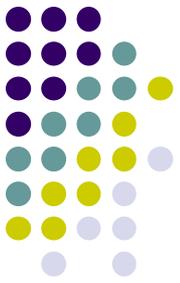


- Prior to use of an investigational drug to treat an individual with a serious medical condition, the physician must obtain:
 - Permission to use an existing IND (often held by a sponsor or ID manufacturer) or a new IND from the FDA; and
 - IRB approval for the single patient use and the associated treatment protocol and consent form
- The IND is held by NCI if the investigational drug is approved for distribution under the NCI's Group C Guideline Program.



To Apply for an IND

- To apply for an IND, the treating physician must submit an application to the FDA.
- The IND application asks for:
 - Contact information for the treating physician,
 - Summary information on the patient,
 - Summary information on the treatment protocol, and
 - Contact information for the IRB.
- Fax the completed application with documentation of the treating physician's credentials and a copy of relevant correspondence with the provider of the ID to the FDA at 301/ 827-4590.
- Keep a copy of the form for your records and to send to the IRB.



To Apply for IRB Approval

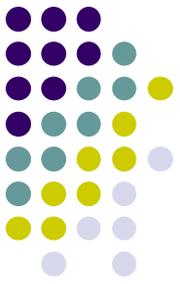
- To apply for IRB approval for Single Patient Use (SPU) of an ID, the treating physician must provide to the UH IRB:
 - The IND application or documentation of an existing IND
 - The SPU protocol (see next slide)
 - The treating physician's CV
 - An investigator's brochure for the ID or a compilation of safety and efficacy findings to date on the study drug
 - A draft consent form



Writing the SPU Protocol

- An SPU protocol must include the following elements, providing sufficient detail for the IRB to conduct a comprehensive human subjects review of the study:
 - Objectives of the protocol
 - Study design, methods and procedures
 - Potential risks and benefits of the protocol
 - Inclusion and exclusion criteria
 - A patient safety monitoring plan
 - Criteria for withdrawal of the patient from the study
 - Procedures to protect the safety, welfare and rights of the patient

Documenting Evidence of the Safety and Efficacy of the ID



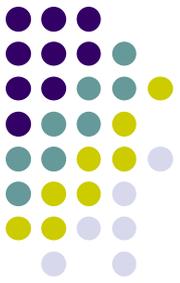
- The physician should ask the company providing the ID for information on the drug's safety profile, including potential risks to patients.
- The company may release an Investigator's Drug Brochure for the ID.
- The physician must present a comprehensive review of all relevant safety information to the IRB.



Drafting the SPU Consent Form

- The Single Patient Use (SPU) consent form must meet all requirements of a research consent form.
- It must include all of the FDA-required elements of a consent form. In addition, it should include the “optional” elements identified by the FDA, as appropriate.

After the SPU has been Approved



- If the IRB approves the SPU, it will issue a letter to the treating physician/PI, indicating its approval for the protocol and consent form. This letter should provide instructions on follow-up requirements.
- The physician must provide progress reports on the SPU to the IRB at intervals determined by the IRB not to exceed one year.
- The physician must notify the IRB of any unanticipated problems occurring to the patient.
- A physician who holds an IND must also provide safety and other information directly to the FDA.