



SOP: EMERGENCY AND TREATMENT USE OF INVESTIGATIONAL DRUGS, BIOLOGICS AND DEVICES

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1 PURPOSE

- 1.1 This procedure establishes the process providers must follow to treat patients with investigational drugs, biologics, and medical devices.
- 1.2 The process begins when a provider determines (1) no generally accepted alternative for treating the condition is available; and (2) there is substantial reason to believe the patient will benefit from use of the investigational drug, biologic, or medical device.
- 1.3 The process ends when the IRB staff has communicated the results to the physician; and if necessary, initiated the non-compliance process.
- 1.4 This policy does not apply to off-label uses of approved drugs, biologics, and medical devices.

2 REVISIONS FROM PREVIOUS VERSION

- 2.1 Updated to include new regulatory provisions and to include pre- and post-review.
- 2.2 Updated to remove the requirement for specific template letters.
- 2.3 Revised for clarity

3 POLICY

- 3.1 The University of Miami requires its health care providers and its IRBs to comply with all applicable regulations of the Food and Drug Administration (FDA) when using investigational drugs, biologics, and medical devices for clinical purposes.

4 DEFINITIONS

- 4.1 Emergency Use: The use of an investigational a drug, biologic, or medical device on a human subject in a life-threatening situation in which no standard acceptable treatment is available, and in which there is not sufficient time to obtain IRB approval ([21 CFR 56.102\(d\)](#)).
- 4.2 Expanded access: The FDA has several specific mechanisms and regulations that allow use of an investigational item outside of a formal clinical trial. This is called *expanded access*.
- 4.3 IDE: Investigational Device Exemption. An IDE application is the document submitted to the FDA for permission to conduct a clinical study using a significant risk device that is new or not approved for a given use. When the FDA approves an IDE application, it assigns an IDE number to the specific use of the device.
- 4.4 IND: Investigational New Drug. An IND application is the document submitted to the FDA for permission to conduct a clinical study using a drug or biologic that is new or not approved for a given dosage, formulation, or indication. When the FDA approves an IND application, it assigns an IND number to the specific use of the item.
- 4.5 Investigational: This term is used to refer to an item that is not FDA-approved for marketing in the United States, or to an item that is being evaluated for a new and not-yet-approved indication, dosage, or formulation.
- 4.6 Off-label use: The clinical use of an FDA-approved drug, device or biologic for a purpose or population that has not been approved by the FDA, or in a route or dose that has not been approved by the FDA. Off-label use is not regulated by the IRB or the FDA; it is subject only to any policies and procedures of state law and the clinician's institution.
- 4.7 Orphan drug: A formal designation by the FDA for drugs primarily intended to treat a rare disease or condition. See the regulations at 21 CFR 316.
Sponsor: The person, company, organization, or other entity that initiates and takes responsibility for a clinical investigation using an FDA-regulated item. The sponsor does not actually conduct the investigation unless the sponsor is a sponsor-investigator. The item is administered, dispensed, or used under the immediate direction of another individual.

5 RESPONSIBILITIES



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5.1 Specific responsibilities for researchers, HSRO staff, and the University of Miami IRB are described throughout this document. However, the researcher responsibilities described here are limited to those associated with IRB review and approval. Researcher responsibilities to the FDA (whether as a sponsor, an investigator, or an investigator sponsor) or to a sponsor are not described.

6 PROCEDURES: Health Care Providers

- 6.1 Healthcare providers are expected to comply with all University of Miami policies and procedures, including any requirements to identify in advance who will pay for the drug, biologic, or medical device and any associated tests and procedures.
- 6.2 Ensure the consent process adequately manages expectations about the possible benefits of the investigational treatment and makes clear what is known about the investigational item.
- 6.3 For Emergency Use
 - 6.3.1 As time permits, complete “WORKSHEET Emergency Use (HRP 322)” and submit to the IRB via secure email or contact an IRB Chair to discuss proposed use.
 - 6.3.2 Obtain IND or IDE by contacting FDA at:
 - 6.3.2.1 (888) 463-6332 or druginfo@fda.hhs.gov for an IND for an investigational drug;
 - 6.3.2.2 (240) 402-8360 or industry.biologics@fda.hhs.gov for an IND for an investigational biologic; and
 - 6.3.2.3 (301) 796-7100 or dice@fda.hhs.gov for investigational devices.
 - 6.3.3 Obtain Sponsor approval of Emergency Use
 - 6.3.4 Report the Emergency Use to the IRB within five (5) business days using “[Emergency Use Request Form](#)”
 - 6.3.5 Submit a protocol if it is possible that the investigational drug, biologic or medical device will be used again in the same patient or in a different patient.
 - 6.3.6 The use of an investigational product for treatment use is not considered research under DHHS regulations.
 - 6.3.7 The emergency use of an investigational drug or biologic, is a clinical investigation, the patient is a participant, and the providers are responsible for submitting reports required by the FDA.
- 6.4 For Non-Emergent Expanded Access (sometimes referred to as “compassionate use”)
 - 6.4.1 For drugs and biologics, obtain either a new IND or as a protocol/supplement to an existing IND.
 - 6.4.2 Submit an application for initial review to the IRB along with a protocol, consent document and other supporting documents.

PROCEDURES: Human Subject Research Office

- 6.5 For emergency use of a drug, biologic, or device in a life-threatening situation:
 - 6.5.1 The Designated Reviewer uses “WORKSHEET Emergency Use (HRP 322)” to determine if the proposed use complies with the FDA requirements.
 - 6.5.2 If the Designated Reviewer has indicated that the proposed use will follow FDA regulations:
 - 6.5.2.1 Complete a “TEMPLATE LETTER: Pre-Review of Emergency Use - Criteria Met (HRP-570),” or equivalent, and send to the treating physician.
 - 6.5.2.2 Set a 5 day deadline for receipt of the 5 day report.
 - 6.5.3 If the Designated Reviewer has indicated that the proposed use will NOT follow FDA regulations, complete a “TEMPLATE LETTER: Pre-Review of Emergency Use - Criteria Not Met (HRP-571),” or equivalent, and send to the treating physician.



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- 6.5.4 If the Designated Reviewer has indicated that the actual use described in the 5-day report followed FDA regulations, complete a “TEMPLATE LETTER: Review of Emergency Use - Criteria Met (HRP-572),” or equivalent, and send to the treating physician.
- 6.5.5 If the Designated Reviewer has indicated that the actual use did NOT follow FDA regulations:
 - 6.5.5.1 Complete a “TEMPLATE LETTER: Review of Emergency Use - Criteria Not Met (HRP-573),” or equivalent, and send to the physician.
 - 6.5.5.2 Manage under “SOP: New Information (HRP-024)” as Non-Compliance.

7 MATERIALS

- 7.1 SOP: New Information (HRP-024)
- 7.2 TEMPLATE LETTER: Pre-Review of Emergency Use - Criteria Not Met (HRP-571)
- 7.3 TEMPLATE LETTER: Review of Emergency Use - Criteria Met (HRP-572)
- 7.4 TEMPLATE LETTER: Review of Emergency Use - Criteria Not Met (HRP-573)
- 7.5 TEMPLATE LETTER: Pre-Review of Emergency Use - Criteria Met (HRP-570)
- 7.6 TEMPLATE LETTER: Review of Device Compassionate Use (HRP-574)

8 REFERENCES

- 8.1 21 CFR §50.23; 21 CFR §50.24; 21 CFR §56.102(d); 21 CFR §56.104(c).
- 8.2 21 CFR §812.36; 21 CFR §812.47.
- 8.3 (FDA Information Sheet Guidance for IRBs, Clinical Investigators, and Sponsors) Frequently Asked Questions About Medical Devices:
<http://www.fda.gov/downloads/RegulatoryInformation/Guidances/UCM127067.pdf>.