MCW IRB Committee Procedures

REVIEW OF EXPANDED ACCESS USE REQUESTS

Unit: Human Research Protections Program (HRPP), Office of Research

Applies to: Institutional Review Board Committees

PURPOSE:
To outline the process and steps the IRB Committee takes in reviewing and evaluating requests for expanded access use of an investigational drug, biologic or device by a physician.

When a patient has a serious or life-threatening condition that is not addressed by current approved treatments, options may exist to use an investigational medical drug, device, or biologic (i.e., one that has not been approved or cleared by FDA) to treat the patient. A variety of FDA mechanisms exist to grant this expanded access, including:

1. Treatment Use
2. Compassionate Use
3. Intermediate-Size Patient Population Expanded Access
4. Single patient Expanded Access
5. Open Label Protocol/IND
6. Humanitarian Use Devices

This policy only addresses expanded use pathways which still require FDA approval prior to expanded access. If a physician needs to treat a patient in an emergency capacity in which no standard acceptable treatment is available and in which there is not sufficient time to obtain prospective IRB approval, please see *IRB Member SOP: Emergency Use of Investigational Drugs, Devices or Biologics*

DEFINITIONS:
Expanded Access: The use outside of a clinical trial of an investigational medical product (i.e., one that has not been approved by FDA). This term is used broadly by the FDA. It can cover treatment use and emergency use. It is often used by the device arm of the FDA synonymously with compassionate use. It is often used by the drug arm of the FDA to address intermediate-size patient population expanded access and single patient expanded access.

Clinical Trial: A research project in which one or more human subjects are prospectively assigned to one or more interventions (which may include placebo or other control) to evaluate the effects of the interventions on biomedical or behavioral health-related outcomes.

Treatment Use: The use of an unapproved drug, biologic or device when the primary purpose is to diagnose, monitor, or treat a patient's disease or condition rather than to obtain information in support of a clinical trial.

Treatment IND/IDE: There are four requirements that must be met before the FDA will issue a treatment IND/IDE:

1. The product is intended to treat a serious or immediately life-threatening disease;
2. There is no satisfactory alternative treatment available;
3. The product is already under investigation, or trials have been completed; and
4. The trial sponsor is actively pursuing market approval.

Compassionate Use: This term is used primarily by the device arm of the FDA. Compassionate use can be for devices that are being studied in a clinical trial under an IDE for patients who do not meet the requirements for inclusion in the clinical investigation but for whom the treating physician believes the device may provide a benefit in treating and/or diagnosing their disease or condition. It can also be used for devices that are not being studied in a clinical investigation (i.e., an IDE for the device does not exist). This provision is typically approved for individual patients but may be approved to treat a small group.

Immediately Life-Threatening Disease: 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.

Open Label Protocol or Open Protocol IND: These are usually uncontrolled studies, carried out to obtain additional safety data (Phase 3 studies). They 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 Institutional Review Board (IRB) review and informed consent.

Humanitarian Use Device (HUD): A medical device intended to benefit patients in the treatment or diagnosis of a disease or condition that affects or is manifested in not more than 8,000 individuals in the United States per year.

PROCEDURE:

1. Under Federal Regulations, the IRB Committee is charged to review a physician's application for expanded access use of an investigational drug, biologic or device, prior to the use.
2. For the review of the expanded use submission, the HRPP office will assign IRB Reviewers in accordance with *Staff: Assigning Primary Reviewers and Use of Consultants*
    a. For projects which involve a Humanitarian Use Device, the IRB Committee considers the project and reviews it in conjunction with the federal regulations regarding the conduct, design and consenting process of these projects.
3. The IRB Committee will review the submission to ensure the appropriate regulatory elements are addressed per the federal regulations. The IRB Committee will complete the *IRB Member Form: Expanded Access Reviewer's Checklist.*
4. The IRB Committee will determine if the submission meets the criteria for approval, per the federal regulations. An IRB decision letter will be issued to the Investigator, along with the consent form in accordance with *Staff: Creation and Processing of IRB Meeting Minutes and Decision Letters.* The IRB Decision Letter will include instructions to the Investigator to file follow up reports regarding the subjects outcome 30 days and 90 days post use.

REFERENCES:

21 CFR 312 subpart I

21 CFR 812.35
21 CFR 812.36

SUPPORTING DOCUMENTS:
IRB Member SOP: Emergency Use of Investigational Drugs, Devices or Biologics
IRB Member Form: Expanded Access Reviewer's Checklist
Staff: Assigning Primary Reviewers and Use of Consultants
Staff: Creation and Processing of IRB Meeting Minutes and Decision Letters

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