Covenant HEALTH **Clinical Research**	**SUBJECT:** Expanded Access and Compassionate Use Policy **PAGE** 1 **OF** 3
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System Policy
To be used by: *Fort Loudoun Medical Center, Methodist Medical Center, Parkwest Medical Center, Roane Medical Center, Fort Sanders Regional Medical Center, LeConte Medical Center, Morristown Hamblen Healthcare System, Cumberland Medical Center, Claiborne Medical Center* (each a "Covenant Health Entity", and collectively, the "Covenant Health Entities")

Keywords: Compassionate Use, Expanded Access, Investigational Products, FDA, Participants, IRB Review

Scope: This policy applies to expanded access and compassionate use requests for investigational products with active ingredients that have not been approved for any purpose in the United States, or are otherwise not available in the United States through normal commercial channels. For the purposes of this policy, an expanded access or compassionate use request is any request for the use of an investigational product outside the scope of a clinical study. The expanded access and compassionate use provision allows access for patients who do not meet the requirements for inclusion in the clinical investigation, but for whom the treating physician believes the investigational product or device may provide a benefit in treating and/or diagnosing their disease or condition. This provision is typically approved for individual patients, but may be approved to treat a small group.

Purpose: The FDA Expanded Access Program (EAP) allows for compassionate use for treatment purposes in patients with serious diseases or conditions when there are no comparable or satisfactory alternative therapies to diagnose, monitor or treat the patient's disease or condition and the sponsor or manufacture agrees to provide the drug or device for treatment purposes.

Policy: According to the FDA, expanded access and compassionate use may be appropriate when all of the following apply:

- Patient has a serious disease or condition, or whose life is immediately threatened by their disease or condition. Immediate life-threatening disease or condition means a stage of disease in which there is reasonable likelihood that death will occur within a matter of months or in which premature death is likely without early treatment. Serious disease or condition means a disease or condition associated with morbidity that has substantial impact on day-to-day functioning.
- There is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition.
- Patient enrollment in a clinical trial is not possible.

- Potential patient benefit justifies the potential risks of treatment.
- Providing the investigational medical product will not interfere with investigational trials that could support a medical product's development of marketing approval for treatment indication.

Investigational drugs, biologics or medical devices have not yet been approved or cleared by FDA and FDA has not found these products to be safe and effective for their specific use. Furthermore, the investigative medical product may, or may not, be effective in the treatment of the condition, and use of the product may cause unexpected serious side effects.

Under the 2009 final EAP rule, expanded access to investigational drugs for treatment will be available to:

- Individual Patient IND, including Emergency Use IND [21 CFR 312.310] commonly held by treating physician or investigator for treatment of an individual patient.
- Intermediate Population Treatment IND [21 CFR 312.315] commonly held by the sponsor or manufacturer for use in population smaller than typical of treatment IND or treatment protocol. The investigational drug for intermiate population treatment INDs may be in active development or may be an FDA approved drug that is unavailable or in limited supply.
- Large Population Treatment IND or treatment protocol [21 CFR 312.320] commonly held by the sponsor for widespread treatment use. For a large population treatment INDs, the sponsor or manufacturer must be pursuing marketing approval.

Procedure: A physician may seek expanded access and compassionate use of an investigational medical product once it has been determined that there are no available clinical trials for the patient. When enrollment is not possible or feasible, expanded access and compassionate use may be an option for gaining access to an investigational medical product. The physician must confirm the patient's current disease or condition qualifies for expanded access and compassionate use, meaning that the patient has either a serious or immediately life-threatening disease or condition and there is no available comparable or satisfactory alternative available for the patient. The physician must then identify the appropriate expanded access request type. If the patient meets criteria for expanded access, the physician must speak with the sponsor or manufacturer to see if they will provide the investigational medical product for expanded access use. Once permission from sponsor is obtained, both the physician and the sponsor have to work together to request the FDA to approve the medical product for use for the single patient or small group of patients. The FDA requires the reason for which the request is being made, the treatment plan and a consent form signed by the patient.

Prior to treating the patient, the physician must submit the following documents to the IRB for review and approval:

- FDA approval documents of expanded access or compassionate use for medical product
- Letter from sponsor granting permission for use of medical product and their willingness to provide medical product for use
- Treatment plan for medical product use along with sponsor required data to be collected
- Informed consent document to be used

Investigator Responsibilities:
In all cases of expanded access, investigators are responsible for reporting adverse drug events to the sponsor and IRB, ensuring that the informed consent requirements are met, ensuring that IRB review of the expanded access use is obtained in a manner consistent with the requirements of Covenant Health

IRB, and maintaining accurate case histories and drug disposition records and retaining records in a manner consistent with FDA regulations.

In general, expanded access and compassionate use treatment will be discontinued when the investigative medical product becomes commercially available.

Reference(s):
21 CFR 312.300-312.320