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Lakeland Regional Health

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Owner	Trudy Wittenberg: 4388 Institutional Review Board Administrator
Department	HRPP/Research

Research: Expanded Access, Non-Emergency Compassionate Use (Devices)- AD.0202

PURPOSE

The purpose of this policy is to outline the **Principal Investigator's** responsibilities, IRB submission requirements and review process for non-emergency compassionate use of a device. Our policies guide our practices and ensure that we place people at the heart of all we do to deliver the best outcomes and safest care.

APPLICABILITY

This policy applies to Lakeland Regional Health's **Workforce** and Research Personnel engaged in research. This policy applies to use of investigational devices or other interventions that are regulated by the FDA. This policy does not regulate or interfere with the clinical practice of medicine.

POLICY

I. General

A. A Principal Investigator may use an unapproved device for compassionate use after obtaining approval from the convened IRB or IRB Chair/Vice Chair, as appropriate.

B. Expanded access for medical devices is a potential pathway for patients with either a serious, life-threatening disease, or condition, to access an investigational medical device that has not been approved or cleared by the FDA for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available. There are three categories, but this policy will cover Compassionate Use:

1. Emergency Use

i. The requirements and Principal Investigator responsibilities for emergency use of a device are outlined in the Emergency Use Policy.

2. Treatment **Investigational Device Exemption (IDE)**
    i. Treatment investigational device exemptions will be referred for review and approval by the convened board in accordance with the Investigational Device Policy.
3. Compassionate Use (or Individual Patient/Small Group Access)
    i. A Principal Investigator, who must be a licensed physician, may want to access an investigational device that has not received FDA approval or clearance if they conclude that: (1) the patient has a life-threatening or serious condition; (2) no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition exists; and (3) the potential patient benefit justifies the potential risks of the investigational device.
    ii. Unlike emergency use of an unapproved device, prior FDA approval is needed before compassionate use occurs.
    iii. Prior to compassionate use, a Principal Investigator must obtain approval of the Chair or Vice-Chair, or full IRB to satisfy the requirement to obtain clearance from the Institution.

PROCEDURE

I. Requirements for Submission to the IRB (via IRBNet) before compassionate use, regardless of whether there is an IDE for the device or not:
    A. The Principal Investigator must submit (in IRBNet):
        1. A description of the patient's condition and the circumstances necessitating treatment;
        2. 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;
        3. An identification of any deviations in the approved clinical protocol for the IDE that may be needed to treat the patient;
        4. The patient protection measures that will be followed:
            i. A consent form that contains the information required under 21 CFR § 50.25;
            ii. An independent assessment of a physician who is not participating in the study or in the care of the patient that concurs with the planned usage; and
            iii. Authorization from the device manufacturer on the use of the device.
        5. An appropriate schedule for monitoring the patient to detect any possible problems arising from the use of the device, taking into consideration the investigational nature of the device and the specific needs of the patient.

6. Evidence of FDA approval of the compassionate use;
7. If there is an IDE for the device, confirmation of the IDE by the sponsor;
8. If there is no IDE for the device: A description of the device provided by the manufacturer;
9. The Principal Investigator's Brochure (if available);
10. PI's CV;
11. Conflict of Interest for PI; and
12. Confidentiality Agreement for PI.

II. Post-Approval Requirements

A. Following the compassionate use of the device, a follow-up report should be submitted to the FDA by whomever submitted the original compassionate use request to FDA within forty-five (45) days of using the investigational device. The report should present summary information regarding patient outcome and a copy should be sent to the IRB. If any problems occurred as a result of device use, these should be discussed and also reported to the reviewing IRB as soon as possible.

B. The Principal Investigator is responsible for complying with FDA and other regulatory requirements and policies, including, but not limited to, obtaining IRB approval of proposed modification to the treatment plan (unless necessary to eliminate apparent immediate hazard to the participant), prompt reporting of unanticipated problems (link UAP & SAE Policy, link Protocol Deviation & Non-Compliance Policy), submitting continuing reviews and study closure, as applicable.

C. If there is an IDE for the device, the above compassionate use criteria and procedures can also be applied when a physician wishes to treat a few patients rather than an individual patient suffering from **serious disease or condition** for which no alternative therapy adequately meets the medical need. In this case, the IDE supplement (also submitted to FDA) should include the information identified above and indicate the number of patients to be treated. The supplement should also include the protocol to be followed or should 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.

III. IRB Responsibilities

A. IRB Review of Individual Patient Expanded Access/Compassionate Use

1. The IRB Administrator or designee will check all submissions for completeness (link Expanded Access submission checklist) and if anything is missing, the IRB Administrator will communicate with the PI and study team until the submission is complete and ready for review. Incomplete applications or those with personnel who are not current regarding training will not be accepted or distributed for review.
2. The IRB Administrator or designee will complete a worksheet verifying that all of the required documents are in the submission and upload it into IRBNet.

3. The submission will be reviewed by the Chair or Vice Chair, and the reviewer will:
    i. Assess the risks and benefits of treatment for the particular patient involved, whether risks to the patient have been minimized and that such risks are reasonable in relation to anticipated benefits;
    ii. When the request is for a pediatric patient, confirm that adequate provisions are included for soliciting age-appropriate assent from children and permission from a parent or guardian, as required under 21 CFR § 50.55.
    iii. Confirm that the informed consent document contains the information required under 21 CFR § 50.25;
        a. Given that the device used under expanded access is investigational, a statement in the informed consent document indicating that although the primary use of the device is for treatment, the device is investigational and FDA has not determined that the device is safe or effective for use in treating the condition, will satisfy the requirement under 21 CFR §50.25(a)(1) that the informed consent provide a statement that the use of the product "involves research."
    iv. Confirm the validity of the IDE (if applicable) number.
        a. The IRB will confirm the validity of the IDE number by requiring that the Principal Investigator submit the notification of the IDE number assigned from the FDA, a letter from the sponsor, or the commercially or NIH-sponsored protocol containing the IDE number.
4. The reviewer documents their review and determinations.
5. The reviewer establishes the continuing review frequency.
6. The Chair or Vice Chair will provide a letter of approval to the Principal Investigator via IRBNet.
7. At any time, the reviewer may forward the submission to the full IRB board.

DEFINITIONS

Investigational Device Exemption (IDE): IDEs are regulated under 21 CFR Part 812. An approved IDE means that the IRB (and FDA for significant risk devices) has approved the sponsor's study application and the requirements under 21 CFR Part 812, as applicable, are met. An investigational device exemption (IDE) allows the investigational device to be used in a clinical study in order to collect safety and effectiveness data. An approved IDE permits a device to be shipped lawfully for the purpose of conducting investigations of the device without complying with other requirements of the Food, Drug, and Cosmetic Act (FD&C Act) that would apply to devices in commercial distribution.

Principal Investigator: Individual who conducts a clinical investigation (i.e., under whose immediate direction the test article is administered or dispensed to, or used involving a subject) or, in an investigation conducted by a team, the responsible leader of that team. (21 CFR § 56.102)

Serious Disease or Condition: A disease or condition associated with morbidity that has substantial impact on day-to-day functioning. Short-lived and self-limiting morbidity will usually not be sufficient, but the morbidity need not be irreversible, provided it is persistent or recurrent. Whether a disease or condition is serious is a matter of clinical judgment, based on its impact on such factors as survival, day-to-day functioning, or the likelihood that the disease, if left untreated, will progress from a less severe condition to a more serious one.

Test Article: Any drug (including a biological product for human use), medical device for human use, human food additive, color additive, electronic product, or any other article subject to regulation under the act or under sections 351 and 354-360F of the Public Health Service Act (42 U.S.C. 262 and 263b-263n).

Workforce: All LRH employees, volunteers, trainees/students, contractors, and medical staff.

REFERENCES

I. CHOP - Individual Patient Expanded Access for Drugs and Devices
II. CHOP - IND-IDE
III. Investigational Medical Devices | Research (virginia.edu)
IV. 21 CFR PART 50
V. 21 CFR PART 56
VI. 21 CFR PART 312
VII. 21 CFR PART 812

Approval Signatures

Step Description	Approver	Date
	Danielle Drummond: 0001 President & Chief Executive Officer - LRHS	05/2024
	Jonn Hoppe: 1011 Executive VP, Chief Legal Officer-General Cou	05/2024
	Timothy Regan: 0009 President - LRMC/Chief Medical Officer	05/2024

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Sheena Butts: 0016 AVP - Surgical and Procedural Services	05/2024
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Anita Henry: 4622 Sr. Director - Supply Chain	03/2024
Deana Nelson: 4080 SVP - Administration and Corporate Initiative	03/2024
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Andrew Bugajski: 4387 AVP - Research and Sponsored Studies	02/2024