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Research: Expanded Access, Non-Emergency Individual Patient IND (Drugs/Biologics)- AD.0203

PURPOSE

The purpose of this policy is to outline the investigator's responsibilities, IRB submission requirements and review process for non-emergency expanded access/treatment use of a drug/biologic for individual patients. 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 drugs**, biologics, diagnostics, 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. An investigator may use an unapproved drug/biologic for individual patient treatment after obtaining approval from the convened IRB or IRB Chair/Vice Chair, as appropriate.

B. Expanded access for drugs/biologics refers to the use of an investigational drug when the primary purpose is to use the drug to diagnose, monitor, or treat a patient's disease or condition rather than to generate scientific information intended to characterize the safety and effectiveness of a drug. There are three categories:

1. Expanded access for individual patients

i. The requirements and investigator responsibilities for

emergency use of a drug are outlined in Research: Emergency Use of Drugs/Biologics for Individual Patients- AD.0199.

2. Expanded access for intermediate-size populations
    i. Will be referred for review and approval by the convened board in accordance with Full Board SOP.
    ii. An intermediate-size population IND has no fixed numerical requirement, but it is for more than one (1) patient and is generally employed when the investigational drug is not actively being developed for marketing.
    iii. At this time, Lakeland Regional Health (LRH) and the LRH IRB do not participate in intermediate-size populations for expanded access.

3. Expanded access for widespread treatment use through a treatment IND or treatment protocol
    i. Treatment IND - Access to an investigational drug (including a biologic) for treatment use by a large (widespread) population, submitted as a protocol under a new IND. The investigational product must be under active development for marketing. Unless FDA notifies the sponsor that treatment may begin earlier, there is a thirty (30) day waiting period before treatment may begin.
    ii. Treatment Protocol - Access to an investigational drug (including a biologic) for treatment use by a large (widespread) population, submitted as a protocol to an existing IND by the sponsor of the existing IND. The investigational product must be under development for marketing. Unlike other access protocols submitted to existing INDs, there is a thirty (30) day waiting period before treatment may begin, unless FDA notifies the sponsor that treatment may begin earlier.
    iii. At this time, LRH and the LRH IRB do not participate in widespread/large-size populations for expanded access. For guidelines on investigational drugs, see Research: Investigational Drugs- AD.0156.

II. Expanded Access for Drugs/Biologics – Individual Patient
    A. An investigator, who must be a licensed physician, may want to treat a patient with an investigational drug if they conclude that:
        1. The patient has a **serious or immediately life-threatening disease or condition** and has no other comparable or satisfactory therapeutic options; and
        2. The patient cannot obtain the drug under another IND or protocol.
    B. An investigator, who must be a licensed physician, who submits a non-emergency

individual patient expanded access IND may request a waiver from the requirement for full IRB review from the FDA. The waiver may be requested using Form FDA 3926 (by selecting the appropriate box on that form to request a waiver under 21 CFR § 56.105 of the requirements in § 56.108(c), which relates to full IRB review). A physician submitting an individual patient expanded access IND using Form FDA 1571 may include a separate waiver request with the application.

1. If such a waiver is requested, the physician (sponsor-investigator) will need to obtain approval from the IRB Chair or Vice Chair before treatment use begins.
2. If such a waiver is not requested, the single subject use will be referred for review and approval by the convened board (SOP-Research: Full Board Review) before treatment use begins.

C. The IRB can provide approval by the IRB Chair or Vice Chair, for the single subject use provided the investigator holds the IND and the FDA has granted a waiver as requested in the application.

1. Individual patient INDs and individual patient protocols submitted to an existing IND that is held by a commercial sponsor will be referred for review by the convened IRB board.

PROCEDURE

I. Requirements for Submission to the IRB to obtain approval of the Chair, Vice-Chair or Full Board before treatment use begins:

1. The investigator must submit (in IRBNet):

i. A thorough patient history and treatment plan, included in the Form FDA 3926 or in another document; which should include:

a. The proposed dosing schedule, route, and frequency of administration, duration of planned treatment, criteria for discontinuation of treatment, potential adverse events, and planned dose modifications in the event that there are adverse events;

b. The planned monitoring for potential adverse events, response to treatment, and changes in clinical status, as well as proposed modifications to the treatment plan to mitigate risks to patients if appropriate;

c. The key details of the patient's history, including diagnosis and summary of prior therapy (including response to such therapy), as well as information regarding a patient's relevant clinical characteristics (such as comorbid conditions and concomitant medications) that are necessary to assess the potential for increased risks of the drug;

d. A summary of known risks of the drug (this information may be included in the consent form or the Investigator Brochure, rather than the treatment plan, as applicable), and

e. An assessment that the probable risk to the patient from the investigational drug is not greater than the probable risk from the disease or condition. Note: The information provided to the IRB should match what was/will be submitted to the FDA.

ii. A consent form that contains the information required under 21 CFR § 50.25;

iii. The Investigator's Brochure (if available);

iv. **PI's** CV;

v. Conflict of Interest for PI;

vi. Confidentiality Agreement for PI; and

vii. The FDA-issued individual patient expanded access IND.

II. Post-Approval Requirements

A. The PI is responsible for complying with FDA and other regulatory requirements and policies, including, but not limited to, obtaining prospective IRB approval of proposed modification to the treatment plan, prompt reporting of unanticipated problems and non-compliance (see Research: Unanticipated Problems & Serious Adverse Events Reporting- AD.0193 and Research: Protocol Deviations & Non-compliance- AD.0194), and submitting continuing reviews and study closure, as applicable.

III. IRB Responsibilities

A. 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 to 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.

B. 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.

C. The submission will be reviewed by the Chair, Vice Chair, or Full Board, and the reviewer will:

1. 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;

2. 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.

3. Confirm that the informed consent document contains the information required under 21 CFR § 50.25;

i. Given that the drug/device used under expanded access is investigational, a statement in the informed consent document indicating that although the primary use of the drug/device is for treatment, the drug/device is investigational and FDA has not

determined that the drug/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.

4. Confirm the validity of the IND (if applicable) number.
    i. The IRB will confirm the validity of the IND number by requiring that the investigator submit the notification of the IND number assigned from the FDA, a letter from the sponsor, or the commercially or NIH-sponsored protocol containing the IND number.

D. The reviewer documents their review and determinations.

E. The reviewer establishes the continuing review frequency.

F. The Chair or Vice Chair will provide a letter of approval to the investigator via IRBNet.

G. At any time, the reviewer may forward the submission to the full board.

DEFINITIONS

Investigational New Drug: A new drug or biological drug that is used in clinical investigation; See 21 CFR § 312.3(b).

Investigational New Drug Application (IND): A request for authorization from the Food and Drug Administration (FDA) to administer an investigational drug or biological product to humans. Such authorization must be secured prior to interstate shipment and administration of any new drug or biological product that is not the subject of an approved New Drug Application or Biologics/Product License Application.

PI: Principal investigator.

Serious Disease or Condition: A serious or immediately life-threatening 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 § 50
V. 21 CFR § 56
VI. 21 CFR § 312
VII. 21 CFR § 812

Approval Signatures

Step Description	Approver	Date
	Danielle Drummond: 0001 President & Chief Executive Officer - LRHS	03/2024
	Jonn Hoppe: 1011 Executive VP, Chief Legal Officer-General Cou	03/2024
	Timothy Regan: 0009 President - LRMC/Chief Medical Officer	03/2024
	Renee Reed: 4064 Senior Attorney	03/2024
	Rodriguez Dangerfield: 0028 Director - Pharmacy	03/2024
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	Georgia Ann Keriazes: 0729 QI/ Due Pharmacist	03/2024
	Andrew Bugajski: 4387 AVP - Research and Sponsored Studies	02/2024