

The Christ Hospital IRB

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STANDARD OPERATING PROCEDURE

Expanded Access- Investigational New Drugs

POLICY:

This policy outlines the IRB process for expanded access use of investigational new drugs (IND). Expanded access, sometimes called “compassionate use”, refers to the use of an investigational drug or biologic when the primary purpose is to diagnose, monitor, or treat a patient rather than to obtain the kind of information about the drug that is generally derived from clinical trials. The expanded access 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 medical product 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.

All Expanded Access to Investigational New Drugs (IND) must meet the following criteria in FDA regulation 21 CFR 312-305(a)-

1. The patient or patients to be treated have a serious or immediately life-threatening disease or condition, and there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition;
2. The potential patient benefit justifies the potential risks of the treatment use and those potential risks are not unreasonable in the context of the disease or condition to be treated; and
3. Providing the investigational drug for the requested use will not interfere with the initiation, conduct, or completion of clinical investigations that could support marketing approval of the expanded access use or otherwise compromise the potential development of the expanded access use.

There are three categories of expanded access based on the number of people who need access and the level of risk. An expanded access IND submission is required to FDA for each type of expanded access. The submission may be a new IND or a protocol amendment to an existing IND.

Expanded access to investigational drugs for treatment use may be available in the following circumstances:

1. **Individual Patient IND or Protocol, including Emergency Use¹ IND** [21 CFR 312.310] - commonly held by treating physician or investigator for treatment of an individual patient. The product may or may not be under development.

Additional Criteria for Individual Patient Access:

¹ See SOP 1.10 Emergency Use for procedure

- The probable risk to the person from the investigational drug is not greater than the probable risk from the disease or condition; and
 - FDA must determine that the patient cannot obtain the investigational drug under another IND or protocol
2. **Intermediate Population Treatment IND or Treatment Protocol** [21 CFR 312.315] – commonly held by the sponsor (manufacturer) for use in population smaller than typical of treatment IND or treatment protocol. The investigational drug for intermediate population treatment INDs may be in active development or may be an FDA approved drug that is unavailable or in limited supply.

Additional Criteria for Intermediate Population Access:

- The drug is being investigated in a controlled clinical trial under an IND designed to support a marketing application for the expanded access use, or all clinical trials of the drug have been completed
 - The sponsor is actively pursuing marketing approval of the drug for the expanded access use with due diligence; and
 - When the expanded access use is for a serious disease or condition, there is sufficient clinical evidence of safety and effectiveness to support the expanded access use. Such evidence would ordinarily consist of data from phase 3 trials, but could consist of compelling data from completed phase 2 trials; or when the expanded access use is for an immediately life-threatening disease or condition, the available scientific evidence, taken as a whole, provides a reasonable basis to conclude that the investigational drug may be effective for the expanded access use and would not expose patients to an unreasonable and significant risk of illness or injury. This evidence would ordinarily consist of clinical data from phase 3 or phase 2 trials, but could be based on more preliminary clinical evidence.
3. **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 must be pursuing marketing approval.

Additional Criteria for Intermediate Population Access:

- There is enough evidence that the drug is safe at the dose and duration proposed for expanded access use to justify a clinical trial of the drug in the approximate number of patients expected to receive the drug under expanded access; and
- There is at least preliminary clinical evidence of effectiveness of the drug, or of a plausible pharmacologic effect of the drug to make expanded access use a reasonable therapeutic option in the anticipated patient population.

PROCEDURE

INVESTIGATOR/SPONSOR

1. Submits IND Application to FDA- FDA approval is required prior to IRB approval and before expanded access/compassionate use occurs (30 calendar day review timeframe). In order to obtain FDA approval, the sponsor should submit an IND Application on Form 1571, 1572, or 3926 (Emergency and Individual Access only).
2. Upon FDA approval, the following must be submitted to a convened IRB for consideration:
 - a. FDA Approval letter
 - b. Study Application
 - c. Protocol, if applicable
 - d. Disclosure of Financial Interest form

- e. HIPAA Request for Partial Waiver, if applicable, and
- f. Informed Consent form

Note: All requests submitted to the IRB requiring full board review are distributed to all IRB members approximately 2 weeks prior to the meeting date, and therefore must be received in sufficient time for distribution.

3. Submits to the IRB any updates or changes including but not limited to:
 - a. amendments to the protocol/treatment plan
 - b. unanticipated problems
 - c. changes to FDA approval status
 - d. when the expanded access is completed, and
 - e. continuing review report, if treatment continues beyond the IRB approval date.

CONVENED IRB

1. Reviews the request for expanded access and all submission documents
2. Votes to approve, approve with minor or major modifications, table, or disapprove the use
 - a. The IRB may approve the expanded access use for up to 1 year. If treatment continues beyond 1-year, continuing review is required.
3. Performs continuing review for expanded access that continues beyond the approval period.

IRB OFFICE

1. Communicates IRB decision to the investigator in writing.
 - a. Approvals are given a 1-year approval, effective the date of the convened meeting, and expiring on the 1st day of the month, 12 months later.
2. Facilitates continuing review for expanded access that continues beyond the approval period.

REFERENCE:

- 21 CFR 56 IRB Regulation
- 21 CFR 50 Informed Consent of Human Subjects
- 21 CFR 312.310 (Expanded Access for Individual Patients)
- 21 CFR 312.315 (Expanded Access for Intermediate Sized Patient Populations)
- 21 CFR 312.320 (Expanded Access for Widespread Treatment Use Through a Treatment IND or Treatment Protocol)
- IRB Reference Manual 13 Expanded Access to Investigational Medical Products

REVISION HISTORY:

Date Revised	Reason For Change	Revised By
04/06/15	Updated definitions of Expanded Access	Becky
02/06/19	Updated to reflect FDA changes	Emily
03/09/21	Updated to reflect refine procedure and create separate SOP's for IND from IDE	Erica