The Christ Hospital IRB
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STANDARD OPERATING PROCEDURE

Expanded Access to Investigational Devices

1.0 POLICY

This policy outlines the IRB process for expanded access use of investigational devices. An unapproved medical device may normally only be used on human subjects through an approved clinical study in which the subjects meet certain criteria, and the device is only used in accordance with the approved protocol by a clinical investigator participating in the clinical trial. However, there may be circumstances under which a health care provider may wish to use an unapproved device to save the life of a patient or to help a patient suffering from a serious disease or condition for which no other alternative therapy exists. Patients/physicians faced with these circumstances may have access to investigational devices under one of three mechanisms by which FDA may makes an unapproved device available:

- Emergency Use[1]
- Compassionate Use (or Individual Patient/Small Group Access)
- Treatment Investigational Device Exemption (IDE)

These mechanisms can be utilized during a certain timeframe in the Investigational Device Exemption (IDE) process if the criteria are met. FDA approval is required except in the case of emergency use.

Expanded access refers to the use of an investigational medical device when the primary purpose is to diagnose, monitor, or treat a patient rather than to obtain the kind of information about the device 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.

1.1 Compassionate Use (or Individual Patient/Small Group Access)

The compassionate use provision provides a path to accessing investigational devices that have not received FDA approval or clearance for patients for whom the treating physician believes the device may provide a benefit in treating and/or diagnosing their disease or condition. Compassionate use can be for devices that are being studied in a clinical trial under an IDE, or Humanitarian Device Exemption (HDE), for patients who do not meet the requirements for inclusion in the clinical investigation but for whom the

[1] See SOP 1.10 Emergency Use for procedure

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 or HDE for the device does not exist). This provision is typically approved for individual patients but may be approved to treat a small group.

Criteria:

- The patient has a life-threatening or serious disease or condition
- There is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition; and
- Potential patient benefit justifies the potential risks of the investigational device.

The IDE supplement request for FDA approval requires prior concurrence from the IRB chair or his/her designee. In some cases, the TCH IRB will not approve the request until they have approval from the FDA. In such cases, the original request should indicate that IRB approval will be obtained prior to use of the device. Proof of the approval by the IRB Chair will need to be submitted with the follow-up report after the patient is treated.

The physician should not treat the patient identified in the supplement until FDA approves use of the device under the proposed circumstances. Treating physicians must monitor patient(s) receiving compassionate use devices and submit a written summary of the results of the use, including any safety related information, to the IDE sponsor or FDA and the IRB.

1.2 Treatment IDE

In the case of a serious disease, a device ordinarily may be made available for treatment use under this section after all clinical trials have been completed. In the case of an immediately life-threatening disease, a device may be made available for treatment use under this section prior to the completion of all clinical trials.

An approved IDE specifies the maximum number of clinical sites and the maximum number of human subjects that may be enrolled in the study. During the course of the clinical trial, if the data suggests that the device is effective, then the trial may be expanded to include additional patients with life-threatening or serious diseases. This is called a treatment IDE.

A Treatment IDE may occur during the clinical trial or prior to final action on the marketing application in cases where it is appropriate to use the device in the treatment of patients not in the trial under the provisions of the treatment investigational device exemptions (IDE) regulation.

Criteria:

- The device is intended to treat or diagnose a serious or immediately life-threatening disease or condition

- There is no comparable or satisfactory alternative device available to treat or diagnose the disease or condition in the intended patient population
- The device is under investigation in a controlled clinical trial for the same use under an approved IDE, or all clinical trials have been completed; and
- The sponsor of the controlled clinical trial is pursuing marketing approval or clearance of the investigational device with due diligence.

Treatment use of an investigational device is conditioned upon the sponsor and investigators complying with the safeguards of the IDE process and the regulations governing informed consent (21 CFR 50) and IRB review (21 CFR 56). The full IRB reviews the protocol using the same procedures as regular, full IDE protocols.

The sponsor of a treatment IDE must submit progress reports on a semi-annual basis to all reviewing IRB's and FDA until the filing of a marketing application. The progress report must also include the number of patients treated with the device under the treatment IDE, the names of the investigators participating in the treatment IDE, and a brief description of the sponsor's efforts to pursue marketing approval/ clearance of the device.

The sponsor of a treatment IDE is responsible for submitting all other reports required under 21 CFR 812.150, such as unanticipated adverse device effects and final reports. The reports are submitted as supplements to the original IDE application.

2.0 PROCEDURE

2.1 Submitting Physician/Sponsor

2.1.1 Submits the request to FDA. FDA approval is required prior to IRB approval and before expanded access/compassionate use occurs

2.1.1.1 If there is an IDE/HDE for the device, submits an IDE Supplement for Compassionate Use or a treatment IDE application for Treatment Use to FDA (30 calendar day review timeframe).

2.1.1.2 If there is no IDE/HDE for the device, submits a compassionate use request for a single patient to FDA.

2.1.2 Creates a Compassionate Use submission in Mentor IRB. The following documentation should be uploaded with the submission for IRB consideration:

2.1.2.1 FDA Approval letter, or notification that this is pending IRB review

2.1.2.2 IRB Application or Treatment Plan, including proposed length of treatment

2.1.2.3 Protocol, if applicable

2.1.2.4 An independent assessment from an uninvolved physician

2.1.2.5 Authorization from the device manufacturer on the use of the device

2.1.2.6 Notification of institutional approval for the use of the device

2.1.2.7 Proposed Informed Consent form

Note: Submissions must be received prior to the set meeting deadline to be reviewed at the next IRB meeting.

2.1.3 Submits to FDA a follow-up report within 45-days of using the investigational device

2.1.4 Submits to the IRB any updates or changes including but not limited to:

2.1.4.1 amendments to the protocol/treatment plan,

2.1.4.2 written results of the use, including safety information,

2.1.4.3 notice of any problems that occurred as a result of device use

2.1.4.4 changes to FDA approval status,

2.1.4.5 when the expanded access is completed, and

2.1.4.6 continuing review report if treatment continues beyond the IRB approval date.

2.2 IRB Chair Review (For Compassionate Use (or Individual Patient/Small Group Access))

2.2.1 Reviews all submission documents utilizing the IRB Compassionate Use/Expanded Access Checklist

2.2.1.1 Confirms the medical evaluation of the patient's condition that there is no comparable or satisfactory alternative available, including already approved therapies or other clinical trials for which the patient might be eligible

2.2.1.2 Ensures that the informed consent or appropriate permissions will be obtained and documented per 21 CF 50.25

2.2.1.3 Reviews documentation that FDA has made its determinations regarding safety and effectiveness

2.2.1.4 Reviews the treatment plan to determine that it makes adequate provision for ensuring the safety of the patient, including adequate monitoring and appropriate plans for collecting and reporting the data, that the physician is qualified to administer the treatment, and that treatment facility is adequately equipped

2.2.2 Makes either determination

2.2.2.1 (a) Concurs with the use, or (b) determines review frequency. The IRB Chair may approve the expanded access use for up to 1 year. If treatment continues beyond 1-year, continuing review is required.

2.2.2.2 Defers to convened board for full review

2.2.3 Performs continuing review for expanded access that continues beyond the approval period.

2.3 CONVENED IRB REVIEW (for Treatment IDE and Deferrals)

2.3.1 Reviews the request for expanded access and all submission documents utilizing the IRB: Compassionate Use/Expanded Access Checklist

2.3.1.1 Confirms the medical evaluation of the patient's condition that there is no comparable or satisfactory alternative available, including already approved therapies or other clinical trials for which the patient might be eligible

2.3.1.2 Ensures that the informed consent or appropriate permissions will be obtained and documented per 21 CF 50.25

2.3.1.3 Reviews documentation that FDA has made its determinations regarding safety and effectiveness.

2.3.1.4 Reviews the treatment plan to determine that it makes adequate provision for ensuring the safety of the patient, including adequate monitoring and appropriate plans for collecting and reporting the data, that the physician is qualified to administer the treatment, and that treatment facility is adequately equipped.

2.3.2 Votes to approve, approve with minor or major modifications, table, or disapprove the use. The IRB may approve the expanded access use for up to 1 year. If treatment continues beyond 1-year, continuing review is required.

2.3.3 Performs continuing review for expanded access that continues beyond the approval period.

2.4 IRB Office

2.4.1 Reviews the submission utilizing the IRB Office: Compassionate Use/Expanded Access Checklist to:

2.4.1.1 Ensure all ancillary reviews have been completed,

2.4.1.2 Ensure that all required documentation has been uploaded, and

2.4.1.3 Ensure that all key research personnel have valid credentials uploaded to their Mentor Accounts.

2.4.2 Communicates IRB decision to the investigator in writing. 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.

2.4.3 Facilitates continuing review for expanded access that continues beyond the approval period.

3.0 REFERENCES

3.1 21 CFR 56 IRB Regulation

3.2 21 CFR 50 Informed Consent of Human Subjects

3.3 21 CFR 812 Investigational Device Exemption

3.4 IRB Reference Manual 13 Expanded Access to Investigational Medical Products