
EFFECTIVE DATE: *April 22, 2021***PROCEDURE TITLE:***Expanded Access - Investigational Medical Devices**To be reviewed every three years by:
Institutional Review Board***REVIEW BY:** *April 22, 2024*

PROCEDURE

This Procedure implements the requirements of Institutional Review Board Policy No. 1 *Authority of the Institutional Review Board*, which requires the Trinity Health Mid-Atlantic (THMA) Institutional Review Board (IRB) establish policies and procedures to ensure that the THMA's IRB operations fully comply with applicable laws, regulations, professional standards, and the *Ethical and Religious Directives for Catholic Health Care Services*, including promoting the conduct of ethical and compliant research.

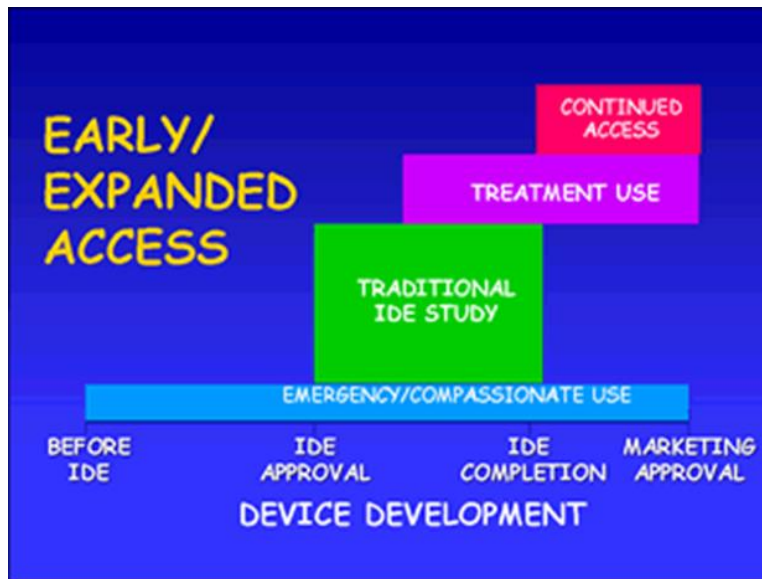
The use of an investigational device outside of a clinical trial for treatment of a patient is called expanded access. If enrollment in an existing clinical trial protocol is not possible (e.g., a patient is not eligible for any ongoing clinical trials, or there are no ongoing clinical trials to address the patient's condition), the physician has the potential to gain expanded access approval for a patient or group of patients as follows:

- Single Emergent Use
- Compassionate Use (individual patient or small group access)
- Treatment Use
- Continued Access

Request for expanded access to an investigational device must be submitted to the THMA IRB. The type of expanded access (emergency or non-emergency use) determines whether an IRB acknowledgement will suffice either before or after the use *or* whether prospective IRB review and approval prior to the use is required. At the earliest opportunity the clinician will notify the IRB of his/her intent to use or use of an investigational device *in an emergency* (but no less than 5 working days after the use). Informed consent will be obtained from the patient or his/her legally authorized representative unless the federal research requirements for waiver or exception from the informed consent requirements are satisfied.

Also see THMA IRB policy: *Devices*.

Below is a timeframe for the availability of these four options:



I. Emergency Use Expanded Access

Emergency use is the use of an investigational device for a patient in an emergency situation where there is not sufficient time to obtain FDA approval prior to the use. Emergency use of an investigational device is intended to provide patients and physicians with access to devices intended to treat life-threatening or serious diseases or conditions when there is no available alternative and no time to obtain FDA approval.

Emergency use may apply even if the investigational device is being studied in a clinical trial under an IDE:

- if a physician needs to use the device in a manner inconsistent with the approved investigational plan; or
- a physician who is not part of the clinical study, wishes to use the device to treat a patient with a life-threatening or serious disease or condition.

Emergency use of an investigational device may occur before an IDE is approved and when a device is not being studied under an IDE.

A. Criteria

The following conditions and criteria must be met:

- 1) The patient has a life-threatening or serious disease or condition that needs immediate treatment;
- 2) No generally acceptable alternative treatment for the condition exists; *and*
- 3) Because of the immediate need to use the device, there is no time to use existing procedures to obtain FDA approval for the use.

If all of the above criteria are met, an unapproved device may be used in an emergency situation without *prior* approval by the FDA.

B. Determinations and patient protections

The FDA expects the physician to make the determination that the patient's circumstances meet the above criteria, to assess the potential for benefit from the use of the unapproved device, and to have substantial reason to believe that benefits will exist.

The THMA IRB asks that physicians notify the IRB of the intended emergency use of an investigational device prior to use, if this is feasible given the emergency, so that the IRB may be aware and review the physician's determination that the criteria and conditions were met.

In the event that a device is used in circumstances meeting the criteria listed above, the physician will follow as many patient protection procedures as feasible given the nature of the emergency, such as obtaining:

1. Informed consent from the patient or a legally authorized representative;
2. Clearance from THMA or your department;
3. Concurrence of the IRB chairperson;
4. An independent assessment from an uninvolved physician; *and*
5. Authorization from the device manufacturer.

C. Reporting the emergency use to the FDA and the THMA IRB

1. If there is an IDE for the device, the IDE sponsor must notify the FDA of the emergency use within 5 days through submission of an IDE Report ([§812.35\(a\)\(2\)](#)). This follow-up report should include a summary of the conditions constituting the emergency, the patient protection measures that were followed, and patient outcome information.

Additionally, the physician must notify the THMA IRB before the emergency use or within 5 days of the emergency use using the *Emergency Use of a Device* form

2. If no IDE exists, the physician should submit a follow-up report on the use of the device (description of device used, details of the case, and the patient protection measures that were followed) to:
 - a. The IRB using the *Emergency Use of a Device* form
 - b. The FDA by sending the follow-up report to:
Food and Drug Administration
Center for Devices and Radiological Health
10903 New Hampshire Ave
Document Control Center
WO66 Rm G-609
Silver Spring, MD 20993

II. Compassionate Use (or Individual Patient/Small Group Access)

Compassionate use of a device allows one or a small group of patients expanded access to a device during a clinical trial and FDA approval is obtained prior to the use. The FDA recognizes that there are circumstances in which an investigational device is the only option available for a patient faced with a serious or life-threatening disease or condition. The compassionate use expanded access 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 for patients who do not meet the requirements for inclusion in the clinical investigation but for whom the 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 for the device does not exist). This provision is typically approved for individual patients but may be approved to treat a small group.

A. Criteria

The conditions and criteria that must be met for compassionate use of a device are that:

1. The patient has a life-threatening or serious disease or condition; and
2. No generally acceptable alternative treatment for the condition exists.

Prior FDA approval is required before compassionate use occurs.

B. Requesting compassionate use of a device

If a licensed physician would like to obtain an investigational device for an individual patient, the medical device company must first agree to provide the investigational device for compassionate use. FDA cannot require a company to provide an investigational device for compassionate use to proceed. If the device manufacturer agrees to provide the device under compassionate use, there are two different processes to follow to obtain FDA approval, depending on whether or not there is an IDE for a clinical trial for that device:

1. **If there is an IDE for the device**, the IDE sponsor (who may be the device manufacturer or a physician who has submitted the IDE to conduct the clinical study for the device) should submit an IDE supplement requesting approval for a compassionate use under section §812.35(a) in order to treat the patient. The IDE supplement should include:
 - A description of the patient's condition and the circumstances necessitating treatment;
 - 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; and
 - An identification of any deviations in the approved clinical protocol that may be needed in order to treat the patient
 - **The patient protection measures that will be followed:**
 - a) A draft of the informed consent document that will be used;

- b) Clearance from the institution as specified by their policies;
- c) Concurrence of the IRB chairperson;
- d) An independent assessment from an uninvolved physician; and
- e) Authorization from the device manufacturer on the use of the device.

In some cases, the THMA IRB will not approve the request until they have approval from 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 Chairperson will need to be submitted to the FDA with the follow-up report after the patient is treated. At THMA institutional clearance is needed in addition to the IRB Chair concurrence.

2. **If there is no IDE for the device**, the physician or manufacturer will submit the above information to FDA, along with a description of the device provided by the manufacturer. Physicians and manufacturers can contact CDRHExpandedAccess@fda.hhs.gov for assistance.

The physician should not treat the patient identified in the request until FDA approves use of the device under the proposed circumstances. In reviewing this type of request, FDA will consider the above information as well as whether the preliminary evidence of safety and effectiveness justifies such use and whether such use would interfere with the conduct of a clinical trial to support marketing approval.

If the request is approved, the attending physician should devise an appropriate schedule for monitoring the patient, taking into consideration the investigational nature of the device and the specific needs of the patient. The patient should be monitored to detect any possible problems arising from the use of 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 a serious disease or condition for which no alternative therapy adequately meets their medical need. In this case, that the request should include the information identified above and indicate the number of patients to be treated. If there is an IDE for the device, the supplement should 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. Follow-up information on the use of the device should be submitted in a report after all compassionate use patients have been treated.

C. Actions the FDA takes on compassionate use requests

After a compassionate use request is received, FDA will approve, approve with conditions, or disapprove the request. When there is an IDE for the device, compassionate use request IDE supplements have the same statutory 30-day review cycle as other IDE submissions. However, the patient need is considered when reviewing these requests and they are often expedited if necessary. For example, in 2015, compassionate use requests received under an IDE were reviewed in as little as 1 day, and on average in 18 days. Compassionate use

requests received without an IDE were reviewed in as little as the same day as receipt, and on average in 10 days.

D. Reporting the compassionate use to the FDA and the THMA IRB

Following the compassionate use of the investigational device, a follow-up report including summary information regarding patient outcome and any problems that occurred as a result of device use, should be submitted to the:

1. THMA IRB via the *Compassionate Use of a Device* form.
2. FDA

III. Treatment Use

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

A. Criteria:

1. The device is intended to treat or diagnose a serious or immediately life-threatening disease or condition;
2. There is no comparable or satisfactory alternative device available to treat or diagnose the disease or condition in the intended patient population;
3. 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
4. The sponsor of the controlled clinical trial is pursuing marketing approval/clearance of the investigational device with due diligence.

A device that is not approved for marketing may be under clinical investigation for a serious or immediately life-threatening disease or condition in patients for whom no comparable or satisfactory alternative device or other therapy is available. During the clinical trial or prior to final action on the marketing application, it may be appropriate to use the device in the treatment of patients not in the trial under the provisions of the treatment investigational device exemption (IDE) regulations (21 CFR 812.36).

The treatment use provision of the IDE facilitates the availability of promising new devices to desperately ill patients as early in the device development process as possible, before general marketing begins, and to obtain additional data on the device's safety and effectiveness. 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.

B. FDA approval for a Treatment Use IDE

The sponsor must submit a treatment use IDE application to the FDA, Center for Devices and Radiological Health and obtain approval. A licensed practitioner who receives an investigational device for treatment use under a treatment IDE is an "investigator" under

the IDE and is responsible for meeting all applicable FDA investigator responsibilities under 21 CFR 812, 21 CFR 50, and 21 CFR 56.

Once approval is obtained, convened THMA IRB review must take place and IRB approval must be obtained. The treatment use may begin 30 days after the FDA receives the treatment IDE submission and after IRB approval is obtained. The FDA may notify the sponsor in writing earlier than the 30 days that the treatment use may or may not begin. FDA may approve the treatment use as proposed or approve it with modifications.

Note that the FDA may disapprove or withdraw approval of a treatment IDE if:

- The required criteria [§812.36(b)] are not met or the treatment IDE application does not contain the required information [§812.36(c)];
- FDA determines that any of the grounds for disapproval or withdrawal of approval apply [§812.30(b)(1) through (b)(5)]. See [Approval Process, FDA Actions](#), for additional information;
- The device is intended for a serious disease or condition and there is insufficient evidence of safety and effectiveness to support such use;
- The device is intended for an immediately life-threatening disease or condition and the available scientific evidence, taken as a whole, fails to provide a reasonable basis for concluding that the device:
 - may be effective for its intended use in its intended population; or
 - would not expose the patients to whom the device is to be administered to an unreasonable and significant additional risk of illness or injury;
- There is reasonable evidence that the treatment use is impeding enrollment in, or otherwise interfering with the conduct or completion of, a controlled investigation of the same or another investigational device;
- The device has received marketing approval/clearance or a comparable device or therapy becomes available to treat or diagnose the same indication in the same patient population for which the investigational device is being used;
- The sponsor of the controlled clinical trial is not pursuing marketing approval/clearance with due diligence;
- Approval of the IDE for the controlled clinical investigation of the device has been withdrawn; or
- The clinical investigator(s) named in the treatment IDE are not qualified by reason of their scientific training and/or experience to use the investigational device for the intended treatment use.

C. Safeguards

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 institutional review boards (21 CFR 56).

D. Reporting the Treatment Use IDE to the FDA and the THMA IRB

The sponsor of a treatment IDE must submit progress reports on a semi-annual basis to all reviewing IRB's and the FDA until the filing of a marketing application. The dates of these reports are based on the period of time since initial approval of the treatment IDE. *After*

filing of a marketing application, progress reports must be submitted annually in accordance with the IDE regulations.

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 (reports), such as unanticipated adverse device effects and final reports. These must also be filed with the IRB.

IV. Continued Access to a Device

Continued access to a device after the completion of a clinical trial creates a pathway for the potential of participants to continue to have access to the device if there is preliminary evidence that the device will be effective and there are no significant safety concerns.

A. Criteria and conditions

The sponsor of a clinical investigation is permitted to continue to enroll subjects while a marketing application is being prepared by the sponsor and/or reviewed by the Agency if there is:

1. A public health need for the device; **or**
2. Preliminary evidence that the device is likely to be effective and no significant safety concerns have been identified for the proposed indication.

The continued enrollment of participants in a research investigation while a marketing application is being prepared by the sponsor and/or reviewed is known as an "extended investigation". Extended investigations permit participants and/or physicians continued access to the devices while also allowing the collection of additional safety and effectiveness data to support the marketing application or to address new questions regarding the investigational device. The continued access may be applied to any clinical investigation that meets the criteria identified above; however, it is intended to be applied late in the device development process, i.e., after the controlled clinical trial has been completed.

It is important to recognize that there is significant overlap between the treatment IDE regulation and the continued access to a device regulation. Both continued access to a device and the treatment IDE regulation are intended to provide additional access to an unapproved device, once preliminary evidence regarding safety and effectiveness is available to FDA. However, because a treatment IDE can be submitted earlier in the IDE process, i.e., once promising evidence of safety and effectiveness has been collected under the IDE but while the clinical study is ongoing, it could provide access to a wider group of patients at an earlier stage in the IDE process. The treatment IDE regulation also has a more narrow application than the continued access to a device in that treatment use is intended to address only those patients who have an immediately life-threatening or serious disease

or condition whereas the continued access to a device, which is applied after completion of the clinical trial, may be considered for any clinical investigation.

B. IRB review

Convened THMA IRB review and approval is required prior to an FDA approved continued access to a device begins. Submit the New Project Application, protocol, informed consent, and other documents to the IRB.

V. Consent and HIPAA Authorization

Informed consent and HIPAA Authorization must be obtained from each participant (or the Legally Authorized Representative) in advance of the use;

OR

Exception from Informed Consent Requirements [21 CFR 50.23(a)]: FDA regulations permit ***emergency use*** of an investigational device without informed consent where the investigator **and an independent physician**, who is not otherwise participating in the clinical investigation, certify in writing:

1. The patient is confronted by a life-threatening or severely debilitating situation (see definitions above), necessitating the use of the an investigational device;
2. Informed consent cannot be obtained from the patient (because the patient cannot communicate or is incompetent to give consent);
3. Time is not sufficient to obtain consent from the patient's legally authorized representative; ***AND***
4. No alternative method of approved or generally recognized therapy is available that provides an equal or greater likelihood of saving the patient's life.

If, in the investigator's opinion, immediate use of an investigational device is required and if time is not sufficient to obtain the independent physician determination, the investigator should make the determination and, ***within 5 working days*** after the use of an investigational device, have the determination reviewed and evaluated in writing by an independent physician.

VI. Submitting to the IRB

The appropriate forms must be submitted to the IRB within the timeframes indicated for each type:

1. Emergency use of a device - submits the *Emergency Use of an Investigational Device* form before or up to 5 days after the use. Must include an **independent assessment by an uninvolved physician and obtain institutional clearance** (may have Departmental Chair or designee grant clearance).
2. Compassionate Use of a device - after contacting the FDA and obtaining approval, submits the *Compassionate Use of an Investigational Device* form before or up to 5 days after the use. Must include an **independent assessment by an uninvolved physician**

and obtain institutional clearance (may have Departmental Chair or designee grant clearance).

3. Treatment use of a device or expanded access to a device - after obtaining FDA approval, must submit to the IRB a *New Project Application*, protocol, consent document and all other related documents. The *New Study Application* form is located on the IRB forms page of the website. These types of expanded access use must receive full board review (convened IRB meeting) and is considered research. It may be appropriate for your application to be accepted past the deadline to get it to the next IRB meeting, so please contact us to make us aware of your urgent situation: (734) 712-5470. You will also need to meet several FDA requirements - refer to the FDA website.

For Unanticipated Adverse Device Effect reporting, see the THMA policy: *Unanticipated Problems or Adverse Events Reporting*, located on the IRB website.

DEFINITIONS

Immediately life-threatening disease:

This means a stage of a disease in which there is a reasonable likelihood that death will occur within a matter of months or in which premature death is likely without early treatment.

Investigational Device Exemption (IDE):

An investigational device exemption allows the investigational device to be used in a clinical study in order to collect safety and effectiveness data. The FDA grants permission so a device that otherwise would be required to comply with a performance standard or to have pre-market approval can be shipped lawfully for the purpose of conducting investigations of that device. This FDA permission is evidenced by the assignment of an IDE number.

IRB means the Institutional Review Board designated by Trinity Health Mid-Atlantic to represent Trinity Health Mid-Atlantic in the Federal-wide Assurance.

Medical Device:

A medical device is an instrument, apparatus, implement, machine, contrivance, implant, in vitro reagent, or other similar or related article, including any component, part, or accessory, which is:

1. Recognized in the official National Formulary, or the United States Pharmacopeia, or any supplement to them
2. Intended for use in the diagnosis of disease or other conditions, or in the cure, mitigation, treatment or prevention of disease in man
3. Intended to affect the structure or any function of the body of man or other animals.

See the THMA IRB policy: *Devices* for more information.

Ministry means a first tier (direct) subsidiary, affiliate, or operating division of Trinity Health that maintains a governing body that has day-to-day management oversight of a designated portion of Trinity Health System operations. A ministry may be based on a geographic market or dedication

to a service line or business. Ministries include Mission Health Ministries, National Health Ministries, and Regional Health Ministries.

Procedure means a document designed to implement a policy or a description of specific required actions or processes.

Treatment use of a device:

This includes the use of a device for diagnostic purposes.

RESPONSIBLE DEPARTMENT

Further guidance concerning this Procedure may be obtained from the Trinity Health Mid-Atlantic Institutional Review Board.

RELATED PROCEDURES AND OTHER MATERIALS

- [21 CFR 812.36](#)
- FDA [Guidance on IDE Policies and Procedures](#)

APPROVALS

Initial Approval:

Subsequent Review/Revision(s):