SOP 507: EXPANDED ACCESS

POLICY

The Food and Drug Administration's Expanded Access Program allows the use of an investigational medical product (drug, biologic or medical device) for treatment outside of clinical trials when there are no comparable or satisfactory therapies. Expanded access, sometimes called "compassionate use" is not considered a clinical investigation, however, FDA submission and IRB review are necessary. Expanded access does not involve the conduct of "research" as defined at 45 CFR 46 because there is no intent to develop generalizable knowledge. Guidance regarding expanded access to investigational test articles is provided for investigators on the IRB website.

Please note that this policy does not describe the emergency use of a test article (please see SOP 506: Emergency Use of a Test Article and Planned Emergency Research). For single-patient emergency use, investigators should follow instructions for Emergency Use of a Test Article available on the IRB website.

Under the FDA's current regulations, there are three categories of expanded access to investigational drugs for treatment use[1]:

- **Expanded access for individual patients**, including for emergency use
- **Expanded access for intermediate-size patient populations** (generally smaller than those typical of a treatment IND or treatment protocol — a treatment protocol is submitted as a protocol to an existing IND by the sponsor of the existing IND)
- **Expanded access for widespread treatment** use through a treatment IND or treatment protocol (designed for use in larger patient populations)

Prior to treating a patient under expanded access for non-emergent use, the physician must obtain approval from the IRB. Approval from the IRB is intended to protect the rights, safety and well-being of human subjects. In non-emergency situations, treatment may not begin until the IRB has approved the Expanded Access plan.

Expanded access to investigational drugs for treatment for individual patients, may be approved by the IRB Chair or Vice-Chair. Expanded access for intermediate size-patient populations or for widespread treatment will be reviewed by the convened IRB.

Under the FDA's current regulations, there are three types of expanded access for medical devices.[2]

- **Emergency Use**: Use of an investigational device when an individual patient is in a life-threatening situation and needs immediate treatment (there are no alternative options and no time to use existing procedures to get FDA approval for the use).

[1] Expanded Access to Investigational Drugs for Treatment Use – Questions and Answers June 2016, Updated October 2017

[2] Expanded Access for Medical Devices (6/21/2019)

Please contact the IRB Office at (801) 581-3655 or irb@hsc.utah.edu for additional guidance.

- **Compassionate Use (for Individual Patient/Small Group Access**): Use of an investigational device to treat or diagnose and individual or small group of patients with a serious disease or condition when there are no available alternative options.
- **Treatment Investigational Device Exemption:** Use of an investigational device to treat or diagnose a group of patients with a serious or immediately life-threatening disease or condition when the device is also being studied for the same use under an approved Investigational Device Exemption.

Expanded access as compassionate use of an investigational device must be approved by the FDA prior to use. The concurrence of the IRB Chair is required. Expanded access as treatment investigational device exemption will be reviewed by the convened IRB.

PROCEDURES

1. Expanded Access to Investigational Drugs

1.1. Investigators must submit a new study application in ERICA. Applications must include the following:
- Documentation of the FDA determination of expanded access
- Investigator's Brochure or a package insert for the drug
- Informed consent document

1.2. The IRB staff pre-reviews the application to determine that the submission is complete. If the FDA has provided an acknowledgement that a single patient IND may proceed, the IRB staff pre-reviewer will assign the application to the IRB Chair or Vice-Chair. The IRB Chair or Vice-Chair reviews the application for expanded access plan for use of an investigational medical product.

1.3. If the IRB Chair or Vice-Chair agrees with the single patient expanded access plan, the treatment may be acknowledged. If the IRB Chair or Vice-Chair has questions about or believes that the treatment justifies discussion at a convened board meeting, it may be referred to the convened IRB for review. If the IRB Chair or Vice-Chair disagrees with the expanded access plan, it should be referred to the convened IRB for review. The convened IRB may approve or disapprove the expanded access plan.

1.4. Expanded access for intermediate size-patient populations or for widespread treatment will referred to and reviewed by the convened IRB.

1.5. Emergency use for single-patient use will be handled according to the policy outlined in SOP 506: Emergency Use of a Test Article and Planned Emergency Use.

2. Expanded Access Plan for Investigational Devices

2.1. Investigators must submit a new study application to the IRB. Applications must include the following:
- Informed consent document
- Documentation of the FDA concurrence with the compassionate use

2.2. The IRB staff pre-reviews the application to determine that the submission is complete.

2.3. Expanded access as compassionate use may be assigned to the IRB Chair or Vice-Chair. The IRB Chair or Vice-Chair reviews the application for expanded access plan for compassionate use of an unapproved device. If the IRB Chair or Vice-Chair concurs with the compassionate use, the approval may be issued. If the IRB Chair or Vice-Chair has questions about or believes that the treatment justifies discussion at a convened board meeting, it may be referred to the convened IRB for review. If the IRB Chair or Vice-Chair disagrees with the compassionate use, it should be referred to the convened IRB for review. The convened IRB may concur with compassionate use or disapprove the plan.

2.3.1. The physician may not proceed with the compassionate use until the FDA concurrence with the compassionate use has been received by the IRB and there is IRB Chair or Vice-Chair concurrence.

2.3.2. The physician must submit reports after the compassionate use and reports of problems as dictated by the FDA.

2.4. Expanded access as a treatment investigational device exemption will be referred to and reviewed by the convened IRB.

2.5. Emergency use of an investigational device will be handled according to the policy outlined in SOP 506: Emergency Use of a Test Article and Planned Emergency Use.