	Standard Operating Procedure Number: 506	Title: Expanded Access (Single Patient, Compassionate Use)
	Version: 2.0	Effective Date: 9/22/2020

1. PURPOSE

This procedure outlines the processes for requesting Expanded Access for a single patient, which is sometimes called “Compassionate Use” with the Avera IRB.

2. DEFINITION

Expanded Access is the use of an investigational medical product (drug, biologic or medical device) outside of a clinical trial to treat a patient with a serious or immediately life-threatening disease or condition who has no comparable or satisfactory alternative treatment options. Expanded Access exists under a larger program of Expanded Access to Investigational Drugs or Devices in order to provide patients with opportunities to be treated with unapproved drugs or devices when certain criteria are met.

FDA regulations allow access to investigational medical products for treatment purposes on a case-by-case basis. Under FDA’s current regulations, there are three categories of expanded access. This SOP is only intended for letter “A” below. The others (B and C) are covered under regular IRB SOPs.


- A. Expanded Access for individual patients, including for emergency use (21 CFR 312.310)
- B. 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) (21 CFR 312.315)
- C. Expanded Access for widespread treatment use through a treatment IND or treatment protocol (designed for use in larger patient populations) (21 CFR 312.320)

3. GENERAL REQUIREMENTS

3.1 Single Patient (non-emergency)

The Avera IRB will consider the Expanded Access request only when the following criteria are met:

- A. The patient has a serious or immediately life-threatening disease or condition;
 - *Immediately life-threatening disease or condition* means a stage of 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.
 - *Serious disease or condition* means a 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.
- B. There is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition;
- C. The potential patient benefit justifies the potential risks of the treatment use;
- D. Patient enrollment in a clinical trial is not possible; and
- E. Providing the investigational medical product will not interfere with investigational trials that could support a medical product’s development or marketing approval for the treatment indication.

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3.2 Emergency Single Patient Expanded Access

In an emergency where there is not sufficient time to secure IRB review prior to beginning treatment, the emergency use of the investigational drug must be reported to the IRB within 5 business days, as required by 21 CFR 56.104(c). Please refer to IRB SOP 413.

4. SUBMISSION REQUIREMENTS

4.1 Submit to IRB (for Single Patient, Non-emergency)

IRB review is required, and the following documents must be submitted for IRB review:

- a. Letter from the PI explaining the rationale for the intended use in the expanded access (single patient compassionate use);
- b. Patient history;
- c. FDA approval of the single patient (expanded access) submission or documentation that FDA approval will be pursued and provided to the IRB upon approval;
- d. Protocol;
- e. Treatment plan that will be used; and
- f. Consent form.

4.2 Additional document for PI to sign

The PI will need to also read, sign and return to the IRB the following form: "Expanded Access Principal Investigator Roles and Responsibilities – Acknowledgment Form."

5. RESPONSIBILITY

IRB Staff (or designee) is responsible for ensuring that IRB reviewers have all the tools and resources needed to complete their research review of the documents described above.

The IRB reviewers (a primary and secondary) are responsible for conducting a thorough review and making all appropriate approval recommendations for consideration by the IRB. All members will be familiar with the protocol in order to properly weigh all concerns.

Contact the PI or the Study Coordinator for questions or further information.

6. APPLICABLE REGULATIONS AND GUIDELINES

Expanded Access:

<https://www.fda.gov/news-events/public-health-focus/expanded-access>


<https://www.fda.gov/media/85675/download>

Physician Request for a Single Patient IND for Compassionate or Emergency Use:

<http://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/ucm163982.htm>

21 CFR 312.310

21 CFR 812.35 and 36

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REVISION HISTORY

Revision Number	Version Date	Approved By	Date Approved
2.0	6/30/2020	Avera Institutional Official	9/22/2020
<ul style="list-style-type: none"> • Changed the title of this SOP to be more in alignment with wording used in regulations. • Noted that although this SOP is for non-emergency use, there is such a need for emergency use and that IRB SOP is referenced above (IRB SOP 413). • Added PI must sign the Expanded Access Principal Investigator Roles and Responsibilities-Acknowledgement Form. • Updated specific regulations and provided updated links on the FDA website. • Other minor edits and administrative changes to align more with regulations. 			
1.0	August 2016	Director HSRP / August 2016	