



MCW Office of Research Standard Operating Procedure

EMERGENCY USE OF INVESTIGATIONAL DRUGS OR BIOLOGICS

Unit: Human Research Protections Program (HRPP), Office of Research

Applies to: Faculty and Staff involved in human research

PURPOSE:

To provide patients and physicians with access to investigational drugs/biologics to address immediately life-threatening situations when there is no available alternative and before a written submission can be made.

FDA has requirements related to all categories of expanded access, including emergency use [21 CFR 312.305]:

- 1) The patient(s) 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.

NOTE: This policy only addresses situations in which there is not sufficient time to obtain prospective IRB and FDA approval. If there is time to obtain prospective IRB or FDA approval, please see *IRB SOP: Expanded Access Use of an Investigational Drug or Biologic*.

DEFINITIONS:

Emergency Use: For an investigational drug/biologic to be used without prior approval, the following conditions must be met:

- The patient has a life-threatening condition that needs immediate treatment.
- No generally acceptable alternative treatment for the condition exists; and
- There is no time to use existing procedures to obtain IRB and FDA approval for the use.

Immediately Life-Threatening Disease: 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.

Serious Disease or Condition: 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.

PROCEDURE:

Emergency Use of an Investigational Drug/Biologic:

1. The treating physician should contact the HRPP office immediately if they are considering using an investigational drug/biologic for an emergency use situation to determine if an IND must be filed or not with the FDA prior to the use. If the patient meets emergency criteria, call FDA to obtain FDA authorization for the expanded access use.
2. The treating physician should also ensure that the Sponsor is willing to provide the investigational drug/biologic for emergency use and request a letter of authorization to cross reference the existing IND held by the Sponsor.
3. When a treating physician has used an investigational drug/biologic to treat a patient with a life-threatening event, the physician must complete and submit an initial eBridge submission within 5 working days of its use.
4. The IRB eBridge submission must include:
 - a. Emergency Use IND number or Authorization from the FDA to ship the investigational drug/biologic; and
 - b. Approval from the Sponsor for use of the investigational product; and
 - c. The consent form that was used to consent the patient, or, if informed consent was unable to be obtained from the patient or his/her legally authorized representative, a letter from a physician not otherwise participating in the intervention certifying that:
 - i. The patient was confronted by a life-threatening situation necessitating the use of the investigational drug or biologic
 - ii. Informed consent could not be obtained because of an inability to communicate with or obtain legally effective consent from the patient
 - iii. Time was not sufficient to obtain consent from the patient's legal representative
 - iv. No alternative method of approved or generally recognizable therapy was available that would provide an equal or greater likelihood of saving the patient's life.
5. The FDA submission to the appropriate Review Division in the Center for Drug Evaluation and Research (CDER) must be submitted within 15 working days. Include:
 - a. Form FDA 3926
 - i. Ensure to not select box 10.b
 - b. Treating physician's CV
 - c. Letter of Authorization from sponsor
6. Following the emergency use of an investigational drug/biologic, the patient should be monitored to detect any possible problems arising from the use of the investigational drug/biologic.
7. If emergency use is approved by FDA for extended duration, the treating physician must submit reports to FDA in accordance with 21 CFR 312.64.
8. Any follow-up reports must be submitted to the IRB, the Sponsor and/or the FDA in which summary information regarding the patient outcome is presented. The MCW IRB requires follow-up reports to be submitted at 30 days and 90 days post-use via eBridge CPR submission. Include completed Form FDA 3926 into the eBridge CPR submission.

REFERENCES:

21 CFR 56.102 (d)

21 CFR 56.104(c)

21 CFR 812.35(a))

21 CFR 312.36

21 CFR 312.300

21 CFR 312.305

21 CFR 312.310

FDA website and guidance documents

SUPPORTING DOCUMENTS:

IRB SOP: Expanded Access Use of an Investigational Drug or Biologic

Effective Date: 04/28/2023

Version number: 7.0

Previous Version/date: 6.0, 06/15/2018

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Approved By

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