This guidance should be used by any physician who wishes to use an investigational drug, device or biologic in a **non-emergency** situation with a single patient. **It is strongly recommended that submission to the FDA and IRB occur simultaneously to improve the efficiency of the submission process.**

COMPASSIONATE USE PATHWAY CRITERIA

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

**Life threatening* *is defined as:*

- *The likelihood of death is high unless the course of the disease is interrupted;*
- *A disease or condition with a potentially fatal outcome, where the end-point is survival.*
- *The disease or condition causes major irreversible morbidity.*

SUBMISSION GUIDANCE AND STEPS FOR OBTAINING APPROVAL

1. The investigator should contact the drug/device manufacturer of the product to determine 1) if the product can be provided, and 2) if it can be administered under an existing IND/IDE. If it is not available through an existing IND/IDE, but the manufacturer is willing to provide the product, the investigator should:

   A. **Drugs/ Biologics**: Apply for expanded access to an investigational drug under a single patient IND

   - If you need assistance with submitting an IND, please reach out to the Office of Clinical Research Regulatory Unit: https://www.med.upenn.edu/clinicalresearch/regulatory-applications.html.
   - FDA Guidance Information: https://www.fda.gov/news-events/expanded-access/expanded-access-information-physicians
   - **NOTE: If expedited IRB review is requested, then box 10b must be checked on FDA form 3926**

   B. **Devices**: obtain an independent assessment by an uninvolved physician and work with the manufacturer to submit the required documentation (see link below) or IDE supplement to the FDA

   - If you need assistance with submitting required documentation to FDA, please reach out to the Office of Clinical Research Regulatory Unit: https://www.med.upenn.edu/clinicalresearch/regulatory-applications.html.
   - FDA Guidance Information: www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/HowtoMarketYourDevice/InvestigationalDeviceExemptionIDE/ucm051345.htm#compassionate. Physicians can also contact CDRHExpandedAccess@fda.hhs.gov for assistance.
   - Following the use of the device, a follow-up report should be submitted by whoever submitted the original expanded access request to FDA. This report should present summary information regarding patient outcome. If any problems occurred as a result of device use, these should be discussed in the follow-up report and reported to the IRB as soon as possible.

2. Submit the Single Patient Treatment Use (SPTU) Application in eIRB, including any necessary supplemental documentation. All SPTU submissions must be submitted through eIRB. The IRB no longer process requests via email.
3. Draft an informed consent form. Written informed consent should be obtained from the patient or a legal representative. A template SPTU consent form can be found here: www.upenn.edu/IRB/forms. Ensure that the consent reflects the use of the product is for treatment and not research. It is acceptable if the consent states that the data may be used for research. If the data is being used for research please include HIPAA language that does

not use the word “research”. The patient /LAR must understand the investigational nature of the product being used. If the patient signs the notice of privacy practices as a clinical patient, there is no need to include the research HIPAA authorization form as part of the consent form.

4. Obtain any other necessary institutional clearances, if applicable or necessary (e.g., execution of a contract).

The physician should not treat the patient identified in the request until FDA and the IRB approves use of the drug/device under the proposed circumstances.