NYU Langone Health

Human Research Protections
Institutional Review Board
1 Park Avenue | 6th Floor | New York, NY 10016
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# Expanded Access (EA) to Investigational (non-Food & Drugs Administration [FDA] approved) Drugs

The purpose of this guidance document is to assist researchers with navigating policy and regulations regarding Expanded Access to **Investigational Drugs** and related Institutional Review Board (IRB) submission requirements. Sometimes called "**compassionate use**", expanded access is a potential pathway for a patient with an immediately life-threatening condition or serious disease or condition to gain access to an investigational medical product (drug, biologic, or medical device) for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available.

**Criteria for all categories of Expanded Access submissions:** *(Additional criteria described in table below must be met depending on the type of Expanded Access submission)*

- Patient has serious or immediately life-threatening disease or condition
- There are no comparable or satisfactory alternatives to diagnose, monitor, or treat the patient
- Enrollment in a clinical trial is not possible
- The potential benefit to the patient justifies the potential treatment risk(s) – the risk(s) associated with treatment is not greater than the probable risk of the disease or condition
- Providing the investigational product will not interfere with clinical trials that could support the product's development or marketing approval for the treatment indication

**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.

**Immediately Life-Threatening Disease or Condition**: a stage of disease in which there is reasonable likelihood that death will occur within a matter of months or in which premature death is likely without early treatment).

*Note that for Emergency Use, FDA regulations require that any subsequent use of the same test article at the institution has prospective IRB review and approval.*

This guidance is based on the FDA Regulations 21 CFR 312 for expanded access of drugs. For further information and additional resources please visit Expanded access resource pages. For guidance on expanded access of devices, please visit the IRB Template Library. For assistance with submission to the FDA, please contact Research Regulatory Services.

| EA Categories | Specific Criteria | IRB Approval Prior to Use? | Required Documents for IRB Submission | Type of RNav Submission | FDA Approval Prior to Use? |
|---|---|---|---|---|---|
| **Individual Patient – Non-Emergency Use** *Single Patient IND (SPIND)*<br><br>(21 CFR 312.310) | • Treatment for 1 patient<br>• Patient has serious disease or condition.<br>• There is sufficient time for IRB and FDA approval prior to treatment. | Yes | • Form FDA 3926<br>• Letter of Authorization<br>• FDA Acknowledgment / Approval Letter<br>• Investigator's Brochure<br>• Treatment protocol*<br>• Treatment IND Consent Form* | New Human Research Study (select Expanded Access when completing smart form) | Yes |
| **Intermediate-Size Patient Populations**<br><br>(21 CFR 312.315) | • > 1 patient, but typically < 100<br>• Investigational product is **not** being developed due to inability to recruit sufficient population for clinical trial;<br>• Investigational product is being tested in a clinical trial, but patients don't meet eligibility criteria, trial is closed to enrollment, or enrollment isn't feasible (e.g., due to geographic proximity); ***or***<br>• FDA-approved product that is otherwise unavailable due to removal from market, failure to meet conditions of approval, or drug shortage ***and***<br>• FDA approval required prior to treatment and subject to 30-day waiting period | **Yes** | • Forms FDA 1571 and 1572<br>• Letter of Authorization<br>• FDA Acknowledgment / Approval Letter<br>• Investigator's Brochure<br>• Intermediate-Size Population Access Protocol<br>• Treatment IND Consent Form* | **New Human Research Study** (select Expanded Access when completing smart form) | **Yes** |
| **Widespread Use** *Treatment IND*<br><br>(21 CFR 312.320) | • Large population, typically 100s-1000s<br>• Investigational product is under investigation to support marketing application for the expanded access use or clinical trials are complete; **and**<br>• Sponsor is actively pursuing marketing approval; **and**<br>• Sufficient evidence of safety and effectiveness to support expanded access use; **or**<br>• Based on available information, FDA can reasonably conclude the investigational product may be effective for expanded access use and won't expose patients to unreasonable and significant risk; **and**<br>• FDA approval required prior to treatment and subject to 30-day waiting period | **Yes** | • Forms FDA 1571 and 1572<br>• Letter of Authorization<br>• FDA Acknowledgment/Approval Letter<br>• Investigator's Brochure<br>• Intermediate-Size Population Access Protocol<br>• Treatment IND Consent Form* | **New Human Research Study** (select Expanded Access when completing smart form) | **Yes** |
| **Individual Patient – Emergency Use of Drug** ***Emergency IND (eIND)***<br><br>**(21 CFR 312.310)** | • Treatment for 1 patient<br>• Patient has immediately life-threatening disease or condition (a situation that requires a patient to be treated before written submission can be made)<br>• There is not sufficient time to obtain IRB approval prior to treatment. | **Yes, contact IRB for clearance to use test article** | • Emergency Use Appendix*<br>• Copy of signed Emergency Use consent form* or certification that the use meets exception from informed consent on the Emergency Use Appendix<br>• Patient's de-identified history and treatment plan/rationale for emergency treatment<br>• Investigator's Brochure (as applicable)<br>• FDA acknowledgment/ correspondence | **Reportable New Information (RNI)** to notify of emergency EA use must be submitted within 5 working days post-treatment. | **Yes**, initial authorization from the FDA is required, and written submission to FDA within 15 working days of the initial authorization. |

**Template available on IRB website*

**For questions regarding expanded access submissions, contact: Expanded-Access@nyulangone.org**