



## Physician Checklist to Obtain Expanded Access IND for Treatment of Individual Patient

### Introduction

When there is no comparable or satisfactory therapy option available for an individual patient who has a serious or immediately life-threatening disease or condition, the FDA expanded access program provides a mechanism to use an investigational drug to diagnose, monitor, or treat an individual. The use of an investigational drug for expanded access is primarily to treat patients, not to answer safety or effectiveness questions about the drug (21 CFR 312.300). Use of an investigational drug for a single individual patient, either emergency or non-emergency, is one of the three categories of expanded access regulated by FDA (21 CFR 312.310). The other two categories are expanded access for intermediate-size patient populations (generally smaller than those typical treatment IND) (21 CFR 312.315); expanded access for widespread treatment use through a treatment IND (designed for use in larger patient populations) (21 CFR 312.320). These latter two categories will not be discussed in this document.

This checklist provides physicians regulatory information about the requirements and procedures for submitting, obtaining and maintaining an expanded access IND for an emergency or non-emergency use for an individual patient.<sup>1</sup> This checklist is not intended to serve as regulatory advice; for more information, please contact the appropriate office at your institution.

If the expanded access IND is submitted by a physician, the physician is the IND Sponsor–Investigator. (CFR 312.305(c)(3)). INDs approved for a single individual are intended to be treated in the same way as emergency use as far as further similar use. That is, no further similar uses in other patients can occur unless and until the sponsor obtains a treatment IND.

In addition to physicians and investigators, this checklist may also be useful to IRBs, as a supplement to application forms, and to institutions, to adapt into institutional policies and procedures.

### References

Expanded Access to Investigational Drugs for Treatment Use – Questions and Answers  
<https://www.fda.gov/media/85675/download>

Expanded Access (Compassionate Use)

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

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<sup>1</sup> Note: There is an alternative way to obtain, expanded access for an individual patient: If an IND already exists, the IND Sponsor can submit an individual patient access protocol to that existing IND. These submissions must be made by the existing sponsor of the existing IND. This scenario is not further discussed in this checklist.



## Individual Patient IND

The following steps should be followed to obtain an Individual Patient IND:

1.  **Contact Sponsor/Manufacturer** to make sure they will provide drug and provide a Letter of Authorization (LOA) to reference existing IND application or Drug Master File (whichever is applicable).
2.  **Submit Individual Patient IND request (via telephone, fax, or email) to FDA. Please note there is no required format for submitting this information to FDA, but the following information must be included:**
  - a. **Individual (or Single) Patient Expanded Access** (also called a Single Patient IND)
    - i. Written request to include the following:
      1.  Statement that this is a request for an individual patient IND
      2.  Letter from treating physician with brief clinical history (e.g., diagnosis, disease status, prior therapy, response to prior therapy, rationale for requesting proposed treatment)
      3.  Patient Information: Proposed treatment plan/protocol (dose, route, duration, monitoring procedures, modifications (e.g. dose reduction) for toxicity, references).
      4.  Product Information: Product Name, Dosage Form, Route of Administration, Dosing Regimen
      5.  Chemistry, Manufacturing and Controls (CMC)/Pharmacology/Toxicology Information
        - a. Requirement may be met by submitting the LOA from company to use information previously submitted by the company to FDA.
      6.  Informed consent statement documenting that informed consent and approval for the use by an appropriate IRB will be obtained prior to initiating treatment.
      7.  Investigator qualification statement (e.g., CV or info specifying training, experience, and licensure of treating physician)
      8.  FDA Form 1571 (indicate treating physician as sponsor). [Form](#) and [Instructions](#). Form FDA 3926 (specific to individual patient IND submissions) may also be used.
      9. FDA Form 1572
      10.  Contact telephone number and fax number for treating physician/IND Sponsor



Reference:

<https://www.fda.gov/drugs/investigational-new-drug-ind-application/physicians-how-request-single-patient-expanded-access-compassionate-use>

## **b. Emergency Use**

i. Must meet all criteria:

1.  Patient's case is an emergency: Disease is life-threatening or will lead to severe disability
2.  No approved therapeutic alternative is available
3.  Risk of complications from the disease is higher than the risk of toxicity from this investigational treatment
4.  IND supplier (e.g., product's manufacturer) agrees to supply the drug and to provide LOA
5.  Drug is not available by other means (approved drug, ongoing trial, another expanded access program)
6.  There is insufficient time to obtain IRB approval and a report of the use is provided to the IRB within five working days of the Emergency Use.

ii. Can be done over the phone, but follow up with written request within 15 working days of initial authorization.

1. Normal business hours: 8:00 am-4:30 pm EST
  - a. Phone: 301-796-3400 or 888-463-6332 or the appropriate Review Division
  - b. Fax: 301-431-6356 (call before faxing)
  - c. Email: [cdereops@fda.hhs.gov](mailto:cdereops@fda.hhs.gov)
2. After business hours and all weekends
  - a. FDA Emergency Call Center: 866-300-4374 or 301-796-8240

References:

Eligibility tool:

<http://www.fda.gov/downloads/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/InvestigationalNewDrugINDApplication/UCM343034.pdf>

Timeline:

<http://www.fda.gov/downloads/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/InvestigationalNewDrugINDApplication/UCM343024.pdf>



### 3. **FDA Determination**

- a. **Approved/Active IND:** A number will be assigned to the application. The IND sponsor (treating physician) should provide this IND number to the drug supplier, so the supplier may ship the drug to the treating physician. The FDA will either allow the treatment use to proceed or not allow it to proceed (put the application on clinical hold). The IND is considered active (treatment with the drug may proceed) 30 days after FDA receives the IND submission or upon earlier notification to the physician by the FDA.
- b. **Disapproved:** If the treatment use is not allowed to proceed (e.g., a clinical hold is placed on the application), FDA will notify the physician of this decision initially by telephone. The call will be followed by a written letter that provides the reasons for FDA's denial of the request.

### 4. **IRB Submission / Reporting**

- a. **Expanded Access for Non-Emergency:** IRB review and approval are required if there is time for the IRB to review the submission prior to investigational drug administration. Contact your reviewing IRB as soon as possible for submission requirements and ask for information about the anticipated IRB review timeline. If the submission cannot be reviewed by the IRB in a timely manner given the patient's situation, you may need to consider an Emergency Use. IRB Submission requirements typically include:
  - i. Letter from the investigator explaining the rationale for the intended use addressing the regulatory requirements set out by the FDA.
  - ii. FDA approval of the single patient (expanded access) submission.
  - iii. Letter from the sponsor/manufacturer agreeing to the use
  - iv. Protocol or a reference protocol that will be used
  - v. Consent Form
  - vi. Investigational Brochure

The reviewing IRB determines whether or not the use of the unapproved investigational drug is permissible.

- b. **Emergency Use:** The regulations permit a one-time use per institution of an investigational drug without prior IRB review. If there is not enough time to conduct IRB review the investigator must communicate the emergency situation to the reviewing IRB for guidance. Consent of the patient is required unless the following condition can be met:
  - i. PI and a physician who is not otherwise participating in the clinical investigation have certified all of the following:



1. The patient is confronted by a life-threatening situation necessitating the use of the test article.
  2. Informed consent cannot be obtained from the participant because of an inability to communicate with, or obtain legally effective consent from, the participant.
  3. Time is not sufficient to obtain consent from the participant's legal representative.
  4. There is no available alternative method of approved or generally recognized therapy that provides an equal or greater likelihood of saving the life of the participant.
- ii. If there is not sufficient time to obtain an independent physician's determination that these four conditions can be met, the PI can make this determination, and have it reviewed and evaluated by an independent physician within five working days.

**Reporting of the Emergency Use to the IRB is required of the investigator within five business days after use in a patient.** The IRB reviews the report of the Emergency Use.

5.  **Investigator's Responsibilities** – Given that the physician is now an IND holder, he/she must manage the paperwork of the IND (submit SAEs, amendments, etc.) as mandated by the FDA.

FDA Reference:

<https://www.fda.gov/drugs/investigational-new-drug-ind-application/ind-application-procedures-investigators-responsibilities>



## FDA Resources

1. Investigator-Initiated Investigational New Drug (IND) Applications:  
<https://www.fda.gov/drugs/investigational-new-drug-ind-application/investigator-initiated-investigational-new-drug-ind-applications>
2. Individual Patient Expanded Access Applications:  
<https://www.fda.gov/media/91160/download>
3. Information for Sponsor-Investigators Submitting INDs:  
<https://www.fda.gov/drugs/investigational-new-drug-ind-application/information-sponsor-investigators-submitting-investigational-new-drug-applications-inds>
4. Emergency IND Application Timeline:  
<https://www.fda.gov/drugs/investigational-new-drug-ind-application/emergency-investigational-new-drug-eind-applications-antiviral-products>
5. CDER Offices and Divisions:  
<https://www.fda.gov/about-fda/center-drug-evaluation-and-research/cder-offices-and-divisions>