

How to Request Treatment Use via FDA's Expanded Access Process (Non-Emergency)

- Discuss available treatment options with the patient and decide, along with your patient, whether a clinical trial or treating with an investigational drug, biologic, or medical device outside a clinical trial is appropriate. This will depend upon the patient's medical history, whether there are approved therapies available with which the patient has not been treated, and whether the patient is eligible to enter a clinical trial for the product. Only patients with a serious or life-threatening disease or condition, as defined below, are eligible for expanded access consideration.
 - **A 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 determined by clinical judgment, based on the condition's impact on clinical outcomes such 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 months or in which premature death is likely without early treatment.
- Select the investigational product that you want to use for treatment. This may occur in several ways: The patient brings information about the investigational product to you; you consult clinicaltrials.gov or [Reagan-Udall Foundation Expanded Access Navigator](#) to find which investigational products are available through a company's expanded access program; you contact the drug, biologic, or device company.

Every company is required by the 21st Century Cures Act to publish its policy regarding expanded access and provide contact information for requests. This information is typically available on the company's website, clinicaltrials.gov (Search under "Study Type" for "expanded access studies."), or [Reagan-Udall Foundation Expanded Access Navigator](#). The policy should also provide contact information to make a request. If you are unable to find contact information for a company, begin by calling its regulatory affairs office for information.

A company that does not have a listed expanded access program may be willing to provide its product outside of its ongoing clinical trial(s) and, therefore, you should contact the company.

- As the treating physician, you also are responsible for determining that the risks to the patient from the investigational product are not greater than the risks from the disease or condition. This determination should be based on the information about the investigational product

available to you and your knowledge of the patient’s clinical situation, and may be made in conjunction with the product manufacturing company.

- Follow the company’s instructions for requesting an individual patient IND or IDE (treatment use). Different companies have different requirements and forms. But, for the most part, you should expect to provide:
 - The investigational product you are requesting.
 - The patient’s medical history, including age, disease, and treatment history. Expect to be asked about concomitant medical conditions or medications or other medical complications.
 - Reason for requesting the product (pharmacological rationale).
 - Treatment plan (dosing, duration of treatment, etc.)
 - Any proposed special monitoring or precautions.

Note: Not all companies are able or open to providing their investigational product to patients outside of clinical trials.

- If the company agrees to make the investigational product available, it will notify you and will issue a “Letter of Authorization” (LOA) that will permit FDA to cross-reference certain proprietary but necessary information from the company’s existing IND or IDE application. You can find [an example of the LOA here](#).
- Once you receive the LOA, you must submit a request for an IND or IDE for the treatment use to FDA. Start by contacting the appropriate review division within FDA ([See List](#)) to confer with the medical officer connected with the product about putting together the application to request the IND or IDE.
- If the medical product is a drug or biologic, submit [FDA Form 3926](#), along with your curriculum vitae and the LOA. When accessing Form 3926, you may see a “please wait” message. In that case, right click (or control click on Mac) and select the “save as” option to download the document as a PDF to your computer before opening it. Form 3926 is specifically designed for individual patient IND requests. If you are applying for an IDE, provide a written treatment plan or protocol that includes the same information requested in FDA Form 3926.

Form 3926 asks for the following information:

- Patient’s initials and date of submission.
- Clinical information.
- Treatment information.
- LOA (from manufacturer), submitted as an attachment.
- Physician’s qualification statement.
- Physician name, address and contact information.
- Request for authorization to use Form 3926 and request for IRB waiver of full-board review (simple check box on the form).

- Certification statements, confirming that treatment will not begin without prospective IRB review and documentation of informed consent, once you receive confirmation from FDA.
 - Physician’s signature.
- FDA allows almost all requests to proceed. The agency may contact you for additional information if unanswered questions persist. Although it rarely happens, FDA may deny a request if the patient is deemed eligible and able to participate in a clinical trial for the condition for which treatment is sought, if there are insufficient safety data upon which to make a determination, or the treatment has no valid rationale for the patient. If the determination is to proceed, FDA will send you a letter with the IND number and any concerns, recommendations, or stipulations.
- While you are waiting for FDA’s determination, contact your IRB office to make them aware that the expanded access request will be coming to them shortly for review. If you don’t have access to an IRB, contact Clinical Research Pathways at info@clinicalresearchpathways.org or call 404.386.8982 to find an IRB that will conduct the review for you. Note: It is not necessary to wait for FDA approval before submitting your expanded access request to the IRB. However, the IRB review process might go more quickly if you already have FDA approval in hand.
- After you receive FDA approval, submit Form 3926 (if the IRB accepts this form) or the treatment plan, a draft informed consent document, and any other documents (e.g., IND or IDE number) required by the IRB for review. If using Form 3926 for a drug or biologic, be sure to check the box on the form requesting a waiver of the requirement for the full IRB to review the request. The IRB chair or a designee will review the request. Expanded access requests involving medical devices are generally reviewed by the IRB chair without a request from the treating physician. Remember: Even if you have an IND or IDE number from FDA, treatment may not begin until IRB approval and informed consent from the patient have been secured.
- Follow any special handling requirements for the investigational product. It must be labeled as investigational, and you will be required to keep track of it. Depending on the manufacturer’s requirements, you may also have to return any unused product or document its destruction.
- Once you have received IRB approval, and the patient has reviewed and signed the informed consent document, you may begin to treat the patient.
- Until you complete treatment of the patient, remember that you have regulatory responsibilities to fulfill with FDA, the product manufacturer, and IRB because you are considered a “sponsor” by FDA. Review the list of responsibilities above. One of the key responsibilities is to report serious and unexpected adverse reactions (adverse events) that are believed to be related to the use of the investigational product to the product manufacturer and FDA. You also are required to provide summary outcome data to FDA. This information should be submitted as an attachment to the review division that originally issued the IND or IDE number. For an IND, use FDA Form 3926, selecting the appropriate checkbox under field no. 9, *Contents of Submission*, to indicate the kind of information or report that is being submitted to the existing IND.