

How to Request Single Patient Expanded Access (“Compassionate Use”) of an Unapproved Investigational Drug

When a physician wants to submit a Single Patient Expanded Access (“Compassionate Use”) request to obtain an unapproved investigational drug for an individual patient, he or she must first ensure that the manufacturer (pharmaceutical company) is willing to provide the investigational drug for expanded access use. If the manufacturer agrees to provide the drug, there are several steps the physician must follow with the manufacturer, the FDA, the UTHSC IRB, and affiliate institution (if applicable).

Emergency Use Requests

In an emergency situation, the request to use an unapproved investigational drug may be made via telephone or other rapid means of communication, and authorization to ship and use the drug may be given by the FDA official over the telephone. In these situations, known as emergency IND (eIND) requests, shipment of and treatment with the drug may begin prior to FDA’s receipt of the written IND submission that is to follow the initial request. An [emergency IND timeline](#) is available online to guide clinicians through the process. Local procedures are outlined in the UTHSC IRB SOP for [Emergency Use](#).

Non-Emergency Requests

In a non-emergency situation, a written request (IND) for individual patient use of an investigational drug must be submitted to the FDA and approved by the UTHSC IRB and affiliate institution, if applicable.

MANUFACTURER/PHARMACEUTICAL COMPANY

DRUG REQUEST FORM

Some manufacturers/pharmaceutical companies may ask the physician to provide a drug request form. Contact the company to find out about their process for requesting expanded access and what information you will need to provide. You can find a pharmaceutical company’s contact information via the company’s website or clinicaltrials.gov. If a company requires a drug request form, it may include:

- The patient’s clinical history
- The rationale for why this treatment may be effective
- The physician’s regulatory knowledge of the product and experience using experimental treatments
- A proposed treatment plan (which may be determined with the pharmaceutical company)

REQUIRED DOCUMENTATION

If the pharmaceutical company grants the request to use the investigational drug, the company’s representative will provide required documentation such as a [Letter of Authorization \(LOA\)](#).

The physician will then submit a copy of this required documentation to the FDA and the UTHSC IRB to verify the pharmaceutical company has approved the physician’s use of the investigational treatment for single-patient expanded access IND.

THE FDA

In a non-emergency situation, a written request (IND) for individual patient use of an investigational drug must be submitted to the FDA. The investigational drug may be shipped and treatment of the patient may begin 30 days after the application is received by FDA or earlier if notified by the FDA that treatment may proceed. These non-emergency requests are known as individual patient INDs, or single patient expanded access requests. A [guide to initiate and maintain non-emergency requests](#) is available for physicians online.

FORM FDA 3926

The FDA requires the submission of Form FDA 3926, Individual Patient Expanded Access Investigational New Drug Application (IND), by the physician or the physician's representative. This form is designed specifically for use by physicians when submitting requests for single patient expanded access to investigational drugs, including in emergencies. This form requires:

- A brief clinical history of the patient
- Investigational drug name
- The name of the manufacturer/pharmaceutical company that will supply the drug
- Proposed treatment plan using the investigational treatment
- Information concerning the physician's training, experience, and licensure
- Indication that the physician will obtain informed consent from the patient
- Certification that the physician will receive IRB approval
- Copy of the LOA

Upon receipt of the single patient expanded access application, the FDA will review to make a decision. The FDA will either allow the treatment to proceed or place the IND on hold. FDA may contact the physician to request more information or clarification in order to avoid placing the IND on hold.

Treatment with the drug may proceed 30 days after FDA receives the IND submission or upon notification of the physician by FDA, whichever comes first. IRB approval and informed consent must be obtained before treatment begins. If the treatment use is not allowed to proceed (i.e., a clinical hold is placed on the application), FDA will notify the physician of this decision initially via a telephone call. The call will be followed by a written letter that provides the reasons for FDA's denial of the request.

Active INDs will be assigned an IND number. The drug supplier may request the IND number in order to ship the drug to the treating physician.

UTHSC INSTITUTIONAL REVIEW BOARD (IRB)

IRB SUBMISSION

Once the manufacturer (pharmaceutical company) has agreed to provide the investigational treatment and Form FDA 3926 has been submitted to the FDA, the physician must submit an application to the UTHSC IRB via [iMedRIS](#). For more information, about the IRB application and the documents to include see the UTHSC IRB SOP [Compassionate/Treatment Use](#).

Institutional Review Board (IRB)

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INFORMED CONSENT FORM

The informed consent form provides patients with information about the protocol, including patient expectations and known possible outcomes and risks. Under local policy, the informed consent must follow the [UTHSC IRB Consent Form Template](#) and be submitted to the IRB for review and approval. Following the IRB's approval, the treating physician must review the informed consent form with the patient. The FDA also may ask to see the informed consent form.

INSTITUTIONAL APPROVAL

Institutional approval may be required prior to the administration of the investigational treatment. If the investigational treatment will be administered at one of these institutions, contact the corresponding administrator to meet institutional requirements.

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Administration of the investigational drug for an individual patient in a non-emergency situation may not proceed until the physician has received approval from the manufacturer, the FDA, the UTHSC IRB, and affiliate institution (if applicable).

REPORTING DOCUMENTS

As the patient undergoes treatment with the investigational treatment, the physician must follow the treatment protocol agreed upon with the IRB and the manufacturer. The physician must report the following to the FDA via **Form FDA 3926** and the UTHSC IRB via [iMedRIS](#):

- Serious and unexpected adverse events (view guidelines for determining adverse events [here](#))
- Changes to the treatment plan
- Annual Reports
- When treatment is completed, submit a summary/final report of the expanded access use