



U.S. Food and Drug Administration

# SINGLE PATIENT EXPANDED ACCESS:

## PHYSICIAN FACT SHEET and APPLICATION CHECKLIST

### What is Expanded Access?

Expanded access is the use of an investigational drug outside of clinical trials to diagnose, monitor, or treat patients with serious or life-threatening diseases or conditions for which there are no comparable or satisfactory therapy options available.

When possible, it is preferred that a patient be given an investigational drug as part of a clinical trial rather than through expanded access. This is because clinical trials are designed to generate data that may lead to the product's approval and, consequently, wider availability of the drug. However, patients may be able to receive the investigational product through expanded access when patient enrollment in a clinical trial is not possible (for example, the patient is not eligible for any ongoing clinical trials or there are none available).

### Obtaining the investigational drug

To obtain expanded access for your patient, first contact the pharmaceutical company developing the drug. Sometimes, the company will provide the drug to patients according to a pre-established protocol. If not, you should ask the company for approval to obtain its drug. The company does so by issuing a Letter of Authorization (LOA).

### Requesting expanded access from the FDA

To request access to an investigational drug for your patient, you must then submit an application to the FDA for expanded access on your patient's behalf. Form FDA 3926 can be used for this application. The expanded access process also includes requesting approval from an Institutional Review Board (IRB), and obtaining informed consent from your patient for the use of the investigational drug. Once the request is authorized by FDA, you will be responsible for managing the patient's medical care.

Ensuring patient safety is a priority; FDA must determine that the potential benefit justifies the potential risks of the use of the investigational drug. Even with safeguards, there may be unknown risks, since there is limited information available about the investigational drug. Your patient may not receive expanded access if the drug company does not provide the drug or if the FDA denies the request. However, FDA has historically granted expanded access to almost all the requests it receives.

If your patient needs the drug on an emergency basis, before a written request can be submitted, FDA can grant the request over the phone and your patient can begin treatment after you receive the medication from the drug company. However, you must still submit an expanded access application to FDA within 15 days and notify an IRB within 5 days of initiation of treatment.

**Contact: [DrugInfo@fda.hhs.gov](mailto:DrugInfo@fda.hhs.gov) or 1-855-543-3784 with any questions.**

#### More Information:

- [FDA Information for Physicians: Expanded Access](#)
- [FDA Guidance: Expanded Access to Investigational Drugs for Treatment Use -Questions and Answers](#)
- [Application for Individual Patient Expanded Access](#)
- [FDA's Expanded Access Contact Information, including FDA review divisions](#)

## Follow the steps below to request expanded access to an investigational new drug for your patient.

### 1. Ensure your patient meets the eligibility criteria for expanded access

- They must have a serious or immediately life-threatening disease or condition; there must be no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition; and they generally must be unable to participate in a clinical trial (see [clinicaltrials.gov](http://clinicaltrials.gov) for a list of many clinical trials being conducted around the world).
- You must determine that the probable risk from the investigational drug is not greater than the probable risk from the disease or condition.
- If your practice includes multiple patients who might be good candidates for the investigational product, consider whether an expanded access IND for an intermediate-size population, rather than multiple single patient INDs, would be more efficient.

### 2. Obtain a letter of authorization (LOA) from the drug manufacturer

- Contact the drug manufacturer/company to request use of the drug outside of the clinical trial setting. FDA may be able to help identify the contact. The manufacturer must decide whether to provide the drug to treat your patient under expanded access.
- If the manufacturer agrees to provide the drug for expanded access, submit a Letter of Authorization from the drug company to the FDA with your IND submission. A template of this letter can be used and is available [here](#).

### 3. Fill out the “Individual Patient Expanded Access Investigational New Drug Application” form (Form FDA 3926) and submit it to FDA

- Submit the request for your patient. See the guidance [Individual Patient Expanded Access Applications: Form FDA 3926](#) for instructions.
- For emergency requests, you may contact 855-543-3784 and follow the instructions on [FDA's Expanded Access Contact Information page](#). After 4:30 pm EST weekdays and all day on weekends, contact the FDA Emergency Call Center at 866-300-4374.

### 4. Request Institutional Review Board (IRB) approval

- If you work for an academic medical center, use the IRB procedures in place for your institution. If you are in private practice, seek IRB approval through a local university, hospital or an independent IRB.

### 5. Discuss the risks of the investigational drug treatment with your patient and obtain informed consent

- Informed consent must be obtained before initiating treatment, unless one of the exceptions in 21 CFR part 50 applies.

### 6. Await Authorization from FDA and the IRB

- Your patient may begin treatment 30 days after FDA receives the request, unless you receive earlier notification from FDA that the treatment may proceed. Typically FDA responds to these requests in a matter of days (or hours for emergency requests). You must also receive IRB approval before treatment can begin.
- Historically, FDA has approved 99% of expanded access requests. However, this is not a guarantee that yours will be approved.
- Once your request is approved by FDA, notify the drug company and arrange to obtain the drug.
- In certain circumstances, the drug company may be able to [charge the patient](#) for the cost of the drug, or it may elect to cover the cost.
- Any additional costs for administering the drug and monitoring its use will depend on the patient's insurance coverage and do not require FDA authorization. FDA has no authority to require that the Centers for Medicare and Medicaid Services (CMS) or any private health insurance company reimburse for investigational drugs for which FDA has authorized charging. It is important that you and your patient consider the cost of the investigational drug and the medical services associated with its use that are not covered by third-party payers such as insurance or Medicare.

### 7. Begin treatment and monitor the patient

- You are required to adhere to the monitoring procedures described in the treatment plan you outlined in the Form FDA 3926, including adverse event reporting. You may also have to submit a summary of the results of the treatment.