CANCER FACTS

National Cancer Institute • National Institutes of Health
Department of Health and Human Services

Access to Investigational Drugs: Questions and Answers

1. What is an investigational drug?

An investigational drug is one that is under study but does not yet have permission from the Food and Drug Administration (FDA) to be legally marketed and sold in the United States.

FDA approval is the final step in the process of drug development. The first step in the process is for the new drug to be tested in the laboratory. If the results are promising, the drug company or sponsor must apply for FDA approval to test the drug in people. This is called an Investigational New Drug (IND) Application. Once the IND is approved, clinical trials can begin. Clinical trials are research studies to determine the safety and measure the effectiveness of the drug in people. Once clinical trials are completed, the sponsor submits the study results in a New Drug Application (NDA) or Biologics License Application (BLA) to the FDA. This application is carefully reviewed and, if the drug is found to be reasonably safe and effective, it is approved.

2. How do patients get investigational drugs?

By far, the most common way that patients get investigational drugs is by participating in a clinical trial sponsored under an IND. A patient's doctor may suggest participation in a clinical trial as one treatment option. Or a patient or family member can ask the doctor about clinical trials or new drugs available for cancer treatment.

Another way of learning about new drugs being tested in clinical trials is through the National Cancer Institute's (NCI) PDQ® database. This database contains information on a large number of ongoing studies. Individuals can search this database on their own at http://cancer.gov/clinical_trials or they can call the NCI's Cancer Information Service at 1–800–4–CANCER (1–800–422–6237). Information specialists can search the database and provide a list of trials for individuals to share with their doctor.

3. Are there other ways to get investigational drugs?

Less common ways that patients can receive investigational drugs are through an expanded access protocol or by a mechanism known as a special or compassionate exception.

Expanded Access

Expanded access protocols are available for a limited number of investigational drugs that have been well studied and are awaiting final FDA approval for marketing. Expanded access allows a wider group of people to be treated with the drug. The purpose of an expanded access program is to make investigational drugs that have significant activity against specific cancers available to patients before the FDA approval process has been completed.

The drug company or IND sponsor must apply to the FDA to make the drug available through an expanded access program. There must be enough evidence from studies already completed to show that the drug may be effective to treat a specific type of cancer and that it does not have unreasonable risks. The FDA generally approves expanded access only if there are no other satisfactory treatments available for the disease.

Special Exception/Compassionate Exemption

Patients who do not meet the eligibility criteria for a clinical trial of an investigational drug may be eligible to receive the drug under a mechanism known as a special exception or a compassionate exemption to the policy of administering investigational drugs only in a clinical trial. The patient's doctor contacts the sponsor of the investigational agent and provides the patient's medical information and treatment history; requests are evaluated on a case-by-case basis. The FDA must approve each request to provide the drug outside a clinical trial. There should be reasonable expectation that the drug will prolong survival or improve quality of life.

These are some questions that are considered when determining if a patient may be a candidate to receive an investigational drug as a special exception:

- Is the patient ineligible for a clinical trial?
- Have standard therapies been exhausted?
- Is there objective evidence that the investigational agent is active in the disease for which the request is being made?
- Can the drug potentially benefit the patient?
- What is the risk to the patient?

In some cases, even patients who qualify for treatment with an investigational drug on a "compassionate basis" might not be able to obtain it if the drug is in limited quantity and high demand.

4. Are all investigational drugs available through an expanded access or special exception mechanism?

No. The drug company or sponsor decides whether to provide an investigational drug outside the clinical trial setting. Availability may be limited in part by drug supply, patient demand, or other factors.

5. What is the NCI's role in providing access to investigational drugs?

The NCI acts as the sponsor for many, but not all, investigational drugs. When acting as sponsor, the NCI provides the investigational drug to the physicians who are participating in clinical trials of the drug. A physician who wishes to treat a patient with the investigational drug as a special exception must request the drug from the NCI. The request must include the patient's age, sex, diagnosis, date of diagnosis, previous cancer therapy, current clinical status, intended dose and schedule of the requested drug, any proposed concomitant cancer drugs or other therapies, and pertinent laboratory data. These requests are reviewed on a case-by-case basis.

6. Who can provide access to investigational drugs being developed by pharmaceutical companies?

In the case of investigational drugs sponsored by a drug company, the drug company in collaboration with the FDA provides access to the drug. The process is similar to that described above.

A request to treat a patient with an investigational drug outside a clinical trial must be made to the drug company and to the FDA. The request to the FDA is sent as general correspondence to the appropriate reviewing division where the IND application is filed. The drug company can provide the name of the appropriate reviewing division. (FDA reviewing divisions are prohibited from divulging proprietary information such as whether a sponsor has filed an IND or the status of an IND.)

7. Are there specific criteria used to determine whether patients can receive an investigational drug outside the clinical trial setting?

Generally, patients must meet the following criteria to be considered for treatment with an investigational drug outside the clinical trial setting:

- have undergone standard treatment that has not been successful
- be ineligible for any ongoing clinical trials
- have a cancer diagnosis for which an investigational drug has demonstrated activity and is being studied in ongoing Phase 2 or Phase 3 protocols

The potential benefits of receiving the drug should outweigh the risks involved.

8. What should patients do if they are interested in receiving an investigational drug through a special exception or expanded access mechanism?

Patients interested in gaining access to investigational drugs should talk to their physician about available options. Physicians can make requests for special exceptions by contacting the study sponsor. Physicians will be required to follow strict guidelines, including gaining approval from their Institutional Review Board and obtaining "informed consent" from the patient. Informed consent is a process that includes a document to be signed by the patient which outlines the known risks and benefits of the treatment, as well as the rights and responsibilities of the patient.

9. What are the costs involved in receiving an investigational drug?

In general, the drug is provided free of charge. However, there may be other costs associated with the treatment. Patients should check with their insurer about coverage of these costs prior to beginning treatment.

10. What are some of the potential drawbacks to receiving an investigational drug?

There are some potential drawbacks to receiving an investigational drug. It is not known whether an investigational drug is better than standard therapy for treating a disease, and a patient who is receiving an investigational drug may not receive any benefit from it. Side effects (both long-term and short-term) from the drug may not be fully understood, especially if the drug is in early phases of testing. Finally, a patient's health insurance company may not pay expenses associated with receiving the investigational drug.

11. How can patients find out more information about a specific investigational drug?

Patients can find out more about a specific drug by contacting the drug company that is developing the drug. Information may also be available from the Cancer Information Service at 1–800–4–CANCER (1–800–422–6237).

12. What other resources are available on this topic?

The following list of resources may be helpful:

- NCI's Cancer.gov Web site has a feature titled *Understanding the Approval Process for New Cancer Drugs: Summary*, which can be found at http://cancer.gov/clinical_trials/doc_header.aspx?viewid=d94cbfac-e478-4704-9052-d8e8a3372b56 on the Internet.

- FDA Center for Drug Evaluation and Research Web site has *Oncology Tools*, which contains a variety of information related to cancer including a section on access to unapproved drugs. That Web address is http://www.fda.gov/cder/cancer/index.htm.

- CTEP (the Cancer Therapy Evaluation Program at NCI) has a Web site titled *Developing Cancer Therapies*, which can be found at http://ctep.cancer.gov.

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Sources of National Cancer Institute Information

Cancer Information Service

Toll-free: 1–800–4–CANCER (1–800–422–6237)
TTY (for deaf and hard of hearing callers): 1–800–332–8615

NCI Online

Internet

Use http://cancer.gov to reach the NCI's Web site.

LiveHelp

Cancer Information Specialists offer online assistance through the *LiveHelp* link on the NCI's Web site.

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