

FDA Expands Access to Investigational Drugs



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Seriously ill patients who lack good treatments sometimes want to try a promising drug that is still under development. These patients often hope this drug will provide them with a lifeline where none had existed before.

Because of this need, FDA has had rules in place since 1987 that have allowed patients to have access to investigational drugs under certain circumstances, even though the safety and effectiveness of the drug has not been fully established.

On August 12, 2009, FDA announced changes to the rules to make them broader and clearer for the patient and the treating physician, while still preserving the integrity of clinical trials designed to find out whether a drug

has a desired effect on some disease or condition.

These changes were described in the Federal Register (available online) and include:

- changes in the expanded access rule to explain the procedures and standards for patients who want access to investigational drugs
- changes to the charging rule to explain when a drug manufacturer can charge a patient for an investigational drug, in a clinical trial or

expanded access program, and what costs a manufacturer can recover when charging

The Importance of Clinical Trials

It is understandable that people with a disease with no good treatment and their treating physicians are very interested in trying a new drug under development, especially if the early results of a clinical trial suggest that the drug shows promise. FDA's Expanded Access program provides a means to obtain investigational drugs.

This need, however, must also be balanced with the need to protect patients, and the need to ensure that the drug continues to be developed in a scientifically valid and reliable way. At this early stage, there is only limited data on a drug's effectiveness and safety. It is still essential that a clinical trial continue to obtain the rest of the data.

"Clinical trials are the most important part of the drug development process in determining whether new drugs are safe and effective, and how to best use them," says Janet Woodcock, M.D., director of FDA's Center for Drug Evaluation and Research.

"We understand that some patients have run out of options and want to try something that is not fully tested, and we want to support them in these situations without exposing them to undue risks," Woodcock says. "But we also need to make sure that, ultimately, all patients get a treatment that has been shown to work. The clinical trial process gives everyone the full picture on the safety and effectiveness of a drug before it is used in the population at large."

Below is a description of the final rules.

Expanded Access Rule

To permit treatment of a patient with an investigational drug under an expanded access program, FDA generally must be satisfied that

- The patient's disease or condition has no satisfactory approved therapy. An example of this is a rare

type of cancer that has no known or approved treatment. Or, it may be the case that the available treatments did not work for the patient.

- The potential benefit for the patient justifies the potential risks. An example of this is the potential for longer survival with a disease or condition
- The expanded availability of the untested drug will not interfere with that product's development. For example, access to an investigational drug shouldn't interfere with enrollment in clinical trials needed to demonstrate the drug's safety and effectiveness

Among other things, the expanded access rule will

- explain the several different kinds of access that are possible, including access for individual patients, for small numbers of patients, and for large numbers of patients in what are called "treatment protocols"
- ensure safeguards to protect patients
- preserve the ability to develop meaningful data on the drugs available under expanded access

Charging Rule

The charging rule permits drug manufacturers to charge patients for an investigational drug in clinical trials or that's being made available for expanded access. Charging in clinical trials will be allowed under very limited circumstances, but will be permitted for most expanded access uses.

In some cases, a company could not develop a drug unless it is able to charge for the drug in clinical trials. Allowing charging for expanded access helps provide access to investigational drugs that manufacturers may not be able to offer without being able to charge.

FDA revised this rule to provide more explicit criteria for when charging should be permitted in clinical trials and for drugs available for expanded access.

Among other considerations, the

revisions are meant to

- make the process of obtaining authorization to charge more transparent
- specify what costs can be recovered by drug manufacturers:
 - only the direct costs of the drug when charging for a drug in a clinical trial
 - the direct costs of the drug plus the costs of administering the expanded access program when charging for a drug for an expanded access use

FDA has launched a new Web site (www.fda.gov/ForConsumers/ByAudience/ForPatientAdvocates/AccessToInvestigationalDrugs/default.htm) where patients can learn about options for investigational drugs. The site explains the various options that patients may use to explore the possible use of investigational drugs with their health care professional.

Topics addressed on this site include

- deciding whether to seek access to an investigational drug
- clinical trials and investigational drugs
- access to investigational drugs outside of a clinical trial (expanded access). [FDA](#)

This article appears on FDA's Consumer Updates page (www.fda.gov/ForConsumers/ConsumerUpdates/default.htm), which features the latest on all FDA-regulated products.

For More Information

Final Rules for Expanded Access to Investigational Drugs for Treatment Use and Charging for Investigational Drugs
www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/InvestigationalNewDrugINDApplication/ucm172492.htm