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FDA seeks to ease access to new drugs

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The Food and Drug Administration said Monday that it is proposing to expand access to experimental drugs for seriously sick patients, and would allow drug companies to charge for as yet unapproved therapies.

For decades the FDA has allowed some patients to take drugs that are still under investigation and awaiting government approval.

But the guidelines for when those drugs could be used were not explicit or broad enough, the FDA said, nor was there enough awareness among doctors and patients of what options were available.

The new rules are intended to give physicians clear directives on when to grant access to medications, even perhaps at the very earliest stage of development, in cases in which the potential benefit is deemed to outweigh the risks.

The goal, the FDA said, is to give "many more patients" access to unapproved medicines.

For example, until now the rules limited the use of such drugs to certain groups.

Additionally, the FDA said it was moving to address "inequities in access" to experimental drugs. In situations outside the most sophisticated teaching hospitals, it has been possible for viable treatment options to get overlooked.

"We expect that clearly articulating procedures and standards for expanded access will result in more patients with serious or immediately life-threatening diseases or conditions getting the earliest possible access to these therapies," the FDA said.

In some situations in recent years, according to patient advocates, drugs that appeared to give significant hope to the seriously ill were withheld for further testing.

One example they cite is Erbitux, which was not widely distributed until final FDA approval even though it had shown efficacy early on in treating some forms of cancer.

Use of these unapproved drugs, even those of unknown safety, will be reserved for the most grave medical problems, said Dr. Rachel Behrman, deputy director of the FDA's office of medical policy.

"We are not talking about the temporary relief of minor pain," Behrman said. "We are talking about serious diseases."

The FDA would also allow researchers to charge patients for experimental drugs to recover the costs of development, but not a profit.

This is intended to make drugs more available in cases in which their high cost might deter a small pharmaceutical company from offering an unproven treatment.

"FDA hopes this proposal will increase awareness in the health-care community of the range of options available for obtaining experimental drugs for seriously ill patients," said Dr. Janet Woodcock, the agency's deputy commissioner for operations.

The proposal, which is open for comment for 90 days, codifies and makes formal policies that have grown up over the years.

Use of unproven drugs has been a hot-button issue for decades. AIDS activists have long complained that the approval process for drugs is too slow.

The FDA noted that tens of thousands of patients have already received unapproved drugs for treatment of HIV, cancer and heart disease.

However, the existing regulations did not adequately describe the full range of programs available, the agency said in statement.

One health advocate sounded a warning about the proposal to use drugs that have not been proven risk-free.

"None of these drugs do we know are safe and effective," said Dr. Sidney Wolfe, editor of WorstPills.org, a part of Public Citizen.

"You may be doing people more harm than good," he said.

Wolfe noted that experimental drugs for cancer and AIDS often have toxic side effects that can present a danger to patients.

But Frank Burroughs, president of the Abigail Alliance for Better Access to Developmental Drugs, said the FDA does not go far enough in assuring quick access to all drugs that could help patients.

Burroughs said that in many cases it is quickly apparent that a drug is safe and effective yet still is not made available to people who would benefit from it.

"A good example is Erbitux," Burroughs said. He said that Erbitux quickly displayed effectiveness in treating cancers of the head, neck and colon.

"Abigail was my daughter," Burroughs said. "She died in 2001 after being unable to get Erbitux."

Next year a federal court is scheduled to hear a case brought by the Abigail Alliance and the Washington Legal Foundation aimed at making experimental drugs more accessible to patients.