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FDA May Broaden Access To Experimental Drugs

**Agency Seeks to Balance
New Options for Gravely Ill
With Protection of Patients**

By ANNA WILDE MATHEWS
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The Food and Drug Administration is moving to broaden access to experimental drugs for people with serious illnesses, with efforts that would represent a middle ground in the emotional debate over when unproven treatments should be available to patients with no other options.

The FDA is preparing a proposal that would clarify how doctors and drug companies can make certain drugs, not yet FDA-approved, available to small groups and individual patients with dangerous diseases and no other treatment options, according to people with knowledge of the matter. Agency officials have also drafted a proposal that would lay out more situations in which companies could charge patients for access to experimental drugs, though not for a profit, these people said.

Both proposals are subject to approval by the White House's Office of Management and Budget and could change before they are released.

The FDA proposals, which wouldn't represent a major revamping of the current system, don't go as far as the most aggressive patient activists wish. On the other side, some doctors warn that the agency needs to make sure there is reason to believe a drug could work before patients start taking something that may not help.

Rachel Behrman, deputy director of the FDA drug center's office of medical policy, says the agency is trying to increase access to experimental drugs and ensure that companies will continue to gather evidence to win full approval for their medicines. "We are striking a balance to ensure patients have access to the drugs they need when they need them, while at the same time safeguarding the integrity of the drug-development process and safeguarding the individual," she says.

Scott Gottlieb, an FDA deputy commissioner, says the FDA is working with the National Cancer Institute to get oncologists involved in large simple studies that would supplement traditional clinical trials. These would "enable people to get access to the drug before approval, and we get some incremental information we might not otherwise get," he says.

Typically, patients' most direct path to getting an experimental drug is to enroll in a clinical trial. But a patient may not meet the criteria to participate. For decades, FDA rules have also allowed "treatment" access to unapproved drugs. But the regulations can be difficult to navigate.

Under the new plan expected to be proposed by the FDA, the agency will lay out a formal pathway for small groups and individual patients to get access to unapproved drugs, according to people familiar with the matter. Though this has happened in the past, the new rule is expected to clarify the FDA's standards, making the process easier.

The proposed rules also lay out more specific circumstances under which patients could be charged for an unapproved drug, which aren't completely clear in the current regulation, according to people with knowledge of the matter. For instance, patients could be charged for approved drugs made by other companies and taken with the experimental treatment in a clinical trial.

Companies and researchers wouldn't be allowed to make a profit. Asking patients for such payments can be controversial, since insurers may not be willing to cover unproven remedies.

The FDA changes wouldn't go nearly as far as a plan pushed by the Abigail Alliance for Better Access to Developmental Drugs and the **Washington Legal Foundation**, two nonprofits that favor aggressive access policies. In a petition to the agency, they suggested a new FDA "initial approval" pathway that would let drug makers sell their experimental medicines at a profit, though with some restrictions, after they had shown potential efficacy in even a small number of patients. "We've got to have some incentive for pharmaceutical companies to participate" in the access programs, says Frank Burroughs, president of the Abigail Alliance.

The FDA's Dr. Behrman says that the agency believes making a profit on an investigational drug "is wrong for a variety of reasons, including that it can be a disincentive to develop the drug and it could put patients at risk."

The agency is appealing a May decision by the U.S. Court of Appeals for the District of Columbia that the Constitution protects terminally ill patients' access to "potentially life-saving investigational new drugs." The new proposals were developed before the ruling emerged.

On the other side, some researchers worry that patients may actually expose themselves to more risk by taking unproven drugs. A different petition to the FDA, filed by the National Coalition for Cancer Survivorship and the American Society of Clinical Oncology, presses the agency to clarify its current rules. "We have to be very careful not to expose desperate patients with cancer to therapies that might have no benefit whatsoever and could have unanticipated toxicities," says Richard Schilsky, associate dean for clinical research at the University of Chicago.

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