FDA Eases Rules on Access to Investigational Psychotropic Drugs

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The FDA's new rule on "expanded access programs" would allow pharmaceutical companies to give seriously ill patients broader access to investigational drugs outside of clinical trials. A limited number of expanded access programs were created in the past under sketchy FDA rules; the 2 new allied rules—one on the conditions drug companies must meet to create a program, the other on how they can charge for the drugs—ostensibly give pharma a wider berth. Moreover, psychotropic drugs can be provided under the clarified policy.

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When the FDA was considering changes to its policy (in part prompted by a lawsuit), it contended with the issue of whether the use of psycho-tropic drugs fits in the definition of "serious medical condition"—with which a patient must be afflicted before a drug company can make an investigational agent available outside a clinical trial. The health insurance industry made an effort to convince the FDA to exclude mental health conditions from serious medical conditions.
In her comments to the FDA after the agency proposed a rule in March 2007, Karen Ignagni, president and CEO of America's Health Insurance Plans (AHIP), pressed the agency to include a definition in the final rule that said, "A serious disease or condition is one which is persistent, substantially disabling, progressive, and likely to result in death within 6 to 12 months." She noted that schizophrenia and chronic depression are among the conditions that "cause disabling health effects and suffering for a period of time without death occurring prematurely or in a matter of months." AHIP was concerned about exposure of its insurance company members to wide claims from policy holders for reimbursement for expensive, investigational drugs obtained through

expanded access programs, which might multiply because of the FDA rule liberalization.

In its final rule, published on August 13, 2009, the FDA rejected the AHIP's proposed definition. Instead, it adopted a definition that whether a disease or condition is serious "is a matter of judgment, but generally is based on its impact on such factors as survival, day-to-day functioning, or the likelihood that the disease, if left untreated, will progress from a less severe condition to a more serious one." This definition, the FDA added, is consistent with the criteria in a 1999 Institute of Medicine report entitled "Definition of Serious and Complex Medical Conditions." One example the report cited was "conditions that may require frequent monitoring, such as schizophrenia and other psychotic illnesses."

Although access to investigational psychotropic drugs may be significant to patients with schizophrenia and depression and their psychiatrists, in the context of this rule making, it was a side issue. The FDA's review of its decades-old rules was driven by a lawsuit filed by a patients' advocacy group called the Abigail Alliance. Frank Burroughs, president of the alliance, noted that the FDA estimated that only 3095 additional patients will get investigational drugs as a result of the paired final rules, which were essentially written under the Bush administration. "We're happy that the number is in the plus column, but the FDA missed an opportunity," he stated.

The alliance was scheduled to meet with Margaret Hamburg, MD, the new Obama-appointed FDA commissioner, in late September to press her to expand access further.

The FDA's main concern in liberalizing vague rules (that had been in place for 2 decades) was ensuring that any wider availability of investigational drugs through expanded access programs did not hurt enrollment in clinical trials. "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," says Janet Woodcock, MD, director of the FDA's Center for Drug Evaluation and Research. "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."

Wayne Goodman, MD, professor and chairman of psychiatry at Mt Sinai School of Medicine in New York City, said, "I have mixed feelings about expanded access, reflecting a delicate balance between a detrimental effect on recruitment into clinical trials and need for more options." Before joining Mt Sinai, Goodman served as director of the division of adult translational research and treatment development at the NIMH. He noted, however, that some patients cannot gain entrance to clinical trials because of comorbid psychiatric or medical conditions.

One option raised by Goodman but not considered by the FDA would be to allow clinical trial participants who are classified as "nonresponders" to placebo to get open-label access after the trial ends. In open-label access, both the patient and physician know the identity of the drug the patient receives. "Such a change would have [the] effect of improving access to study drug, albeit to those subjects who qualify for entry into a clinical trial," explained Goodman. One potential problem with this approach, however, is that the FDA generally prefers to keep the identity of all clinical trial participants under wraps until all clinical trials for a particular drug are completed. Goodman suggested this concern could be skirted if a company gave the names of nonresponders to a third party who might run an open-access program, which would be the equivalent of an expanded access trial.

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