FDA: Patient Access to Investigational Drugs

As a cancer physician, I often cared for patients who found themselves in very desperate medical situations. When all available approved treatment alternatives have been exhausted, many patients are willing to try investigational or restricted therapies – but then learn they are not eligible for the protocols that govern their use. What are such desperate patients to do?

My Take is that once approved treatment regimens have been exhausted, desperately ill patients should have appropriate access to any reasonable treatment that has the potential to be of benefit and yet doesn't present unjustifiable risks. The best way to provide these drugs is through clinical trials, where the rigor and discipline of the trial enables patient response, side effects, and outcomes to be carefully measured to determine safety and effectiveness in a systematic way. When this is not possible, there can be an alternative pathway to receiving the investigational therapy.

The FDA has called this pathway "single patient access," often referred to by the public as " compassionate use." We have a long history of helping patients gain access to potential treatments this way. For example, just in FDA's oncology division, we review and approve hundreds of requests each year for single patient access to investigational drugs.

More recently, we have encountered circumstances in which a drug is approved and is commercially available, but can only be used under very strict rules – these are called an approved Risk Evaluation and Mitigation Strategy (REMS). But we have adapted "single patient expanded access" to this category of drugs as well in an effort to do everything appropriate to serve these unfortunate patients.

So – how can patients gain access to an investigational drug or a restricted drug through these mechanisms? To permit such treatment use of an investigational drug, the company must first agree to make the drug available. Then, the FDA, in conjunction with the patient's treating physician, must determine, among other things that the potential benefit justifies the potential risks of the treatment use and those potential risks are not unreasonable in the context of the disease or the condition to be treated; and that providing the investigational drug for the treatment use will not interfere with the initiation, conduct, or completion of clinical investigations that are required to support marketing approval of the investigational drug for the greatest number of patients who can benefit. A similar path is taken when considering use of restricted commercially available drugs.

Drugs under investigation, in either a clinical trial or an expanded access program, don't offer a guarantee of success, but they do offer an option. Information about clinical trials of investigational drugs and expanded access options, including contacts and locations, is available through the ClinicalTrials.gov web site. At the FDA, we have an Office of Special Health Issues which you can call and where we have trained personnel to listen to your story and assist you with the necessary information. Our number to call is 301-827-4460.

At the end of the day, benefitting patients is our goal at FDA. And how we achieve it requires a structured and disciplined process to facilitate getting the right drug or medical product to the right patient in the right way – to get the best possible outcome. FDA

Andy

Andrew C. von Eschenbach, M.D.
Commissioner of Food and Drugs

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