

January 12, 2007

Division of Dockets Management (HFA-305)
Food & Drug Administration
5630 Fishers Lane, Room 1061
Rockville, MD 20852

RE: Docket No. 2006N-0062
RIN 0910-AF14
Expanded Access to Investigational Drugs
for Treatment Use: Proposed Rule

Dear Sir or Madam:

The National Organization for Rare Disorders (NORD) is pleased that the FDA has issued a Proposed Rule to clarify the mechanisms under which patients may have access to investigational drugs outside of controlled clinical trials. NORD is a national non-profit voluntary health agency representing an estimated 25 million Americans with rare "orphan" diseases. Treatments for these disorders are generally known as "orphan drugs," and they are usually the first and only treatments for very serious and life-threatening diseases.

The Rule

The proposed rule suggests that the term "Expanded Access" should be used to describe a group of three methodologies that would allow access to investigational drugs: The first would allow access to individuals, or a small group of individuals, who have no other available treatment for their serious or life-threatening disease; the second would allow access by an intermediate size population; and the third (Treatment IND) would allow broad access for the largest populations.

We are pleased that FDA recognizes that in the past, access to investigational drugs had favored cancer and HIV, while people with other serious and life-threatening diseases have felt ignored. Clarifying the rules based on population size instead of diagnosis is logical and equitable, as long as it does not interfere with the conduct of controlled clinical trials. We also concur that a basic stipulation for any expanded access program should be a mandatory listing on the Clinicaltrials.gov Website.

Limitations

Unfortunately, FDA admits that it cannot force sponsors to offer expanded access to patients outside of clinical trials. Thus, expanded access may remain an unfulfilled dream to patients who will know the expanded access mechanism exists, yet will still be unable to obtain the drug.

We agree that risks and possible benefits must be well characterized before access to an investigational drug can be granted, and all human subject protections must be in place. Since the great majority of investigational drugs do not achieve marketing approval because they are either unsafe or ineffective, FDA must do its utmost to prevent a “therapeutic misperception” that experimental drugs will definitely help desperate patients who have no other treatment options. Patients should be counseled, and informed consent documents must reflect that they cannot expect to personally benefit from the drug, but the knowledge gained from the experiment will help other patients in the future.

Expanded access should be permitted no sooner than the completion of Phase II trials so that risks and potential benefits can be characterized from the results of preceding trials. The data from Phase II trials must be compelling before access is allowed. Otherwise Phase II trials should be fully enrolled or completed before broader access is permitted, and only if relative safety and effectiveness is probable.

We are pleased that the agency has made provision for small populations such as rare diseases, drugs not approved in the USA but used in other countries, and drug shortages. However, the proposed rule does not mention an element of the original Treatment IND regulation: The ability of companies to charge patients for the cost of the investigational drug.

The original IND regulation allowed companies to recoup their development costs by charging patients for the cost of the drug without a profit. Unfortunately, we learned from those experiences that in general, health insurance and Medicare/Medicaid will not pay for investigational drugs except for Group C cancer medications. The National Cancer Institute designates Group C drugs, but no other class of drugs is categorized this way.

Therefore, if companies are permitted to charge patients for an investigational drug under any expanded access scenario, only the wealthy will have access. This is a very serious problem, and we understand that FDA may be hesitant to address it. We would like to see a requirement that companies choosing to charge for investigational drugs put aside a quantity of the drug for needy patients who cannot afford to pay. There is no other equitable way to address the issue of reimbursement, nor to require insurers to pay. The more expensive a drug or biologic is, the less likely that reimbursement will be available. Additionally, if lab tests or imaging procedures are required during therapy, and if insurance will not pay, sponsors should be required to cover those costs to prevent patients from skipping those tests or procedures.

We hope these comments are helpful. Again, we thank the agency for the proposed rule, which will make expanded access programs more understandable and uniform.

Very truly yours,

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