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Comments of The Patients' Coalition

CDER Stakeholders Meeting

August 17, 1998

Delivered on behalf of the following Patients' Coalition members:

- AIDS Action
- American Foundation for AIDS Research
- Aplastic Anemia Foundation of America
- Center for Medical Consumers
- Committee for Children
- Cystinosis Foundation
- Hemochromatosis Foundation
- Human Rights Campaign
- Myasthenia Gravis Foundation of America
- National Organization for Rare Disorders
- National Women's Health Network
- Project Inform
- Treatment Action Group
- Tri-State Sleep Disorders Center
- United Church of Christ, Office for Church in Society
- United Parkinson Foundation
- Wilson's Disease Association

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Good Afternoon. My name is Scott Sanders with the American Foundation for AIDS Research and I have been asked by the Patients' Coalition to deliver these comments on behalf of the coalition. The Patients' Coalition came together several years ago because of concerns that the needs of patients with serious and life-threatening illnesses were being ignored or, in some cases, misrepresented during the early discussions about possible changes to the Food, Drug and Cosmetic Act. These groups then joined together with other consumer organizations, united in the common desire to see that new products are thoroughly and adequately researched pre- and post-approval and that the FDA's authority as a regulatory agency not be diminished. It was our belief then -- and it remains so today -- that the changes necessary at the FDA could best be accomplished without legislation and certainly without legislation lowering the standards and authority of the FDA, as was done in FDAMA.

Our task today and the FDA's task in the coming years is to define the FDA's strategy for meeting its legislative mandates in the coming years.

ADVOCATE FOR MORE RESOURCES

First, the FDA must make an assertive effort to get more resources. The Center directors must carry the message to the Acting Commissioner and to the new Commissioner, when confirmed, that she or he must be a vocal advocate -- within the Administration, in the Congress and before the American people -- for the resources the FDA must have to meet its legislated responsibilities. There is simply no way for the FDA to do its job with the resources it has now.

Certainly one strategy that you have heard today that deserves your attention is for the FDA to work more constructively with its stakeholders, as is done by many other federal agencies, to build support for adequate funding levels. One concrete step that the FDA must take in this planning process is to generate a realistic budget for meeting its legislative mandate. By enacting Sec. 406, the Congress gave FDA the perfect venue for developing a budget estimate that reflects the professional judgment of the FDA leadership. The FDA would be seriously remiss if it conducted such a planning exercise without a budget attached. If there is one message that you take away today it should be to work within the agency to insure that FDA leadership seizes the opportunity that it has been given by the Congress to document what resources the agency needs to do its job.

REASSERT REGULATORY AUTHORITY

Second, the FDA must forcefully reassert its role as a regulator. The skewed debate of the past three years has shifted the perception of what the FDA's role in our society and economy is and we fear that it has also shifted the FDA's own perception of what its role is. The FDA is first and foremost a regulatory agency with a primary responsibility to protect and promote public health. While the agency should never unnecessarily act in ways that are harmful to industry, it is not appropriate for FDA to compromise its mission in order to support, through its decisions, the financial well-being of a particular company or type of company. The FDA's job is to make sure that the regulated industry follows the rules that are designed to protect public health.

For the regulatory process to work there must be two separate roles, one for the regulator and the other for the regulated. These parties can and should communicate frequently and work cooperatively, but their missions and their roles are distinct. To be credible and respected, the FDA's regulatory process must maintain its clear independence from the regulated industry. As the FDA moves forward with developing the Congressionally-mandated plan, it must keep this perspective at the forefront.

One pointed example of the impact of the prolonged lack of adequate resources and the skewed perspective at the FDA is question number 5 in the list provided by CDER: "How should CDER balance the need for strong and timely pre-market review programs with the need for effective post-market inspection, surveillance and enforcement programs?" That's like asking us to find a balance between building safe aircraft and providing adequate maintenance over the course of the plane's life. It is simply not appropriate to balance pre- and post-market responsibilities against one another.

If the FDA is to fulfill its mission, it must do both fully and energetically. The solution to the problem is not to cut back on either one, but to find the will and the resources to do both. Certainly, the death and injury figures cited in the FDA's own Message to FDA Stakeholders tell us very clearly the price that the American people are paying as result of trying to find a balance between these two important responsibilities. Certainly, the current facts dictate the need for a greater commitment to drug safety.

CREATE OFFICE OF DRUG SAFETY

The single most significant step that CDER could take to begin to make meaningful progress toward fulfilling its surveillance and adverse event reporting responsibilities is to create an independent Office of Drug Safety with the resources and authority to do its job.

The current situation is alarming. Currently, the FDA has fewer than 60 employees and a budget of \$6 million to monitor the safety of 3,200 different approved drugs in the marketplace. A staff of less than 60 is unequal to the challenge of reducing deaths and serious injuries from approved drugs. A study recently published in the Journal of the American Medical Association showed that 106,000 Americans died and another 1.3 million injured as a result of adverse reactions to properly prescribed medications. For the record, this study and several other relevant studies with equally alarming results are attached. The extremely limited staff that the FDA has to deal with this tremendous problem is in sharp contrast to the 4,000 inspectors the Federal Aviation Administration has to monitor the safety of 11 major and 70 smaller air carriers and private pilots, an industry which in 1996 had accidents resulting in a total of 945 deaths.

The time has long since come for CDER to establish an Office of Drug Safety with its own advisory committee to consider safety questions about already approved drugs. The office should have the funds and capacity to use all major tools of public health prevention including case control studies, patient surveys, and data from existing health care information systems, and the MedWatch system. The office should be required to assess and publish an annual detailed

analysis of drug-related injuries and deaths to monitor progress towards improved drug safety, and to measure any problems with newly approved drugs. As a part of the planning process required by FDAMA, the FDA should determine what resources and staffing would be needed for this office to do its job and the FDA's leadership should be on Capitol Hill making the case. We can assure you that patient and consumer groups would be right behind you to make the case as well and we hope that the regulated industry would do the same. Drug safety is in everyone's best interest.

STRENGTHEN POST-MARKETING RESEARCH AND SURVEILLANCE

Another area where CDER must strengthen its authority and effectiveness is in compelling drug sponsors to conduct the post-approval trials that are agreed upon at the time of approval. This is especially important for priority drugs intended for the treatment of serious and life-threatening diseases, which have seen a marked reduction in pre-approval regulatory requirements for data to demonstrate the safety and efficacy of the product for the proposed use. Many of these drugs are approved very quickly under "accelerated" NDAs. Many members of the Patients' Coalition advocated forcefully for this mechanism and have seen the impact it has had on moving drugs through quickly. It was never the goal to see those drugs approved without continued research. Patients desperately need post-approval data to confirm the early indications of effectiveness and address on-going safety concerns and issues such as dosing and regimens.

CDER must develop a stronger system for compelling sponsors to conduct controlled trials to confirm clinical efficacy and expand upon the limited knowledge base that formed the basis on which accelerated approval has been granted. Post-marketing research must get done. A medical officer should be responsible for monitoring the conduct and completion of all agreed upon post-marketing research for each approved drug. Monitoring and completion of this research must be a top priority. As more and more drugs are approved on less and less data, the manufacturers must be held accountable for the research that they commit to doing as a condition of their approval. We acknowledge that the FDA's ability to successfully compel manufacturers is hampered by the lack of appropriate enforcement mechanisms, such as civil monetary penalties. The unwillingness of Congress to include such authority in FDAMA is a major failing of the legislation, but the FDA must move forward to develop and implement a system that will ensure that this critical research gets done. .

Additionally, the FDA, in conjunction with industry, should identify drugs currently in use for which adequate safety and dosing information in certain populations are not available and then develop a means of obtaining that information through aggressive surveillance of current practices.

In a small victory for patients, the status of individual, post-approval studies is now the subject of a public reporting requirement. As required by the statute, these public reports must include "information ...to establish the status of a study described ... and reasons, if any, for failure to carry out the study." To be useful and to meet the requirements of the statute, the information provided must be of sufficient detail to be meaningful. If a study has been halted, the report

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should say why it was halted; if it was stopped because of adverse reactions, for example, that should be stated along with a listing of those reactions and their numbers. If a study is in progress but not meeting projected milestones because of poor enrollment, then that should be reported. Congress included this provision so that patients and consumers could effectively monitor the progress of committed phase IV studies. The FDA must not cripple this provision by unnecessarily limiting the information that is publicly reported.

FAST TRACK APPROVAL

As I stated, there has been a significant shift toward approving new products on less data and that will continue with the implementation of the fast-track provisions of FDAMA. This provision, like the current accelerated approval regulations, includes some important safeguards, such as a fast-track withdrawal mechanism. All drugs approved under the new fast-track mechanism in FDAMA should be subject to all provisions of the section. The proposal that some fast-track products be exempt from the requirements of the provision is inappropriate and clearly in conflict with the statute.

ACCESS TO INFORMATION

Patient and consumer groups need better and easier access to information about the research on and the regulation of the products they are taking or might take, once the products are approved. Members of the Patients' Coalition have numerous stories of trying to get basic, non-proprietary information from the FDA only to meet with roadblocks.

I can give one example from a recent series of events in my office. We are trying to gather very basic information on drugs approved through the Treatment IND process so we could gain a better understanding of how well that process has worked in the past, in order to be able to provide better input as to how that system might be improved in the future. At the suggestion of FDA employees, we prepared a FOIA request for the information we needed. One thing that several individuals told us to ask for, which should be easily available, was the "summary basis of approval." We hoped that information would answer a question regarding the information submitted in the NDAs for these drugs. In return we received a terse letter stating, "The Food and Drug Administration has not prepared Summary Basis of Approval for any approvals in the past several years. These documents are no longer prepared and therefore are no longer available." Some of the information we requested was on drugs approved ten years ago. Upon talking to someone else, we learned that the FDA now prepares an alternative to the summary and that we should now request that information separately. It would have been far more helpful if the FDA had just provided the alternative information, as it was clear from the request the type of information we needed. Most consumer and patient groups have very limited resources and this type of response from the FDA only makes it that much harder for us to do the work that we need to do.

This example is representative of the difficulty that many patient and consumer groups have in getting information from the FDA, but there are many examples that are more serious. To address these problems and to help the public be appropriately informed, the FDA should

comment publicly on non-proprietary information in new drug and device applications, including deficiencies in applications and petitions. It should also comment on rejected or withdrawn applications. Such openness in the process will create mutual responsibility in the review of applications and petitions. Sponsors will be responsible for submitting quality applications and petitions, with complete data and information, while the FDA will be held accountable for reviewing complete applications and petitions within the mandated time frames. In addition, if a product is pulled from the market as the result of safety concerns, the FDA should be required to publicize or, at least, acknowledge the reason for the product's unavailability. This has not always been the case.

The FDA is at a crossroads. We hope that CDER and the agency as a whole will seize the opportunity that FDAMA-required plan presents to put forth a complete picture of the programs and resources that will be required for the FDA to fulfill all of its legislated responsibilities on behalf of the American people.