

FDA'S DRUG APPROVAL PROCESS: UP TO THE CHALLENGE?

STATEMENT OF ANDREW SPERLING, DIRECTOR OF LEGISLATIVE ADVOCACY ON BEHALF OF THE NATIONAL ALLIANCE FOR THE MENTALLY ILL BEFORE THE COMMITTEE ON HEALTH, EDUCATION, LABOR & PENSIONS UNITED STATES SENATE MARCH 1, 2005

Chairman Enzi, Senator Kennedy and members of the Senate HELP Committee, I am pleased to offer this testimony on behalf of the 210,000 members and 1,200 affiliates of the National Alliance for the Mentally III (NAMI). As the nation's largest organization representing children and adults with severe mental illnesses and their families, NAMI would like to thank you for convening this important hearing.

Balancing Safety & Efficiency

NAMI welcomes the recent discussions about how we can make our drug approval system better. At the same time, we are mindful of the fact that people living with life altering diseases such as schizophrenia, bipolar disorder and major depression are given hope for recovery and a full life because of the advances of scientific discovery and development. NAMI supports reform that makes drugs safer, but we also want to warn against those that might unintentionally slow down the flow of new advanced treatments for major mental illnesses -- or even worse, discourage their creation altogether. The only way to prevent such unintended consequences is to have a thoughtful policy discussion not about safety alone, but safety in combination with benefit and efficiency. While we of course want safer drugs, NAMI believes strongly that any safety reforms absolutely must be matched with efforts to enhance the FDA's level of efficiency, scientific expertise, and overall capacity to fulfill its numerous mandates.

NAMI respects the Committee's scrutiny of recent concerns regarding drug safety, and we share your commitment to assuring that information about risks associated with drugs – especially medications to treat mental illness – is identified and disseminated as early as possible. However, it is important to always keep in mind that beneficial drug products will always carry a certain amount of risk. No drug is ever 100% safe.

Just like patients and their caregivers must weigh the benefits and risks associated with a particular product when deciding whether or not to use it to treat an illness, we feel that the FDA must continue to carefully weigh the benefits and risks associated with a product when deciding whether or not to grant approval. For that reason, NAMI would advise against any effort that creates new regulations or bureaucracy that isolates or further separates either the drug safety function or the drug efficacy function from the overall drug review process. Safety and efficacy must never be viewed in isolation from each other. The FDA's review process should remain structured in a way that emphasizes the benefit-risk balance of a medicine as a basis for approval.

Drug reviews that are not based on this delicate balance will almost certainly discourage research on new therapies for illnesses such as schizophrenia, bipolar disorder and major depression. It may become very difficult to get a drug approved for such serious illnesses if the regulatory hurdles for safety are too high because those drugs are likely to have some level of side effects, and they are likely to be used in a patient population that is disabled and vulnerable to adverse reactions.

Of even greater concern is how an overemphasis on safety might have a devastating impact on our ability to move forward on early detection and early intervention – or perhaps even a cure for the major severe mental illnesses. It is important to note that all of the major clinical interventions for illnesses such as schizophrenia and bipolar disorder are palliatives. These medications help control the disabling symptoms and the illness and allow the patient to increase or maintain functioning. They do not however "cure" the illness and long-term adherence to treatment is often the most difficult barrier.

The conundrum is that no single medication works the same for every patient. This is especially the case with atypical antipsychotic medications that are the mainstay for treating schizophrenia. These agents are not interchangeable and react differently for individual patients both in terms of efficacy in addressing the major symptoms of the illness, and in their side effect profile. A drug that cannot be tolerated by one patient, may be the only agent that is effective for another. The concern is that if the regulatory hurdles for safety are too high, it will be very difficult for doctors, patients and their families to access the full range of approved treatments, even though they may help hundreds of thousands, if not millions, of people living with this devastating illness to achieve recovery.

Who is going to invest billions of dollars into the research and development of new cancer products if they are not likely to be approved because they might not be considered safe enough, regardless of their benefits? Or, if they will face an even longer and less efficient review process?

Improving FDA's Performance

NAMI agrees that the FDA's role in evaluating and monitoring safety should be strengthened. But more importantly, we want to ensure safer and more effective drugs moved through the system as efficiently as possible so they can be used as soon as possible by those who need them most, such as people living with severe mental illnesses.

NAMI would prefer strategies and solutions designed to improve the FDA's capacity in the areas of safety, efficacy, and efficiency simultaneously. At the very least, any effort to improve one aspect of these factors alone should not be implemented without careful consideration of how the other two might be impacted. And this means that FDA's budget must be considered accordingly.

NAMI supports the 5 key "Pillars of Safety" that have been recommended to the committee by our colleagues at the National Patient Advocate Foundation as critical to reforms at the FDA:

- 1- Safety and Efficacy must continue to be the foundational elements of the FDA regulatory process. Safety cannot exist in a vacuum apart from efficacy.
- 2- Mechanisms to enhance existing structures and processes for post market safety monitoring and adverse event reporting must be explored.
- 3- Efforts to bring even greater efficiency and scientific expertise to the FDA's review and monitoring processes must continue; such efforts must be done in a manner that empowers the Agency to keep pace with the rapid advancements now occurring in areas such as genomics.
- 4- FDA must continue to work with industry, patient groups, physicians, hospitals, academia, and other government agencies to enhance the critical path.
- 5- The FDA must be sufficiently resourced in order to insure more effective pursuit of its existing mandates. Additional resources are even more essential if FDA is to successfully implement a comprehensive suite of reforms.

NAMI is encouraged by the FDA's plan to allocate more than \$70 million over five years to support enhanced monitoring and surveillance of risks that may be associated with drug products already on the market. However, no drug is without risk; and it always has been an unfortunate but unavoidable fact that some adverse effects may not become apparent until after a drug has been in wide or extended use. We can hope to minimize such adverse effects and enhance the agency's capacity to report them, but we must also

accept certain risks associated with beneficial drug products. Moreover, without new monies, every dollar the FDA shifts towards new regulations and infrastructure for safety is money taken away from programs that allow the agency to more effectively and efficiently evaluate risk and benefit together.

Conclusion

Mr. Chairman, thank you for the opportunity to share NAMI's views on this important issue. We look forward to working with the Committee to ensure that any safety reforms at the FDA are balanced with efforts to enhance the FDA's level of efficiency, scientific expertise, and overall capacity to fulfill its mission.