Summary of Statement of Diane E. Thompson
on Behalf of the Alliance for Drug Safety and Access
House Energy and Commerce, Health Subcommittee Hearing on "Assessing the Safety of our Nation's Drug Supply"
May 9, 2007

Today, I will be testifying on behalf of the Alliance for Drug Safety and Access (ADSA), a coalition of 11 patient and provider organizations.

We have the opportunity before us to both maintain timely access of patients to new therapies, while strengthening oversight of drugs already on the market. We believe that with sufficient resources both goals are achievable. Simply put, we do not accept that patients should have to choose between safety and speedy access to new medications.

FDA has virtually no authority to compel drug manufacturers to continue to study the safety of products after they have been approved, force changes to drug labels if dangerous side effects are uncovered, or require that the results of clinical trials be shared with the patients who make them possible. Giving FDA these authorities and flexible tools to enforce them, including civil money penalties, as legislation pending before the Committee would do, ultimately benefits both patients and drug manufacturers.

We believe that the core of any effort to improve drug access and safety must be a shift to a "life-cycle" paradigm, with an emphasis on the continuing pursuit of knowledge about a drug's risk-benefit profile and timely communication of that information to patients and providers. We ask the Committee also to adopt the IOM's recommendation that the Office of Surveillance and Epidemiology (OSE) be given a greater role in drug review and the development of safety plans.

We ask the Subcommittee also to ensure that any drug safety legislation includes mechanisms for greater public input and transparency. Given that no one stands to benefit or lose more than patients in drug safety decisions, patients must be given a significant role in the assessment and management of drug risks.

Any new authority of FDA to require studies of post-market safety concerns must include unambiguous authority to require studies on-label uses of a drug. Any effort to reform the drug safety system that fails to address one-fifth of the use of drugs in real-world settings leaves a significant safety gap. Children would be left at particular risk by the failure to clarify this authority, since as much as three-quarters of pediatric prescribing is off-label.

In our view the Subcommittee must make the public dissemination of trial results a cornerstone of its drug safety efforts.

FDA must be given the resources it needs to accomplish its critical mission through a combination of an increase in user fees targeted to drug safety activities and an increase in appropriations. The need for new authorities and for increased funding are so inextricably linked, legislation to improve the safety and availability of pediatric drugs and devices, as part of a single legislative package.

Statement of Diane E. Thompson

Vice President for Public Policy and Communications

Elizabeth Glaser Pediatric AIDS Foundation

on Behalf of the Alliance for Drug Safety and Access

House Energy and Commerce, Health Subcommittee Hearing

on "Assessing the

Safety of our Nation's Drug Supply"

Wednesday, May 9, 2007

Mr. Chairman, Mr. Deal, and members of the Subcommittee, thank you for the opportunity to participate in today's hearing. I am Diane Thompson, Vice President for Public Policy and Communications at the Elizabeth Glaser Pediatric AIDS Foundation. Today, I will be testifying on behalf of the Alliance for Drug Safety and Access (ADSA), a coalition of 11 patient and provider organizations. Collectively, members of ADSA advocate on behalf of over 30 million patients, including those suffering from HIV/AIDS, Parkinson's disease, spinal cord injuries, paralysis, multiple sclerosis, leukodystrophies, Tourette Syndrome, and over 6,000 known rare diseases. In addition, our members represent over 100,000 providers of care to children and individuals with mental illnesses.

As a representative of the Elizabeth Glaser Pediatric AIDS Foundation, I am also proud to offer the perspective of an organization that has been focused on speeding patient access to safe medicines since its inception in 1988. This issue is at the heart of our mission -- the Foundation's creation was sparked by Elizabeth Glaser's outrage over the lack of safe and effective options for treating her two HIV-infected children. Although Elizabeth's efforts were too late to save her daughter, Ariel, who died from AIDS at the age of 7, her legacy includes her son Jake, now 22 years old, and the thousands of HIV-infected children around the world who now have the chance to grow up healthy and even start families of their own, thanks to the search for lifesaving pediatric medicines that Elizabeth Glaser and the Foundation championed.

I would like to thank the Chairman, the Ranking Member, Mr. Waxman, Mr. Markey, and other members of the Subcommittee for your leadership on this issue, for moving beyond the headlines to examine our nation's current drug safety system and discuss meaningful solutions to ensure that the Food and Drug Administration (FDA) remains the world's gold standard for public health protection. Your task is not an easy one and we appreciate the historic nature of this undertaking.

We have the opportunity before us to both maintain timely access of patients to new therapies, while strengthening oversight of drugs already on the market. We believe that with sufficient resources both goals are achievable. Simply put, we do not accept that patients should have to choose between safety and speedy access to new medications.

Patients with serious illnesses understand that bringing drugs to market in a timely way means that not every risk can be identified in advance. What they also demand, however, is sufficient information for themselves and their providers to assess risks and benefits on an ongoing basis — which often means further testing of the drug after approval. Yet, the FDA has virtually no authority to compel drug manufacturers to continue to study the safety of products after they have been approved, force changes to drug labels if dangerous side effects are uncovered, or require that the results of clinical trials be shared with the patients who make them possible.

Giving FDA these authorities and flexible tools to enforce them, including civil money penalties, as legislation pending before the Committee would do, ultimately benefits both patients and drug manufacturers. Allowing FDA to require additional testing of drugs postmarket could actually allow the FDA to approve drugs more quickly, knowing it will have the ability to act if there are new safety concerns once the drug is in the hands of patients. Also, by giving FDA the flexibility to impose fines for non-compliance, we can avoid the worst possible outcome for everyone: pulling a drug from the market that still holds some benefit for some group of patients.

We believe that the core of any effort to improve drug access and safety must be a shift to a "life-cycle" paradigm, with an emphasis on the continuing pursuit of knowledge about a drug's risk-benefit profile and timely communication of that information to patients and providers. This approach, which is recommended by the Institute of Medicine (IOM), has been included in drug safety legislation introduced by Mr. Waxman and Mr. Markey. In our view, individualized risk evaluation and mitigation strategies, rather than a one-size-fits-all approach to patient safety, will be key to the appropriate balancing of drug risks and benefits that is so critical to patients with life-threatening illnesses.

To further improve the depth and breadth of input into drug safety decision making, we ask the Committee also to adopt the IOM's recommendation that the Office of Surveillance and Epidemiology (OSE) be given a greater role in drug review and the development of safety plans. The lack of communication and cooperation between that office and the Office of New Drugs,

highlighted in both the IOM report and a March 2006 report by the Government Accountability Office, is deeply troubling. At minimum, we recommend that the Committee formally assign OSE staff a role in the review of new drugs applications and post approval regulatory actions, as the IOM recommends.

We ask the Subcommittee also to ensure that any drug safety legislation includes mechanisms for greater public input and transparency. The history of our Foundation and of the broader HIV/AIDS community is the story of the power of patients' contributions to scientific decision making. Although they began as three mothers around a kitchen table with no formal training in science and medicine, Elizabeth Glaser and the other founders of the Foundation ultimately changed the accepted thinking of both the National Institutes of Health and FDA about the risks of not studying AIDS drugs in children – a success story that is repeated throughout the histories of patient organizations. Given that no one stands to benefit or lose more than patients in drug safety decisions, we ask that you consider a significant role for patients in the assessment and management of drug risks.

We also urge the Committee to clarify that any new authority of FDA to require studies of post-market safety concerns is not confined to on-label uses of the drug. In our efforts to improve the drug safety system, we need to pay particular attention to not only what happens inside the FDA, but also what goes on in the real world. A recent study found that 21% of prescriptions written

in 2001 were for off-label uses. Any effort to reform the drug safety system that fails to address one-fifth of the use of drugs in real-world settings leaves a significant safety gap.

Children would be left at particular risk by the failure to clarify this authority, since as much as three-quarters pediatric prescribing is off-label. Thanks to the efforts of many on this Subcommittee, there are mechanisms available to both encourage and require manufacturers to study their products for children. However, there are gaps in those mechanisms. The existing pediatric study requirement does not apply to off-label uses. While the existing incentives can be applied to off-label studies, they are voluntary -- and we are seeing that manufacturers are increasingly opting not to conduct the studies FDA requests. Unambiguous authority to require such studies when the off-label use is significant will help ensure that children too can reap the benefits of an improved drug safety system.

In our view the Subcommittee must make the public dissemination of trial results a cornerstone of its drug safety efforts. The establishment of a results database would be a significant step forward in giving patients and providers additional information with which to assess benefits and risks. By linking the registration of new trials with final outcomes, this database also could help prevent selective reporting of positive results and the problems that have resulted from the withholding of negative trial results. And, not incidentally, given that clinical trials could not exist without patients' willingness to give of their time and health, such a mechanism could help restore patients' trust in the integrity of the clinical trials process.

While we work toward providing the FDA additional authorities and enforcement tools, we must acknowledge that chronic under-funding is severely straining the ability of the Agency to perform even its current functions. Years of essentially flat funding, coupled with new challenges such as increasingly global markets, the threat of bioterrorism, and the promise of personalized medicine, have left the Agency struggling to meet its obligation to protect the public health. We – Congress, the Administration and patients – must work together to give the FDA the resources it needs to accomplish its critical mission. We suggest the combination of an increase in user fees targeted to drug safety activities and an increase in appropriations. Because we believe that the need for new authorities and for increased funding are so inextricably linked, we strongly recommend the Subcommittee consider these issues, along with legislation to improve the safety and availability of pediatric drugs and devices, as part of a single legislative package.

Mr. Chairman, you have before you a historic opportunity to finally match our nation's success in speeding new therapies to patients with a system that can better ensure the safety of those products once on the market. We appreciate your interest in patients' and providers' perspectives on these critical issues and look forward to working with you to accomplish these goals.

Thank you again for the opportunity to share our views.