

**Statement of Diane E. Thompson  
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on Behalf of the Alliance for Drug Safety and Access**

**Senate Health Education, Labor and Pension Committee Hearing on Building a 21<sup>st</sup>  
Century FDA: Proposals to Improve Drug Safety and Innovation  
November 16, 2006**

Mr. Chairman, Senator Kennedy, and members of the Committee, 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.

First, let me begin by thanking the Chairman, Senator Kennedy, Senator Dodd and other members of the Committee for your leadership on this issue, for moving beyond the headlines to take on the difficult task of crafting bipartisan legislation to truly reform our nation's drug safety system. We certainly appreciate the magnitude of the task and the historic nature of this undertaking.

We also appreciate the efforts you and your staff have made to incorporate the recommendations of our coalition. We know that you share our interest in both continuing the timely access of patients to new therapies and strengthening oversight of drugs already on the market. And, 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. However, what they also demand is sufficient information for them and their providers to continue to assess risks and benefits — which often means further testing of the drug after approval. Yet, as the report by the Institute of Medicine (IOM) so clearly illustrates, the Food and Drug Administration (FDA) has virtually no authority

to compel drug manufacturers to continue to study the safety of products after they have been approved, to force changes to drug labels if dangerous side effects are uncovered, or to require that the results of critical studies be shared with patients and providers. In addition, at current funding levels, FDA lacks the resources to successfully accomplish many activities it is authorized to undertake, including effective collection and analysis of post-market safety data.

Giving FDA these authorities and flexible tools to enforce them, as legislation pending before the Committee would do, ultimately benefits both patients and drug manufacturers. Allowing FDA to require additional testing of drugs when there are clear signals of safety problems 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 were pleased to see that S. 3807 essentially contains these critical elements. Perhaps most importantly, it frames them in a context of a risk-based approach. That model, 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. Similarly, we welcome the IOM's vision of applying a "life-cycle" paradigm to drug risks and benefits, with its emphasis on the continuing pursuit of knowledge about a drug's safety profile and timely communication of that information to patients and providers. As recommended by the IOM, we hope that this will include the significant improvements to FDA's capacity to collect and analyze safety data through passive and active surveillance systems, as well as through prospective studies.

We are concerned however, that S. 3807 lacks sufficient mechanisms to elicit much needed patient and provider input. Some of the most critical patient safety decisions under the new structures proposed in the legislation will be those that relate to the development of risk evaluation and management strategies (REMS) plans. Yet, the bill currently assigns the responsibility for developing those plans and resolving related disputes solely to FDA and to an internal board composed entirely of federal employees, with no opportunity for input from outside experts, patients, or providers.

The history of our Foundation and of the broader HIV/AIDS community is the story of the power of patients' contributions to scientific decisionmaking. 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 greater role for patients in the development of REMS plans and resolution of REMS disputes. Specifically, we recommend that an existing or new advisory committee be utilized rather than the Drug Safety Oversight Board. Such a committee could draw on more diverse expertise, including the voices of patients and providers, and could make its deliberations public, which would be an important step in improving public trust in the process.

To further improve the depth and breadth of input into drug safety decisionmaking, we ask the Committee to adopt the recommendation of the IOM 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 urge the Committee to formally assign OSE staff a role in the review of new drug applications and postapproval regulatory actions, as the IOM recommends.

We also urge the Committee to clarify that the 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. As the IOM report notes, 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 1/5 of the use of drugs in real-world settings would create a significant safety gap.

Children would be placed at particular risk by the failure to clarify this authority, since as much as ¾ of pediatric prescribing is off-label. Thanks to the efforts of Senators Dodd, Clinton, and DeWine, 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.

We applaud the significant focus placed by S. 3807 on the public dissemination of trial results through a clinical trials database. The establishment of such a database would be a significant step forward in providing patients and providers with additional information with which to assess benefits and risks. By linking the registration of new trials with final outcomes, this database could also help prevent selective reporting of positive results and further revelations about 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.

However, in our view, a number of additions should be made to the database established by S. 3807 to ensure that it is as comprehensive and complete an accounting of trials as possible. We endorse the IOM recommendation that the database incorporate Phase II trials. We also believe that to satisfy the objective of providing patients and researchers with the full body of evidence on a drug or a class of drugs, there must be an element of retroactivity, perhaps beginning with trials of already approved products -- both for the approved use and for any uses that were studied but not approved.

Following the recommendations of a previous report by the IOM in July of 2005 on the post-market safety of pediatric medical devices, we also ask that device clinical trials be added to the

database. From the point of view of patients it is irrelevant whether a new therapy comes in the form of a drug or a device; the results of all such studies should be made publicly accessible. And, finally, while we endorse the concept of a single, comprehensive, national database that provides “one-stop-shopping” for patients and providers, until the concerns noted previously are remedied, we do not support pre-empting any efforts by states to also collect this information.

We applaud the inclusion of civil money penalties in S. 3807 as a critical step in providing FDA with graduated, flexible enforcement authority. However, we are concerned that the current penalties are too low to have much impact, particularly for higher sales products, and ask that they be increased. To ensure compliance with the requirements of the clinical trials database, we ask that the authority for FDA to impose fines for other types of violations also be applied to this section.

Finally, we agree with the IOM recommendation that specific safety-related performance goals be added when the Prescription Drug User Fee Act (PDUFA) is reauthorized next year. Clearly the experience from PDUFA thus far is that deadlines generate attention and focus. Even with additional funding, if postmarket activities without performance goals have to compete with pre-market functions with performance goals, we would be concerned they would remain an afterthought.

Obviously, the drug safety reforms proposed by both S. 3807 and the IOM create considerable new responsibilities for the FDA. For FDA to succeed in implementing these reforms, it is essential that new and expanded safety activities be explicitly paired with increased resources. We would suggest a combination of an increase in user fees targeted to drug safety activities and an increase in appropriations. We also recognize that it may be necessary to prioritize the reforms that can be implemented in the short- and long-term depending on the availability of new resources and we look forward to working with the Committee to do so.

Mr. Chairman, the Committee has before it an 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’ perspectives on these critical issues and look forward to working with you over the next year to accomplish these goals. Thank you again for the opportunity to share our views.