The Prescription Drug User Fee Act
FDA Public Meeting
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Statement of Steve Gibson
The ALS Association

Good afternoon. My name is Steve Gibson and I am Vice President of Government Relations and Public Affairs for The ALS Association.

The Amyotrophic Lateral Sclerosis (ALS) Association is pleased to submit comments to the Food and Drug Administration as it considers the reauthorization of the Prescription Drug User Fee Act. We strongly support the Agency in this endeavor and believe that the Agency's recommendations, if enacted, will help expedite the development of safe and effective medicines for diseases such as ALS.

The ALS Association is the only national not-for-profit health association dedicated solely to the fight against ALS, or Lou Gehrig's disease. In addition to serving as a resource for ALS patients and their families, The Association advocates for increased funding for ALS research and other health care policies that respond to the needs of people with ALS and their families. The ALS Association also is among the world's largest private sources of funding for ALS-specific scientific research, having awarded nearly $40 million in the past decade to fund research seeking to identify the cause, means of prevention and cure for ALS.

ABOUT ALS

ALS is a fatal, neurodegenerative disease that attacks nerve cells and pathways in the brain and spinal cord, ultimately resulting in a loss of voluntary muscle control, paralysis and death. It is a particularly cruel disease. As the disease progresses, its victims become trapped inside a body they no longer can control – unable to walk, talk, breathe or even blink an eyelid. There is no known cause of ALS, nor is there a cure. The disease can strike anyone, regardless of their age, gender, race or ethnicity. And it is always fatal, in an average of two to five years after diagnosis.

Although the average life expectancy for people with ALS is just a few years, many patients are living longer, more productive lives thanks to recent advances in research and medical science. Indeed, a wide variety of medications and other technologies are available to help treat and manage the symptoms of ALS. However, there currently is only one drug on the market specifically approved to treat ALS. Riluzole, which was approved in late 1995, has demonstrated only modest effects, prolonging life by just a few months and only in some patients. Therefore, The Association welcomes any efforts by FDA to examine ways to speed innovation and the development of new products that could benefit people with ALS.

The comments we are providing are intended as a preliminary assessment of our thoughts on PDUFA. We look forward to working with the FDA, Members of Congress and other concerned

stakeholders on this initiative and to providing the Agency additional input as it continues to explore new approaches in the drug development and review process.

ADEQUATE FDA RESOURCES

In recent years, it has been obvious to most observers that FDA has been under-staffed and under-funded. The ALS Association believes the PDUFA plan put forth last month will provide a much-needed increase in staff and resources and will help the Agency to meet its demands and ensure that patients have timely access to safe and effective medicines.

We also believe Congress has an important role in this process not simply to act promptly in reauthorizing PDUFA, but also to ensure that sufficient government funds are provided to help the Agency keep pace with its personnel, training, and information technology needs -- to enable the FDA to fulfill its mission on behalf of the American public, particularly those living with serious and life threatening illnesses whose lives quite literally can depend upon the ability of the Agency to facilitate access to new technologies. In the event the current mix of industry fees and federal resources are not sufficient, we urge Congress to provide additional support through the appropriations process.

DRUG SAFETY

The foundation of our drug development system is the ability to demonstrate safety and efficacy in new medicines, and, with few exceptions, this standard has served patients well. We applaud FDA for including new drug safety initiatives in its PDUFA proposal, including the addition of resources to ensure postmarket safety, and rapidly identify and even prevent adverse events. The Agency's recent announcement to improve the drug safety system beyond the scope of PDUFA indicates the FDA's commitment to addressing concerns about drug safety and strengthening the public's confidence that the drugs they take are safe and that risks are identified and communicated.

However, while drug safety is an important concern, people with ALS view the issue from a much different perspective than most Americans. That's because people with ALS will die if they do not have access to the latest drugs and biologics that show promise in treating a disease for which there currently is no effective treatment. To people with ALS, the mere ability to access to a new drug is itself a matter of drug safety. After all, ALS patients face a greater risk to their lives by not having access to new treatments. Therefore, we urge FDA and Congress to proceed with caution in this area. FDA and Congress must recognize that the practice of medicine is not a one-sized-fits-all proposition and that the assessment of benefit and risk must take into account the needs of individual patients. To place excessive emphasis on risk without taking due account of drug benefit, including the severity of the underlying disease, would be a disservice to people with ALS and the more than 100 million Americans living with serious and life-threatening diseases.

DRUG DEVELOPMENT and REVIEW

As I have mentioned, people with ALS currently have only one treatment option. However, they hold onto the hope that our nation's scientists and researchers will develop new treatments for the disease that can slow its progression, improve quality of life and, ultimately cure and even prevent the disease from arising.

That's why it is so critical that PDUFA promote drug development and expedite drug reviews and approvals, including for products not specific to ALS. After all, people with ALS can benefit from advances in the treatment of other neurological conditions such as MS, Parkinson's and Alzheimer's.

We strongly support the timelines and goals outlined in PDUFA concerning prompt review and evaluations of investigational drugs. Delays in this area translate into denied access to potential life-saving therapies for ALS patients--to withhold a drug in order to obtain an unreasonable amount of data could cause a countless number of patients to suffer due to the lack of access to new treatments.

ENHANCED TECHNOLOGY

Information technology is at the heart of the drug development process and the product review process. Our ability to collect, analyze and understand the enormous amounts of information gathered during the clinical phases and post-marketing period will play a vital role in determining the viability of potential products, and the long term safety and efficacy of approved products. It is imperative the FDA have modern information technology systems—we believe the PDUFA plan is a significant step forward in this regard.

CRITICAL PATH

The ALS Association is particularly pleased that the Critical Path Initiative will receive full-time permanent staff and funds as part of PDUFA. Critical Path will facilitate partnerships between the FDA, industry and the public that will promote new ideas to advance medical science. It takes advantage of new technologies to ensure the Agency employs a 21st century approach to finding treatments and cures for diseases such as ALS. Importantly, Critical Path aims to speed the drug development process, not simply the review process, and will provide new opportunities to quickly bring ALS therapies from the lab to the bedside.

However, we also are concerned that FDA has not proposed sufficient funding and resources in order for the potential of Critical Path to be fully realized. While we are pleased that new resources are provided, we hope that the Agency and Congress will work with us and other concerned stakeholders to establish a funding mechanism to support Critical Path.

CONCLUSION

The ALS Association applauds the FDA for its efforts to improve the drug development and review process and to establish policies that promote manufacturer involvement in the research that can lead to product development.

We encourage rapid consideration and adoption of the new PDUFA. We are concerned that delays in this process could hurt morale at the Agency and slow down the review and approval process.

However, we look forward to working with the Agency and Members of Congress on this important issue and hope that Congress follows FDA's lead and takes a balanced approach to

PDUFA reauthorization by providing resources that will enhance drug safety while improving access to breakthrough treatments and encouraging drug development.

We appreciate the opportunity to comment on this important initiative.