



Friends of Cancer Research | 2231 Crystal Drive, Suite 200 | Arlington, VA 22202 | www.focr.org

Statement of Friends of Cancer Research

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Introduction

On behalf of Friends of Cancer Research, I want to thank you for the opportunity to comment on the proposed recommendations for the reauthorization of the Prescription Drug User Fee Act (PDUFA). Friends of Cancer Research is a non-profit organization that over the past ten years, has pioneered innovative public-private partnerships, organized critical policy forums, educated the public, and brought together key communities to develop collaborative strategies in the field of cancer research.

An essential component of accelerating progress for the treatment of all medical conditions is a rigorous and efficient drug review process at the U.S. Food and Drug Administration (FDA). It is of the utmost importance that we work to improve that process to ensure patient safety, provide access to new therapies, and foster the development of innovative new treatments. We support the timely reauthorization of PDUFA and strongly urge Congress to demonstrate their commitment to the future health of all Americans through increased federal appropriations for FDA.

PDUFA Provides Promise to Cancer Patients

PDUFA is a successful program that benefits all stakeholders. It enables FDA to receive vital funds to support their drug review activities; product sponsors have their drugs reviewed more expeditiously; and most importantly, patients receive life-saving treatments in a timely manner.

The efficiency and expediency that PDUFA introduced to the drug review and approval system has been particularly important for patients with cancer and other life-threatening illnesses, where the ability to receive new drugs as quickly as possible is literally a matter of life and death.

Prior to passage of PDUFA, the drug lag between the US and Europe was approximately 1 ½ years, allowing European patients to benefit from new medical innovations years before they became available in the US. Now, thanks to PDUFA, US patients are usually the first to receive the benefits of new drugs. Since PDUFA's inception in 1992, approximately 100 new cancer therapies have been approved and made available to

patients in a fraction of the time it used to take to get FDA approval. For patients who are holding out hope for a new treatment, there are over 200 new drugs currently in development for cancer; it is critically important to these patients and their families that our drug review and approval process maintains its ability to deliver these promising new therapies as rapidly as possible, while maintaining the delicate balance between the benefits and risks associated with their use.

In discussions about drug safety and the benefit-risk tradeoff, it is useful to remind ourselves that patients' willingness to accept risk is not constant across patient populations served by the drug review and approval process – and this must be reflected in the system. Cancer is a disease that exists on a continuum from late-stage terminal illness, to early detection, to prevention. Patients suffering from life-threatening and/or seriously debilitating illnesses are willing to accept greater risk in the treatments available to them than are patients who are suffering from less serious conditions. All patients deserve accurate information so that they can work with their doctor to decide upon the best course of treatment to meet their individual needs.

Enhancements in the Drug Safety System

It is important to note that PDUFA's success in eliminating the drug lag and providing new medicines to patients in a timelier manner has been accomplished without compromising safety. In fact, since the first authorization of PDUFA, only 3.5% of approved drugs have been subject to safety-based withdrawal. This is similar to the ratio of pre-PDUFA safety-based withdrawals of 3.1%.

Obviously, no drug is 100% safe or 100% effective in 100% of patients. And, it is impossible to know everything about a drug before it is used widely by patients. To approve drugs without sufficient data on safety and efficacy would be irresponsible. Similarly, 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. The FDA is charged with determining the delicate balance of benefits and risks of drugs from a population standpoint before approving them for use. This tremendous responsibility should be accompanied by the resources necessary to do the job.

Increasing the regulatory authority of FDA through new legislation should be done with careful consideration. An increase to the agency's authority could result in unintentional consequences such as restricting or slowing access to life-saving treatments by patients in need, further increasing the already expanding cost of healthcare, or discouraging future innovative product development.

Regulatory authority is only as effective as the underlying evidence that it generates. An automated and routine approach to drug monitoring is needed to improve the agency's ability to identify the risks of marketed new drug products earlier and to evaluate these in the context of the health benefits provided by the product. Addressing the underlying needs to increase personnel, enhance scientific training, advance information technology,

identify new data sources, and further integrate emerging science into the overall regulatory process will lead to this systematic approach to safety surveillance.

Increased regulatory authority will not substitute for a system of routine and automated drug safety surveillance, and continued chronic under-funding will certainly not lead to one.

The recent PDUFA agreement between FDA and industry representatives is a good starting point. As a part of this reauthorization, significant new funds from user fees will be applied to post-market safety enhancements. We are extremely supportive of these programs and others that FDA has recently announced. Programs designed to enhance communication, identify adverse events earlier, expand database interaction, and review proprietary names to reduce medication errors will no doubt benefit the public.

Congress Needs to Appropriately Fund FDA

Industry user fees alone are not sufficient to fund the growing workload of the FDA and position it for success in the 21st century. It is essential that Congress increase its appropriations for FDA to address the agency's underlying needs that were previously mentioned in the form of resources, training, IT, new data sources, and advanced science. Through our membership in the Coalition for a Stronger FDA, Friends of Cancer Research is advocating for increased congressional funding of FDA.

The rate of user fee increases has far surpassed the rate of congressional appropriations for human drug review. This fact raises questions about our elected officials' commitment to the health of nearly every American.

Even with increased user fees, the lack of sufficient funding has caused other programs at FDA to suffer in order to sustain the increasing cost of and personnel for drug reviews. It is clear that proper funding is essential to ensure efficient and accurate review, however the agency cannot rely solely on private funding. As industry has increasingly supported the cost of review programs, the public's confidence in the agency has diminished: many today see the influence of the pharmaceutical and biotechnology industry at FDA as excessive. For FDA to remain a review agency independent of industry, it desperately needs congressionally-allocated resources to function effectively and to be perceived by the public as an independent agency. An increase in the level of direct appropriations will enhance overall FDA productivity and promote its continued success and improved perception of its independence among the general public.

Support for Critical Path

Protecting Americans and promoting public health will only occur through a stronger and science-based FDA. The agency has committed itself through the development of the Critical Path Initiative to modernize the drug development process. During a time of potentially revolutionizing scientific discovery, it is crucial to utilize the expertise at FDA and ensure that the agency play an integral role in emerging science. A central goal of

the Critical Path Initiative is to provide tools to identify patients who will most likely respond to particular treatments, thereby decreasing the risk to benefit ratio. If that can be accomplished, it will result in new ways to diagnose, treat, cure or prevent disease and allow life-saving therapies to reach patients faster while reducing the overall cost of healthcare in the country.

The PDUFA IV recommendations provide additional support for the Critical Path Initiative, but it, too, is a starting point. We commend the FDA and industry for jointly recognizing the importance of focusing on science. We also recognize the need for all stakeholders to work in partnership to enhance the capabilities of the FDA.

Conclusion

Friends of Cancer Research supports the PDUFA IV agreement and encourages lawmakers to pass it before the expiration of PDUFA III. We view the increased user fees in PDUFA as a starting point and urge Members of Congress to do their part to ensure FDA has the sustainable resources it needs to fulfill its mission. We would be happy to provide you with any assistance you need in ensuring that this PDUFA IV package becomes law.

On behalf of the nearly 1.5 million Americans diagnosed with cancer every year, I thank you for this opportunity to testify.