

Statement at the House Committee on Energy and Commerce
Subcommittee on Health
Hearing on the Prescription Drug User Fee Act

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Thank you Mr. Chairman and members of the Subcommittee on Health. My name is Alan Goldhammer, Ph.D., and I am the Deputy Vice President for Regulatory Affairs at the Pharmaceutical Research and Manufacturers of America (PhRMA), a trade association representing the leading research-based pharmaceutical and biotechnology companies. PhRMA members alone invested an estimated \$43 billion in 2006 in discovering and developing new medicines. We thus have a keen interest in ensuring that the Food and Drug Administration (FDA) has adequate resources to perform its critical functions of ensuring the safety, effectiveness and availability of new medicines for American patients.

Having participated in each of the four previous user fee negotiations, I bring to the Subcommittee today a full historical perspective of the Prescription Drug User Fee Act (PDUFA) and the need for expeditious re-authorization. Last year the Food and Drug Administration (FDA) and industry representatives spent nine months integrating comments from public stakeholders and discussing how the PDUFA program could be improved to continue to meet the FDA's central mission of protecting and promoting the public health. The outcome of those discussions will be the focus of my testimony today.

Reauthorization of PDUFA is one of the more important legislative issues facing Congress this year. Since its enactment in 1992, PDUFA has brought about tangible benefits to patients, the FDA, and the pharmaceutical industry. FDA's appropriated resources have been augmented by industry user fees, providing the Agency with sufficient resources to conduct reviews of new pharmaceuticals in a thorough and timely manner assuring widespread patient access.

It is important to put the PDUFA program in historical context. Initially it was unclear whether the program would succeed or not. Thus, the legislation contained a five year sunset provision. This has worked well, providing the necessary time to gauge the effectiveness of the program, and allowing all stakeholders to reflect on what can be further done within the confines of user fees to protect and promote the public health, the central mission of FDA.

Since its original passage in 1992, PDUFA has been a crucial program not only for FDA and the pharmaceutical industry, but also – and most importantly – for patients. Prior to passage of PDUFA-I in 1992, the average review time for a new drug application (NDA) had increased to over 30 months (even though the statute calls for a 6 month review time), and there was a significant backlog of pending NDAs at the Agency. As a result, life-saving medications routinely were available to patients in Europe well before they

were available to patients in the United States. With the increased funding provided under the PDUFA program, FDA was able to hire additional staff and quickly eliminate the backlog of pending NDAs. In addition, FDA made great strides to complete its reviews of new NDAs in a more timely manner, which not only added predictability to the drug review process but, more importantly, benefited patients by providing quicker and more widespread access to life-saving medications, such as treatments for HIV infection.

In PDUFA-II, the program was enhanced by increasing FDA resources in return for improved interactions during the drug development process. PDUFA-III addressed FDA's needs for sound financial footing and increased resources that could be directed towards drug safety. Both of these reauthorizations also directed funding towards information technology infrastructure so that both FDA and industry could realize the benefits of electronic regulatory submissions.

Throughout the PDUFA programs of the past 15 years, the exacting standards by which FDA evaluates NDAs have not been altered. What has been altered is the level of resources available for FDA to perform its critical function of reviewing safety and effectiveness of potentially life-saving medications. With more resources provided by PDUFA, FDA has been able to complete its rigorous reviews more quickly and efficiently. The outcome of this review, however, is not affected by PDUFA funding and, depending upon the scientific data, may be a decision to approve the drug or to *not* to approve the drug. That decision is FDA's based on the information in the license application. User fees are not earmarked for specific applications and certainly are not contingent upon approval of the drug. They go into FDA's general budget and simply are used to hire additional staff to allow FDA to perform its critical drug review functions while maintaining the same exacting standards for safety and efficacy.

Furthermore, each reauthorization has focused on issues critical to the FDA's mission. Enhancements to PDUFA have always been carefully structured, responding to both the Agency's and the public's needs so that access to important new therapies is not impaired but in fact facilitated.

The Agency's PDUFA-IV proposal is no exception and contains important new provisions and resources to:

- enhance and modernize the FDA drug safety program,
- add a new user fee program to give FDA additional resources to review and provide advisory opinions on direct to consumer television advertisements,
- improve drug development, and
- provide more stable financing for the program.

Although the industry-funded part of the drug review process will increase during the PDUFA-IV years, patients will be well served by a more predictable drug review process and assurance that the robust drug safety office within the Agency will be enhanced and modernized.

The substantial new funding provided to enhance and modernize the FDA drug safety system – nearly \$150 million dollars over the next five years – will continue to assure that FDA's pre- and post-market safety assessment system is the world's best. When the Institutes of Medicine (IOM) report on the US drug safety system was issued last fall,

the recommendations applicable to PDUFA were carefully examined. I believe that this PDUFA agreement substantively addresses all relevant recommendations that could be addressed through a combination of user fees and guidance development.

These additional resources will be used to reduce FDA's reliance on the spontaneous reporting of adverse events and increase use of modernized techniques and resources, such as epidemiology studies and large medical databases, to identify risks more quickly and accurately. We need to be able to use new IT systems, access to electronic health records, new algorithms for detecting drug safety signals, as well as new approaches to validating drug safety signals. Funding is there to move towards this future.

The FDA's PDUFA proposal also provides funds to allow FDA to develop guidance on best epidemiology practices that will serve as a base for agency, academia, and industry use. This guidance is intended to serve the public's interest by assuring that studies reporting drug-associated signals of risk do so based on defined minimum scientific standards. FDA and industry also need a process to identify risk management and risk communication tools that are effective. Industry will benefit by having a list of risk management tools that work, simplifying the development of drug-specific risk management plans. Nobody wants to spend time and resources on approaches that will not benefit patient care. This PDUFA agreement provides resources to accomplish this.

Significant resources are spent by companies late in a drug's life cycle monitoring for adverse events. It is rare that significant new safety issues are identified this late and such resources could be better allocated to other drug safety activities. FDA will also conduct research during PDUFA-IV to determine the best way to maximize the public health benefit associated with collecting and reporting adverse events. We hope that this will lead to a better deployment of resources.

A key patient safety initiative is the allocation of a portion of this funding to improving the trade name review process. Trade names are reviewed within FDA's drug safety office to help ensure that new trade names cannot be confused with existing trade names in an effort to reduce possible medication errors. FDA will now have additional resources to review trade names during drug development and provide industry with guidance on "good naming practices." This will improve the predictability of the trade name review process.

The FDA's PDUFA proposal also includes a new user fee for direct-to-consumer television advertisements. In 2005, PhRMA issued a set of voluntary guiding principles regarding direct to consumer advertising. In those guiding principles, PhRMA member companies committed to submit all new DTC TV ads to FDA prior to public dissemination to ensure that FDA's suggestions could be addressed before the ad was seen widely by the public. The proposed new user fee would ensure that FDA has the resources to review TV advertisements voluntarily submitted to FDA in accordance with the guiding principles and thus demonstrates the industry's commitment to those principles and to vigorous self-regulation.

This PDUFA proposal also continues forward with suggested improvements to the drug review process. FDA will implement the good review management principles that were formulated during PDUFA-III. FDA will communicate to sponsors a timeline for discussing labeling and post-market commitments in advance of the action date. This will improve the predictability of the drug review process and lead to more meaningful

post-market studies that are appropriate for the new drug.

Funding is allocated for the purpose of expediting drug development. This will permit FDA staff to be directly involved in external activities such as partnerships and consortia that are generating data and information that will create new paradigms for drug development. In return, FDA commits to developing draft guidance in areas related to safety assessment, clinical trial design, and the use of biomarkers. In addition, FDA will participate in workshops and other public meetings to explore new approaches to a structured model for benefit/risk assessment. The results of these interactions will be used to assess whether pilot(s) of such new approaches can be conducted during PDUFA-IV. Collectively, this will lead to new paradigms leading to more efficient and accurate drug development resulting in earlier patient access of important therapies.

Finally, it is important that we continue to assure that FDA is appropriately funded through a combination of appropriations and user fees so that the drug review program can address America's public health needs with the development of new medicines. A considerable amount of time was spent looking at increased workload within FDA, how it is measured, and how an appropriate workload adjuster can be constructed. This will provide the sound financial footing needed to continue keeping FDA's drug and biological review program strong throughout the PDUFA IV years.

The PDUFA program is vital to ensuring that FDA has the necessary resources to perform its critical functions of fostering drug development and innovation and protecting the public health. The PDUFA-IV proposal in particular will provide FDA with substantial new funding to enhance its oversight over drug safety and DTC advertising while ensuring that the drug review program is as robust and efficient as possible so that patients are not left waiting for needed cures. We urge you to reauthorize the PDUFA program in a timely manner for the benefit of FDA, the industry and, most importantly, patients.