Statement PhRMA

PhRMA Statement at the Food and Drug Administration's Prescription Drug User Fee Act Public Meeting

February 16, 2007

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The passage of the 1992 Prescription Drug User Fee Act (PDUFA) brought about tangible results in a very short period of time. At that time, drug reviews were averaging over 30 months and there was a backlog of pending reviews at the Agency. The additional resources provided the Agency eliminated the backlog and allowed FDA to complete reviews of priority applications in six months and standard applications in twelve months. This added predictability to the drug review process but more importantly benefited patients. Important new therapies were approved in a timely manner, providing widespread access.

At the time PDUFA-1 was negotiated, it was not clear whether the program would succeed or not. Thus, the legislation contained a five year sunset provision. In retrospect this has been a good thing. It provides the necessary time to gauge the effectiveness of the program, but also allows 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 the Food and Drug Administration.

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.

It is important to stress that throughout the PDUFA programs of the past 15 years, the exacting standards by which FDA evaluates New Drug Applications 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 may or may not be a drug approval. That decision is FDA's based on the information in the license application.

The Agency's PDUFA-IV proposal 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 excellent drug safety office within the Agency will be enhanced and modernized.

PhRMA believes that 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 gold standard. PhRMA believes that this PDUFA agreement substantively addresses all relevant recommendations of the IOM Drug Safety report.

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. 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. 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.

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 to 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.

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 to 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 for drug development leading to earlier patient access of important therapies.

Finally, there are a number of proposed technical adjustments for financing the PDUFA program over the next five years. It is PhRMA's hope that collectively these will provide the sound financial footing needed to continue keeping FDA's drug and biological review program strong.