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*The National Professional
Society of Pharmacists*

STATEMENT OF THE AMERICAN PHARMACEUTICAL ASSOCIATION

PRESCRIPTION DRUG USER FEES ACT

FOOD AND DRUG ADMINISTRATION AND STAKEHOLDERS MEETING

PANEL V - PRESENTATIONS BY HEALTH PROFESSIONAL GROUPS

SEPTEMBER 15, 2000

Good afternoon. Thank you for the opportunity to present the views of the nation's pharmacists about the future of the Prescription Drug Use Fee Act (PDUFA) program. I am Dr. John A. Gans, Executive Vice President of the American Pharmaceutical Association, the national professional society of pharmacists. APhA represents more than 50,000 pharmacists, pharmaceutical scientists and pharmacy students. As the health professionals working with patients and their medications (including prescription and over-the-counter medications and dietary supplements) every day, pharmacists rely on a credible drug review and approval process by the Food and Drug Administration (FDA).

My comments will focus on two of the questions posed by the Agency for today's discussion. Specifically, I will discuss the benefits and risks of the user fee program, and the focus on whether PDUFA fees should be used to pay for other costs incurred to ensure that medications in the American marketplace are safe and effective.

Benefits and Risks of the User Fee Program

Pharmacists look to the FDA for an important function: to review and approve the medications used in their practice. Pharmacists know, however, that FDA approval does not mean that medications are risk-free—medications are safe and effective *when used appropriately*.

I raise this caveat because it is important to our discussion. The FDA review process does not yield risk-free products, regardless of the question of PDUFA funding. The nation's drug approval process is based on a system of reviewing clinical trial evaluation and extrapolating those results to the population-at-large. Inherent in this extrapolation is the reality that some problems—and benefits—of products will not be discovered in the clinical

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trials. This system requires ongoing vigilance by pharmacists, other health care professionals and consumers to detect and report adverse events, and requires the FDA to take appropriate action in response to those events.

Evaluation of the impact of PDUFA-funding, then, cannot be based on an assumption that approved drugs will never be withdrawn from the market because of problems detected in broad use. While such withdrawals should factor into the assessment, other considerations include the benefits of shortened review time, the comfort-level of staff with the process, and the any evidence that the shortened review time is compromising data review. Shortening the review time is not necessarily correlated directly with a poorer quality of review, the quality of the review should turn on the data developed and submitted.

As the percentage of funding for the drug and biological review processes from user fees increases, however, the risk for an undue focus on speed of review rather than quality of review increases. To diminish this risk, APhA recommends that a cap on the percentage of program costs covered by fees be considered seriously. Importantly, overall funding for these processes—from fees and appropriations—must be increased to keep pace with the expected expansion of new molecular entities and the emergence of pharmacogenomics. It is unacceptable that, according to the *Federal Register* announcement for this meeting, funding for a program as important as our drug review process was insufficient to keep pace with mandatory across-the-board pay increases.

Use of PDUFA Funds for Other Activities

The scope of activities funded by PDUFA fees is another important question, and APhA recommends that the scope be expanded to address two important activities: oversight of direct-to-consumer advertising and improved efforts in post-marketing surveillance.

Direct-to-Consumer Advertising

Oversight of direct-to-consumer (DTC) advertising activities should be added to the scope of PDUFA-funded activities. The prevalence of DTC-advertising is obvious to

any of us watching television or reading magazines, but the benefits of this expansion are not so readily observable. From my perspective, it is difficult to see the value created for the money being spent. Has the explosion of DTC advertising yielded an improvement in medication use, either through improved compliance or by stimulating consumers to seek medical care for untreated conditions? Or, by contrast, has the DTC explosion yielded an increase in the “casualness” with which our society perceives medication—that there is a tablet to treat everything, with no risks or adverse effects? FDA should work with the professions to develop a methodology for measuring whether adverse events or other problems are more frequent where prescribing has resulted from DTC advertising. An assessment of the impact of DTC advertising on medication use, including prescribing, patient compliance, etc. is essential—and should be a component of PDUFA-funded activities.

Post-Marketing Surveillance

APhA supports the expansion of PDUFA-funded activity to include enhancements in post-marketing surveillance. As I mentioned previously, an inherent component of our drug approval process is that some problems—and benefits—of products will not be discovered in the clinical trials. Medication use in “real life” is far different from the controlled environment of clinical trials, with concurrent use of other medications, OTC products and dietary supplements, as well as personal activities impacting whether medications “work”. Identifying the risks and benefits of medication use in “real life” will likely not benefit from a slower review time: only broad use of the medication will identify some problems.

This reality creates an opportunity for pharmacists and FDA to work together, focused on the profession’s goal to make medications “work”. There are two problems in the important function of post-marketing surveillance at the Agency. First, FDA does not receive a sufficient number of adverse drug reports; if we are to believe published reports regarding the amount of morbidity and mortality associated with drug use. The

Agency needs to work with prescribers and pharmacists, to promote swift reporting of all adverse events to FDA.

Second, current reporting is insufficient as a strategy to identify adverse effects and problems with appropriate prescribing and use of pharmaceuticals. FDA's current system for identifying unknown adverse effects of prescription drugs suffers from a lack of resources to analyze and respond to reports received by the Agency. Use of PDUFA funds to improve this activity is vital to maintain the integrity of our drug review system, a system that relies on surveillance to identify adverse effects of products in "real life". Pharmacists have demonstrated that their active participation in Phase IV studies produces valuable data about the safety and effectiveness of approved products. APhA would like to work with the Agency to use this promising mechanism more often when products are approved.

An additional component of post-marketing surveillance is a new system for higher-risk prescription pharmaceuticals. All of us are aware of the steadily mounting evidence of morbidity and mortality attributable to underuse and misuse of prescription pharmaceuticals. This evidence has spilled over from its historical confinement in the pages of medical journals to play out in the lay media. The media, with the public not far behind, are demanding more accountability of manufacturers, physicians, and pharmacists.

Part of the problem is the fact that economic pressures are pushing health professionals into spending less time with each patient. In addition, the now ubiquitous use of formularies puts prescribers in particular in a position of being pressed to approve the use of drug products with which they have less familiarity than the originally prescribed product. These marketplace trends make it difficult for prescribers and pharmacists alike to remain alert to the risks of every drug they prescribe and dispense. These factors contribute to the problems in identifying adverse effects of medications.

FDA could help this situation considerably by creating, in cooperation with pharmacists and other health care professionals, a standardized format for higher risk products identified as belonging to a category of drugs that demand special attention from clinicians and patients. This new risk management format could be analogous to the standards of the thalidomide use system, the System for Thalidomide Education and Prescribing Safety program (STEPS) oversight program to ensure only specifically-educated physicians and pharmacists used the product. Health professionals would know that a drug in the high-risk category bears special or unusual risks that require close monitoring—and a common system would allow pharmacists to build these services into their practices.

Let me clarify that APhA is **not** supporting the restricted distribution requirements imposed on some recently approved products. The Association has significant concern with arbitrarily restricting products to certain providers, as in the availability of Tiksoyn® in the ambulatory setting through only one pharmacy. Such requirements pose substantial risks for consumers, including problems with drug interaction checking, product availability, and interaction with their pharmacist of choice, and are vigorously opposed by the Association. APhA supports programs that limit medication access to those pharmacists and other providers willing to meet legitimate quality requirements—but in systems where all pharmacists may choose whether or not to participate. Additionally, APhA believes that the standards for determining when products are placed into this system should be developed by an open, public process—not the current system where it appears that manufacturers agree to limits on product distribution rather than having their product “held hostage” by the Agency.

Thank you for the opportunity to present the views of the nation’s pharmacists. Again, let me express our support for the PDUFA program, and underscore our recommendations for increased funding for review processes. However, managing the risk of the powerful technology we call medications is not simply a function of the approval process—the risk must be managed when consumers use these products in real

life. Pharmacists are essential to that management, and we look forward to continuing to work with the Agency, consumers and other health care professionals to make medications “work”. Thank you.