Remarks of Sharon Levine, M.D.
Board Member, RxHealthValue and
Associate Executive Director
The Permanente Medical Group
Kaiser Permanente Health Care Program

On Behalf of

RxHealthValue

Public Meeting on the Prescription Drug User Fee Act Reauthorization

December 7, 2001

Thank you for convening this public meeting and inviting stakeholders once again to provide input to the Food and Drug Administration in advance of reauthorization of the Prescription Drug User Fee Act.

My name is Dr. Sharon Levine. I am here today as a Member of the Board of Directors of RxHealthValue, an organization of consumers, health care practitioners, health plans, and employers. The members of RxHealthValue have come together to sponsor research, educate the public, and recommend private- and public-sector solutions to assure that the public realizes the health and economic value of prescription drugs. A recent New York Times article about RxHealthValue reported that the coalition's combined members represent approximately 135 million Americans' interests in assessing and securing value for the resources they expend on prescription drugs – through taxes, deferred wages, private and public health insurance, or direct purchase.

I am also here as a pediatrician and Associate Executive Director of The Permanente Medical Group, Inc. In this role, I oversee the participation of 3,500 Permanente physicians in the Kaiser Permanente pharmacy program, which serves more than three million Northern Californians. My RxHealthValue colleagues and I are very concerned that without adequate

funding in the future, FDA will not be able to fulfill its most important public health duties -- to assure that prescription drugs used to treat patients do what they are designed to do with minimal risk. This means that the research on prescription drugs must meet the highest standards and that manufacturing conditions ensure high-quality medicines. Moreover, given the FDA's role in conducting post-market surveillance and regulating the marketing of prescription drugs, it is essential that the agency have sufficient resources to oversee these expanding activities. Put simply, the rapidly evolving and growing need to assure patient safety and drug availability is clearly outstripping available funding.

RxHealthValue believes that the vital public health functions performed by FDA are of value to every American, and will increase in importance as pharmaceuticals continue to take a more prominent position in health care. Just last week, for the first time in many years, the Congress passed and the President signed legislation that provides the agency with a budget that includes more than was requested by the Administration for salaries and related costs. We heartily applaud the Congress and the President for taking this important first step to ensuring the FDA's critical public health mission is adequately funded. We urge the Administration in its budget proposal for Fiscal Year 2003 to propose an even larger increase, one that would put the Agency on a path, like the one followed by the National Institutes of Health in the 1990s, to a budget that doubles between now and the end of the decade. Doubling the Agency's funding over seven years is essential if it going to be able to fulfill its critical – and too often underappreciated – public health responsibilities.

If a doubling of FDA appropriations were to occur, it is possible that FDA might have sufficient resources to fulfill its duties without user fees, which is RxHealthValue's preference. We recognize, however, that the environment is such that it is unlikely that such an increase will

be proposed and even if it were, Congress is not likely to enact the new taxes needed to meet this goal. PDUFA financing thus seems to be a fact of life, at least for the immediate future. However, it is critical that the distribution of agency efforts not be distorted by this funding. We are concerned that the goals established under PDUFA have forced the FDA to redirect resources from other vital functions to review of new drug applications. It is not that we think drug review activities are less important, rather that PDUFA may have slanted the allocation of resources within the Agency.

Within the area of prescription drug review, there is significant room for improvement. As currently structured, PDUFA only allows user fees to support the review of new drug applications. Yet the agency, responding to manufacturers, has devoted increasingly significant resources to consulting with manufacturers during the discovery and development phases so that new drug applications meet all requirements. Manufacturers, in effect, are depending on the FDA as if it were a consulting firm. One can imagine the cost to manufacturers of paying private consultants for the same advice that is increasingly being provided free by FDA. We would recommend that FDA develop a distinct capacity to provide technical assistance to manufacturers during pre-application phases. Those manufacturers that call on the FDA for assistance beyond that provided as part of the normal regulatory process should be expected reimburse the Agency for the assistance provided.

Similarly, it is important for FDA to have adequate technical expertise to review rapidly developing new technologies used to develop new drugs. Historically, the FDA has maintained a scientific program to ensure that physicians, pharmacists and other staff involved in reviewing new drug applications have the technical support required to respond to new scientific

developments in the private sector. If appropriated funds are not sufficient, financing this kind of activity out of user fees may make some sense.

Due to funding constraints, the peculiarities inherent in a system partially supported by user-fees, and explosive utilization of recently approved drugs we believe that FDA has been unable to meet fully its increased post-drug-approval regulatory responsibilities. The speed with which many new drugs are adopted in the marketplace is breathtaking. That more and more patients are more and more quickly exposed to more and more drugs with which providers have less and less experience is a key lesson of early research sponsored by RxHealthValue.[1]

Driven by the demands of the Prescription Drug User Fee Act, FDA now acts on new drug applications with great speed under considerable pressure. This can result in inadequate clinical experience with new drugs before they are marketed. Driven by massive promotional efforts, in particular the direct-to-consumer advertising that has become ubiquitous since the loosening of promotional restrictions in 1997, uptake of those drugs is greatly accelerated over the past.

This one-two combination – faster approvals with less clinical information and more rapid market uptake – means that to maintain the same level of public safety as in years past, *more resources, not fewer* must go toward the increasingly important FDA responsibilities of marketing review and surveillance of post-marketing experiences. Under current law, user fees may not be used for these purposes. While user fees float with volume, appropriations do not. Congressional appropriations have been inadequate to finance the scope and depth of activities necessary to protect the public health and oversee the activities of this rapidly growing and increasingly important component of our health care system. And, like the review of new drug

applications, increased vigilance regarding the safety of drugs and the appropriateness of proposed marketing materials are core public health functions of the Agency.

As a result, and responding to RxHealthValue's core mission to ensure that Americans have affordable access to health-improving medications, our coalition members have adopted a consensus recommendation to FDA regarding improvement of post-marketing surveillance of approved drugs. The members of RxHealthValue believe that the importance and urgency of funding improved post-marketing surveillance through a swifter, easier system for direct, voluntary reporting by physicians justify additional funding for these activities. While many members of RxHealthValue feel strongly that improved post-marketing surveillance should be funded through additional appropriations, additional user fees could be established to finance these activities as a part of the reauthorization of PDUFA. If user fees must be the source of funding for post-marketing surveillance, many RxHealthValue members support a new user fee to finance FDA's review and certification of print and broadcast advertising.

I would like to take a few minutes to respond to the specific questions directed to this panel:

> *How can FDA ensure that PDUFA goals are met if there continues to be a funding shortfall? If the funding shortfall persists, should FDA, in order to best protect and promote the public health, set review priorities and, if so, how? Should there be flexibility in setting user fees to cover the increased cost of the program?*

These questions go to some of the basic issues that RxHealthValue was formed to address. As I suggested above, funding for the broad range of FDA activities needs to be balanced. As currently structured, PDUFA has prevented FDA from balancing competing demands, thereby tilting Agency activities toward reviewing new drug applications and thus funneling more-and-more of the Agency's limited resources to this one important activity. Efforts should be made in

---

[1] Sources of Growth In Pharmaceutical Expenditures, Brandeis University Schneider Institute for Health Policy, Prescription Drug Analysis Group and PCS Health Systems, Inc., Stanley S. Wallack, Ph.D. and Cindy Parks

the reauthorization of PDUFA to provide the Administration with the authority and flexibility needed to balance the competing demands for resources and funding.

Responding to a funding shortfall is never simple. The notion of review priorities where some group or individual determines that certain new drugs have potentially greater health value than others is appealing. Yet, it seems that making those determinations could require the Wisdom of Solomon. I would urge the FDA if it pursues this path to involve at every level of consideration groups representing patients, providers, health plans and purchasers in this important discussion.

We would suggest that the Agency attempt to make any necessary, difficult prioritization decisions with the question of health value in mind. That is, applications for drugs to treat now ineffectively treated life-threatening or seriously debilitating conditions should be viewed as the highest priority items. In contrast, so-called "line extensions" intended to bolster a manufacturer's market share in an environment of changing conditions due to loss of patent and market exclusivity – such as active metabolite products (e.g., esomeprazole), combinations of generic products (e.g., metformin/glyburide), extended release products (e.g., metformin) and identical new forms of existing products (e.g., sotalol) should be viewed as very low priority items. New drugs in existing therapeutic classes may or may not provide important clinical advances, so it is difficult to make blanket statements. Similarly, new therapies to treat less than life-threatening or seriously debilitating conditions may provide important benefits and it is difficult to make any conclusions in the hypothetical. This is why continuing input from stakeholder groups is essential if priorities need to be established. An advisory group of stakeholder representatives might be a good first step, if traveling down this road is necessary.

I would like to make one final comment. While more germane to FDA's overall mission than to PDUFA, I think it is important that policy makers recognize that FDA's mission departs from what many patients and health care practitioners think it is or should be. Let me explain. Patients and providers think that FDA either is working not just to determine whether a new drug is safe and effective, but whether it is better – that is more effective or safer – than drugs the Agency has already approved. As the Administration develops a PDUFA proposal to submit to Congress next year, I would urge it to consider seeking a broader mandate from Congress for the Agency, a mandate that would require FDA to make determinations about the relative effectiveness of drugs intended to treat the same condition. This may require more resources for the agency, and more information from manufacturers, but it would represent a significant step forward in ensuring that we gain greater value for our limited health care resources.

While FDA is faced with difficult problems, I should say that I am hopeful for the future. The problems I have addressed are not lost on policy makers. As GAO has pointed out, "[t]he focus on reducing new product review times has slowly shifted resources away from other activities amid a general increase in the use of medical products by the American public."[2] Nevertheless, we think this is a theme that bears repeating to policymakers in Congress and the Administration – and it is important that the Agency also deliver this message. The FDA has many friends in the health care community – and we hope to help assure that FDA has the resources it needs to carry out its myriad and critical responsibilities.

On behalf of the members of RxHealthValue and the many Americans we represent, I want to thank FDA again for convening this meeting. The FDA should be commended for its

[2] General Accounting Office, Major Management Challenges and Program Risks, Department of Health and Human Services, January 2001 (Rept. GAO-01-247).

exhaustive self-examination on funding questions, its laudable openness in seeking out the views and concerns of interested parties beyond the regulated industry and its strong commitment to the public health. Thank you.