



National Health Council

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**Testimony before the
House Energy and Commerce Subcommittee on Health**

PDUFA V: Medical Innovation, Jobs, and Patients

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SUMMARY

The Prescription Drug User Fee Act (PDUFA) has facilitated significant improvements in the drug development process. However, more must be done to encourage the development of new treatments and to ensure that patients with chronic diseases and disabilities have access to these drugs in a timely manner. To meet this need, Congress should

1. Develop as part of PDUFA V a framework for benefit-risk assessment of new drugs that incorporates input from stakeholders, including patients.
 - Historically, benefit-risk assessments have taken the perspective of the greater public good. However, individual patients make judgments based on their own preferences and circumstances.
 - The patient community asks for the development of a qualitative framework for benefit-risk assessment of new drugs that incorporates input from patients and that takes into consideration the size of the population affected, the range of existing treatment alternatives available to those patients, and the risks of living with that specific condition.

2. Reevaluate conflict of interest policies related to the selection of panelists for FDA advisory committees.
 - The conflict of interest regulations play an important role in ensuring transparency and credibility in the FDA approval process. However, they should not delay access to safe and effective medicines, especially if the product is designed to treat a significant unmet need.
 - Conflict of interest restrictions should be reevaluated to ensure the most qualified experts are not precluded from participating in advisory committees.

INTRODUCTION

Good morning Chairman Pitts, Ranking Member Pallone, and distinguished members of the Subcommittee. I am Marc Boutin, Executive Vice President and Chief Operating Officer of the National Health Council (NHC). Today, I speak on behalf of the more than 133 million people living with chronic diseases and disabilities. We appreciate this opportunity to present our views on the reauthorization of the Prescription Drug User Fee Act (PDUFA).

The National Health Council is an umbrella organization of patient advocacy organizations and provides a united voice for people with chronic conditions and their family caregivers. Our core membership includes 50 of the nation's leading patient advocacy organizations, ranging in size from large groups such as the American Cancer Society and the American Heart Association, to smaller organizations such as the Alpha-1 Foundation and the Sjögren's Syndrome Foundation. Other members include professional and membership associations, nonprofit organizations with an interest in health, and major pharmaceutical, medical device, health insurance, and biotechnology companies. Our governance is controlled by the patient advocacy organizations. We provide a place where all stakeholders meet for meaningful and reasoned dialogue.

The NHC represents patients and not consumers. I want to make that distinction because while patients and consumers are part of the same stakeholder group and share many common concerns, we are at opposite ends of the same spectrum. People with chronic diseases and disabilities use the health care system to manage their daily lives. They use the health care system on a continuing basis to stay alive. Consumers are people who use the health care system

largely on an ad hoc basis, so their perception or focus on health care issues and access to new treatments is often very different.

Since its enactment in 1992, PDUFA has facilitated significant improvements in the drug development process, accelerated the delivery of life-altering treatments to patients by reducing review times for new drug applications, and improved patient safety.

Despite this progress, more must be done to encourage the development of new treatments and to ensure that patients have access to these drugs in a timely manner. We need to place the discussion of PDUFA reauthorization into the context of meeting the needs of people with complex health issues; those that are most affected by the availability of new treatments.

So, today, I would like to speak to you about two issues related to PDUFA V:

- 1) Developing a framework for benefit-risk assessment of new drugs that incorporates input from stakeholders, including patients, and
- 2) Reevaluating conflict of interest policies related to the selection of panelists for the Food and Drug Administration (FDA) advisory committees.

BENEFIT-RISK ASSESSMENT OF NEW DRUGS

The NHC strongly supports the work of the FDA to bring safe, effective, high-quality treatments to patients. To fulfill its mission to promote and protect public health, the FDA is responsible for weighing the potential risks of drugs against their benefits. Historically, these benefit-risk assessments are conducted from a population based perspective. But as you know, individual patients make judgments based on their own preferences and circumstances.

Marc Stecker of New York is such an example. Seven years ago, he noticed he was limping. Not long after that, he was diagnosed with multiple sclerosis. Today, he is confined to a

wheelchair. “Because my disease is so aggressive,” Stecker said, “I have been very willing to be equally aggressive in trying to combat it.”¹

A patient with a life-threatening disease with limited treatment options is likely to have a much higher tolerance for “riskier” drugs than health care consumers who are not using medicines to manage their daily lives.

Even among patients with the same disease, risk tolerance may vary based on individual values. A mother in her 40s diagnosed with breast cancer may seek treatments that will help her stay alive to see her children grow up; however, a woman in her mid-80s with the same diagnosis may focus more on the quality of her days ahead, rather than longevity.

While the FDA is committed to serving the greater public good, the agency must also address the individual needs of the entire spectrum – from consumers to patients. Just as we know that different people respond differently to the same medications, so, too, we need to recognize that patients and consumers can have vastly different perceptions of benefits and risks.

In PDUFA V, the patient community asks for the development of a qualitative framework for benefit-risk assessment of new drugs that incorporates input from stakeholders, including both patients and consumers. The NHC recommends developing and implementing a plan to integrate a benefit-risk framework in the drug review process that places the drug’s intended recipient into the equation. Such a framework would be conducted with robust input from all the relevant stakeholders such as patients and health care consumers. Such a framework would increase credibility and, perhaps more importantly, provide context to the review process.

¹ Kroen G, Doctor challenges cause of MS and treatment, National Public Radio, 2011. Available at: www.npr.org/2011/01/31/133247319/doctor-challengescause-of-ms-and-treatment

When evaluating the risk of drugs, the FDA must take into consideration the size of the population affected, the range of existing treatment alternatives available to those patients, and the risks of living with that specific condition. To ensure this perspective is incorporated in decision making, the NHC believes it is imperative that patients are consulted. For patients with a rare or incurable condition, especially those with few or no treatment options, restricting access to a new drug is potentially devastating.

For example, patients with systemic lupus erythematosus (SLE) rely on corticosteroids to alleviate flares. Despite significant side effects, such as osteoporosis, hepatotoxicity, glaucoma, artery damage, weight gain, and serious skin irritation, corticosteroids remain an essential component of the treatment regimen for a patient with lupus because their use can substantially reduce the symptoms associated with inflammation. That is, many patients with SLE accept risks of major negative side effects because the alleviation of symptoms and prevention of flare ups outweigh these other risks.

Far too often adverse events are framed from the point of view of someone without a disease or disability or someone who has never relied upon medication to improve or extend their life. Creating a better system to balance the benefits and risks of new drugs is a complex task. But, the engagement of people with chronic conditions and their representatives will be paramount to making it successful.

To relegate patients to a silent or hidden status in the assessment of new drugs limits the agency from receiving valuable input from those most affected. Their perspective and best judgment should be fully considered and valued. Benefit-risk assessments of a drug may look considerably different when taking in the perspective of a patient with a debilitating chronic

condition – a patient who is willing to take a risk on a medication for a chance at a more healthy and functional life.

CONFLICT OF INTEREST POLICIES

Equally important as acknowledging the differences in the benefit-risk analysis of drugs by the intended user, we must also ensure that the conflict of interest rules are not so strident as to delay the review and approval of new drugs for people with few or no treatment options.

The conflict of interest regulations play an important role in ensuring transparency and credibility in the FDA approval process. However, they should not delay access to safe and effective medicines, especially if the product is designed to treat a significant unmet need. The conflict of interest rules and their application must reflect this much needed balance.

FDA advisory committees provide expert advice that is critical to enabling the FDA to fulfill its mission. The NHC appreciates the need for conflict of interest screening to maintain public trust in the role of advisory committees in providing independent advice to the FDA. However, we are concerned that efforts to maintain the public's trust may now be superseding the need to secure necessary expertise to the detriment of the advisory committee process as a whole.

The NHC is particularly concerned that restrictions currently placed on the agency are creating challenges in convening advisory committees on highly specialized topics, such as rare disorders. On these topics, very few experts exist.

When we look at the unmet needs of targeted and smaller patient populations, there are fewer individuals with the relevant expertise and experience. The FDA is hard pressed at times to identify experts with the appropriate training and background to provide the agency with

informed advice, as experts in these specialized fields are likely to receive research grants for their studies, including grants from pharmaceutical and biotechnology companies, or serve as consultants to industry.

The FDA's conflict of interest policy appears to be in need of re-assessment when considering that nearly one quarter of the more than 600 seats on the FDA's 49 advisory committees remain vacant. According to data published by the FDA², the rate of vacancies on FDA advisory committees was 23 percent as of March 31. For example, at the Center for Drug Evaluation and Research (CDER), vacancies on committees were 24 percent in March. At the Center for Biologics Evaluation and Research (CBER), the vacancy rate was 38 percent. And at the Center for Devices and Radiological Health (CDRH), vacancies were 18 percent. These vacancy rates are alarming in light of the significant responsibility placed on advisory committees to inform FDA decision making. While we understand that there are numerous factors leading to these vacancy rates, it is clear that conflict of interest disqualifications strongly contribute to them.

We are deeply concerned that the challenges in identifying experts for advisory committees are leading to delays in patient access to new treatments. In fact, during a PDUFA V stakeholder meeting held on November 17, 2010, the FDA noted that there have been cases in which late recusals from an advisory committee due to a conflict of interest have led to a meeting cancellation and a delay in the FDA's approval of the application.³ In a communication to individuals inquiring about the FDA's review of a new drug application, the FDA wrote

² <http://www.fda.gov/AboutFDA/Transparency/track/ucm216403.htm>. Accessed on June 30, 2011.

³ <http://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM235777.pdf>. Accessed on June 30, 2011.

“Although FDA strives to have broad representation of appropriate medical and scientific specialties on its advisory committees, optimal representation is often difficult to achieve given the strict conflict-of-interest regulations that apply ...”⁴

We fully support disclosure requirements so that conflicts of interest are transparent. However, conflict of interest rules should not be so strict as to preclude the most qualified experts from serving on advisory committees. Such preclusions can cause delays or cancellations of committee meetings. Patients are anxiously awaiting new treatments to manage, prevent, or delay their disease or disability. We urge you to consider how overly stringent conflict of interest rules may be doing more harm than good.

CONCLUSION

The reality is we are still not getting new treatments to market as quickly as the patient community would like or need. By working together with the FDA and all stakeholders we can do better in getting safe and effective treatments through the regulatory process if we take into consideration the perspective of the intended user – whether it be a person with a chronic condition or an average consumer.

We need a system that is flexible enough to meet the specific needs of the people who will ultimately use the medicine.

To conclude, on behalf of the National Health Council, the patient advocacy community, and the people living with chronic conditions that we represent, we recommend that Congress enact PDUFA V reauthorization legislation which includes the development of a framework for

⁴ <http://blogs.forbes.com/matthewherper/2010/10/22/fda-responds-to-outraged-arena-investors/>

benefit-risk assessment that incorporates meaningful patient input. In addition, the National Health Council also calls on Congress and the FDA to reevaluate conflict of interest policies as they relate to the FDA advisory committees to ensure they do not impede the delivery of new, safe, and effective treatments to patients.

Thank you again for this opportunity to appear before this subcommittee. I look forward to your questions.