



National Health Council

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STATEMENT FROM THE NATIONAL HEALTH COUNCIL ON THE REAUTHORIZATION OF THE PRESCRIPTION DRUG USER FEE ACT (PDUFA) APRIL 12, 2010

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On behalf of the National Health Council, thank you for this opportunity to present our views on the Prescription Drug User Fee Act (PDUFA) as the Food and Drug Administration prepares for the reauthorization of the program.

The National Health Council is an umbrella organization of patient advocacy organizations and provides a united voice for the more than 133 million people with chronic diseases and disabilities 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.

Our governance is controlled by the patient advocacy organizations. But we seek to provide a place where all stakeholders can come and have meaningful and reasoned dialog. Other members include professional and membership associations, nonprofit organizations with an interest in health, and major pharmaceutical, medical device, and biotechnology companies.

The National Health Council represents patients and not consumers. I want to make that distinction because while we have a lot of concerns in common, our perspectives will appear to be almost opposite. This is because our focus is entirely different from consumers. 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 – their focus – on health care issues is often very different.

The National Health Council has been involved with the reauthorization of PDUFA because we, like consumer organizations, are obviously concerned about the safety and efficacy of new treatments. However, we are also concerned about the length of time it takes to bring new treatments to people with chronic conditions – especially for individuals who have no viable treatments or whose treatments are simply not sufficient to sustain them over the long term.

I want to remind this audience that it was the patient community that stood up and said “Enough is enough. We need to do better.” You only have to look to the 1980s and the HIV and AIDS crisis, when the patient community said, “This is not working. You need to speed this up. We’re

**STATEMENT FROM THE NATIONAL HEALTH COUNCIL
ON THE REAUTHORIZATION OF PDUFA
APRIL 12, 2010
Page 2 of 4**

dying, we need treatments, and we need them now.” And as a result, you saw dramatic changes in the regulatory process for new drugs.

At the time, people diagnosed with HIV and AIDS had one treatment that was very hard to find. Today, there are now multiple treatments. And just recently, a new drug came on the market that works in a very different, unique way. If people with HIV or AIDS have a certain biomarker they can receive a drug that has had amazing results with almost no side effects.

We have also seen some treatment advances in the cancer community and for those with heart disease. But the reality is we are still not getting new treatments to market as quickly as the patient advocacy community would like.

The National Health Council has been and continues to be supportive of PDUFA. We have seen great results under PDUFA I, II, and III. The program created the resources that the FDA has effectively used to improve the process for bringing new products to market while still meeting safety and efficacy standards. Our challenge is we’re not seeing the same efficiencies under PDUFA-IV.

That leads the National Health Council to make two primary recommendations for PDUFA-V to better serve the patient community.

The first recommendation is the FDA needs to have patient involvement at all levels throughout the FDA, and especially as full partners in the discussion of changes to PDUFA-V.

Patients are their own experts. One cannot underestimate the knowledge that informed patients have about their conditions. If you have a rare disorder, you probably know more about your condition than anyone else. There’s no one better informed than a patient who has been diagnosed with a serious, life-threatening disease or disability.

Perhaps even more importantly, patients provide credibility to the process. Right now this country is in an age of transparency, and the public wants everything out in the open. The reality is the FDA needs patient involvement to ensure credibility of the outcomes. People who take medications on a daily basis understand there is a risk associated with new drugs, and they are willing to take that risk at the shot of a more normal, productive, and happy life. The patient advocacy community can give that credibility to the process when the agency has to make the tough decisions about the balance between benefit and risk.

Too often the decisions about the benefit and risk of new medications are made by people who are healthy and are not dependent on drugs on a daily basis. The National Health Council urges

**STATEMENT FROM THE NATIONAL HEALTH COUNCIL
ON THE REAUTHORIZATION OF PDUFA
APRIL 12, 2010
Page 3 of 4**

the FDA to include people with chronic conditions – the patient community – in the decision making process, including the development of risk evaluation mitigation strategies (REMS).

- **Any data collection that is required as part of a REMS strategy or any other mechanism, needs to account for the benefits, as well as the risks, to patients.** It is only when you combine the two and frame them in the context of the condition or conditions being treated that you can make any effective judgment about benefit and risk.
- **We call for the Government Accountability Office to conduct an analysis of the impact of safety considerations, including the REMS process, on patient access to new drugs.** The National Health Council is not saying we don't want additional measures for patient safety and efficacy. But such measures should not be impeding the access to drugs. Remember so many people with chronic conditions are living and dying as a result of their condition, while waiting for a treatment.

The patient advocacy community was part of the last PDUFA reauthorization to change the language in the REMS strategy. Specifically, we wanted the safety and efficacy mechanisms to be in place; but we wanted them to be used as a tool in post-market surveillance to ensure safety, not to figure out every single risk in the clinical trial process.

The National Health Council's second overarching recommendation is PDUFA-V needs to encourage the development of innovative trial designs.

There are some who will say PDUFA is a user fee agreement, and that it is simply for the approval of the drug. And I would say to you, "No, it is not."

The patient community came out and demanded change for the approval process of new HIV and certain cancer treatments. We supported PDUFA because it streamlined the process and made it more efficient, and we got products to market more quickly.

The reality is many of the efficiencies under PDUFA have been accomplished because PDUFA has resulted in more FDA staff and resources. Our next opportunity is to figure out how to develop clinical trials that streamline the approval process for new medications that target the specific population that a particular drug is designed to reach.

**STATEMENT FROM THE NATIONAL HEALTH COUNCIL
ON THE REAUTHORIZATION OF PDUFA
APRIL 12, 2010
Page 4 of 4**

For the first time in my professional advocacy career, Japan and, to some extent, Europe now outpace the United States in this space. That makes no sense to me as a patient advocate.

- **We need new methodologies for conducting non-inferiority trials.**
- **We need trial protocols for specific therapeutic areas.**
- **And perhaps most important from the patient perspective, we need biomarker and surrogate endpoint qualification.** We need to figure out how to define the populations we are going to provide treatments to, to make the drugs safe and efficacious.

I would like to close by saying just this: You need to understand that the patient community is getting really frustrated, frustrated in a way I haven't seen in a long time.

The patient advocacy community worked hard to double the funding for the National Institutes of Health. It was where we cut our teeth in advocacy. We expected a commensurate gain in the development in new treatments, and over the last decade we've more than doubled the public and private investment in new treatments. And yet the realization has been that we're getting fewer new patient treatments each year.

The patient advocacy community is getting angry. It's become clear to us that the challenges are at the *back end* of the development process – the regulatory approval end. We need to do better in getting new treatments through the regulatory process.

The AIDS epidemic proved that we can do this. So why should somebody with lupus, or somebody with ALS – Lou Gehrig's disease – or Alzheimer's, or Parkinson's, or lung cancer – why are they not getting the treatments that they need? Why are they still dying?

So on behalf of the National Health Council, I urge you again to

- **Ensure that patient participation is there throughout the process to help you adopt a more balanced benefit/risk assessment.** We will help the FDA make those tough decisions. We understand the risk of our conditions.
- **And second, it's time to improve the regulatory science that speeds the delivery of new, safe, and effective treatments by seeking out and utilizing innovative trial designs in the regulatory approval process.**

Thank you for your time.