



National Health Council

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October 21, 2010

Via e-mail: PDUFAReauthorization@fda.hhs.gov

Re: Potential PDUFA Enhancement Proposals

On behalf of the member organizations of the National Health Council (NHC), I welcome the opportunity to comment on your proposals for potential enhancements to PDUFA. It is clear from your presentation at the stakeholder meeting on September 29 that you have carefully listened to the various stakeholder groups to form your potential enhancements. We thank you for taking into account the concerns of the patient community, and we appreciate the opportunity to further comment on the proposed enhancements and recommend which options are most important to people with chronic diseases and disabilities.

The NHC is the only organization of its kind that brings together all segments of the health care community to provide a united voice for the more than 133 million people with chronic diseases and disabilities and their family caregivers. Made up of more than 100 national health-related organizations and businesses, its core membership includes approximately 50 of the nation's leading patient advocacy groups, which control its governance. Other members include professional societies and membership associations, nonprofit organizations with an interest in health, and major pharmaceutical, medical device, biotechnology, and insurance companies.

As you can imagine, the NHC greatly appreciates *Proposal #3: Patient-Focused Drug Development*. In your presentation you articulated that "informed patients are often their own experts on their disease." We could not agree more. We appreciate your idea to hold meetings involving members of the patient advocacy community to seek input, and we hope that this process is included in benefit/risk calculations. As we have mentioned throughout the debate on policy options for PDUFA V, the amount of risk that a patient is willing to take is greatly dependent on various factors including the severity of the condition and the amount of viable treatment options. Because of this variation, we cannot think of a more important area where input from patients should be sought.

The NHC also appreciates *Proposal #12: Advancing Biomarkers and Pharmacogenomics*. We feel that this proposal could accelerate the delivery of targeted therapies to patients. We see enhancing regulatory science at FDA as one of the most crucial ways to speed the approval process of promising treatments while still adequately determining efficacy and safety. We have advocated for an adaptive system in which biomarkers would be used to target new compounds to specific patient populations. When drug candidates demonstrate the potential to address an unmet need, conditional approval could be granted, which would allow for patients to access promising drugs early. Safety and efficacy outcomes would be closely monitored and would inform whether the drug should be restricted, withdrawn, or

NHC Statement on Potential PDUFA Enhancement Proposals

October 21, 2010

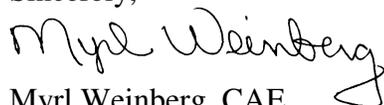
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fully approved. Such a system would facilitate the collaboration of regulators, drug manufacturers, research investigators, and patients, which would promote the generation of more robust data throughout the drug development process. We encourage the FDA to consider going further in this area by evaluating the role that biomarkers can play in facilitating new frameworks for approval, such as the adaptive system we describe above.

The NHC appreciates the opportunity to comment on your proposed options, and we are eager to continue to work with the FDA as you consider enhancements to PDUFA.

Please do not hesitate to contact Kevin Cain, our Assistant Vice President of Government Affairs, if you would like to discuss these issues in greater detail. He is reachable by phone at 202-973-0542 or by e-mail at kcain@nhcouncil.org. You may also reach me on my direct, private line at 202-973-0546 or via e-mail at mweinberg@nhcouncil.org.

Sincerely,

A handwritten signature in black ink that reads "Myrl Weinberg". The signature is written in a cursive style with a large, stylized "W" and a long, sweeping underline.

Myrl Weinberg, CAE
President