National Health Council
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September 27, 2010

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

Re: PDUFA Stakeholder Meeting

The National Health Council (NHC) appreciates the opportunity to submit proposals for enhancements to the Prescription Drug User Fee Act (PDUFA).

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 we have shared in previous stakeholder meetings convened by the FDA, the NHC has recommended that the next PDUFA should:

- **Encourage a balanced benefit/risk assessment process by increasing the opportunities for meaningful patient input:** The NHC recognizes the challenges associated with making judgments on whether a drug's benefits outweigh its risks. Recently, discussions have focused on whether systematic guidelines for assessing the benefit/risk of new drugs should be developed. As FDA considers whether to develop such a framework, the FDA should seek to ensure the perspective of patient community is well-represented in any process regarding its development. Certainly, tolerance of risk can vary across conditions, depending on factors such as the magnitude of benefit, the severity and prevalence of the condition being treated, and the availability of treatment alternatives. But an important perspective that is often lacking in discussions regarding safety is the varying tolerance and perception of risk among patients and consumers. Any benefit/risk assessment must be mindful that the acceptability of risk may differ for a patient living with a chronic disease or disability than a health care consumer. We believe the patient perspective is vital to conversations regarding drug safety and implore the FDA to ensure patient perspectives are incorporated in the development of processes related to drug safety, including the creation of a systematic approach for assessing drug risks and benefits and Risk Evaluation and Mitigation Strategies (REMS).

- **Advance regulatory science to accelerate the delivery of new, safe, and effective treatments to market**: People with chronic diseases and disabilities depend on modern drug therapies to treat their conditions. To fulfill its objective to accelerate the delivery of new treatments, the FDA should evaluate enhancements to its approval process. The NHC believes the FDA can play a vital role in addressing concerns that significant investments in basic research are not yielding a commensurate level of new treatments. We encourage the FDA to consider new frameworks for approval, such as an adaptive system that would allow for early access to promising drugs for unmet needs.[1] Under an adaptive system, 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 fully approved. Such a system would facilitate the collaboration of regulators, drug manufacturers, research investigators, and patients, which would promote more robust learnings throughout the drug development process.

The NHC appreciates the opportunity to provide our comments and to share the perspective of the patient community. We are eager to continue to work with FDA as it considers enhancements to PDUFA.

Please do not hesitate to contact Kevin Cain, our assistant Vice President of Government Affairs, if you or your staff 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,

Myrl Weinberg

Myrl Weinberg, CAE
President

[1] BioCentury, "Regulatory Innovation." Week of September 13, 2010. Volume 18. Number 39.