National Health Council

1730 M Street NW, Suite 500, Washington, DC 20036-4561 ■ 202-785-3910 ■ www.nationalhealthcouncil.org ■ info@nhcouncil.org

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December 4, 2014

The Honorable Margaret A. Hamburg, MD
Commissioner
Food and Drug Administration
5630 Fishers Lane
Room 1061
Rockville, MD 20852

Subject: Food and Drug Administration Activities for Patient Participation in Medical Product Discussions; Establishment of a Public Docket (FDA-2014-N-1698-0001)

Dear Commissioner Hamburg:

The National Health Council (NHC) is pleased to submit comments to the Food and Drug Administration (FDA) regarding strategies to solicit the views of patients during the medical product development process and integrate the perspectives of patients during regulatory decision-making. We appreciate the opportunity to provide input and recommendations to FDA on this important topic and applaud the agency's wide-ranging efforts to transform the regulatory review process into one that incorporates the patient perspective to a greater extent.

The NHC is the only organization that brings together all segments of the health 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 the nation's leading patient advocacy organizations, which control its governance.

We firmly believe that patients are best equipped to provide information on how diseases impact daily life, report on experiences with available therapies, identify expectations for desired outcomes, and outline thresholds for risk. We therefore support meaningful and purposeful engagement of patients in all stages of the development and regulation of medical products, ranging from the early stages of product development to regulatory approval and post-market activities.

FDA has made great strides in implementing programs and initiatives across the agency that seek to collect patient perspectives and experiences to inform product reviews. For example, the agency's Patient-Focused Drug Development (PFDD) program has been especially successful in creating a forum in which patients, caregivers, and patient representatives can share their views and be included in FDA's processes. In addition to capturing the

perspective of patients in the product review process, FDA has succeeded in raising awareness on the importance of patient input in the research and regulatory settings.

While we commend FDA for its progress, we urge the agency to continue to explore ways to enhance the usefulness of these programs to both the agency and the patient community. FDA should critically evaluate its engagement approaches, assess whether there are ways in which patients can be engaged more effectively, and share these learnings with the public.

As the FDA looks to utilize patient information to supplement its decision-making process, it is important that the agency collect the specific types of information it needs through these engagements. Therefore, NHC encourages FDA to offer more detailed guidance on the specific types of patient information that would be useful to the agency in its product reviews. Increasingly, patients, caregivers, and advocates are faced with the challenge of ensuring that they are providing the type of information that will be useful to researchers, product developers, and regulators. Furthermore, in open forums with limited time, such as PFDD meetings, it is critical to be able to deliver this information in an efficient and effective way.

To help address this particular challenge, NHC created a Patient Information Tool to help patient organizations systematically organize issues around key patient populations within their disease area and identify key topics to focus on in PFDD meetings.[1] The aim of the tool is to ensure patient organizations' communications with the agency are comprehensive and, conversely, to help FDA capture the information it needs from patients, caregivers, and patient advocates to inform assessments of benefit-risk for new products.

Similarly, FDA should also increase transparency regarding how patient information is being reviewed internally and incorporated into the agency's decision-making process. Current outputs, such as the "Voice of the Patient" reports from PFDD meetings, robustly capture the discussion and information collected from the engagements; however, how and to what extent this information is being used by FDA is unclear. Additionally, there is a lack of clarity as to how these reports will be updated in the future as new information about the diseases becomes available or if treatment regimens change. Added transparency about how patient perspectives are integrated into these regulatory decisions will help patients better understand how their information is being used and assist them in optimizing their engagement with the agency. Furthermore, being able to see the direct impact of their contributions will help patients feel meaningfully engaged.

Additionally, we believe FDA should increase available opportunities for engagement with the agency outside of formal public meetings (e.g., PFDD). Specifically, we recommend that FDA continue to have regular consultation meetings with the patient community to discuss patient engagement strategies, provide updates, and receive general feedback. Additionally, several different paths currently exist for patient organizations to set up meetings with FDA (e.g., through the Office of Health Constituents, the product review divisions). This leads to confusion about how to engage with FDA and we believe patient organizations, especially those without a current relationship with the agency, would benefit from guidance from FDA and the creation of a single, clear process to initiate a conversation with the agency. This accessibility is crucial to

[1] National Health Council's Patient Information Tool.
http://www.nationalhealthcouncil.org/NHC_Files/Pdf_Files/NHCPatientInformationToolandinstructions.pdf

ensuring a patient-focused regulatory process and will signal equal opportunity for engagement to those in the patient community.

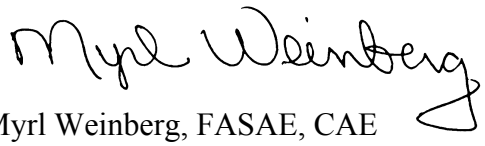
Finally, we believe that FDA can capitalize on its patient engagement work by helping foster patient engagement in the research and development process. Although there is great interest in engaging patients in research and development, patients, caregivers, and patient advocates are engaged inconsistently and in some cases ineffectively. FDA can help companies by:

- Encouraging the development of a common definition of meaningful and appropriate patient engagement
- Providing guidance to product developers regarding appropriate patient engagement methods
- Creating certainty for product developers by offering guidance on the types of patient information that would be useful to the agency and how this information would be incorporated into FDA's decision-making process

The issue of patient engagement across the spectrum of health care, from research to delivery, is one of great importance to both the patient community and the stakeholders that depend on patient information to inform their work. We are encouraged by the progress we see and look forward to continuing to work with the FDA on this important topic.

Please do not hesitate to contact Eric Gascho, NHC's 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 202-973-0545 or via e-mail at egascho@nhcouncil.org.

Sincerely,



Myrl Weinberg, FASAE, CAE
Chief Executive Officer