National Health Council

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Stephen Ostroff, MD
Acting Commissioner of Food and Drugs
Division of Dockets Management (HFA-305)
Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, Maryland 20852

RE: Docket No. FDA-2015-D-1580

Patient Preference Information—Submission, Review in Premarket Approvals (PMA), Humanitarian Device Exemption (HDE) Applications, and *De Novo* Requests, and Inclusion in Device Labeling; Draft Guidance for Industry, FDA Staff, and Other Stakeholders

Dear Dr. Ostroff:

On behalf of the National Health Council (NHC), we are pleased to provide comments on FDA's recent draft guidance for industry entitled "Patient Preference Information—Submission, Review in PMAs, HDE Applications, and *De Novo* Requests, and Inclusion in Device Labeling."

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 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 health insurance companies.

We appreciate the opportunity to provide input on this draft guidance. We commend FDA for taking this first, important step to enable enhanced integration of the patient voice throughout a product's full life cycle. The NHC strongly supports the Agency's ongoing effort to provide more clarity for industry and other stakeholders regarding how patient preference information and patient perspectives, priorities, and experiences (collectively the "patient voice") can inform medical product development and regulatory decision-making.

Engaging patients throughout the *entire* product development process is critical to optimizing product development, ensuring that innovative therapies reach patients faster, and better aligning products with patient preferences. To that

end, we also strongly support FDA's broader efforts to generate data and integrate the patient voice throughout the medical product development and approval processes, including encouraging sponsors to voluntarily submit patient preference information when appropriate.

We recognize that the benefit of this draft guidance extends well beyond industry to other stakeholders, including patients. For example, the draft guidance provides patient groups with a starting point from which to focus their engagement efforts. It also importantly recognizes what the patient voice can bring to the medical product development and approval processes, identifying patients as uniquely positioned as expert partners for FDA and other stakeholders.

Our comments on the draft guidance aim to highlight issues with which we strongly agree with FDA and point out those areas in which we believe additional clarity would benefit patients, patient groups, industry, and other stakeholders. We also hope to facilitate engagement and collaboration to further strengthen the patient voice in the product life cycle in order to ensure that treatments best meet the needs of patients.

Patient Preference Information versus the Patient Voice

FDA's definition of "patient preference information" reflects just one component of the patient voice. The patient voice includes not only patient preference information, but also (and not limited to) the patient's view of the natural history of condition, the impact of the condition on the patient's life, the patient's experience with treatments, and outcomes and priorities important to the patient. Furthermore, in certain cases the patient voice may also include the perspectives of their family caregiver. While we realize that the draft guidance as currently written focuses solely on patient preference information, it is important for the Agency to plan in the future to expand the draft guidance beyond patient preference information to include patient engagement to capture the full patient voice. Further clarity from FDA regarding generation and use during product development and regulatory decision-making of *all* types of patient information will be extremely valuable to all stakeholders.

It is imperative that FDA underscores that eliciting and incorporating the patient voice should optimally occur throughout the entire product life cycle, from understanding the disease, to product ideation and development, to post-market, and not just at the point of regulatory decision-making regarding benefit-risk. Additionally, the NHC agrees that collecting and submitting patient preference information to FDA should be voluntary, as it may not be relevant for all products.

In the draft guidance, Figure 1 (Patient Preference Information in the Total Product Lifecycle) acknowledges this broader role of the patient voice by including a category for "patient-owned data" (comparisons, identified problems, and unmet needs), presumably referring to patient information outside the scope of "patient preference information." While Figure 1 outlines some uses of the patient voice throughout the total product life cycle, references to the benefits of integrating the patient voice (outside of patient preference information) in development and regulatory decision-making are absent from the rest of the draft guidance. In fact, in some instances, FDA appears to use the term "patient preference" interchangeably with the term

"patient voice." NHC believes it critical to define clearly and use terminology consistently to avoid confusion (e.g., "patient preference," "patient voice," "patient-centeredness"). Below are possible definitions that could be used:

Term	Definition
Patient preference	Relative desirability of outcomes or acceptability of risks that differ among alternative health interventions
Patient voice	A patient's overall view of his or her condition, including (but not limited to) the natural history of the condition, the impact of the condition on the patient's life, the patient's experience with treatments, outcomes important to the patient, priorities, and other patient preferences and perspectives
Patient-centeredness	The intersection of health outcomes, patient experiences, and patient goals/aspirations

Meaningful Engagement with Patients

We agree with FDA's recommendation that product sponsors should meet with the Agency early and often to discuss collection and submission of patient information to ensure it is valuable to product sponsors and acceptable to FDA. In addition, the NHC would welcome any clarity regarding the current uncertainty and lack of predictability regarding stakeholder (in particular manufacturers of these products) engagement with patients. Companies are concerned their interactions with patients are at risk of being construed as discussions of unapproved medicines or unapproved uses of approved medicines. Thus, they may forgo collecting invaluable input from patients to inform earlier-stage research decisions, such as outcome endpoint selection or clinical trial design. To encourage fruitful collaborations, FDA should provide clear guidance to create a more predictable environment that supports industry-conducted activities to engage patients. Only with such an environment can the benefits of meaningful patient engagement be fully realized.

Qualities of Patient Preference Studies

The NHC welcomes clarity as to what constitutes valid scientific evidence, and we agree with the factors laid out in the draft guidance as appropriate for FDA to consider when determining whether submitted patient preference information constitutes valid scientific evidence.

Specifically, the NHC agrees that caregivers, parents, or guardians provide an important perspective and their voices should also be considered, as appropriate. However, the guidance currently only stipulates that caregiver, parent, or guardians' preferences should be considered "in situations when the patient may not be able to provide the patient preference perspective." We believe it is more accurate to expand the circumstances or contexts under which caregiver, parent, or guardians' preferences can and should be considered. For example, caregivers'

preferences can be critical in certain patient populations where they are particularly affected or to support continuity when it is anticipated in the progression of some diseases that patients will be unable to directly provide their perspective.

Additionally, we strongly believe that the development of patient preference studies should incorporate the patient voice and that patients should be involved in all aspects of the design of patient preference information studies. For example, the outcomes of interest and prioritization of outcomes being tested (with the exception of side effects patients would not be aware of) should come from patients, not clinicians and researchers. We believe prioritizing patients' outcomes of interest and appropriately weighting their perspective in other aspects of study design are critical to increasing the collection of valuable patient preference information.

Submission of Patient Preference Information to FDA

Many stakeholders, other than product sponsors, are beginning to seek patient input early on in, or even before, a product's development process. For example, information on disease progression or unmet medical need can be collected to help ensure not only that those products are designed to meet patient needs, but also that the studies conducted to inform regulatory approval and eventual clinical use are capturing information that is highly relevant and specific to the end-users themselves: the patients. However, the draft guidance as written does not include specific instructions for non-product sponsor submission of relevant patient information. The NHC believes that to optimally encourage submission of patient preference information or other aspects of the patient voice, especially from non-product sponsors, FDA should outline a specific route/process/contact for stakeholders who wish to share patient information with FDA. We believe that explicitly specifying a mechanism for how patient groups might directly submit patient preference information to the Agency will reduce confusion and encourage submissions. Similarly, we strongly support FDA's plans to obtain its own patient information whenever possible.

Use of Patient Preference Information to Approve Products for a Subset of Patients

We would also like to voice our strong support for FDA's acknowledgment that it may be "appropriate [for FDA] to approve a PMA, approve an HDE application, or grant a *de novo* request for use of a device by a subset of the population for which an indication is requested when valid scientific evidence shows that the probable benefit of the device outweighs probable risks for that subset." Bringing promising treatments to individuals suffering from chronic diseases and disabilities remains a critical priority for the patient community. When FDA determines it is appropriate, we agree that the patient voice can and should serve as powerful support for allowing access to a safe and effective treatment to a subset of patients. Furthermore, to protect patients, the NHC supports FDA imposing conditions of approval in appropriate circumstances, such as when a product is approved only for a subset of patients, and conditions of approval are required to mitigate risk and facilitate use in only those patients for whom the product's benefits are expected to outweigh its risks based upon evidence in a subgroup.

Patient Preference Information in Device Labels

The NHC fully supports FDA's recommendation to include in the product label descriptions of patient preference studies considered during FDA's approval assessment. In addition to supporting patients and providers in making more informed, patient-centered treatment decisions, enhanced label information may also inform development of other products by providing other sponsors with valuable information and examples of studies that FDA determined constituted valid scientific evidence.

Importantly, label information must be clear, accurate, relevant, and understandable to patients. Furthermore, patients must also be included in labeling discussions to tailor the patient preference study information included on the label to that which patients want, need, and understand. We agree with FDA's encouragement of the pre-testing of draft labeling with representative user populations in order to "ensure that it is usable, appropriate, comprehensible, unbiased, and complete." We also agree that sponsors should submit to FDA "a plan for how they intend to communicate patient preference information to patients and health care professionals," as this is critical to ensure patient and clinician understanding and communications.

Finalizing the Draft Guidance and Implementation

Given the great benefit that guidance from FDA holds for all stakeholders and recognizing that FDA will carefully consider all comments submitted to the docket for this draft guidance, the NHC encourages FDA to move as quickly as possible to finalize and implement this draft document. Equally important is to ensure that the concepts outlined in the final guidance are effectively communicated to and consistently applied by internal FDA reviewers.

We again thank FDA for this opportunity to provide our comments on this draft guidance, which we believe represents an important first step in working toward broader incorporation of the patient voice throughout the medical product life cycle, from start to finish. Please do not hesitate to contact Eric Gascho, 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 telephone at 202-973-0545 or via e-mail at egascho@nhcouncil.org.

Sincerely,

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Chief Executive Officer