



# National Health Council

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January 8, 2019

The Honorable Scott Gottlieb, MD  
Commissioner, Food and Drug Administration  
5630 Fishers Lane, Rm. 1061  
Rockville, MD 20852

Re: Patient Engagement in Medical Device Clinical Trials Discussion  
Document (FDA-2018-N-4171)

Dear Commissioner Gottlieb:

The National Health Council (NHC) is pleased to provide comments on the Food and Drug Administration (FDA)'s solicitation for feedback on patient engagement in medical device clinical trials, including the Center for Devices and Radiological Health (CDRH) Patient Engagement Advisory Committee (PEAC)'s Discussion Document from its November 15, 2018, meeting. The NHC appreciates the opportunity to provide input to the FDA on this important topic and supports CDRH's plans to develop draft guidance on patient engagement in clinical trials in FY2019.

Founded in 1920, the NHC is the only organization that brings together all segments of the health community to provide a united voice for the more than 160 million people in the United States with chronic diseases and disabilities and their family caregivers. Made up of more than 125 diverse national health-related organizations and businesses, the NHC's core membership includes the nation's leading patient advocacy organizations, which control its governance and policy-making process. Other members include professional and membership associations; nonprofit organizations with an interest in health; and representatives from the pharmaceutical, generic drug, health insurance, device, and biotechnology industries.

The NHC shares CDRH's commitment to enhancing patients' roles and leveraging their perspectives to inform decisions throughout the medical product development and regulatory approval processes. We applaud CDRH's efforts to seek stakeholder input, clarification, and consensus on key aspects of patient engagement (e.g., terminology, value and impact, and challenges or barriers, etc.) to inform future guidance development. The NHC has actively engaged with the FDA and other stakeholders over the past several years to help ensure that the patient voice is meaningfully represented. Most recently, the NHC provided extensive input on Center for Drug Evaluation and Research's (CDER's) planned series of guidances on Patient-Focused Drug Development (PFDD), the first of which focuses on *Collecting Comprehensive and Representative Input Guidance for Industry, Food and*

*Drug Administration Staff, and Other Stakeholders.*<sup>1,2</sup> Through our work to date, the NHC has sought to build consensus across a range of issues to advance the dialogue on patient engagement, from identifying key priority areas and topics for guidance development to providing feedback and suggestions on a common glossary of terms.

To reduce confusion and inefficiency, definitions, guidance, and policies should be aligned between CDER, Center for Biologics Evaluation and Research (CBER), and CDRH, unless it is on something specifically idiosyncratic to that division. Without this alignment, the patient community will not have the resources to be able to effectively represent their constituents across the FDA. Most patients and patient groups have significant resource constraints. Despite their strong desire to engage with the FDA and sponsors in medical product development, most have limited resources to do so as effectively as they would like. Use of varying terms, definitions, and processes across the FDA centers may place additional resource demands on these groups that, instead of enhancing engagement, creates barriers to engagement as an unintended consequence. Harmonization across centers, wherever possible, will serve to enhance patient groups’ ability to engage.

***I. Definition of Patient Engagement***

The NHC agrees with the FDA that a common definition of “patient engagement,” and what it entails in the product-development process, is necessary to establish a shared understanding of key concepts and to ensure that work progresses in a cohesive, consistent manner. We appreciate that CDRH’s proposed definition of patient engagement and some of the concepts in the discussion document appear to be closely coordinated with and reflective of existing FDA work.<sup>3</sup> However, there is not complete alignment, which will present challenges for stakeholders in the future. For example, the proposed definition of patient engagement in the discussion document differs from the definition proposed in CDER’s draft guidance on “Patient-Focused Drug Development: Collecting Comprehensive and Representative Input. Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders” (see Table 1). While we agree with the two distinct roles for patients (patient advisors, patient research participants), this terminology differs from the language described in CDER’s PFDD glossary.<sup>3</sup> To promote efficiency and demonstrate alignment, CDRH could adopt CDER’s PFDD glossary, deviating only as necessary due to specific, unique characteristics of medical device as compared to drug development.

**Table 1. FDA proposed definitions of patient engagement<sup>3</sup>**

CDRH	<i>Activities that involve patient stakeholders sharing their experiences, perspectives, needs, and priorities to <b>help inform the design, the implementation, and dissemination for medical product development and assessment.</b></i>
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<sup>1</sup> FDA. Patient-Focused Drug Development: Collecting Comprehensive and Representative Input Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders. Draft guidance. June 2018. <https://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm610442.pdf>

<sup>2</sup> NHC. Comments on Patient-Focused Drug Development Guidance: Methods to Identify What Is Important to Patients and Select, Develop, or Modify Fit-for-Purpose Clinical Outcome Assessments (Docket No. FDA-2018-N2455). <https://www.nationalhealthcouncil.org/public-policy/letters-comments/nhc-comments-patient-focused-drug-development-guidance-methods>.

<sup>3</sup> FDA. Patient-Focused Drug Development: Collecting Comprehensive and Representative Input Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders. Draft guidance. June 2018 <https://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm610442.pdf>

CDER	<i>Activities that involve patient stakeholders sharing their experiences, perspectives, needs, and priorities that <b>help inform FDA’s public health mission. Such activities may include (but are not limited to): testimony at Advisory Committee meetings, submission to regulations.gov public docket; meetings attended by patients, FDA, and other stakeholders; other correspondence with FDA; interactions through social media; and interactions with or information from patient representatives or patient advocates.</b></i>
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In addition to leveraging existing frameworks, definitions, and other concepts already developed for PFDD, we encourage CDRH to take into consideration specific recommendations and concerns that may have been raised through previous dialogue and feedback solicitations (e.g., recent comments to CDER’s planned PFDD guidances)<sup>4</sup>. For example, the NHC recommends that the FDA reiterate and clarify that not all patient-reported outcomes (PROs) are patient centered and not all patient-centered outcomes are reported by patients, as confusion appears to be highly prevalent among many stakeholders. For an outcome measure to be patient centered, it must capture a concept(s) patients identify as being highly important to them – these measures can be PROs, another type of Clinical Outcome Assessment (COA), or even a clinical measure. Understanding this distinction is important to help stakeholders differentiate among PROs developed without patient input versus those developed with patient input and partnership.

## II. *Value and Impact*

We agree with the benefits of patient engagement outlined in this section and encourage CDRH to further emphasize the benefits and impacts that patient involvement and the patient perspective can bring beyond simply the operational aspects of clinical trials, particularly around quality and relevance of data. For example, better quality data can result when patients play an active role early in the process, especially before and as part of protocol development. Patient perspectives may help inform the selection of endpoints or formulation of questions so that they are most relevant to or understandable to patients. These are distinct impacts that should be highlighted and emphasized.

## III. *Challenges*

While we agree that the challenges highlighted in the discussion document exist, we believe the document does not capture the full range of challenges that may affect the success of patient engagement in clinical trials. For example, investigators’ reluctance to engage patients may stem from underlying cultural or organizational barriers that must first be acknowledged. Further, specific actions from the FDA or other stakeholders, such as consensus building or other forms of communication, could help to resolve some of these barriers outside of FDA guidance. The NHC has explored many of these challenges to patient engagement in research, development, and review of medical products through multi-stakeholder forums; we encourage CDRH to leverage these findings in its guidance development process.<sup>5</sup>

<sup>4</sup>NHC. Comments on Patient-Focused Drug Development Guidance: Methods to Identify What Is Important to Patients and Select, Develop, or Modify Fit-for-Purpose Clinical Outcome Assessments (Docket No. FDA-2018-N2455). <https://www.nationalhealthcouncil.org/public-policy/letters-comments/nhc-comments-patient-focused-drug-development-guidance-methods>

<sup>5</sup> NHC. Dialogue: Advancing Meaningful Patient Engagement in Research, Development, and Review of Drugs. September 22, 2015. <https://www.nationalhealthcouncil.org/sites/default/files/PatientEngagement-WhitePaper.pdf>.

#### IV. *Approaches*

We commend CDRH for outlining specific examples for patient engagement in clinical studies in this section. However, we recognize this is an emerging field; thus, new methods for engaging patients are still being developed and tested. We strongly encourage CDRH to align with other stakeholders who are also doing work in this area, including CDER. Similarly, external stakeholders, such as the Medical Device Innovation Consortium (MDIC), the Clinical Trials Transformation Initiative (CTTI), and the Patient-Centered Outcomes Research Institute (PCORI) have all developed numerous frameworks, resources, and materials on patient engagement methods and the science of patient input.<sup>6,7,8</sup> While CDRH may already be working with some or all entities, we recommend that the future guidance build off of and align with these organizations' existing work.

#### V. *Conclusion*

Lastly, the NHC recommends CDRH create opportunities to more systematically gather stakeholder input to inform the framework for the planned guidance. We recognize that the purpose of this discussion document is to provide a very brief overview and summarize each of the questions posed. However, we believe that more targeted questions related to the proposed contents of the guidance, as well as opportunities for ongoing dialogue to further prioritize topics of importance, will better guide stakeholder input and help ensure that the guidance ultimately addresses topics of greatest relevance to the patient and medical device communities. The NHC previously undertook such an effort to establish consensus on the general areas that should be covered in the guidance for PFDD.<sup>9</sup> We therefore encourage the FDA and CDRH PEAC to continue to engage external stakeholders as it seeks to develop the framework for its guidance, and to create opportunities for patients and stakeholder to provide targeted input.

The NHC fully supports CDRH's ongoing efforts to enhance patient engagement in medical product development and looks forward to continuing to engage with the agency as it develops guidance documents on these topics.

Please do not hesitate to contact Eric Gascho, our Vice President of Policy and 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](mailto:egascho@nhcouncil.org).

Sincerely,



Marc Boutin, JD  
Chief Executive Officer

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<sup>6</sup>MDIC. Additional Resources & FAQ's about Conducting Patient Preference Studies.

<https://mdic.org/resource/additional-resources-faqs-about-conducting-patient-preference-studies/>

<sup>7</sup>CTTI. Project: Patient Groups & Clinical Trials. <https://www.ctti-clinicaltrials.org/projects/patient-groups-clinical-trials>

<sup>8</sup>PCORI. The Value of Engagement. <https://www.pcori.org/about-us/our-programs/engagement/value-engagement>

<sup>9</sup>NHC and Genetic Alliance. Integrating the Patient into the Drug Development Process: Developing FDA Guidance. Meeting Summary. May 4, 2016. <http://www.nationalhealthcouncil.org/sites/default/files/NHC-GA-Patient-Engagement.pdf>.