National Kidney Foundation®

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September 10, 2018

The Honorable Scott Gottlieb, MD
Commissioner, Food and Drug Administration
Division of Dockets Management (HFA-305)
5630 Fishers Lane, Room 1061
Rockville, MD 20852

Re: Patient-Focused Drug Development: Collecting Comprehensive and Representative Input; Draft Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders; Availability (FDA-2018-D-1893-0001)

Dear Commissioner Gottlieb,

The National Kidney Foundation is pleased to comment on the Patient-Focused Drug Development: Collecting Comprehensive and Representative Input; Draft Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders. We understand this is the first in a series of four guidance documents that aim to inform stakeholders on opportunities to collect patient experience data and other patient input that will be used to guide new drug and device development and regulatory decisions. The National Kidney Foundation is the largest, most comprehensive and longstanding, patient centric organization dedicated to the awareness, prevention and treatment of kidney disease in the US. In addition, we have provided evidence-based clinical practice guidelines for all stages of chronic kidney disease (CKD), including transplantation since 1997 through the National Kidney Foundation Kidney Disease Outcomes Quality Initiative (NKF KDOQI). We offer the following comments on this draft guidance.

Patient Engagement

We appreciate that the Patient Focused Drug Development (PFDD) guidance documents will build from lessons learned in PFDD meetings and ideally create an integrated approach across the FDA to evaluating the use of patient experience data in regulatory decisions and holistically by medical product developers to incorporate what matters most to patients in development of new products. The National Kidney Foundation has convened two recent externally led (EL) PFDD meetings in rare forms of kidney disease – complement 3 glomerulopathy (C3G) and Alport Syndrome (in collaboration with the Alport Syndrome Foundation) – and have participated in the 2016 FDA led PFDD for organ transplant recipients. EL-PFDD meetings have and will continue to have an important role in engaging patients early to ideally help shape

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medical product developers thinking around development of new therapies that address the challenges most important to patients. However, we are hopeful that with this guidance, PFDD will become better incorporated into FDA decision making and medical product development holistically and not as a standalone activity.

Patient Experience Data

The National Kidney Foundation has a long history of advocating for expanded opportunities to include patient perspectives in decisions throughout the lifecycle of medical products - from product development through post-market regulations. Only patients who are living with a serious health condition can attest to how that condition and any current treatments they are taking affect their daily lives, what symptoms are most concerning to them, and what trade-offs regarding risks and benefits they are willing to make. We appreciate FDA's recognition of the important role patient experience data has in medical product development and approval. The National Kidney Foundation also strongly supports the acknowledgements that patient experience data can include the natural history of disease, symptom burden, experience with treatments, patient preferences, values and social needs. We also appreciate the recognition that the patient experience should be defined from the patient's perspective, but patient experience data can be enriched by input from clinicians and patient partners – including non-clinician caregivers and patient advocacy organizations.

The National Kidney Foundation also supports that the guidance allows for the collection of patient experience data by patient advocacy organizations as these are often the organizations who are already interacting with and providing support to patients to help them with their disease. They also tend to be the organizations already engaged in trying to foster innovation to develop cures and new therapies that will alleviate the burdens of diseases. These organizations are most often the best positioned to provide detailed information on patient experiences.

In this draft guidance FDA acknowledges that patients should be engaged throughout the medical product development cycle, including pre-development and early development. Most of this document tends to focus on patient engagement in clinical trials. The National Kidney Foundation suggests that FDA provide additional details throughout this guidance on how patient experience data can be incorporated prior to the clinical trial. In addition, at an August 10, 2018 meeting convened by the National Health Council, Commissioner Gottlieb stated that this patient guidance needs to be incorporated into the workflows of medical product developers and the FDA and not a standalone activity. We agree with this statement and request that the FDA share further details on how the agency will ensure this draft guidance and subsequent PFDD guidance documents will assist developers in incorporating PFDD concepts

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into the medical product development cycle and in regulatory decisions regarding approval of clinical and pivotal trial design.

FDA also acknowledges that patients should be considered partners with medical product developers and not subjects. The National Kidney Foundation fully supports this acknowledgement and appreciates its inclusion in the guidance. Patients as partners can be further defined in this document to ensure that stakeholders understand that patients should be engaged in forming the study questions and not just answering the questions. This is important to ensuring that what matters most to patients is incorporated into the development and regulatory approval process. FDA should also clarify that patients as partners means that feedback loops are established with patients so that patients know how their experience data will be used and the timeline for the study. Patients should also be updated regularly on the status of the study and the end results should be shared.

Methods for patient engagement data collection

The National Kidney Foundation appreciates that the guidance addresses quantitative and qualitative data collection methodology and allows for mixed methods research. The examples provided in the methodology table are helpful in illustrating opportunities for collecting patient experience data. For example, allowing a survey with open-ended questions can be helpful in ensuring that what matters most to patients is captured in the research. While close ended questions are important and helpful in quantification, used alone they may not accurately or entirely reflect what is most important to patients.

Representativeness

Establishing guidance around representativeness in patient engagement data collection and use is important and we appreciate its inclusion in this document. This is where relying on PFDD meetings to collect patient data has its limitations. Those who are willing and able to participate and attend a PFDD meeting may not reflect the average patient or may exclude a subpopulation that has a greater disease burden or social risk factors that are barriers to participation. This issue was raised by patients during the PFDD meeting of patients who have had an organ transplant, which was held in September 2016. During that meeting participants highlighted that they were among the most activated patients and likely more adherent to current therapies. They recognized their experiences may not be generalizable to all patients. As this example illustrates, patients and patient advocacy organizations can be helpful in identifying representativeness considerations some of which are unique to a specific condition. The FDA should include additional factors on what constitutes a good process and additional guiding questions for considering representativeness to include in this document. We suggest FDA include additional guiding questions and examples of good and bad processes from the

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National Health Council's Patient Representativeness Roadmap and Rubric to provide additional clarity.[1]

The National Kidney Foundation appreciates the opportunity to comment on this draft guidance. We look forward to providing input on subsequent, forthcoming PFDD guidance documents. Please contact Tonya Saffer, Vice President for Health Policy at tonya.saffer@kidney.org, or 202-244-7900 extension 717 with any questions.

Sincerely,

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Kevin Longino
CEO and Kidney Transplant Patient

Kerry Willis
Kerry Willis, Phd
Chief Scientific Officer

[1]https://www.nationalhealthcouncil.org/sites/default/files/Representativeness%20in%20Patient%20Engagement.pdf