National Kidney Foundation®

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June 8, 2017

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: Enhancing Patient Engagement Efforts Across the Food and Drug Administration; Establishment of a Public Docket; Request for Comments (FDA-2017-N-0455)

Dear Commissioner Gottlieb:

The National Kidney Foundation (NKF) strongly supports the establishment of an Office of Patient Affairs at the Food and Drug Administration (FDA) and appreciates the opportunity to comment on this proposal. NKF has previously advocated for expanded opportunities at FDA to include patient perspectives in decisions throughout the lifecycle of medical products - from product development all the way 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 and what trade-offs regarding risks and benefits they are willing to make. NKF 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, NKF has provided evidence-based clinical practice guidelines for all stages of chronic kidney disease (CKD), including transplantation since 1997 through the NKF Kidney Disease Outcomes Quality Initiative (NKF KDOQI). NKF offers the following comments on the proposed Office of Patient Affairs.

- The Office of Patient Affairs should be positioned to provide bidirectional education – including education to patients on the FDA regulatory process and opportunities for its staff to engage and to readily receive input and education from patients on their experiences and health challenges.
- The Office of Patient Affairs should develop processes and best practices for patient engagement and serve as a facilitator for medical product centers to engage directly with patients
- The Office of Patient Affairs should develop a scientific process for using patient input in medical product decisions
- The Office of Patient Affairs should not be a single point of entry for patient engagement
- The Office of Patient Affairs should be a data aggregator of patient input and publicly share lessons learned from patients

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- The Office of Patient Affairs should include patients and patient organizations in its creation and to further define its objectives and responsibilities.

NKF agrees that the bidirectional education objectives for the proposed Office of Patient Affairs (the Office) are appropriate. We agree that the Office should take the lead on engaging patients and connecting them to opportunities to inform medical product decisions across the agency. We also agree that the Office should lead patient education, communication and outreach to ensure the FDA is approachable for patients and that a diverse representation of the patients engage with FDA. NKF believes the Office should also develop a process and best practices for FDA medical product centers to gain a better understanding of the patient experience, gathering patient perspectives on what is clinically meaningful, and assessing attitudes towards benefit and risk and the tolerance for uncertainty. We emphasize that in this regard the Office should develop processes and best practices and facilitate their use across FDA, not be solely accountable for all patient engagement activities. For effective patient engagement to occur, it needs to become ingrained in the culture of FDA, and all medical product centers should be accountable for successful patient engagement. The creation of the Office of Patient Affairs should not create a silo for all patient engagement activities. Patients and patient advocacy organizations should continue to be able to develop and maintain relationships directly with medical product centers. FDA staff engaged with medical product development and regulatory decisions benefit from direct engagement with patients.

Many FDA staff involved in sensitive decision making have never interacted with a patient who has the condition. This is what has made the patient focused drug development (PFDD) sessions valuable. By participating in the PFDD for Organ Transplant Recipients, in September 2016, NKF saw first-hand the value and impact on FDA staff of hearing directly from patients about their experiences with immunosuppressive drugs. In fact, a separate meeting this past April was convened and provided another opportunity for FDA staff to learn from organ recipients' experiences with antibody mediated graft rejection. Hearing directly from patients allows FDA scientists to better understand and factor into decision making the real effect medical products have on patients' lives. Given that medical products are only as valuable as they are perceived to be by the end-user, the patient, it would be ideal if all FDA staff involved in medical product decision making and regulations could hear directly from the patients about their needs, goals, experiences, and willingness to accept risks.

NKF also supports the Office of Patient Affairs objectives to enhance the science of eliciting and integrating patient input into FDA decision making across medical product centers. Our hope is this will create a transparent and consistent approach to how patient input is incorporated into FDA decisions across a product lifecycle. Transparency and communication are key to ensuring patients participate and provide input and can clearly see the value of their time and effort.

For reasons stated above, we disagree with the proposal that the Office of Patient Affairs be a single point of entry for the patient community. NKF does agree that the Office of Patient Affairs would be well positioned to navigate and link patients to opportunities to engage with medical product centers across FDA and that the Office should serve as a central entity for receiving information from patients and

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analyzing the data to enhance learnings stemming from patient engagement. This could be established through feedback loops with the medical product centers who should be directly engaging patients. NKF also agrees that the Office should have responsibility for establishing platforms to encourage patient participation in FDA medical product decisions, including leveraging online channels to reach larger numbers and a greater diversity of patients.

The data the Office collects on patient input and lessons learned should also be shared with the patients who participate and with the public. The Office could also aggregate patient input data from across medical product centers to identify broad lessons learned.

NKF strongly supports the proposal to create an Office of Patient Affairs, but many details remain to be worked out. We appreciate that the Office will evaluate its performance on a biennial basis to allow the patient community to provide feedback. NKF also encourages the agency to establish a formal process to engage the patient community in further defining the objectives and responsibilities for the Office and in developing processes and best practices for patient engagement across FDA. The National Kidney Foundation would be a willing and strong partner for the Office in these activities.

NKF has been involved in several initiatives to engage patients in medical product research and regulations. For example, In September 2010, NKF and the FDA cohosted a workshop[1] to discuss patient reported outcomes (PRO) as a surrogate endpoint in clinical trials of chronic kidney disease (CKD)-related therapies. One conclusion of the workshop was that more research was needed to determine appropriate PROs for nephrotic syndrome, anemia secondary to kidney disease, and autosomal dominant polycystic kidney disease. Workshop participants agreed that qualitative research, such as conducting patient focus groups, patient interviews, and developing tools to permit clinical trials to target symptom burden associated with the disease state is important in evaluating the benefit to patients of a particular therapy. In addition, we are an Advisory Board Member of the Patient Focused Medicine Development (PFMD), which is a global coalition of patients, patient advocacy organizations, non-profit organizations, physicians, health insurers, pharmaceutical companies, and medical device manufacturers dedicated to establishing global best practices on how to engage patients on medical product development. In our role with the coalition NKF senior leadership and patients who are members of our Kidney Advocacy Committee are working with other PFMD members to produce guidelines and best practices for better patient engagement from phase one though clinical trials and up to market launch. Patient engagement and a continuous feedback loop to patients is included at every step. The priorities of PFMD are summarized in a recent paper.[2]

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[1] Perrone R, et al. Patient-reported outcomes in clinical trials of CKD-related therapies: Report of a symposium sponsored by the National Kidney Foundation and the US Food and Drug Administration. Am J Kidney Dis. 2013 Dec;62(6):1046-1057.

[2] Boutin, Marc et al. Culture and Process Change as a Priority for Patient Engagement in Medicines Development, Therapeutic Innovation & Regulatory Science, 51:1, page(s): 29-38, January 1, 2017.

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NKF appreciates the opportunity to comment on the proposal for the development of an Office of Patient Affairs and welcomes future opportunities to collaborate with the FDA on the creation of this new Office.

Sincerely,

Kerry Willis

Kerry Willis, PhD
Chief Scientific Officer