



National  
Kidney  
Foundation®

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September 14, 2018

Ed Simcox  
Chief Technology Officer  
U.S. Department of Health and Human Services  
KidneyX, c/o Ross Bowling  
200 Independence Avenue SW, Room 624D  
Washington, D.C. 20201

Re: KidneyX Requests for Information.

Dear Mr. Simcox,

The National Kidney Foundation is pleased to offer the following comments in response to the Requests for Information issued on August 16, 2018. 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).

The National Kidney Foundation appreciates the U.S. Department of Health and Human Services focus on spurring innovation in new treatments for people with kidney disease through its public-private partnership to create KidneyX. We support KidneyX goals to improve the quality of life for people living with kidney disease through spurring innovative medical product development and creating pathways to ensure patients have access to new products. While we appreciate the requests for information as an opportunity to inform priorities and unmet needs we strongly encourage KidneyX to create an infrastructure that supports continuous patient engagement and we offer the following recommendations.

1. Following the patient engagement processes and recommended best practices of organizations like the Patient Centered Outcomes Research Institute and the Patient Focused Medicines Development will help KidneyX will ensure that that development of medical products competing for prizes through KidneyX engage patients early in their

process of development.<sup>1,2</sup> KidneyX should set the example for patient engagement by reviewing existing literature on symptoms patients face and conducting focus groups and surveys of patients, families, and caregivers to determine unmet needs. KidneyX should ensure representativeness in its outreach to patients, provide transparency in the purpose for the outreach, and communicate back to patients who participate how their information was used and the end results. To also lead by example, Kidney X should include patients as judges and give them at least an equal role in decision making as other stakeholders. To the extent possible, KidneyX should recruit or work with patient advocacy organizations to recruit diverse patients with different backgrounds, lifestyles and experiences with kidney disease to review prize competitors. To achieve representativeness, the patient judges experiences should align with the overarching topic/objectives for the awards. For example, the first round of awards for KidneyX will focus on renal replacement therapies. Patient judges for that round should have various experience with different types renal replacement therapies. Patients who have had successes and failures with renal replacement therapy and family members of patients who have decided to withdraw from renal replacement therapy are a couple of examples of people who have lived experiences with ESRD to contribute to decisions on prize winners.

2. The Food and Drug Administration has issued the first in a series of draft guidance documents for Patient-Focused Drug Development. While categorized under drug development, the guidance document addresses patient engagement in medical product development more broadly. The first guidance document addresses collecting comprehensive and representative input through patient experience data. Future draft guidance documents will continue to address how stakeholders can collect patient experience data and other relevant patient information to inform product development and regulatory decision making. These guidance documents will hopefully provide a roadmap to encourage product developers to collect and use patient experience and other patient information to inform medical product development from concept through market entry. Product developers competing for KidneyX prizes should be required to respond to how they plan to or have incorporated patient experience data into their product design, further development of the product, clinical trial phases and post market surveillance. The FDA guidance documents can serve as a resource to these developers.
3. Patients should be included as partners in product development, not merely subjects.

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<sup>1</sup> <https://www.pcori.org/engagement/what-we-mean-engagement>

<sup>2</sup> <http://patientfocusedmedicine.org/the-patient-engagement-quality-guidance>

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Patients should be engaged in forming study questions and design, not just answering the developer's questions. This is important to ensuring that what matters most to patients is incorporated into the development and regulatory approval process. 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. KidneyX should embed these principles into the prize competitions.

While we appreciate that the RFI is collecting information from patients as well as other stakeholders and relying on the Kidney Health Initiative's Renal Replacement Therapy Roadmap to inform priorities, the National Kidney Foundation believes greater representativeness in patient outreach is needed to help KidneyX focus and prioritize topics for the prize competition. It appears the first round of prizes would allow for nearly any innovation in dialysis technology to qualify. Also, we note that technical challenges like fluid removal appear in the same list as broad quality of life improvements, like "increasing mobility and physical activity". Given such a diversity of categories we believe it will be quite difficult to prioritize, in a patient centered way, awards that address unmet needs as defined by patients. There are several studies published and ongoing, for example Standardised Outcomes in Nephrology (SONG) initiative,<sup>3</sup> that have engaged patients and prioritized unmet needs. Lessons learned in recent externally led (EL) and FDA led kidney disease and organ transplant PFDD meetings have also helped inform priorities for product development by patients living with these conditions. The National Kidney Foundation has convened two recent EL PFDD meetings in rare forms of kidney disease – complement 3 glomerulopathy (C3G) and Alport Syndrome (in collaboration with the Alport Syndrome Foundation) – and has 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 medical product developers thinking around development of new therapies that address the challenges most important to patients. KidneyX should review the reports from these meetings available on the FDA website.<sup>4</sup> In addition there is further outreach to patients to achieve representativeness in soliciting feedback on priorities for KidneyX that should be conducted prior to the first competition.

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

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<sup>3</sup> <http://songinitiative.org/>

<sup>4</sup> <https://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm368342.htm>

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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 the opportunity to comment on KidneyX plans for prize competitions and would be pleased to further collaborate with KidneyX to help achieve our mutually shared goals of encouraging patient-centered innovation in product development that improves outcomes for people with kidney disease.

Please contact Tonya Saffer, Vice President for Health Policy with questions at [tonya.saffer@kidney.org](mailto:tonya.saffer@kidney.org) or 202.244.7900 extension 717.

Sincerely,  
*Kevin Longino*

Kevin Longino  
CEO and Kidney Transplant Recipient