Integrating Patient Perspectives into Medical Device Regulatory Decision Making to Advance Innovation in Kidney Disease

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Understanding patient perspectives is critical to developing tailored care approaches that address patients' needs and wants, with the ultimate goal of improving patient satisfaction and quality of life. Methods to better measure patients' experiences living with their condition and its diagnosis, management, and treatment are also evolving. Tools are being developed that can robustly measure how patients feel and function, and quantitatively capture the relative value patients place on risks and benefits associated with treatments. With these tools in hand, the health care enterprise that includes medical product regulators, health care providers, and payers is uniquely poised to systematically incorporate the patient voice into its daily operations. This article highlights novel approaches the Center for Devices and Radiological Health (CDRH) at the Food and Drug Administration (FDA) has taken to establish systematic, sustained mechanisms for bringing the patient voice to bear in medical device evaluation.

The CDRH regulates medical devices used in the care and treatment of people with kidney failure, such as dialysis systems, hemodialyzers, and peritoneal and vascular access devices, to help promote and protect the health of these patients. To help support regulatory decisions, the CDRH has committed to incorporating the patient perspective into all regulatory efforts, as appropriate (1). The FDA's concerted efforts to incorporate patient input into the evaluation of medical devices began a decade ago, with CDRH staff identifying a need to make patient centrism more integral to their work. Through national town hall discussions and additional conversations with patient groups, CDRH's vision statement became "patients in the United States have access to high-quality, safe, and effective medical devices of public health importance first in the world," where "patient" is the first word because they are the group most affected by regulatory decisions. To involve patients in the regulatory process, the CDRH has encouraged the medical device industry and others to engage with patients and collect patient-focused outcomes. This commitment to putting patients first was clearly reflected in the 2015 public workshop held by the Kidney Health Initiative (KHI), where the CDRH participated in conversations about ways patients living with kidney disease could play a critical role in developing and evaluating technologies to treat their condition (2).

The CDRH's commitment to including the patient voice was further reflected in the 2016–2017 Strategic Priority of "Partnering with Patients," (3) during which more than 90% of the CDRH's staff engaged with patients, and the CDRH created and annually convenes the Patient Engagement Advisory Committee, a formal mechanism to receive recommendations from patients, caregivers, and patient advocates about general, scientific matters related to medical devices (4). From the patient-listening sessions to advisory committee testimonies, to structured measurement of patients' experiences with medical devices, the CDRH continues to elicit the insights of patients. Data on patient-reported outcomes (PROs) and patient preferences (also called health preferences) can be integrated into the total body of scientific evidence used to support regulatory decisions on medical devices.

PROs measure how a patient feels, functions, and survives. PROs such as fatigue and physical function are important end points to be captured in kidney disease clinical trials. Efforts such as the International Standardized Outcomes in Nephrology initiative that collect input from kidney disease patients, caregivers, and health professionals may lead to the development and improved assessment of outcomes that are important to patients (5). The FDA has issued guidance documents detailing factors that lead to well-defined and reliable assessments of symptoms and physical function in a way that they can be aggregated to support the evaluation of safety and effectiveness for medical products (6). Assessing outcomes that are important to patients can help support the evaluation of medical devices and improve discussions that health care providers have with their patients.

While PROs assess how a patient feels and functions, patient-preference information reflects the relative values that patients place on benefits and risks of their treatments. Patient-preference information is defined by the CDRH as "qualitative or quantitative assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among

alternative health interventions" (7). The CDRH has been a leader in advancing the science of patient preferences and incorporating them into medical device benefit-risk decisions. The CDRH partnered with the Medical Device Innovation Consortium, a public-private partnership focused on medical device regulatory science, publishing a framework and a catalog of methods for measuring patient preferences (8). The 2016 FDA guidance document on voluntary submission of patient-preference information by the industry was developed to encourage submission of patient-preference information to the CDRH, to outline recommended qualities of patient-preference studies, to provide recommendations for collecting and submitting patient-preference information to the CDRH, and to discuss the inclusion of patient-preference information in CDRH decision summaries and device labeling (7). In December 2017, the FDA, including medical product centers for devices (CDRH), drugs (Center for Drug Evaluation and Research), and biologics (Center for Biologics Evaluation and Research), cohosted a workshop with academic Centers of Excellence in Regulatory Science and Innovation on advancing the use of patient-preference information as scientific evidence in medical-product evaluation (9). The CDRH is helping to advance the methodologies used to measure patient-preference information by conducting a number of studies with academic centers and other research partners. From prostate cancer to uterine fibroids, to chronic pain and adolescent scoliosis, to kidney disease, the CDRH is working to advance the application of well-conducted patient-preference studies.

Patient-preference studies have been used to expand labeled indications for medical devices and support performance goals for clinical trials (10,11). As an example, a home hemodialysis device cleared for use with a care partner present had its labeled indication modified to include use without a care partner, and other mitigations of risk, on the basis of the patient-preference information collected by the industry sponsor (11). The development of this study was initiated through early interactions among industry, the FDA, and the patient community during the 2015 KHI Patient Engagement Workshop (2). Referencing the FDA guidance document that included recommendations for quantitative patient-preference studies, the industry was able to conduct a patient preference study that was used as valid scientific evidence to help support the evaluation of the home hemodialysis device (7). In summary, patient-preference studies that are "all about patients," with good study design, conduct, and analysis, can be impactful.

Collaborative efforts between the FDA and the KHI to include the patient voice in innovative kidney failure treatments are underway. These efforts include not only conducting a patient-preference study but also integrating that study into a registry platform. The patient-preference study is focusing on attributes important to patients for emerging kidney replacement therapies and potentially could be used to develop performance targets for innovative technologies. By testing and evaluating ways to better integrate the collection of patient data, we advance opportunities to use it as an integral part of regulatory and health care decision making.

In conclusion, kidney disease has become a catalyst for acknowledging, better defining, and measuring patient preferences. Patients are the inspiration that spurs innovations in technology and scientific methods. The CDRH is aligned with this goal to better engage with patients, understand their perspective, and proactively integrate these perspectives into the total product life cycle of medical devices to help protect and promote patient-centric public health.

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References

1. Benz HL, Saha A, Tarver ME: Integrating the voice of the patient into the medical device regulatory process using patient preference information. *Value Health* 23: 294–297, 2020
2. Hurst FP, Chianchiano D, Upchurch L, Fisher BR, Flythe JE, Castillo Lee C, Hill T, Neuland CY: Stimulating patient engagement in medical device development in kidney disease: A report of a Kidney Health Initiative Workshop. *Am J Kidney Dis* 70: 561–569, 2017
3. US Food and Drug Administration: 2016-2017 Strategic priorities center for devices and radiological health. Available at: https://www.fda.gov/media/95317/download. Accessed June 22, 2020
4. Hunter NL, O'Callaghan KM, Califf RM: Engaging patients across the spectrum of medical product development: View from the US Food and Drug Administration. *JAMA* 314: 2499–2500, 2015
5. Tong A, Manns B, Wang AYM, Hemmelgarn B, Wheeler DC, Gill J, Tugwell P, Pecoits-Filho R, Crowe S, Harris T, Van Biesen W, Winkelmayer WC, Levin A, Thompson A, Perkovic V, Ju A, Gutman T, Bernier-Jean A, Viecelli AK, O'Lone E, Shen J, Josephson MA, Cho Y, Johnson DW, Sautenet B, Tonelli M, Craig JC; SONG Implementation Workshop Investigators: Implementing core outcomes in kidney disease: Report of the Standardized Outcomes in Nephrology (SONG) Implementation Workshop. *Kidney Int* 94: 1053–1068, 2018
6. US Food and Drug Administration: Patient-reported outcome measures: Use in medical product development to support labeling claims. Available at: https://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-reported-outcome-measures-use-medical-product-development-support-labeling-claims. Accessed June 22, 2020
7. US Food and Drug Administration: Patient preference information—Voluntary submission, review in premarket approval applications, humanitarian device exemption applications, and *de novo* requests, and inclusion in decision summaries and device labeling. Available at: https://www.fda.gov/downloads/medicaldevices/deviceregulationandguidance/guidancedocuments/ucm446680.pdf. Accessed June 13, 2020
8. Medical Device Innovation Consortium (MDIC): Patient Centered Benefit-Risk Assessment: (PCBR). Available at: https://mdic.org/project/patient-centered-benefit-risk-pcbr/. Accessed June 22, 2020
9. Benz HL, Lee TJ, Tsai JH, Bridges JFP, Eggers S, Moncur M, Shaya FT, Shoulson I, Spatz ES, Wilson L, Saha A: Advancing the use of patient preference information as scientific evidence in medical product evaluation: A summary report of the Patient Preference Workshop. *Patient* 12: 553–557, 2019

10. RTI Health Solutions: New system for ear tube insertion approved by FDA: General anesthesia not required. Available at: https://www.rtihs.org/news-and-events/new-system-ear-tube-insertion-approved-fda-general-anesthesia-not-required. Accessed June 22, 2020
11. NxStage: NxStage Medical's NxStage one home hemodialysis system for solo dialysis - K171331 and the 510(k) summary, 2017. Available at: https://www.accessdata.fda.gov/scripts/cdrh/cfdocs/cfPMN/pmn.cfm?ID=K171331. Accessed June 22, 2020

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