

Overview of Various Components of the Science of Patient Input

Advancing the Use of Patient-Reported, Real-World Evidence for Medical Device Evaluation of Innovative Products for the Treatment of Kidney Failure Using Strategically Coordinated Registry Networks

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In the United States, 746,557 individuals live with ESKD, with approximately 70% receiving dialysis (1). Dialysis, although life sustaining, is a life-changing therapy for patients. In a survey of patients with CKD on dialysis, 51.9% of patients reported that their decision to be treated with dialysis reflected physicians' preferences and 13.9% stated it reflected family members' preferences rather than personal choice (2). Another study found that patients with CKD may consider markers of "freedom," such as time on dialysis, convenience, and effect on family, as being more important than life expectancy (3). These studies highlight the need to re-evaluate decision making around the initiation of dialysis and involve patients in discussions about prognosis and goals of care. This sentiment of shared decision making is increasingly being reflected in our evolving health care ecosystem.

At the Food and Drug Administration (FDA) Center for Devices and Radiologic Health (CDRH), our mission is to protect and promote public health, which means that people with medical illness are at the heart of what we do. All too often, the health care systems focus more on what they can do for patients instead of understanding what patients would like the health care system to do for them. Since 2016, when CDRH established "partnering with patients" as a strategic priority (4), there has been an increase in the need for research-based methods and tools to identify the effectiveness of incorporating patient input and its effect on public health. The goals of this strategic priority are to promote a culture of meaningful patient engagement by facilitating CDRH staff interaction with patients and to increase the use and transparency of patient input as evidence in our decision making. Accompanying the efforts to integrate patient perspectives into our thinking is the development of the science of patient perspectives to ensure that the data generated are of regulatory grade (*i.e.*, are fit for use and constitutes valid scientific evidence). CDRH has publicly stated in the strategic priority document that "to successfully achieve a mission and vision in service of patients, we must interact with patients as partners and

work together to advance the development and evaluation of innovative devices, and monitor the performance of marketed devices" (4). Only in that way can partnering with patients truly reach its desired goal of developing, regulating, and delivering innovative and better health care.

In this series of short perspectives from several viewpoints, the authors examine the various components of the science of patient input. This begins with reviewing how FDA/CDRH developed patient engagement as a strategic priority, how it has used patient perspective information survey data in the past, how it might consider novel approaches to foster innovation, and why this is important (5). This is followed by learning how the world of financial engineering and Bayesian decision analysis, as proposed by Andrew Lo and his team at the Massachusetts Institute of Technology (MIT), can use the data gathered in the survey to create systematic, transparent, and repeatable estimates of appropriate "P values" that could be used in clinical trials (6). Next, the Kidney Health Initiative (KHI; a public-private partnership between the American Society of Nephrology and the FDA, established in 2012) describes how it intends to support the development of a new patient perspective information survey through its interactions with Research Triangle Institute, its Patient and Family Partnership Council, and others following methodology curated by the Medical Device Innovation Consortium through the Patient Centered Benefit Risk Project (7). Finally, the most important stakeholders, the recipients of health care, describe what this entirely new scientific process means to them, their health, and their future (8).

This is just the beginning of this new scientific approach to the discovery of how partnering with patients can reach its desired goals. Only vigorous and robust scrutiny of this work by others will yield further information on how this field of science should develop and progress.

- Article 1 discusses how the FDA uses patient perspective information in regulatory decision making (with references to FDA's guidance documents and experience with obesity devices) (5). The authors

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Tarver and Neuland (5) were supported by the FDA/CDRH patient perspectives team and the renal review team.

- Article 2 discusses how the MIT Bayesian decision analysis approach from Chaudhuri and Lo (6) has enabled this work and is helping to create objectivity, transparency, and reproducibility for regulatory decision making (with references to the Parkinson work in patient perspective information). The authors Chaudhuri and Lo (6) were supported by Lo's group at MIT.
- Article 3 discusses how KHI is supporting the development of a new patient perspective information survey for patients with ESKD. The authors Flythe and West (7) were supported by their group at KHI.
- Article 4 by Conway and Knight (8) discusses how patients view this entire process and changes at FDA and in this field of study.

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