Legitimization and Incorporation of Patient Preferences

The Arrow that Hit the Achilles Heel of Status Quo Kidney Care

Paul T. Conway and *Richard Knight*

CJASN 16: 645–647, 2021. doi: https://doi.org/10.2215/CJN.11780720

We hope that at the point that readers see this *CJASN* article, they will have already read the three accompanying *CJASN* articles on patient preference information and patient-centered innovation in KRT (1–3). These four articles represent a combined effort to chronicle the amazingly rapid application of the emerging science of patient insights, and the substantive effect it has already had as a legitimate body of evidence for policy and regulatory decision making within the realm of kidney disease and KRT. Each article provides information on the ascendancy of kidney patients within the scientific and policy processes.

We believe, as kidney patients and advocacy leaders for the American Association of Kidney Patients within the national policy arena, that our fellow authors have accurately captured one of the most significant transformations to the underlying basis of kidney medicine and innovation policy and regulatory decision making in the past 50 years. History demonstrates that the achievement of seemingly distant national policy visions often depends upon the evolution, legitimization, and application of new sciences, disciplines, and technologies years in advance. Public attention and imagination are easily captured by a compelling vision and a well-planned communications strategy. We know this from our careers in national issue campaigns, service in four presidential administrations, and through multiple staff roles in the United States Congress. But it is the hard work of scientists, researchers, innovation leaders, and strategic thinkers outside the limelight, thinking beyond process, whose persistence and courage as change agents produce the results necessary to maintain public and stakeholder interest and achieve a vision.

2020 marked the 1-year anniversary of the unapologetically optimistic vision for American kidney care articulated through the bipartisan July 10, 2019 Executive Order on Advancing American Kidney Care (https://www.whitehouse.gov/presidential-actions/executive-order-advancing-american-kidney-health/), signed by President Trump. The Executive Order served as a framework for significant improvements in kidney health outcomes and a new era in kidney treatment innovations, including disease prevention, precision medicine, artificial wearable and implantable kidneys, and new devices, diagnostics, and biologics. Most significantly, the Executive Order armed kidney patients with greater care choice, and reasserted patients into their rightful place as decision makers for their own care. We are certain that this bipartisan vision will continue under President Biden, because the tools and processes within the kidney ecosystem necessary to realize the vision were in place before 2019.

Over the past decade, the Food and Drug Administration (FDA), led by the FDA Center for Devices and Radiologic Health (CDRH), has pursued a purposeful strategy to capture, quantify, and value patient preference insights across multiple disease populations, and incorporate them into regulatory deliberations and decisions. Substantial efforts have been made to engage kidney patients so as to better understand patient burdens of disease and actual or perceived barriers to better health outcomes. Particular attention has been paid to issues of risk tolerance and acceptance levels among patients for new therapeutics and devices. The search for a "*P*" value for patient preferences, and insights on the trade-offs patients may be willing to make between benefits and risks of new therapeutics, is ongoing. How these data will be modeled to accelerate innovations in new KRTs is outlined in "Incorporating Patient Preferences *via* Bayesian Decision Analysis" (2).

The FDA knew that the government alone is not, nor can it be, the primary driver for changing status quo kidney care or improving outcomes through innovation. In 2012, the FDA utilized one of government's most powerful tools, the power to convene, and entered into a collaborative agreement with the American Society of Nephrology to launch the Kidney Health Initiative (KHI). In 7 years, KHI became the largest, fully representative collaborative in the kidney ecosystem. KHI formally included kidney patient advocacy organizations from the start, and its diverse membership includes multiple federal government agencies, established care providers, start-up enterprises, and nearly every pharmaceutical, device, and diagnostic company involved in kidney disease. KHI and FDA have leaned forward to provide those interested in developing the next generation of commercially viable KRTs with a detailed study on the path forward, or the "how," for innovation leaders *via* the KHI "Technology Roadmap for Innovative Approaches to RRT" (4). The roadmap, coupled with a highly inclusive, forthcoming major patient community preference survey, will

American Association of Kidney Patients

Correspondence: Paul T. Conway, Policy and Global Affairs, American Association of Kidney Patients, 6339 Crooked Oak Lane, Falls Church, VA 22042. Email: conwaypault@gmail.com

expand opportunities to embed insights from patient life experiences into the next generation of therapies envisioned by the 2019 Executive Order. The *CJASN* paper "Using Patient Preference Information to Inform Regulatory Decision Making: An Opportunity to Spur Patient-Centered Innovation in Kidney Replacement Therapy Devices" provides details on these strategic developments to benefit kidney patients and support the broader vision of disrupting the status quo (3).

In conjunction with KHI efforts, the FDA and CDRH have pursued an aggressive internal and external strategy to incorporate patient insights in their studies and formal decision making, as appropriate. The CDRH encourages innovation leaders to seek out and directly incorporate patient insights at the start of any new endeavor, far in advance of new submissions to the FDA. This is part of the larger CDRH plan to involve patients across the full product development life cycle, including clinical trial design, so that they are more representative of populations affected by disease. The *CJASN* paper "Integrating Patient Perspectives into Medical Device Regulatory Decision Making to Advance Innovation in Kidney Disease" discusses some of the specific steps the FDA has taken to engage and sustain kidney patient community and advocacy organization involvement.

FDA and KHI efforts have had significant spillover effects on efforts other federal agencies have launched to substantively incorporate kidney patient insights. The National Institutes of Health, National Institute on Diabetes and Digestive and Kidney Diseases has anchored much of the Kidney Precision Medicine Project (KPMP) on patient insights as experts in kidney disease and risk acceptance for clinical research, including elective biopsies. This includes patient involvement in formal decision mechanisms, including the KPMP Community Engagement Committee and the KPMP External Expert Panel that guides the project. At the Centers for Medicare and Medicaid Services and the Center for Medicaid and Medicaid Innovation, kidney patients are regularly enlisted to chair or serve on technical evaluation panels aimed at making dialysis quality measurements more relevant to patient decision making and consumer preferences. The Veterans Administration, a long-time leader in telemedicine, has demonstrated renewed efforts to engage kidney patients and share their insights with other federal agencies, as appropriate.

In practical terms, what does this research and scientific activity around patient preference, as chronicled across this group of *CJASN* papers, really mean for kidney patients, their caregivers, and the future of status quo kidney care? The power of the answer is clear in its simplicity. The lives of kidney patients, disproportionately from minority communities, will improve as a new generation of therapeutics and care options, shaped by the insights of patients like themselves, are made available. As authors, we believe a new generation of care solutions, developed with the science of patient insights, will drive diversification in care choices and a concurrent demand for coverage by payors, both federal and private, upon their approval by the FDA.

Unfortunately, for kidney patients caught in status quo kidney care, the pursuit of aspiration is severely limited by kidney disease itself. The disease can take a brutal toll on the human body and spirit, especially when kidneys fail, as *CJASN* readers who care for patients know first-hand. But to us, as the authors and national American Association of Kidney Patients advocacy leaders with decades of both dialysis and transplant experience, there are two factors that exacerbate the negative physiologic and potential psychologic effects that accompany kidney disease. First, many patients are put on care regimens, like in-center dialysis, without being asked what their aspirations are, or without having a complete discussion about which type of treatment will best position them to achieve their larger plans, like obtaining a kidney transplant or continuing their work. Second, status quo technologies severely limit the types of care presently available to home and in-center therapies, such as peritoneal dialysis and hemodialysis and kidney transplant (pre-emptive for those fortunate enough to find a donor ahead of dialysis). These technologies are over 50 years old and, apart from organ transplantation, have serious drawbacks to the freedoms necessary to live a truly active and full life.

At the American Association of Kidney Patients, we redefined ideal and quality kidney disease therapies as solutions that best enable patients to fully pursue their aspirations. Aspirations are not merely short-term goals: we mean the ability to pursue a career, travel, start a family, have children, own a home, retire securely, and live an active life despite having to manage a life-threatening disease. We take pride in the fact that our coauthors in this *CJASN* series are change agents working together to transform the kidney care status quo. Each has consistently welcomed kidney patients, including us, into their professional endeavors in highly substantive roles: not as spectators, but as expert collaborators whose insights are highly valued and respected. Through their efforts, and their allied networks, we are highly optimistic about the future of kidney care for all patients, especially those yet to be diagnosed.

Disclosures

P.T. Conway reports roles with the following entities: Chair, Patient Engagement Advisory Committee, US Food and Drug Administration; Co-Chair, Global Innovations in Patient-Centered Kidney Care Summit, American Association of Kidney Patients/George Washington University School of Medicine and Health Sciences; Board Member, Kidney Health Initiative; External Expert Panel, Kidney Precision Medicine Project (KPMP), National Institutes of Health/National Institute of Diabetes and Digestive and Kidney Diseases (NIH/NIDDK); Contract Management Board, NIH/NIDDK, United States Renal Data System (USRDS); Member, Nephrology Specialty Board, American Board of Internal Medicine; Member, Patient Advisory Board, Center for Dialysis Innovation, University of Washington; Moderator, KidneyX 2019 Summit; and Policy Stakeholder, Executive Order on Advancing American Kidney Health. R. Knight reports roles with the following entities: Member, Advisory Council, NIH/NIDDK; Co-Chair, Strategic Plan Stakeholder Engagement Subgroup, NIH/NIDDK; Co-Chair, Community Engagement Committee, KPMP, NIH/NIDDK; Member, Visiting Committee, Scientific Registry for Transplant Recipients; Member, Patient Advisory Committee, Network 5, Centers for Medicare and Medicaid Services; and

Policy Stakeholder, Executive Order on Advancing American Kidney Health.

Funding

None.

Acknowledgments

The authors extend their gratitude to Dr. Murray Sheldon, Associate Director for Technology and Innovation for the FDA, for the invitation to participate in this project and his sense of urgency to improve the lives of all kidney patients. We express our sincere appreciation to the professional experts and fellow contributors to this project; each are dedicated to fostering greater treatment innovation and care choice for kidney patients through the use of patient preference data. Finally, we remember the lives of our friends and fellow kidney patients whose courage and expertise contributed to the national pivot toward the application of patient preference science, before their own lives were ended by kidney disease. To Brian Hess, Bill Murray, Celeste Lee, Bill Peckham, Maile Robb, Angela Davis, Sonya Cochran, and Jack Reynolds—Fair Winds and Following Seas—your lives mattered, your work continues, and your legacy endures.

The content of this article reflects the personal experience and views of the author(s) and should not be considered medical advice or recommendation. The content does not reflect the views or opinions of the American Society of Nephrology (ASN) or *CJASN*. Responsibility for the information and views expressed herein lies entirely with the author(s).

References

1. Tarver ME, Neuland C: Integrating patient perspectives into medical device regulatory decision making to advance innovation in kidney disease. *Clin J Am Soc Nephrol* 16: 636–638, 2021 10.2215/CJN.11510720
2. Chaudhuri SE, Lo AW: Incorporating patient preferences *via* Bayesian decision analysis. *Clin J Am Soc Nephrol* 16: 639–641, 2021 10.2215/CJN.12110720
3. Flythe JE, West M: Using Patient preference information to inform regulatory decision making: An opportunity to spur patient-centered innovation in kidney replacement therapy devices. *Clin J Am Soc Nephrol* 16: 642–644, 2021 10.2215/CJN.11930720
4. Kidney Health Initiative: Technology Roadmap for Innovative Approaches to Renal Replacement Therapy, 2018. Available at: https://www.asn-online.org/g/blast/files/KHI_RRT_Roadmap1.0_FINAL_102318_web.pdf. Accessed September 11, 2020

Published online ahead of print. Publication date available at www.cjasn.org.

See related articles, "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," "Integrating Patient Perspectives into Medical Device Regulatory Decision Making to Advance Innovation in Kidney Disease," "Incorporating Patient Preferences *via* Bayesian Decision Analysis," and "Using Patient Preference Information to Inform Regulatory Decision Making: An Opportunity to Spur Patient-Centered Innovation in Kidney Replacement Therapy Devices," on pages 634–635, 636–638, 639–641, and 642–644, respectively.