

Importance of Patient-Reported Outcome Measures on Clinical Practice in Patients With Kidney Disease

Rebecca Frazier and John Devin Peipert



Patient-reported outcomes (PROs) are health assessments provided directly from patients that measure patients' health-related symptoms and experiences in order to inform health care delivery.¹ There has been increasing

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recognition of the importance of patient-centered care in nephrology. A primary goal of patient-centered care is to elicit patients' concerns and values with regard to their own health care and let these values guide clinical decision-making.² Research on patients with kidney disease shows that they often prioritize symptoms and functionality over laboratory measurements and clinical guidelines, which nephrologists have traditionally used to direct clinical care.³ Prior research indicates that nephrologists often do not comprehensively address patients' symptoms, in part due to clinicians underappreciating symptom severity and patients underreporting symptoms.⁴ Patient-reported outcome measures (PROMs) can be used in clinic to systematically assess patients' concerns and quantify their experiences. PROMs can monitor disease progression, assess symptoms, track side effects, and evaluate psychological well-being. When compared to usual clinical care, PROM use in clinics can enhance symptom management and improve patients' health-related quality of life (HRQOL).^{5,6}

PROMs can also be used to incorporate the patient experience in medical research. The Food and Drug Administration (FDA) has advocated for PRO use in supporting medical product claims in clinical research.¹ In particular, nephrotic syndrome has been highlighted by the FDA and National Kidney Foundation as a kidney disease that may be amenable to symptom assessments with PROMs, and they have called for the development of PRO endpoints for clinical trials assessing interventions for this syndrome.⁷ While there has been increased use of PROMs in the past decade, they are still underutilized in nephrology research and clinical care.⁸ Developing disease-specific PROMs that address patients' concerns and reflect clinical changes may expand PROM use in nephrology, optimize evaluation of new treatments, and ultimately improve health care delivery.

Focal segmental glomerulosclerosis (FSGS) and minimal change disease (MCD) are glomerular diseases that exist along one pathophysiologic spectrum. These diseases cause nephrotic syndrome, characterized by high levels of proteinuria and significant edema, and can lead to irreversible kidney damage.^{9,10} FSGS and MCD and their treatments can cause significant physical and psychological morbidity. Clinicians often use levels of proteinuria and serum creatinine to monitor disease status, but this approach fails to

capture the physical and psychological burden of the disease. While general PROMs exist for chronic kidney disease and disease-specific PROMs exist for adults with FSGS, no measure yet exists that evaluates HRQOL in the FSGS/MCD spectrum in both children and adults. As described in this issue of *Kidney Medicine*, Dr Carozzi and her colleagues are developing a comprehensive, disease-specific PROM to fill this void.¹¹ Compared to generic measures, disease-specific instruments are often more clinically relevant and better capture changes in clinical status, and thus may be used to monitor disease severity and guide treatment. In this manuscript, the authors complete the initial part of survey development, called domain development, to determine the symptoms and experiences that are important to patients as related to their kidney disease. This work, when developed into a PROM, will expand the available disease-specific PROMs for this debilitating disease.

The development of a PROM is complex and requires both qualitative methods and quantitative analysis for the instrument to be clinically useful. The first step in instrument development is to identify specific domains to be assessed based on the survey's objectives, the disease of interest, and the target population.¹² Central to domain development is ensuring content validity, which is the ability of the instrument to measure all relevant facets of the concept of interest.¹² The FDA advocates patient input in content validation so that the domains accurately reflect patients' experiences.¹ Once the domains are comprehensively described, PRO question generation can occur. The questionnaire is then statistically analyzed to ensure that the instrument has reliability and validity and is responsive to change. An instrument is reliable if patients respond the same way consistently over time. An instrument is valid if the instrument assesses what it is intended to measure. Finally, an instrument is responsive to change if the PROM scores improve when the patient clinically improves. While quantitative analysis of a new PROM helps ensure a robust instrument, in the absence of adequate content validation it becomes clinically meaningless. Ensuring that the instrument measures concepts meaningful to patients is essential.

In this manuscript, Carozzi et al¹¹ identify several domains that affect adults and children with FSGS and MCD. They broadly categorize HRQOL into physical health, mental health, and social health domains. In the physical health domain, patients reported significant medication side effects (particularly with steroids), difficulties with attending numerous medical appointments, and symptoms such as swelling, pain, and insomnia. Many of these symptoms are likely routinely assessed by physicians clinically as part of their nephrology care, such as

swelling and pain. However, some may be underappreciated, such as the burden of medical appointments. A PROM addressing physical health burdens associated with FSGS and MCD would comprehensively assess patients' experiences and allow physicians to better address these concerns. In addition, increased recognition of patients' side effects with medications may change prescribing practices, such as more reliance on steroid-sparing treatments, which may increase patients' HRQOL.

The authors also highlight the significant mental (including emotional) and social health burdens that these diseases inflict on patients. The researchers found that emotionally, most patients worried about how their kidney disease would affect their future and experienced feelings of sadness, depression, anxiety, and frustration. Socially, every patient interviewed had experienced decreased attendance at recreational activities due to their illness and felt that their disease affected their work or school life. Most also noted an impact on their relationships with family and friends. Patients' mental and social health may not be routinely explored by clinicians, as many physicians lack training in these areas and thus may focus more on physical symptoms or laboratory indicators of disease. However, these mental and social factors may affect patients' HRQOL more than physical symptoms. In addition, many of these issues may have long-lasting consequences, such as difficulties with school attendance and performance, more than physical symptoms, which are often transitory.

The use of disease-specific PROMs in FSGS/MCD to assess patients' HRQOL would give clinicians tools to evaluate the less-recognized factors associated with these diseases and allow for interventions. It would also give clinicians new endpoints to assess disease severity and treatment response, such as an overall social health score, that they could use in conjunction with more routine clinical assessments. However, addressing mental and social health in clinic may necessitate a shift in clinical practice patterns, as many nephrologists may not have the training or resources to address these issues in the clinical setting. This may include an increased reliance on multidisciplinary care, such as collaborations with social workers, case managers, and mental health professionals. It may also have implications for medical education, as physicians may need training on how to approach patient-centered care. These changes may be initially challenging, but they would allow for a more comprehensive approach to clinical care with improved patient outcomes.

ARTICLE INFORMATION

Authors' Full Names and Academic Degrees: Rebecca Frazier, MD, and John Devin Peipert, PhD

Authors' Affiliations: Division of Nephrology and Hypertension, Department of Medicine (RF); Center for Translational Metabolism and Health, Institute for Public Health and Medicine (RF), Northwestern University Feinberg School of Medicine, Chicago, Illinois; Jesse Brown Veterans Administration Medical Center, Chicago, Illinois (RF); and Department of Medical Social Sciences,

Northwestern University Feinberg School of Medicine, Chicago, Illinois (JDP).

Address for Correspondence: Rebecca Frazier, MD, Department of Nephrology, Northwestern University, 633 N. St. Clair, 18th floor, Chicago IL 60611. Email: rebecca.frazier@northwestern.edu.

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