

Establishing Meaningful Patient-Centered Research Outcomes for Patients with PKD

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INTRODUCTION Polycystic kidney disease (PKD) is an irreversible inherited disease that causes permanent worsening of kidney function. Despite being rare, PKD is the most common genetic cause of chronic kidney disease. We initiated “Meaningful Patient-centered Outcomes With Relevance for patients with PKD” (EMPOWER PKD) with PCORI funding to engage patients with PKD regarding: priority health outcomes, insurability (health and life insurance), and patient activation.

METHODS We utilized semi-structured focus groups. A pre-piloted guide allowed for both conversational flow and consistency in questions among groups. Six groups with seven to eleven participants lasted a duration of two hours. A multi-voting technique determined which patient-important outcomes were of highest priority. We audio-record each group and transcribed the conversations verbatim for thematic analysis using grounded theory.

RESULTS Fifty-seven people participated in the study. Of these, 46 people reported having PKD (82%), and seven people reported being caregivers (13%). The mean age of participants was 53.3 years (range 19–80 years). The diversity of participants included 91% white, 4% African American, 4% American Indian, 2% Latino, and 52% were female.

Six focus groups yielded 70 outcomes of importance that can be classified into six categories. The highest valued outcome was slowing the progression/symptoms of the disease followed by keeping kidneys healthy, and diet modifications. Patients were less concerned about mortality than they were about their kidney health.

CONCLUSIONS This project is a first step to ensure that the preferences of patients and families with PKD are acknowledged and used to inform research. Early diagnosis and detection are important topics, as are the effect of diagnosing PKD on insurability decisions. We learned this effect is a main factor that hinders patient activation in self-management. We will evaluate factors to determine the level of patient activation that will be key to patient involvement in future studies.

Patient Important Outcomes

