Patient and Other Stakeholder Engagement in Patient-Centered Outcomes Research Institute Funded Studies of Patients with Kidney Diseases


Abstract
Including target populations in the design and implementation of research trials has been one response to the growing health disparities endemic to our health care system, as well as an aid to study generalizability. One type of community-based participatory research is “Patient Centered-Research”, in which patient perspectives on the germane research questions and methodologies are incorporated into the study. The Patient-Centered Outcomes Research Institute (PCORI) has mandated that meaningful patient and stakeholder engagement be incorporated into all applications. As of March 2015, PCORI funded seven clinically-focused studies of patients with kidney disease. The goal of this paper is to synthesize the experiences of these studies to gain an understanding of how meaningful patient and stakeholder engagement can occur in clinical research of kidney diseases, and what the key barriers are to its implementation. Our collective experience suggests that successful implementation of a patient- and stakeholder-engaged research paradigm involves: (1) defining the roles and process for the incorporation of input; (2) identifying the particular patients and other stakeholders; (3) engaging patients and other stakeholders so they appreciate the value of their own participation and have personal investment in the research process; and (4) overcoming barriers and challenges that arise and threaten the productivity of the collaboration. It is our hope that the experiences of these studies will further interest and capacity for incorporating patient and stakeholder perspectives in research of kidney diseases.

Clin J Am Soc Nephrol • • • •, 2016. doi: 10.2215/CJN.09780915

Introduction
The notion that there is value in including members of the target population in the research team is not novel and some areas of medical research have a long history of patient involvement but it has been gaining broad scientific attention in the last decade (1,2). There are multiple terms and definitions used for this type of research. One of the most widely used terms is “community-based participatory research” (CBPR), which is often defined as a collaborative approach to research that equitably involves all partners in the research process and recognizes the unique strengths that each brings (3).

There are several factors that explain the increasing utilization of CBPR approaches in recent years (4). Researchers have begun to acknowledge that researcher-driven models have not successfully addressed the variety of health disparities that have become hallmarks of modern medicine. Furthermore, communities themselves are weary of being passive participants and are asserting their voices in setting the research agenda. With engaged partners, the process of disseminating the results back to the community is incorporated into the initial study framework, ensuring that the results have the highest potential impact and benefit for the community (5).

The term “Patient-Centered Research” can be used when a particular patient population is defined as the target CBPR community. Although coined several decades ago, the term achieved more widespread adoption following the Institute of Medicine’s landmark report Crossing the Quality Chasm (6). Specifically, patient-centered care is defined as:

“Health care that establishes a partnership among practitioners, patients, and their families (when appropriate) to ensure that decisions respect patients’ wants, needs, and preferences and that the patients have the education and support they need to make decisions and participate in their own care.”

Patient-centered research is primarily concerned with addressing the questions of most importance to patients and enhancing decision making through the generation of evidence that is reliable and actionable for a patient’s unique circumstances. Traditional randomized controlled trials are costly, slow, and produce results that are often difficult to translate to routine practice (7–9). There is need for more
Defining the Role of Patients and Other Stakeholders in Research

The first step to incorporating patient and stakeholder input into the research program is to have a clear vision of their desired roles. In the funded nephrology studies, patients’ roles even within their contributions to the study design were quite diverse, from providing data to helping develop meaningful power calculations, to reviewing the study protocol, to being active participants in the development of study questions and methodologies (Table 2). A major challenge to meaningful incorporation of patient and other stakeholder input is the usually tight timeline required for grant submission. Some of the studies had previous ongoing relationships with groups of patients and some were formed de novo for the purposes of the application. While all of the studies incorporated the patient perspective, the specific methodologies and scope of that involvement varied across studies.

Illustrative Example of the Scope of Patient Involvement (Hynes study)

In the Bringing Care to Patients: A Patient-Centered Medical Home for Kidney Disease study, the patient is emphasized as the key member of the healthcare team (17). Notably, patients and other stakeholders are invited to participate in quarterly discussion groups at each dialysis center to guide implementation of the intervention. The participants’ input helps the research team plan, implement, and evaluate the new care model, particularly with respect to availability of the primary care physician, pharmacist, and health promoters during dialysis treatment. Their input also helps researchers to gain insight into patient and family member educational needs and interests.

Illustrative Example of the Scope of Patient and Stakeholder Involvement (Mehrotra study)

During the early planning phase, conference calls were held with patient groups and dialysis providers in order to receive their input in developing the essential elements of the study design. In practice, both Patient and Stakeholder Councils contributed to the overall study design, and assisted in the development of each intervention and the selection of primary and secondary outcome measures. The Patient Council assisted the coinvestigators in developing a semistructured interview to ascertain study participants’ experiences with each intervention upon completion of participation. The dialysis provider partners facilitated communication with facility administrators, medical directors, and nephrologists in participating dialysis facilities to ensure smooth implementation of the protocol without interfering with clinical care.

Illustrative Example of the Roles of Patient and Stakeholder Involvement (Cope study)

The NephCure Kidney Network (NKN), was established to address critical gaps in primary nephrotic syndrome research infrastructure by its development as a patient-powered research network. The framework for patient and family engagement in health care was adapted from Carman et al. (18) to achieve both depth and breadth of involvement from the nephrotic syndrome community in creating and sustaining the NKN. In a patient-powered research network, control of the research process is held largely by patients in combination with other stakeholders, including caregivers, advocates, clinicians, and researchers. The active involvement of the patient perspective in the network ensures a central role for patient participation in governing the network and its uses, identifying and prioritizing research questions, recruiting patients to participate, defining data elements for
Table 1. Studies funded by PCORI through March 2015 targeting patients with kidney diseases

<table>
<thead>
<tr>
<th>ID</th>
<th>Study Title</th>
<th>Primary Investigator(s)</th>
<th>Primary Goals of Study</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Shared Decision-Making and Renal Supportive Care</td>
<td>Lewis Cohen/ Mark Unruh</td>
<td>Aim 1: Is palliative care team intervention effective in improving nursing home residents’ end-of-life outcomes, such as pain, shortness of breath, in-hospital deaths, hospitalizations, and presence of advance directives? Aim 2: Is palliative care team intervention effective in improving nursing home staff end-of-life care processes and outcomes, such as assessment of symptoms, skills in delivering care, communication, teamwork, and satisfaction?</td>
</tr>
<tr>
<td>2</td>
<td>NephCure Kidney Network for Patients with Nephrotic Syndrome</td>
<td>Elizabeth Cope</td>
<td>Aim 1: Establish, implement, and maintain the infrastructure for the NKN for patients with primary nephrotic syndrome, a Patient-Powered Research Network and member node of PCORnet. Aim 2: Establish and execute a long-term sustainability plan for maintaining the NKN and its ancillary research program as a resource for clinical research of most importance to patients with these rare and devastating diseases.</td>
</tr>
<tr>
<td>3</td>
<td>Improving Patient Quality of Life and Caregiver Burden by a Peer-Mentoring</td>
<td>Nasrollah Gahramani</td>
<td>Hypothesis 1: Peer mentoring will lead to improved health-related quality of life among patients with CKD. Hypothesis 2: Peer mentoring will lead to improved engagement among patients with CKD. Hypothesis 3: Peer mentoring will lead to improved caregiver burden among caregivers of patients with CKD.</td>
</tr>
<tr>
<td>4</td>
<td>Bringing Care to Patients: A Patient-Centered Medical Home for Kidney Disease (PCMH-KD)</td>
<td>Denise Hynes/Michael Fischer/Anna Porter/Marian Berbaum/Jose Arruda</td>
<td>Aim 1: Establish processes for patient and stakeholder input to develop and refine the care model, project processes, and PCMH-KD training curricula. Aim 2: Implement a new PCMH-KD care model at two dialysis centers over a two year intervention period using a quasi-experimental design. Aim 3: Evaluate and compare the effectiveness of the PCMH-KD model to the current CMS-mandated care model for primarily improving patient and caregiver reported outcomes and avoidable healthcare use.</td>
</tr>
<tr>
<td>5</td>
<td>Treatment Options for Depression in Patients Undergoing Maintenance Hemodialysis (ASCEND)</td>
<td>Rajnish Mehrotra/ Susan Hedayati/ Daniel Cukor</td>
<td>Hypothesis 1: An engagement interview increases the acceptability of treatment for depression in hemodialysis patients. Hypothesis 2: There is no significant difference in the efficacy of individual cognitive behavioral therapy or anti-depressant drug therapy for depressed hemodialysis patients.</td>
</tr>
<tr>
<td>6</td>
<td>Reducing Health Disparity in CKD in Zuni Indians</td>
<td>Vallabh Shah</td>
<td>Hypothesis 1: The Zuni Health Initiative can integrate an innovative approach to home-based kidney care utilizing tribal Community Health Representatives, Point of Care technology, telemedicine, and text messaging in conjunction with patient preferences and Patient Activation Measures into the chronic care model to improve the detection and treatment of CKD and related risk factors. Hypothesis 2: This model is generalizable to other high-risk communities, e.g., rural New Mexicans served by ECHO, and American Indians in Guadalupe, AZ being studied by NIDDK, NIH-Phoenix.</td>
</tr>
</tbody>
</table>
the authors concluded that identi-
cation are needed prior to engagement. In a qualitative
research question and whether formal training and edu-
cation of relevant literature about stakeholder engagement and inter-
views with research leaders, Guise et al. (20) concluded that explicit and consistent use of terminology about stakeholders
was largely absent. The context and scope of the research
topic will, to a great extent, determine the de-
velopment of the target audience and continued repeated
engagement, and sought different depths of engagement
depending on a variety of factors including where they
were in their own “patient journey”, their own health, and
perceived ability to contribute meaningfully. To accommo-
date varying patient preferences, it is suggested that pa-
patients and other stakeholders should have the opportunity
to be involved in structured roles independently through-
out the continuum of engagement (consultation, involve-
ment, and shared leadership) rather than encouraging all
patients to seek shared leadership. This approach allows
patients to adjust the depth of their involvement over time
to fit their own needs.

Identification of Relevant Patient and Other Stakeholder Participants

Once the proposed roles of patients and stakeholders are
defined, the task of identifying and recruiting the specific
patients and stakeholders begins (19). In an extensive review
of relevant literature about stakeholder engagement and inter-
views with research leaders, Guise et al. (20) concluded that explicit and consistent use of terminology about stakeholders
was largely absent. The context and scope of the research
topic will, to a great extent, determine the definition of each
specific stakeholder, as well as the roles of the individual
stakeholders at various stages of the research.

The next step in the process of stakeholder engagement is
to determine baseline knowledge about the preliminary
research question and whether formal training and edu-
cation are needed prior to engagement. In a qualitative
study of patients who had been engaged as stakeholders,
the authors concluded that identification of the most
appropriate stakeholders (characterized by confidence, mo-
tivation, intelligence, and focus on helping others) is
more important than the type of training they receive (21).
As part of the paradigm shift to early engagement of stake-
holders, it seems appropriate to involve the stakeholders in
the decision about the type and extent of training they de-
sire. Desired engagement plans include early needs assess-
ment of the target audience and continued repeated
involvement of stakeholders (22,23).

Depending on the exact nature of the study, it may be
particularly important to include perspectives from patient
groups that have not traditionally been included in re-
search (such as minority, elderly, and frail). Most of the
nephrology studies have a formal Patient Council that
interacts regularly with study leadership. Beyond that,
studies utilized a variety of venues and modalities to solicit
patient input, including direct survey of patient represen-
tatives, open solicitation of comment from target patient
populations, national advocacy groups, tribal councils, and
through clinical staff.

Illustrative Example of Patient and Stakeholder Identification (Cohen study)

Although the idea of inviting representatives from the
national kidney patient advocacy organizations to be part
of the project was initially entertained, it was decided
instead to recruit patients and families from the clinics that
would be studied. Two Patient Advisory Boards were
created that were involved in all aspects of the project,
including the two research studies. A Stakeholders Ad-
visory Board was also instituted whose membership in-
cluded representatives from the three proprietary dialysis
organizations that operate the clinics, hospice administra-
tors, dialysis nurses, a nephrologist, and social workers.

Illustrative Example of Patient and Stakeholder Identification (Tentori study)

Study investigators worked closely with a stakeholder
advisory panel that was comprised of nine patients and
family members, clinical nephrologists, social workers, and
representatives from patient advocacy organizations. Given
that priorities and needs are different for various patient
types, they ensured that patient representatives included a
range of ages, employment status, and experience with
dialysis modalities. Patient members were highly functional
patients, with a high level of involvement in their own
health care decisions and often engaged in other research
or education programs. The entire council collaborated in
developing and testing interview protocols, and were key in
developing the content and layout of the study’s decision
aid. Throughout the study, the stakeholders provided in-
sight on how best to implement the study, as well as de-
tailed feedback on the focus of analysis of results.

<table>
<thead>
<tr>
<th>ID</th>
<th>Study Title</th>
<th>Primary Investigator(s)</th>
<th>Primary Goals of Study</th>
</tr>
</thead>
<tbody>
<tr>
<td>7</td>
<td>Empowering Patients on Choices for Renal Replacement Therapy (EPOCH-RRT)</td>
<td>Francesca Tentori</td>
<td>Aim 1: Identify outcomes most important to kidney patients with different characteristics. Aim 2: Compare the impact of hemodialysis and peritoneal dialysis on patient-centered outcomes. Aim 3: Compare measures related to the decision-making process between patients receiving and not receiving a tailored decision aid.</td>
</tr>
</tbody>
</table>
### Table 2. Examples of patient engagement across the different studies

<table>
<thead>
<tr>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Shared Decision Making and Renal Supportive Care (SDMRSC)</td>
<td>Patients undergoing hemodialysis and families of patients.</td>
<td>Referred by social workers or clinicians.</td>
<td>12</td>
<td>Initially monthly, then quarterly. Reply to the queries from investigators and partner in development of study goals, methodology and dissemination plan.</td>
<td>They were most involved in the planning process, but remain important sources of input.</td>
<td>No</td>
<td>Representatives from dialysis provider organizations, hospices, nephrologists, and dialysis social workers.</td>
</tr>
<tr>
<td>2</td>
<td>NephCure Kidney Network for Patients with Nephrotic Syndrome (NKN)</td>
<td>Adult patients and parents of minors with primary nephrotic syndrome (n=6) and patient advocates (n=4).</td>
<td>Call for nominations through advocacy organization’s website.</td>
<td>10</td>
<td>Weekly e-digest; monthly steering committee calls; monthly workgroup calls. Codevelop policies, operational plans; aid in implementation (peer-to-peer recruitment); review ancillary research projects; aid in communications and dissemination.</td>
<td>Roles did not change, but degree of involvement did. Originally, Steering Committee members also were required to be on ≥1 workgroup, modified approach to route interested patients to different roles (governance versus implementation).</td>
<td>No</td>
<td>Practicing clinicians (n=3), researchers (n=3), representatives from industry (n=2).</td>
</tr>
<tr>
<td>3</td>
<td>Improving Patient Quality of Life and Caregiver Burden by a Peer-Mentoring Program for patients with CKD and Their Caregivers</td>
<td>Patients with CKD (stage ≥4 or ≥5) and caregivers.</td>
<td>Referred by social workers or clinicians.</td>
<td>5</td>
<td>Initially in focus group setting (for proposal review); during conduct of the study, within the context of periodic meetings. Review the proposal; serve as members of the community advisory board (oversight of conduct of study) and members of the Patient and Caregiver Advisory Group.</td>
<td>No</td>
<td>Representatitives of patient advocacy organization;pliers; representatives of hospital administration; representatives of healthcare organizations.</td>
<td></td>
</tr>
<tr>
<td>-----</td>
<td>-------------------------------------------------</td>
<td>-----------------------------------------------------------------------------------------------</td>
<td>--------------------------------------------------------------------------------------------------</td>
<td>-----------------------------------</td>
<td>-----------------------------------------------------------------------------------------------------------------</td>
<td>-----------------------------------------------------------------------------------------------------------------</td>
<td>-------------------------------------------------</td>
<td>-----------------------------</td>
</tr>
<tr>
<td>4</td>
<td>PCMH-KD Health System Intervention</td>
<td>Patients with ESRD receiving hemodialysis at one of two outpatient dialysis facilities and their caregiver/family member.</td>
<td>Patients and caregivers recommended and/or who volunteered.</td>
<td>Varied quarterly, ranging from 4–7 per session.</td>
<td>Stakeholder groups led by a trained facilitator 3–4 times per year. Patient representative met with the study team on an ad hoc and regular basis.</td>
<td>Stakeholder discussion group participants shared experiences during the moderated discussions, reviewed study protocol documents, met with Project Coordinator, and suggested format changes for the discussion group.</td>
<td>Modified format of discussion groups to obtain feedback to report to dialysis center management.</td>
<td>Clinicians, representatives of dialysis center, and Service Chief.</td>
</tr>
<tr>
<td>5</td>
<td>ASCEND Trial</td>
<td>Patients from dialysis facilities and national advocacy organizations for patients with kidney disease.</td>
<td>Study leadership reached out to national organizations and patients in target dialysis facilities.</td>
<td>9</td>
<td>Patient council phone calls, facilitated by one of study PIs, and one of the patients served as the head of the council.</td>
<td>Council was most active in the design phase of the trial, then frequency of meetings decreased.</td>
<td>Represents Dialysis Providers Organizations, NIDDK, Council of Nephrology Social Workers, American Nephrology Nurses Association, National Renal Administrators Association.</td>
<td></td>
</tr>
<tr>
<td>6</td>
<td>Zuni Health Initiative – Home-Based Kidney Care</td>
<td>Patients are from the community of Zuni Pueblo.</td>
<td>Tribal governor and his council members, leaders from community as part of TAP and Zuni IHS clinician.</td>
<td>Zuni Governor and his council (9 members). TAP includes 5 community stakeholders</td>
<td>Group sessions with patients every 3 months. Patient preferences in kidney care: compliant to lifestyle changes; exercise and nutritional regimen and adherence to prescribed medicine.</td>
<td>None</td>
<td>Zuni Tribal Governor and his council; TAP; Zuni IHS hospital; Zuni wellness Program; NIDDK Phoenix; ECHO program.</td>
<td></td>
</tr>
</tbody>
</table>
Illustrative Example of Patient and Stakeholder Identification (Ghahramani study)

The researcher had a long-standing relationship with a local patient advocacy organization. As a member of the Board of Directors of the organization and a long-time instructor for the organization’s patient and caregiver mentoring program, he transformed a meaningful partnership into the base of the advisory council. A patient who also served on the board was engaged to provide the patient’s perspective on the research question. Similarly, a dialysis social worker, who worked closely with the advocacy organization and cared for patients on a regular basis, was in a unique position to serve as a research consultant drawing on long standing awareness of the day-to-day problems of patients with ESRD.

In dialogues with patient representatives from across the studies, one of the themes that emerged was that were more likely to provide input if they felt their contribution was valued. The patient representatives were more responsive if they could see their input incorporated into the study. The specific mechanism in which patient contribution is shared with study leadership should be explicitly stated and the reactions to the suggestions should also be patients shared. The more transparent the dialogue, the more likely the patients were to feel empowered in their roles. Furthermore, it would appear that having a specific individual from the study team serve as the liaison to the patient representatives serves to unify the experience for the patients. That person can, but need not, be the Principal Investigator for the study, but should be someone empowered to represent the research team to the patients, and the patient council to study leadership.

Challenges of Patient and Other Stakeholder Engagement

The stages of stakeholder engagement in research include: identification of the relevant stakeholders, definition of their roles, engagement, and maintaining engagement (14). There are both patient and system level barriers to effective engagement. The contextual factors in patient/stakeholder engagement are numerous and complex and include individual characteristics (e.g., age, self-efficacy, and literacy), disease characteristics (e.g., perception, stigma/taboo, comorbidities, and treatment demands), characteristics of the setting (e.g., type of provider, data driven care, incentives, information availability), and cultural/contextual norms. A common challenge is maintaining stakeholder engagement throughout the course of the study. In a qualitative analysis of multistakeholder projects in The Netherlands, patient involvement was rarely sustained (24). Determinants of involvement of patients and stakeholders include patient interest, attitude of the clinician, and insight about appropriate methods (25). Trust, usually based on prior relationships, is a key factor in maintaining engagement. For example, a researcher’s ties with a patient advocacy organization can greatly facilitate engagement of that organization in various stages of the proposed research. The trust and working relationship between the researcher and the advocacy organization is likely to generate trust with patients and other stakeholders such as caregivers. Some of the challenges

<table>
<thead>
<tr>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>7</td>
<td>EPOCH-RRT</td>
<td>Patients and family members</td>
<td>9</td>
<td>Regular meetings, conference calls, and emails with patient advisory council</td>
<td>Served as a key component to inform the research and provide constructive feedback to the design and utility of study materials. Helped train interviewers.</td>
<td>None</td>
<td>Health care providers.</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

Table 2. (Continued)
in engaging stakeholders include: lack of a shared language with investigators and limited research training on the part of the patients; resistance of researchers toward questions generated by nonscientists; the optimal timing and level of stakeholder involvement; and ambiguities about ethical aspects of the engagement process. There are special ethical considerations in community-based research, and these issues may be further complicated if the “community” is not one in the traditional sense of the word, but rather a group of disparate people brought together by sharing a common illness.

Illustrative Example of the Challenges Associated with Patient Participation (Cohen study)

In preparing for the first meeting of the advisory board, the project coordinator made repeated contact with the seven potential members, proposed a mutually acceptable annual honorarium, discussed in detail how to participate in the inaugural conference call, and after considerable negotiation arrived at a mutually acceptable time. However, at the agreed upon hour, only two patients called in. During the ensuing conversations, it became apparent that all of the patients and families wished to take part in the project but none of them had ever participated in a conference call. Even members who were comfortable with computer technology found this daunting, unsure of how to get on the call and were unclear as to what to say or not say. They required practice, individual support, and further explanations before each was prepared to try again. For the next scheduled conference call, it was gratifying when six out of the seven members called in on time.

Illustrative Example of Patient and Stakeholder Barriers (Hynes study)

A key challenge for patient participation in the study’s patient council was due to the nature of the disease and its general toll on patients and their family members. For example, confirming attendance for a planned discussion group and arranging transportation was time consuming, and often uncertain in guaranteeing a quorum in advance of the session. The investigators also learned that ESRD patients’ attendance could be uncertain due to variability in disease progression, symptom exacerbation, and complications. Because of this, about 25% more patients were invited than were expected to attend a given session. Having a large percentage of patients who were primarily Spanish speaking was another important consideration. The investigators planned for bilingual staff and separate English and Spanish sessions, and held a session in both Spanish and English to accommodate as many patients as possible.

It has become clear that there cannot be a “one size fits all” model for stakeholder engagement. While it is useful to share models found to be successful in different situations, specific research situations often present different engagement challenges. Strategies for engaging patients and other stakeholders must incorporate an understanding of the unique environments in which patients exist and the potential barriers these environments pose and address them systematically.

Benefits of Patient and Other Stakeholder Engagement

Constructing a base of patients and other stakeholders who are ready to actively participate in multiple phases of the research process is a challenging and time consuming endeavor. However, we believe it is also one that will tangibly benefit the research and often the researcher. Successful engagement can provide the research team an opportunity to share in colearning, empathy, trust, and shared values with their patient populations, and provide them with a new perspective on the importance of the work. While we do not yet have empirical data on the ways patient input has affected health outcomes, we do have many examples of specific contributions made to the studies.

Illustrative Example of the Benefits of Other Stakeholder Involvement (Mehrotra Study)

After receiving the contract to evaluate treatment options for depression, but before the study was implemented, final approval from the dialysis provider organizations was needed. As the study proposed some novel treatment delivery options, the dialysis provider organizations did not have policies in place to address some of the needs. Their final approvals were secured in part due to the advocacy of their leadership representatives who had been part of the study calls during the planning of the study. Investigators were convinced that having the buy-in and input from the stakeholders from the start of the study was the key factor in their motivation to find creative solutions to accommodate the study at their respective sites.

Illustrative Example of the Benefits of Patient Involvement (Tentori Study)

Only patients and their caregivers have direct experience of living with kidney disease; consequently, investigators found the input from the advisory panel was invaluable in implementing each step of the study. As an example, the advisory panel provided extensive feedback on the protocol for qualitative studies, interview scripts, and data interpretation. The panel had strong opinions and was extremely vocal in the design and content development of the decision aid, to ensure that all aspects of the decision aid would be appropriate for the target audience of predialysis CKD patients and their caregivers.

Illustrative Example of the Benefits of Patient Involvement (Shah Study)

During the first phase of a pilot study by the Zuni Health Initiative, community members were engaged to identify perceived barriers to healthy lifestyles and healthcare utilization. Structural barriers that were identified included: the difficulty and expense of traveling to and from appointments; the difficulty of recruiting and retaining qualified healthcare providers; the perceived misdiagnosis or late diagnosis of disease; the perceived rationing of healthcare services; language barriers; and limited patient education. Additional psychologic barriers included anxiety; fear of chronic disease; reluctance to participate in self-care; resistance to dietary change; and reluctance to engage in regular exercise. With this
information, the research team had a fuller appreciation of the challenges they would face in the implementation of their intervention.

Benefits and Challenges of Patient Stakeholder Involvement – Patient Perspective

Many of the patient representatives had very positive reactions to their time spent collaborating on the research projects. The representatives listed benefits to the studies due to their contributions, but also listed many personal benefits. One of the themes that emerged was the sense of empowerment that the study provided. Many patients feel overwhelmed by their illnesses and that their control over its course and prognosis is poor. However, being involved in research allows them to feel that their illness experience has more meaning if it can affect the larger community.

The most discussed challenges were finding time to prepare and participate in between the patients’ medical demands and doctors’ appointments. Beyond the scheduling two other themes emerged – the need for appropriate orientation and the need for transparency. One representative commented, “As the only lay person on the team and being the newcomer to a group of colleagues is a challenge. Fish out of water! Learning the research and medical lingo, with all their acronyms is a huge challenge!” An orientation session providing the background of the research team, its goals, and some of the relevant medical terminology, would have circumvented some of these difficulties. A different patient representative commented that she did not understand how the group was using her suggestions. “Am I a true partner here, and credit will be shared, or am I more of a consultant working in a think tank?” she wondered. Clear expectations set out at the beginning of the formation of the input mechanism are required for everyone to feel valued.

Discussion

Our collective experience suggests that successful implementation of a patient- and stakeholder-engaged research paradigm involves: (1) defining the roles and process for the incorporation of input; (2) identifying the particular patients and stakeholders to build a foundation of key stakeholders who appropriately represent the target treatment population and its caregivers; (3) engaging patients and stakeholders so they appreciate the value of their own participation and have personal investment in the research process; and (4) overcoming barriers and challenges that arise and threaten the productivity of the collaboration. Engaging patients may include educating them so they understand the research process and how it is connected to changes in clinical practice, and are therefore equipped to make meaningful contributions (31). Foundations of trust and mutual respect are key elements to being able to successfully navigate all of the foreseeable and unforeseeable challenges that present themselves over the course of a collaboration that may span many years and involve a great diversity of people engaged in the research. The numerous examples of patient engagement in research presented here suggest a growing movement within medicine in general, and specifically in those treating kidney disease.

We believe that the nephrology research community could serve as a model for implementing the ideals of CBPR and patient-centered methodologies. The treatment and research teams often develop long-term meaningful relationships with their patient populations due to the severity of the illness, its chronicity, and the substantial time burden associated with treatment. These relationships form the foundation of meaningful collaboration. This focus on patient engagement may be particularly meaningful in communities that have been traditionally disenfranchised by medical research. As there are many health disparities in renal disease, patients with kidney diseases from underserved communities may be particularly motivated to have a voice in their research and clinical agenda. There are similar advantages in engaging with dialysis provider organizations early in the research process. As dialysis centers are independent entities, it has been a logistic challenge for researchers to recruit enough patients to participate in trials. The meaningful involvement of provider organizations may also help the generalizability of the study, as it will aid in the implementation, dissemination, and sustainability of study findings. Researchers who wish to engage in stakeholder-engaged research must be genuinely open to incorporating perspectives that are legitimately different than their own, to overcoming any preconceived biases about the importance of this input, and must be able to lead a team that is more diverse than most research teams.

By working together to develop a culture of patient-centered research, we can improve and refine the selection of research questions and outcomes to be studied, enhance accrual and participant retention, and more appropriately disseminate and implement changes in clinical management identified by the research. Kidney research benefits from expanding the potential funders and from its successes. It is our hope that the innovations introduced by PCORI funded research will help patients and families and stimulate further interest in research and participation in kidney disease.

Acknowledgments

This work was (partially) supported through distinct PCORI Awards to L.M.C., E.L.C., N.Ghaemmaghami, D.M.H., R.M., V.O.S., and F.T. D.M.H. is also supported by a US Department of Veterans Affairs Health Services Research and Development Service Research Career Scientist Award (RCS-98-352).

All statements in this report, including its findings and conclusions, are solely those of the authors and do not necessarily represent the views of PCORI, its Board of Governors, or Methodology Committee. The opinions expressed in this paper do not necessarily reflect those of the National Institute of Diabetes Digestive and Kidney diseases, the National Institutes of Health, the Department of Health and Human Services, the Department of Veterans Affairs, and the government of the United States of America.

We would like to thank all of the patient stakeholders that contributed to the development of this manuscript and make our shared research agenda possible.

Disclosures

L.S. and F.T.: The Empowering Patients on Choices for Renal Replacement Therapy project uses data from the Dialysis Outcomes and Practice Patterns Study (DOPPS), an observational study conducted by Arbor Research Collaborative for Health. The DOPPS...
Program is supported by Amgen, Kyowa Hakko Kirin, AbbVie Inc., Sanofi Renal, Baxter Healthcare, and Vifor Fresenius Medical Care Renal Pharma, Ltd. Additional support for specific projects and countries is also provided in Canada by Amgen, BHC Medical, Janssen, Takeda, Kidney Foundation of Canada (for logistics support); in Germany by Hexal, German Society of Nephrology, Shire, WiNe Institute; for Peritoneal Dialysis Outcomes and Practice Patterns Study in Japan by the Japanese Society for Peritoneal Dialysis. All support is provided without restrictions on publications. All research grants are made to Arbor Research Collaborative for Health and not to individuals directly. M.U. receives general research support from Dialysis Clinic Inc.

References


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

This article contains supplemental material online at http://jcn.asnjournals.org/lookup/suppl/doi:10.2215/CJN.09780915/-/DCSupplemental.