



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

**PCORI Funded Projects:
Sample Engagement Plans from
Methods Portfolio**

August 6, 2014



About PCORI

PCORI is committed to transparency and a rigorous stakeholder-driven process that emphasizes patient engagement. PCORI uses a variety of forums and public comment periods to obtain public input to enhance its work.

Our Mission: PCORI helps people make informed healthcare decisions and improves healthcare delivery and outcomes by producing and promoting high-integrity, evidence-based information that comes from research guided by patients, caregivers, and the broader healthcare community.

Our History: PCORI was authorized by the Patient Protection and Affordable Care Act of 2010 as a nonprofit, nongovernmental organization. PCORI’s purpose, as defined by the law, is to help patients, clinicians, purchasers, and policy makers make better informed health decisions by “advancing the quality and relevance of evidence about how to prevent, diagnose, treat, monitor, and manage diseases, disorders, and other health conditions.”

The statutory language defining PCORI is broad and authorizes research that will support a strong patient-centered orientation, inform better choices among alternative treatment and prevention strategies, and direct attention to individual and system differences that may influence strategies and outcomes. PCORI was designed to produce knowledge through the analysis and synthesis of existing research and the support of new research.

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PCORI Funded Projects: Sample Engagement Plans



Introduction

PCORI seeks to support research that includes meaningful involvement of patients and other stakeholders in all the steps of research. From time to time, PCORI is asked for information about patient and stakeholder engagement plans. PCORI recognizes that patient and stakeholder engagement plans can take multiple forms and will vary depending on the nature of the research, including the hypotheses, design, conduct, and dissemination of the research.

To enhance understanding of different models of patient and stakeholder engagement, we have selected sample engagement plans taken from actual funded projects in our Improving Methods for Conducting Patient-Centered Outcomes Research portfolio. To ensure privacy, the names of patient and stakeholder partners have been removed. However, we are committed to recognizing the valuable work of PCORI patient and stakeholder partners and look forward to publicly featuring that work in other ways.

These engagement plans are provided solely as examples for educational purposes; they do not reflect all engagement and stakeholder plan models, and do not reflect PCORI's endorsement. Incorporation of similar engagement plans in a research proposal will not guarantee funding of the proposal. PCORI may update this resource from time to time.

—PCORI Engagement Team
August 6, 2014



Engagement Plan A:

A Method for Patient-Centered Enrollment in Comparative Effectiveness Trials: Mathematical Equipoise

PUBLIC ABSTRACT

Comparative effectiveness research (CER) provides evidence about what treatments are best for which patients. In CER, comparative effectiveness randomized clinical trials (RCTs) are the gold standard of evidence. But patient enrollment is challenging, so RCT samples are often too small and not representative of usual patients, and many needed trials are never done. Thus, there are major gaps in good evidence to inform care decisions.

Objectives: This project aims to further develop our method for patient-centered enrollment in comparative effectiveness RCTs. Intended to be embedded in electronic health records (EHRs) in usual clinical settings, its objective is to identify patients for whom, based on their individual characteristics, there is insufficient evidence to favor one treatment, called “clinical equipoise,” the ethical and scientific basis for enrolling a patient in an RCT. “Mathematical equipoise” (ME) uses mathematical models to predict patient-specific outcomes of treatment options for comparison. When the predictions are equivalent, suggesting equipoise, enrollment in an RCT that compares the treatments can be considered. When the predictions suggest one treatment is likely best, trial enrollment would be inappropriate, but the predictions can inform decision making. In creating the mathematical predictive models for our ME prototype for heart attack, we used RCT data, which are ideal for making predictive models. But for many treatments, there are no previous RCTs—the very situation for which RCTs are needed, which ME could support. Thus, for widespread use of ME, predictive models will need to be built on data from clinical registries, EHRs, and other non-RCT data. Thus, this project will further develop the ME method by applying it to an important clinical treatment question for which there are no previous RCTs, total knee replacement (TKR) versus nonsurgical treatment for knee osteoarthritis (OA).

Methods: First, we will create a consolidated database from non-RCT sources on knee OA outcomes that will allow creation of predictive models for TKR and nonsurgical treatments. We will include views and needs of stakeholders for decision support for knee OA, to capture key pain and physical function outcomes. We then will develop multivariable mathematical models that predict patient-specific outcomes of TKR and nonsurgical treatment, adjusting for the inherent differences in the component databases. Also, we will further develop the ME decision support user interface to ensure that it is responsive to the needs of patients and physicians and that it supports their shared decision making for both RCT enrollment and treatment.

Anticipated Impact: Development of the ME method so that it can address important clinical problems for which RCTs have not been done—especially when ultimately widely incorporated in EHRs—should



greatly enhance the number, scope, and impact of patient-centered comparative effectiveness trials.

BRIEF SUMMARY OF ENGAGEMENT PLAN

We recognize that engagement of patients and stakeholders in research design and conduct is a key and innovative strategy for ensuring that the research question and the conduct of the research remain patient-centered. For this reason patients, patient advocates, healthcare providers, researchers, and EHR experts participated in the design of this proposal. Our organizational structure incorporates a stakeholder panel that will participate in the proposed research, adding relevance and transparency and informing the study design and conduct, and the analysis, interpretation, and dissemination of the project results. Our use of the stakeholder panel builds on a framework for engaging stakeholders in research recently published by the Project Director with other researchers and national thought leaders. Our stakeholder panel was designed to include six patient and patient advocate stakeholders representing people at risk for knee OA because of existing OA in other joints, people actively considering treatment options for their existing knee OA, and patients who had TKR for OA. Because two of our current members are actively considering treatment options at the time of grant submission, we will recruit two additional members based on the composition of the group when the project begins. The stakeholder panel also includes seven providers such as rheumatologists, primary care physicians, orthopedic surgeons, and a physical therapist who currently care for knee OA patients, some of whom will have a dual role in representing researchers. Involvement of the stakeholder panel in the study design and overall project is detailed within each aim below and then summarized further below.

COMMITMENT TO PATIENT/STAKEHOLDER ENGAGEMENT

To develop this project we began by identifying and interviewing four stakeholder groups: 1) patients with or at risk of having knee OA, 2) arthritis patient advocates, 3) clinicians, and 4) researchers. We also recognize the role of other stakeholders such as payers and policy makers and will make every attempt to involve them in the dissemination of the study results and future ME studies.

Patients: This study has relevance to patients with knee OA who face repeated decisions about their treatment because of the chronic and progressive nature of the disease and who might benefit from decision support or value participation in an RCT. We sought out a number of people with OA to discuss the proposed project and determine how the study could be most relevant to their needs. Our choice of comparators and outcomes, improvement in physical function, and pain relief reflects the primary concerns of the patients we interviewed, which is supported by the literature.

“It comes down to a final decision about quality of life. It would be helpful to have more information about what’s ahead for me.” “I have OA in other joints so I know I’m at risk for problems with my knees. I decided to participate in the OA to contribute to research, to finding answers.”

Arthritis Patient Advocates: We connected with the largest advocacy organization for people with knee OA, and leadership and advocates voiced support for the project and suggested adding a physical



therapist to the panel and increasing the number of clinician members from six to seven.

“Involving patients in determining the direction for research in OA is critical. We have many people who want to participate in studies. We try to connect them to researchers the best we can.”

Clinicians: Because knee OA treatment is a preference-sensitive condition, clinicians caring for people with knee OA must engage patients in discussions and provide options for treatment. Primary care physicians we spoke with expressed interest in decision support based on individual characteristics to support patient treatment discussion. Clinicians suggested we include only study databases with one-year followup.

“In the office, I ask them what activity is most important to them to be able to maintain or get back to doing. My recommendations for treatment are very specific to improving their activity level or resolving their pain.”

Researchers: Researchers supported methods to improve recruitment of research patients into trials and described the advantages to applying the ME method to other clinical research topics.

“As a person who does clinical trials, this method could improve and save time screening for study eligibility based on the inclusion and exclusion criteria “

ENGAGEMENT OF STAKEHOLDERS, PATIENTS, AND CLINICIANS

The stakeholder panel will participate in the development and conduct of the study and throughout the project in the development of the database, the math equipoise model, the design and testing of the MEDS user interface, and dissemination of the study results. The panel is composed of six patients and patient advocates, and seven clinicians and researchers. The panel will meet quarterly throughout the three years, and individual members will be interviewed about specific study questions as they occur and will participate in the design of the user interface. Because not all panel members reside locally, conference calls will be arranged for meetings and interviews at no cost to the participants. Patient stakeholders choosing to attend in person will be compensated for parking. Stakeholder¹ panel members are listed in the budget justification and letters of support. In addition to the panel, 12 patients and clinicians will be recruited from within the rheumatology, orthopedic, and general medicine clinics to participate in the development of the MEDS through an iterative design process and usability testing. Testing will take place in the clinic area and will be coordinated with existing visits in order not to incur additional expenses.

¹ In this context, “stakeholder” includes patients.



Engagement Plan B:

Applying Methods of User-Centered Design to Achieve Patient-Centered Care

PUBLIC ABSTRACT

More and more, people are taking an active role in their health care by sharing health decisions with their doctors, nurses, and other healthcare professionals. This is called shared decision making. In 2012, an article published in the *New England Journal of Medicine*, an important and widely read journal, referred to shared decision making as “the pinnacle of patient-centered care.”

Shared decision making means that patients and their loved ones need to understand the risks and benefits of different options, and healthcare professionals need to understand what is important to different patients or families. What is important to one person may not be what is important to another, even if they have the same health condition.

To help everyone understand medical information and clarify what is important to them, research teams work to create patient decision aids. Patient decision aids are booklets, DVDs, or websites that explain the pros and cons of different health choices and help guide people through the process of making decisions, with patients, families, and healthcare professionals all working together. Patient decision aids are often designed so that patients can go through them on their own and be more prepared to talk with their healthcare professionals.

To create decision aids, some researchers start by asking questions and getting opinions and ideas from patients and their healthcare professionals. This might help them develop a patient decision aid that is better designed for the concerns and needs of the patients who will use it. Other researchers do not talk to patients or their healthcare professionals until they have completely finished creating the decision aid, and still others might not get feedback from patients or healthcare professionals at all.

In this project, we want to understand better how different research teams involve patients and their healthcare professionals when they develop patient decision aids. We will see, for example, if involving patients earlier in the process might help create better decision aids, or if involving healthcare professionals earlier in the process might mean that more doctors offer decision aids to their patients. We will find ways to measure how well research teams involve patients and others. We will also suggest ways for research teams to describe better how they involve patients and others in their projects.

Our team is a mix of patients, caregivers, healthcare professionals, and researchers. Our goal with this project is to work together to identify and show how best to involve patients and others in research projects about patient decision aids. We believe that this will help research teams create patient decision aids that are as helpful as possible for all the patients who will use them. This will help to improve the experiences and outcomes of people who are facing difficult health decisions.



PATIENT AND STAKEHOLDER ENGAGEMENT IN PROPOSAL AND PROJECT

Our team consists of a carefully assembled, diverse group of patients, stakeholders, and academics, each of whom brings an important perspective to this work. In inviting people to participate in the research team, we considered not only expertise and perspective, but also style of interaction in order to ensure that those who are more junior (i.e., junior faculty and patients and stakeholders who are newer to participation in research teams) are the types of people who are comfortable speaking up in a group. Our core aim regarding patient and stakeholder engagement is that the work we produce will reflect the richness of the diverse perspectives of all team members. In order to achieve this aim, we have constructed a realistic work plan, with levels of commitment that allow us to strategically seek input from those who have less time to devote to this project while making the best possible use of the expertise of those who have more time available to commit to this project.

Because this project is concerned with tools that support patient engagement in decisions about their health across a wide range of clinical conditions, potential patients and stakeholders are many. Therefore, patient representatives on our team have personal insight across a wide variety of health and personal situations. (We note that two of them will serve on the steering committee.) Stakeholders are two clinicians—one family physician, one nurse practitioner—and one patient decision aid developer. Our stakeholders bring additional insight about the implementation of patient decision aids in practice and engaging people from vulnerable populations.

The principal investigator has been formally discussing this project with patient and stakeholder team members over the past six months. These discussions helped to shape the study design and research questions in innumerable ways. Broadly, these discussions contributed to a greater focus on how vulnerable populations—such as those who have mental health concerns, who lack stable housing, who have lower literacy, or who have unrelenting caregiving responsibilities—face additional barriers to participation in research. As a patient/caregiver representative on our team aptly described in her letter of support, people who may be an important stakeholder group for research because of their higher needs may also be “just barely clinging to the life raft” and thus lack the time or capacity to engage in research and advocacy.

Such discussions influenced this proposal on two levels. First, in our specific aims and research questions, we have included a greater focus on who is not at the table, and what barriers people in vulnerable populations or with greater needs face to participation. Second, we have also incorporated such issues into our own patient and stakeholder engagement plans. We have structured our budget to ensure that all team members will be compensated in order that they might be free to choose to meaningfully participate in this project without it (literally) costing them or otherwise negatively affecting on their lives. We have also budgeted for the provision of caregiving services during our in-person meetings in order to facilitate participation by patients and stakeholders on our team who have caregiving



responsibilities. Another specific example of patient influence on this proposal is reflected in an issue raised by a patient representative regarding how patient representatives' gradual adoption of a researcher mindset, as it were, is one way in which patients' voices fail to be fully represented. We incorporated this issue in Research Question 1b and in our interview guide in Phase 4, and will pay particular attention to this issue within our own team.

We have carefully designed our work plan such that all team members will be involved in 4-14 meetings or teleconferences (including one in-person meeting) per year. Each of these group interactions will occur at a key decision point for the project. This will help ensure that decisions are taken as a group, taking full advantage of all team members' expertise. We will use strategies of team engagement and listening, including facilitation techniques in teleconferences, to ensure that feedback is solicited from all team members and regular updates and consultations via email. In formally defining levels of engagement with specific time estimates, we have ensured that patients and stakeholders are represented across levels, within the constraints of the time and focus each individual is able devote to this project. All team members will be invited (though not required) to co-author publications generated from this project, and we will especially seek the input of patient and stakeholder team members regarding implementation and dissemination.

It must be noted that many of our team members play multiple roles. For example, the steering committee includes a scientific representative who has also been a practicing primary care physician for 23 years and a patient representative who holds a doctorate in philosophy, and the principal investigator for this application has lived with serious chronic illness since childhood and personally faced difficult health situations and decisions as part of that experience. Because it is difficult to "unlearn" a scientific perspective, and because we feel it is reaching when researchers attempt to claim that we are all (or all will be) patients, we do not classify scientific members who are also patients or stakeholders as such. However, we are aware that this blurring of lines between roles creates a spectrum of perspectives on our team rather than clear divisions among patients, stakeholders, and researchers. In some ways, this role fluidity is positive, because it avoids wide divisions and allows for people to serve as skilled translators between perspectives. We note, however, that this duality of roles is something to which we will pay close attention throughout the project to ensure that everyone remains conscious of the importance of bringing the full range of patient, stakeholder, and researcher perspectives to bear in our discussions and team decisions.