



## PATIENT-CENTERED COMPARATIVE EFFECTIVENESS RESEARCH CENTER

### What do PCORI and its collaborators say about patient and stakeholder engagement?

**Overview:** The Patient-Centered Outcomes Research Institute (PCORI) has many valuable resources on its website that provide insight on patient-engagement and give practical advice on the “how to” of patient engagement. Some of these resources were written by the PCORI methodology committee while others were written by PCORI contractors. Below is a summary of many of the quotations from these documents. This is not meant to be a substitute for PCORI’s resources as the context of these statements is very important. However, it is an introduction to what PCORI say about patient engagement. Please see the references for more information on this subject.

#### Why is patient-engagement required in PCOR?

- “PCOR starts from the vantage point of individuals facing health decisions. Every step of the design, conduct, analysis, and dissemination of PCOR should be directed towards informing health decisions that affect outcomes that are meaningful to a specific group of patients. From the earliest phases of defining a research topic and formulating a study question; then identifying a study population and choosing interventions, comparators, and outcomes to measure; through the conduct of a study and analysis of results; and ultimately to the dissemination of research findings into clinical practice, researchers should ensure PCOR results accurately and effectively inform health decisions important to patients. This requires patient engagement throughout the research process.”<sup>i</sup>
- “Compelling reasons, both practical and ethical, support engaging patients in healthcare research. Patients are the ultimate user of healthcare research findings and the most important stakeholder.”<sup>v</sup>
- “Although engaging patients is justified for ethical and moral reasons (considering that they are the ultimate user of research evidence); we found ample evidence suggesting that this engagement may also improve study design (by choosing outcomes more meaningful to patients or designs that are more culturally sensitive or consistent with patients’ context), execution (improving subject recruitment and retention) and translation (better implementation, dissemination and uptake).<sup>v</sup>

#### Principles for engaging patients in research

- “Engage patients and surrogates in all research phases (benefits outweigh the risks)”<sup>ii</sup>
- It is important that “members of the public are empowered to become active, respected participants in the project.”<sup>i</sup>
- “Initiate patient and surrogate engagement as early as possible in the research project and as frequent as feasible”<sup>iii</sup>
- “Utilize a framework for engaging informants”<sup>iii</sup>
- “Select representatives that are similar to the community or population in which the study results are intended to apply”<sup>iii</sup>
- “Engage patients based on the research questions being asked and the overall aims of the research”<sup>iii</sup>
- Ensure “the engagement process is transparent and includes a conflict-of-interest statement.”<sup>i</sup>
- Ensure “the process used to invite and select participants is inclusive and balanced in terms of ethnicity, gender, age, disease burden, and socioeconomic status.”<sup>i</sup>
- It is important that “the roles and relationships for researchers and lay participants are clarified at the beginning of each project.”<sup>i</sup>
- It is important that “the public is engaged using appropriate, validated, and diverse methods by staff experienced in PCOR or patient-centered care.”<sup>i</sup>
- “The process is sustainable and establishes a culture of improvement. There are measures for quality control of patient participation to ensure that the integrity of the process of patient involvement is maintained over time and across different projects.”<sup>i</sup>
- It is important to have a “transparent involvement process”<sup>iii</sup>

### **Important components of patient engagement**

- **Seeing patients as partners:** “Researchers should not see themselves at a “higher” level than the study participants, but rather should be genuinely willing to partner with study participants, and understand the life ways and life needs of hard-to-reach study participants.”<sup>iv</sup>
- **Respect:** “Commit to meaningful engagement at multiple points, treat patients as equal colleagues (reflecting the idea that everyone possesses expertise), and develop trust.”<sup>iii</sup>
- **Communication:** “Actively communicate in a variety of contexts, develop relationships to foster two-way knowledge exchange, keep the patient perspective in mind, and be flexible and transparent.”<sup>iii</sup>
- **Dedicating sufficient resources:** “Patient engagement is resource and time intensive. Devote funding and financial support in terms of incentives, provide reimbursements for patients and support for researchers. Develop transparent processes throughout in terms of how those processes will be operationalized. Utilize facilitation skills.”<sup>iii</sup>

### **Importance of engaging patients early**

- “With early and frequent engagement of patients, caregivers, and community-based healthcare providers, research results [are] more meaningful to patients from a holistic standpoint.”<sup>iv</sup>
- “Members of the community/patients should be involved early (drawing up the initial protocol or even the proposal) so that they achieve immediate empowerment; which should be one of the primary aims of research”<sup>v</sup>

### **Importance of “pre-engagement” as an initial engagement step**

- “Pre-engagement” of stakeholders builds trust and motivates meaningful engagement. Prior to implementing a PCOR study, investigators should utilize a period of “pre-engagement” when recruiting research participants and partners. This allows time to assure that both partnering institutions and PCOR participants comprehend the study and have sufficient time to have their questions answered and concerns addressed. In addition, research participants often wish to discuss participation with family and friends; pre-engagement allows the potential PCOR participant to make an informed decision and feel comfortable participating so that those who choose to participate are better equipped and motivated to make meaningful contributions to PCOR. This “pre-engagement” may also apply to other phases of PCOR research involving patient engagement, such as partnering to identify and frame the research question or to disseminate findings.”<sup>iv</sup>

### **Identifying specific populations and health-decisions affected by research**

- “To produce information that is meaningful and useful to people when making specific health decisions, research proposals and protocols should describe: 1) the specific health decision the research is intended to inform; 2) the specific population for whom the health decision is pertinent; and 3) how study results will inform the health decision.”<sup>i</sup>

### **Measure Outcomes that People in the Population of Interest Notice and Care About**

- “Identify and select outcomes the population of interest notices and cares about (e.g., survival, function, symptoms, health-related quality of life) and that inform an identified health decision. Define outcomes clearly, especially for complex conditions or outcomes that may not have established clinical criteria. Provide information that supports the selection of outcomes as meeting the criteria of “clinically meaningful,” “patient-centered,” and “relevant to decision-makers,” such as patient and decision-maker input from meetings or surveys or published literature relevant to the question of interest. Select outcomes based on input directly elicited from patient informants, persons representative of the population of interest, either in previous studies or in the proposed research.”<sup>i</sup>

### **Identify, Select, Recruit, and Retain Study Participants Representative of the Spectrum of the Population of Interest Facing the Health Decision of Interest**

- “Research proposals and subsequent study reports should describe: 1) the plan to ensure representativeness of participants; 2) how participants are identified, selected, recruited, enrolled, and retained in the study to reduce or address the potential impact of selection bias; 3) efforts employed to maximize adherence to agreed-on enrollment practices; and 4) methods used to ensure unbiased and systematic data collection from all participants. If the population of interest includes people who are more difficult to identify, recruit, and/or retain than other study populations (for example, individuals historically underrepresented in health care research such as those with multiple disease conditions, low literacy, low socioeconomic status, or poor health care access, as well as racial

and ethnic minority groups and people living in rural areas), then specify plans to address population-unique issues for participant identification, recruitment, and retention.”<sup>i</sup>

- “PCORI must ensure that research proposals and protocols clearly identify the relevant patient populations and those health decisions that will be affected by the research.”<sup>i</sup>
- “Researchers must ensure that study participants are representative of the spectrum of the population facing the health decision of interest.”<sup>i</sup>
- “When applicable, research proposals should describe how these stakeholders will be identified, recruited, and retained.”<sup>ii</sup>
- “Recruitment for PCOR studies should involve the full spectrum of individuals impacted by the medical condition or health-related question being examined, including hard to reach patients.”<sup>iv</sup>
- “We suggest selecting representatives that are as similar as possible to the community or population in which the study results are intended to be applied. This includes relevant ethnic minorities, elderly, young, disabled, incarcerated and any other special or vulnerable populations impacted by the research. Empiric evidence exists to suggest that the engagement of all these categories of patients or their surrogates is feasible in most cases.”<sup>v</sup>
- “Recruit via a range of settings”<sup>v</sup>

### **Methods of Engaging Patients**

- Participation in the formulation of research questions;<sup>ii</sup>
- Defining essential characteristics of study participants, comparators, and outcomes;<sup>ii</sup>
- Study procedure development (e.g., determining consent procedures)<sup>v</sup>
- Identifying and selecting outcomes that the population of interest notices and cares about (e.g., survival, function, symptoms, health-related quality of life) and that inform decision making relevant to the research topic;<sup>ii</sup>
- Recruitment (through social networks, etc.)<sup>v</sup>
- Agenda setting<sup>v</sup>
- Monitoring study conduct and progress; and<sup>ii</sup>
- Data collection (through patient administered interviews)<sup>v</sup>
- Analysis<sup>v</sup>
- Interpretation of findings<sup>v</sup>
- Designing/suggesting plans for dissemination and implementation activities<sup>ii</sup>
- Actually disseminating information (presentation, manuscript, pamphlet, social media, plan for future study)<sup>v</sup>
- Implementing results in practice (developing decision aid tools, developing clinical practice guidelines)<sup>v</sup>
- Evaluation (Evaluation of process measures, adherence and uptake of interventions, plan for future research)<sup>v</sup>

### **Practical advice for engagement**

- “Develop written agreement clearly detailing expectations of engagement”<sup>v</sup>
- “Have a single contact person on the research team”<sup>v</sup>
- “Adequate authorship credit should be given to those individuals intimately involved in the process, regardless of their background or affiliation.”<sup>v</sup>

### **Importance of Engaging Hard-to-Reach Populations**

- Hard-to-reach patients: those that “belonged to a group that does not typically participate in research due to cultural or socioeconomic barriers or physical or cognitive impairment.”<sup>iv</sup>
- “With greater involvement of patients from all walks of life, results would be more applicable to the entire spectrum of Americans and results would be implemented more rapidly across healthcare delivery systems.”<sup>iv</sup>

### **Advice for Engaging Hard-to-Reach Patients**

- “Partner with individuals, groups, associations, networks”<sup>ii</sup>
- “Bring PCOR to communities where people live”<sup>iv</sup>. “Meet in places where people are —i.e., public spaces, community-specific spaces, semi-private spaces (clinics and churches), and private spaces (housing and shelters)”<sup>ii</sup>
- “Utilize Media (each with varying ranges of geographic reach) such as billboards, public transportation advertising, flyers in billing statements, radio, TV, movie theatres, and social media”<sup>iii</sup>

- “Attend to components of building and maintaining trust: Pre-engage (to understand the community); Relate (be genuinely willing to partner); Communicate (simple questions/avoid making judgments); Be there (keep “coming back” and interacting with participants)”<sup>ii</sup>
- “Ensure that patients understand the research process—use plain language, provide information in chunks, ask open-ended questions, be transparent, and develop simple consent forms”<sup>iii</sup>
- Understand that “‘Outcomes’ is an unfamiliar term for patients and some clinicians”<sup>iii</sup>
- “Framing and phrasing of questions is critical for eliciting patients’ views”<sup>ii</sup>
- “Community-based PCOR requires flexibility, compromise and time”<sup>iii</sup>
- “Patients do not like being asked repeated questions”<sup>iii</sup>
- “Realize that people may not be willing to openly disclose medical information”<sup>iv</sup>
- “Recognize that people make healthcare choices and participate in research based upon who they are as individual persons, not just as patients”<sup>iv</sup>
- “Provide education on exactly what is meant by the term ‘research’”<sup>iv</sup>
- “Make a sincere effort to give back to the community”<sup>iv</sup>
- “Trust is the backbone upon which the structure of all PCOR must be built” This can be: identification of communities and individual patients; developing partnership based upon trust; explaining throughout the process about research in general, the relationship and expectations of all members of the partnership, and what patients will be asked to do if they participate in PCOR studies; respecting participants at all stages of recruitment and actually doing the research; and maintaining trust by giving back to the community and PCOR participants so that as future PCOR projects are conceived and implemented, the trust between researchers and participants facilitates expansion of the PCOR partnership.”<sup>iv</sup>
- “In designing a research study, investigators need to consider the possibility that individuals may not be willing to openly disclose medical information out of fear of being ostracized from their community.”<sup>iv</sup>
- “Even when we used the phrase ‘health outcome,’ we heard that the term “outcome” is not in the vocabulary of most hard-to-reach patients.”<sup>iv</sup>

#### **Engage Patient Informants, Persons Representative of the Population of Interest, in All Phases of PCOR**

- “Engage people representing the population of interest and other relevant stakeholders in ways that are appropriate and necessary in a given research context...People representing the population of interest include individuals who have the condition or who are at risk of the condition, and as relevant, their surrogates or caregivers. Other relevant stakeholders may include clinicians, administrators, policy makers, or others involved in health care decision making.”<sup>iii</sup>
- “Research proposals should 1) describe how patient informants will be: identified, recruited, and retained; involved in determining the study design and monitoring of its conduct; and involved in dissemination of research results, and 2) state how the research process will follow PCOR principles of trust, transparency, co-learning, respect, and partnership. Patient informants include individuals who have the condition or who are at risk of the condition, and, as relevant, their surrogates or caregivers. At a minimum, patient informants should be engaged in formulating research questions; defining essential characteristics of study participants, comparators, and outcomes; monitoring study conduct and progress; and disseminating results.”<sup>i</sup>
- “To be patient-centered, PCORI and researchers must also engage “informants” in the design, conduct, and dissemination phases of research. Informants are people who are representative of a specific population but are not necessarily study participants. For some populations—for example, children or cognitively impaired persons—informants also include surrogates and caregivers. Although not the focus of this standard, informants representative of other stakeholder groups may also be engaged in the research process.”<sup>i</sup>
- “A defining principle of PCOR is ensuring that the patient’s voice and perspective drive every step of the research process, including prioritizing the research questions, designing and conducting the research, and implementing the results in practice.”<sup>ii</sup>

#### **Use Patient-Reported Outcomes When Patients or People at Risk of a Condition Are the Best Source of Information.**

- “When patients or people at risk of a condition are the best source of information regarding outcomes of interest, then the study should employ patient-reported outcome (PRO) measures in lieu of, or in addition to, measures derived from other sources. Proposals should describe: 1) the concept(s) underlying each PRO measure (e.g., symptom or impairment) and how it is meaningful to, and noticed by, patients in the population of interest; 2) how the concept relates to the health decisions the study is designed to inform; 3) how the PRO measure was

developed, including how patients were involved in the development; and 4) evidence of measurement properties including content validity, construct validity, reliability, responsiveness to change over time, and score interpretability, including meaningfulness of score changes in the population of interest with consideration of important subgroups. If these measurement properties are not known, a plan for establishing the properties must be provided.”<sup>i</sup>

- “Many (though not all) meaningful and important patient-centered outcomes, such as symptoms, are best reported by patients themselves. Pain and some other outcomes cannot reliably or accurately be assessed by any means other than direct patient report. If informants from the study population identify outcomes that can only be ascertained by self-report, then inclusion of patient-reported outcomes is essential to patient-centeredness. Even when other sources can provide meaningful outcomes data, patient reports represent the patient perspective and so add value.”<sup>i</sup>
- “Development of PROs requires patient input. Establishing content validity by ensuring that a proposed measure captures what patients actually think and feel is a critical early step in the development process.”<sup>i</sup>

## **Trust**

- “Trust is the key overarching element for PCOR”<sup>ii</sup>
- “By establishing and maintaining trust between researchers and patient communities, patients are motivated and empowered to actively participate in a learning healthcare system.”<sup>iv</sup>
- “In order to make Patient Centered Outcomes Research (PCOR) more meaningful to patients and their caregivers, we recommend that all PCOR be conducted with a focus on building and maintaining trust, which is achieved via pre-engagement and then through a process involving continuous partnering with the community. The goal is to foster full and meaningful partnering between researchers and participants (i.e. patients and their communities and health care providers) in the research process.”<sup>iv</sup>
- “Establishing trust with PCOR partners and recruitment populations builds bridges for open and enduring engagement. Building and maintaining trust has emerged as a key factor in the decision process of hard-to-reach individuals considering participation in the research process and should be a major consideration when recruiting and interacting with study participants. Furthermore, there is a need for trust to develop between academic researchers and physician communities (e.g. primary care) from whom researchers expect to recruit patients.”<sup>iv</sup>
- “The existence of trust is key to motivating partners and communities to participate and to enrolling patients in PCOR. Building trust is not a step in the patient engagement process, but rather an overarching paradigm that needs to be ubiquitous throughout PCOR efforts.”<sup>iv</sup>

## **Communication**

- It is important to communicate with patients and other stakeholders in a “culturally and linguistically appropriate, repetitive and frequent”<sup>ii</sup> manner.
- “Keeping questions simple and simply asking questions without judgment are helpful in eliciting responses from study participants. Active listening, reading body language, and showing passion for the meaningfulness of the research topic are non-verbal ways to build trust.”<sup>iv</sup>
- “People make health care choices and participate in research based upon who they are not only as patients, but as individual persons. Being viewed as a person, and not just a patient removes the stigma of having an illness or health conditions and addresses the multiple medical and non-medical conditions that influence informed decision making and participation in research.”<sup>iv</sup>
- “Investigators should demonstrate utmost respect for every member of each research team and approach every stage. The academic PI must never talk down to or personalize any criticism of the work of CBO participants.”<sup>v</sup>

## **Training and Support**

- Ensure that you provide adequate “support for patient engagement—i.e., facilitation, processes, food, parking, childcare”<sup>ii</sup>
- “Researchers need training on how to engage”<sup>ii</sup> as well
- “Include engagement efforts in research budgets”<sup>ii</sup>
- “In order to effectively engage individuals, especially hard-to-reach patients, researchers need to provide education on exactly what is meant by the term “research.” In addition, there should be an explanation of the purpose of research in general and the specific PCOR project as well as what participation in the research project entails. Researchers often assume that participants have a general conceptualization of research and immediately

begin to talk about the specific project; the very word “research” carries very negative connotations in some communities.”<sup>iv</sup>

- “Clearly [lay] out the structure and budget, [be] transparent about the work that is expected and how the structure addresses the time issues; [highlight] the potential benefits of participation; [provide] some no-strings-attached financial incentives.”<sup>v</sup>
- “Provide compensation”<sup>v</sup>

### **Follow Up, Dissemination, and Implementation**

- “Participants want to be kept abreast of research progress and want a celebration or recognition at the conclusion of a study. Participants are aware of the fact that they ‘give more to the researcher than they get in return’ and would like some feedback at various points throughout the research progress. In short, participants want the researcher to ‘give back’ to the community in terms of feedback on the study.”<sup>iv</sup>
- “Once research concludes, a plan for dissemination should be implemented to share the findings of the research study with participants and their community/organization in a timely manner in order to promote meaningful participation in future research studies. As a component of this dissemination, researchers need to be aware that research occurs in the community as well and that the setting of any follow-up should be appropriate for the patient group participating.”<sup>iv</sup>
- “The dissemination method should be personalized and suitable for the patient’s characteristics and abilities. Language should avoid technical jargon and non-applied information. It is also helpful to include members of the community to address language and cultural issues.”<sup>v</sup>
- “Research participants should be routinely provided with the results of studies to show the potential impact of findings.”<sup>v</sup>
- “The implementation process should involve patients in every step of the way and for this to be successful they need to receive as much information as possible... Appropriate support is critical to facilitate effective patient engagement in the implementation phase, overall providing clear guidance on their roles and responsibilities within the group and ensuring the opportunities to attend training events for all guideline/project development group members”<sup>v</sup>

### **Barriers to patient engagement**

- “Time constraints (researcher perspective): Patient engagement is time consuming and lengthens the duration required to complete the study”<sup>v</sup>
- “Engagement may evolve to become a tokenistic endeavor designed to tick a required box”<sup>v</sup>
- “Time constraints (patients’ perspective)”<sup>v</sup>
- “Funding needed to engage patients”<sup>v</sup>
- “Poor attendance when no compensation”<sup>v</sup>
- “Unclear expectations (differing researcher and patients goals of the study; consumers' unfamiliarity with research programs and research programs' unfamiliarity with consumers; negative attitudes and poor working relationships; difficulties in communication)”<sup>v</sup>
- “Lack of informant knowledge/skills in technology and communication methods”<sup>v</sup>
- “Perceived lower status of patients/informants compared to investigators”<sup>v</sup>

### **References**

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