

# The Patient-Centered Outcomes Research Institute: Research to answer questions important to patients and providers

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The Patient-Centered Outcomes Research Institute (PCORI) funds comparative clinical effectiveness research to provide reliable evidence to help patients and their healthcare providers (HCPs) make informed decisions. All the projects PCORI funds must involve patients, as well as other healthcare stakeholders, including HCPs such as nurse practitioners (NPs), throughout the research process. NPs, with their invaluable experience in interacting with patients and their families, are playing important roles in helping PCORI choose which projects to fund and in leading and guiding research projects. This article provides an overview of PCORI's work and describes how NPs can become involved in this work.

**KEY WORDS:** Patient-Centered Outcomes Research Institute, PCORI, patient-centered outcomes research, comparative effectiveness research, clinical research, nursing research



When nurse practitioners (NPs) meet with patients or their family members, they are likely to be asked many difficult questions.

Common inquiries include:

- What will happen to me?
- What are my options, and what are their potential benefits and harms?
- What can I do to improve the outcomes that are most important to me?
- How can I make the best decisions about my health and healthcare?

Answering these questions requires NPs to use the best available evidence and to translate findings from clinical research into information that patients can act on.

However, many research findings are not directly relevant to patients' individual circumstances, and they do not measure outcomes most important to patients and their families. To address this problem, the **Patient-Centered Outcomes Research Institute<sup>A</sup>** (PCORI) funds research to help patients and their families and caregivers—as well as nurses, NPs, physicians, and others in the healthcare community—answer relevant questions.

## What is PCORI?

PCORI is an independent research institute whose establishment was authorized by Congress in 2010. PCORI was created to improve the quality and relevance of research evidence available to help patients, family members, caregivers, health-care providers (HCPs), employers, insurers, and policy makers reach informed decisions about health and healthcare. This work is accomplished by funding patient centered outcomes research (PCOR), primarily comparative clinical effectiveness research (CER) (Box). PCOR considers patients' needs and preferences to focus on outcomes that are most important to them, whereas CER directly compares existing healthcare interventions, considering potential benefits and harms, to determine which ones work best for which patients.

PCORI is implementing a new model of research. Although traditional research often uses patients as study participants, it does not routinely seek input from patients, caregivers, HCPs, or the larger healthcare community. In the PCORI model, patients and other stakeholders serve as active team members throughout the research process.<sup>1</sup> In addition, the institute engages patients, HCPs, and other healthcare stakeholders in its funding decisions and in other activities.<sup>2</sup>

PCORI has been building a rich and diverse research portfolio since awarding funding in 2012 for an initial round of Pilot Projects, studies designed to address a broad range of questions about methods for engaging patients in the health research and dissemination process. As of June 27, 2015, PCORI has funded 400 **projects**<sup>B</sup> in 39 states plus the District of Columbia and Quebec.

## What are PCORI's goals?

For all the advances that clinical studies have produced, many questions remain about which approaches to disease prevention, diagnosis, treatment, and health-care delivery work best in specific situations. Patients, along with their caregivers and HCPs, require more information that they can understand and readily use. To help meet this need, PCORI has **three overarching goals**<sup>C</sup>:

- To increase the quantity, qual-

ity, and timeliness of useful research information;

- To speed implementation and use of research evidence; and
- To encourage research funded by others to be patient-centered.

PCORI-funded studies engage patients and other stakeholders in planning and conducting studies, analyzing results, and disseminating research findings. For example, these individuals may provide input regarding which interventions

## Definitions

**Comparative clinical effectiveness research (CER)** compares outcomes of two or more approaches to healthcare. The purpose of this comparison is to determine the effectiveness, in terms of both risks and benefits, of the approaches and discover what works best for which groups of people. Preventive measures; diagnostic tests; pharmacologic, surgical, or behavioral interventions; or approaches to healthcare delivery may be compared.

**Patient-centered outcomes research (PCOR)** seeks answers to real-world questions about what works best for patients based on their concerns or circumstances. Findings from this type of research help patients and their caregivers assess healthcare options and make better-informed decisions. Researchers engaged in PCOR seek the voices of patients, providers, and other healthcare stakeholders, who are engaged throughout the research process.

**Patient-centeredness** describes how a research project aims to answer questions or examine outcomes that matter to patients and caregivers. A project may be considered patient-centered if:

- The research question (the comparison) reflects a choice or choices faced by and important to patients, their caregivers, or other healthcare stakeholders.
- The study protocol includes outcomes important and relevant to patients.

**PCORI's Methodology Standards**<sup>Q</sup> include guidance on patient-centeredness. These standards require the engagement of persons who have the condition being examined—or who are at risk for the condition—and their caregivers and other healthcare stakeholders; identification, selection, recruitment, and retention of study participants representative of the spectrum of the population of interest, including hard-to-reach populations such as those with poor healthcare access, low socioeconomic status, and/or low literacy; and use of patient-reported outcomes when patients or persons at risk for a condition are the best source of information.

are practical and which outcomes should be measured.

With their invaluable experience in interacting with patients and their families, NPs are playing important roles in helping PCORI choose which projects to fund and in guiding research projects as they unfold.<sup>3,4</sup> Currently, PCORI has two nurses in leadership positions: One, an NP, serves on the Board of Governors and chairs the Engagement, Dissemination, and Implementation Committee, and the other chairs the Methodology Committee. Twelve nurses serve on multi-stakeholder advisory panels and many others review applications for research funding and serve on research teams around the country. PCORI welcomes input from NPs interested in any type of PCOR.

### What types of research does PCORI support?

PCORI developed a framework to guide funding of research that will help patients and caregivers make better-informed health decisions. This framework addresses five research priority areas:

- Assessment of prevention, diagnosis, and treatment options;
- Improving healthcare systems;
- Communication and dissemination research;
- Addressing disparities; and
- Accelerating PCOR and methodological research.

PCORI funds studies that investigators propose on a wide range of topics within these areas.

PCORI supports studies in a variety of formats. Some are randomized controlled trials, in which researchers randomly assign participants to an intervention and then measure particular outcomes. However, this powerful

clinical tool may be impractical for assessing rare outcomes, effects that take a long time to develop, variation in response between subgroups, or the interventions that work best under usual circumstances (rather than the ideal circumstances of many traditional clinical trials).

To address these challenges, PCORI funds pragmatic clinical studies. These studies are conducted in routine clinical settings and are often large. Study participants represent the breadth of the relevant pa-

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tient population. PCORI provides a list of topics of particular interest for pragmatic clinical studies.

The institute occasionally posts announcements seeking proposals for research on a specific topic. PCORI has not typically specified a research design in these targeted funding announcements, preferring to let investigators propose a means to address the questions presented. Projects currently under way that were funded under targeted announcements include testing the effectiveness of transitional care, preventing injuries from falls in the elderly, and treating severe

asthma in African-American and Hispanic/Latino populations.

Under its priority of accelerating PCOR, the institute is funding the development of PCORnet, the National Patient-Centered Clinical Research Network. Designed to support large-scale, efficient CER, this initiative brings together clinical data networks based in health-care systems and in patient-directed organizations. PCORnet will enable large-scale research to be conducted with enhanced efficiency in real-world healthcare delivery systems.

When funding research, PCORI pays particular attention to conditions that affect large numbers of people across a range of populations; conditions that place a heavy burden on individuals, families, specific populations, and society; and rare diseases, which are difficult to study. In addition, PCORI gives priority to racial and ethnic minorities, older adults, children, and low-income and rural populations.

About 9% of PCORI's current studies focus on women's health problems. These studies range from comparing options for management of uterine fibroids to engaging communities in the fight to reduce preterm birth. Other studies focus on imaging modalities for breast cancer survivors, decisions related to contralateral prophylactic mastectomy, prevention of unnecessary cervical cancer screening and treatment, and contraceptive counseling. In addition, PCORI is investigating how to facilitate translation of research findings into everyday practice by advanced practice nurses and other HCPs.

### How is PCORI improving engagement in research?

A guiding principle of PCORI is that engagement of healthcare stake-

holders can encourage research to be more patient-centered, useful, and reliable, and ultimately lead to greater adoption of research results. Stakeholders are defined by PCORI as the broad range of “communities” that have a stake in the effectiveness of our healthcare system. These communities include not only patients, caregivers and family members, and HCPs—and the organizations that represent them—but also payers (public and private insurers), purchasers of health benefits (e.g., employers), the life sciences industry, policy makers, hospitals and health systems, and training institutions. However, many researchers do not have experience working with community partners as they plan, conduct, and disseminate research. In addition, patients, HCPs, and other stakeholders may lack experience in research activities. Because engagement in research can take many forms, PCORI is exploring how best to meaningfully involve individuals, communities, institutions, and organizations in clinical research.

One way PCORI engages multiple voices is by requiring that all its governing and advisory groups (e.g., its Board of Governors, advisory panels, and application review teams) include patients, HCPs, researchers, and other stakeholders. All participants have equal voices in decisions, which include refining and prioritizing research questions and recommending engagement efforts.

To support engagement, PCORI is developing tools for creating and maintaining research partnerships. One of these tools, the **PCORI Engagement Rubric<sup>D</sup>**, provides guidance on ways to involve patients, their families, and other stakeholders in conducting research and pro-

vides sample engagement plans from funded research.

Promising practices for meaningful engagement in the conduct of research are being explored through funding opportunities such as the Eugene Washington PCORI Engagement Awards and the Pipeline to Proposal Awards. These awards enhance the capacity of patients and other stakeholders to participate in and support research.

## PCORI convenes representatives from across the healthcare community for information exchange, discussion, and partnership building via webinars, workshops, and roundtables.

### How can NPs participate in PCORI activities?

PCORI invites NPs to participate in a wide variety of activities that will help the institute meet its goals. These activities range from suggesting a research question to serving on an advisory panel to directing a research project. NPs, with their expertise in patient-centered care, can help inform research processes and facilitate interaction with patients. Specific ways for NPs to participate in PCORI activities include the following:

### Suggest a patient-centered research question<sup>E</sup>

PCORI's selection of research questions starts with gathering suggestions from patients, HCPs, and other healthcare stakeholders. With the unique lens through which they view health and healthcare, NPs are well positioned to identify gaps in healthcare evidence. Professional organizations such as the National Association of Nurse Practitioners in Women's Health have research agendas<sup>5</sup> that may inspire NPs to propose potential topics.

After a topic is submitted to PCORI (readers can click on the heading of this section to do so), it is assessed for patient-centeredness, disease burden, potential for improving practice, timeliness, and potential impact. Topics are prioritized by the multi-stakeholder advisory panels and selected by PCORI's Board. Suggested research questions have resulted in funding announcements in areas of special interest for women's HCPs, including treatment options for uterine fibroids, effectiveness of transitional care, and obesity treatments for underserved populations.

### Participate in PCORI events<sup>F</sup>

PCORI convenes representatives from across the healthcare community for information exchange, discussion, and partnership building via webinars, workshops, and roundtables. In June 2014, PCORI held a webinar entitled **PCORI in Practice: Highlighting Opportunities for Nurses<sup>G</sup>**, which is archived on the PCORI website. In January 2014, representatives of nursing professional societies attended a PCORI roundtable to share ideas on which programs, activities, and information would

be most useful to members of those societies. More information about upcoming events can be found on the website or by subscribing to **PCORI email alerts<sup>H</sup>**.

### **Review funding applications<sup>I</sup>**

Each research application received by PCORI is evaluated by a panel of two scientists, one patient, and one other stakeholder, such as a nurse. Scores from all types of reviewers have equal weight. First, applications are scored via an online system. Then all the reviewers meet in person to discuss the applications. Training/support for reviewers includes an online training program, mentoring from experienced reviewers, and communication with PCORI Merit Review Officers. Reviewers are compensated, and travel and related costs for the in-person meeting are reimbursed.

Currently, 4% of PCORI reviewers are nurses. NPs are encouraged to apply to become reviewers. Frontline expertise and experience in direct patient care across the age continuum in a variety of settings can help PCORI fund user-driven research that is patient-centered, useful, and usable, as well as scientifically rigorous.

### **Serve on advisory panels<sup>J</sup>**

Applying to one of PCORI's advisory panels is another way that NPs can get involved. These multi-stakeholder panels include patients, caregivers, HCPs, policy makers, researchers, and payers. The seven PCORI advisory panels address these areas:

- Assessment of Prevention, Diagnosis, and Treatment Options
- Improving Healthcare Systems
- Addressing Disparities
- Communications and Dissemination Research

- Patient Engagement
- Clinical Trials
- Rare Disease

The first four advisory panels above align with PCORI's National Priorities and Research Agenda and help the institute prioritize topics submitted. The lattermost three panels provide advice and guidance to the institute and its awardees.

One of the authors (Annie Lewis-O'Connor), who is an NP, served on the Advisory Panel on Improving Healthcare Systems. With her perspective on nursing and advanced nursing practice, Lewis-O'Connor contributed to discussions about prioritizing research topics for PCORI funding.

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She was able to educate panel members about opportunities in healthcare that are not well understood, as well as about burdens that have a major impact on health and health outcomes. Lewis-O'Connor notes that participating on a PCORI advisory panel provides an innovative opportunity to collaborate with patients and other healthcare stakeholders.

### **Apply for a Pipeline to Proposal Award<sup>K</sup>**

Patient engagement in research requires making new connections between researchers and patients, HCPs, and other stakeholders. PCORI's Pipeline to Proposal Awards build and strengthen such

relationships, particularly in communities that have been underrepresented in research. This program builds the capacity for community partnerships to create research questions and submit PCOR proposals that can be considered for PCORI funding. In addition, the program increases the number of patients, researchers, and other stakeholders ready to participate in PCOR and explores methods for engaging and communicating with patients, researchers, and other stakeholders. Three of PCORI's first 30 Pipeline to Proposal Awards went to projects led by nurses. Many other projects have nurses as important partners.

Among the projects for which a nurse is the principal investigator, one created a community team to identify opportunities for CER to reduce infant mortality. In an ongoing project, a community/clinical/academic partnership focused on asthma is creating a sustainable pathway for patient and parent input on research opportunities and building infrastructure to conduct CER. In another project, a community work group is targeting increased capacity of Mexican American families to identify strategies to promote healthful eating and physical activity, thereby reducing health disparities related to obesity. The *Table* lists examples of PCORI-funded research led by nurse scientists.

PCORI recently announced a second round of Pipeline to Proposal Awards to 47 projects, 5 of which are led by nurses. One project of special interest to nurses is titled **Assessing Health Outcomes in Rural Areas Where Nurse Practitioners Provide Primary Care<sup>L</sup>** and another is titled **Developing a Student/Family-Centered School Health Collaborative<sup>M</sup>**.

**Table.** Examples of PCORI-funded research led by nurse scientists

| Year awarded | Project title   | Principal investigator              | Organization                                       | State      | Type of award  |
|--------------|---|-------------------------------------|--|------------|--|
| 2015         | Engaging the Women's Health and Research Community in PCORI's Women's Health Research Focus                       | Beth Battaglino, RN                 | Healthy Women                                      | New Jersey | Engagement   |
| 2014         | Developing and Testing "Best Practices" in Training for Academic/Community Research Partnerships                  | Pamela Williams, JD, PhD, RN        | University of Arkansas for Medical Sciences        | Arkansas   | Engagement   |
| 2014         | Patient-Centered Physical Activity Coaching to Improve Outcomes in COPD: A Pragmatic Trial                        | Huong Q. Nguyen, PhD, RN            | Kaiser Foundation Research Institute               | California | Primary Research: Improving Healthcare Systems                               |
| 2014         | Patient and Provider Engagement and Empowerment Through Technology (P2E2T2) Program to Improve Health in Diabetes | Heather Margaret Young, MS, PhD, RN | The Regents of the University of California, Davis | California | Primary Research: Improving Healthcare Systems                               |
| 2013         | Building a Community of Safe Sleep for Infants  | Lauren Thorngate, PhD, RN, CCRN     | MultiCare Health System                            | Washington | Pipeline to Proposal   |
| 2013         | Mobilizing Community Engagement for Health in a Southern New Mexico Border Region Colonia                         | Becky Keele, PhD, PHCNS-BC, RN      | New Mexico State University                        | New Mexico | Pipeline to Proposal   |
| 2013         | Puget Sound Asthma Coalition: A Community, Clinical, and Academic Partnership                                     | Julie Postma, PhD, BSN              | Puget Sound Asthma Coalition                       | Washington | Pipeline to Proposal   |
| 2013         | Rare Epilepsy Network (REN)   | Janice M. Buelow, PhD, RN           | Epilepsy Foundation                                | Maryland   | PCORnet  |
| 2013         | Bringing Care to Patients: A Patient-Centered Medical Home for Kidney Disease                                     | Denise Hynes, MPH, PhD, RN          | University of Illinois at Chicago                  | Illinois   | Primary Research: Improving Healthcare Systems                               |
| 2013         | Patient Outcomes of a Self-Care Management Approach to Cancer Symptoms: A Clinical Trial                          | Susan McMillan, NP, PhD             | University of South Florida                        | Florida    | Primary Research: Assessment of Prevention, Diagnosis, and Treatment Options |

*continued...*

**Table.** Examples of PCORI-funded research led by nurse scientists (continued)

| Year awarded | Project title   | Principal investigator                 | Organization   | State          | Type of award                            |
|--------------|---|--|--|----------------|--|
| 2012         | Reducing Health Disparities in Appalachians with Multiple Cardiovascular Disease Risk Factors | Debra Kay Moser, DNSc, RN              | University of Kentucky                                 | Kentucky       | Primary Research: Addressing Disparities |
| 2012         | Involving Nursing Home Residents and Families in Acute Care Transfer Decisions                | Ruth M. Tappen, EdD, RN, FAAN          | Florida Atlantic University                            | Florida        | Pilot Project                            |
| 2012         | CAPE: Patient-Centered Quality Assessment of Psychiatric Inpatient Environments               | Kathleen Delaney, PhD, RN, NP          | Rush University MedicalCenter/ Rush College of Nursing | Illinois       | Pilot Project                            |
| 2012         | Decision Support for Symptom and Quality of Life Management                                   | Mary E. Cooley, PhD, RNP, MSN          | Dana-Farber Cancer Institute                           | Massachusetts  | Pilot Project                            |
| 2012         | A Community Partnership Approach For Advancing Burden Measurement in Rare Genetic Conditions  | Pamela Holtzclaw Williams, JD, PhD, RN | Medical University of South Carolina                   | South Carolina | Pilot Project                            |

### Apply for an Engagement Award<sup>N</sup>

Readers who have ideas about ways to increase meaningful engagement of patients and health-care stakeholders in the research process can apply to lead projects awarded to their organizations by the Eugene Washington PCORI Engagement Awards program. Within this program, Knowledge Awards increase evidence about how patients and other stakeholders view, receive, and make use of patient-centered CER. Training and Development Awards build capacity among patients and stakeholders to participate as full, meaningfully engaged partners in PCOR and CER. Dissemination Awards develop and strengthen channels for disseminating and implementing study findings. In addition, PCORI provides support for conferences, workshops, and other for-

mal meetings to facilitate knowledge sharing and explore issues related to PCOR and CER.

Examples of Engagement Awards led by nurses include engaging the women's health and research communities in PCORI activities and testing best practices in training for academic/community research partnerships. Other Engagement Awards include preparing a network of community health centers to implement PCOR in their practices and connecting parents of children with complex health conditions to each other and their HCPs to address common challenges.

### Become a PCORI Ambassador<sup>O</sup>

The goal of PCORI's volunteer Ambassador program is to help patients, HCPs, organizations, and other stakeholders share the promise of PCOR with their com-

munities and promote sharing and use of information generated from PCORI-funded projects. Ambassadors do not speak or act on behalf of PCORI but, rather, are partners aligned with its principles. They play an important role in building a PCOR community.

For now, PCORI invites only those individuals and organizations that have participated in its activities to become PCORI Ambassadors, but the institute plans to open the application process to others in the near future. Ambassadors complete an online training program that provides information on PCORI, its engagement program, and the Ambassador role. PCORI provides a toolkit that includes slides, talking points, and guidance on writing letters to the editor, opinion pieces, and blog posts. Among PCORI's more than

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