Improving Quality and Safety through Patient Centered Outcomes Research: Understanding the PCORI vision and mission

Mary A Blegen  PhD RN FAAN
Professor Emerita, School of Nursing, University of California San Francisco

Objectives

• Describe the research agenda of the patient centered outcomes research institute (PCORI)
• Compare basic research, clinical research, and comparative effectiveness research
• Explain ways in which research can be made patient centered

Patient Centered Outcomes Research Institute (PCORI)

• “The purpose of the Institute is to assist patients, clinicians, purchasers, and policy-makers in making informed health decisions by advancing the quality and relevance of evidence concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed through research and evidence synthesis that considers variations in patient subpopulations, and the dissemination of research findings with respect to the relative health outcomes, clinical effectiveness, and appropriateness of the medical treatments, services, and [other] items.”
Origin and Funding

- PCORI is funded through the Patient-Centered Outcomes Research Trust Fund (PCORTF), which was authorized by Congress as part of the Patient Protection and Affordable Care Act of 2010 and receives income from two funding streams: the general fund of the Treasury and a small fee assessed on Medicare, private health insurance and self-insured plans. (Currently funded through Sept 30, 2019)

PCORI Strategic Imperative

- **Engagement**: Engage patients, caregivers, and other stakeholders in entire research process, from topic generation to dissemination and implementation of results
- **Methods**: Develop and promote rigorous patient-centered outcomes research methods, standards, and best practices
- **Research**: Fund a comprehensive agenda of high-quality patient-centered outcomes research and evaluate its impact
- **Dissemination**: Disseminate patient-centered outcomes research to all stakeholders and support its uptake and implementation
- **Infrastructure**: Promote and facilitate the development of a sustainable infrastructure for conducting patient-centered outcomes research

Background / History

Types of Research

- **Basic Research** – laboratory, basic processes
- **Clinical Research** – Efficacy of treatments, procedures, diagnostic approaches
- **Comparative Effectiveness Research (CER)** - conduct and synthesis of systematic research comparing different interventions and strategies to prevent, diagnosis, treat and monitor health conditions
Why Comparative Effectiveness Research (CER)

- Evidence necessary to inform decisions is incomplete or unavailable
- More than half the treatments delivered today do not have clear evidence of effectiveness (efficacy only)
- Practice patterns, costs and outcomes differ markedly across areas in the U.S.

Effectiveness not Efficacy

<table>
<thead>
<tr>
<th>Effectiveness</th>
<th>Efficacy</th>
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<tbody>
<tr>
<td>Does it work?</td>
<td>Can it work?</td>
</tr>
<tr>
<td>Concerned with the benefits achieved in the actual practice of healthcare with typical patients and providers</td>
<td>Concerned with the benefits achievable from a therapy or an intervention under ideal conditions such as those found in RCT</td>
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<tr>
<td>Observations not manipulations. Use typical patients with adherence problems, comorbidities, competing demands and costs</td>
<td>Patients in the study meet stringent screening for inclusion/exclusion criteria and staff follow-up to maximize compliance</td>
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<tr>
<td>Risk adjustment and statistical controls for confounded effects of patient, provider and system factors</td>
<td>Experimental Design with randomization</td>
</tr>
<tr>
<td>Difficult to find the effect</td>
<td>May overestimate the effect</td>
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Characteristics of CER also called “Outcomes Research”

- Study of patients in typical day-to-day clinical care – not under controlled conditions in an RCT
- Observational not experimental studies
- Identify what works for which patients under what circumstances
- Needs different data sources and data analysis methods
- To ensure its relevance to everyday healthcare delivery, must be based in large national clinical and administrative data bases and research networks
Data Analytic Methods
Without Randomization and Control of treatment and comparison groups, need methods that control confounding variable effects
- Risk Adjustment
- Propensity Scores
- Inverse probability weighting
- Instrumental Variables – 2SLS
  (two stage least squares)

What PCORI Adds
*Patient-Centered Outcomes Research*
... considers patients' needs and preferences and focuses on outcomes most important to them. ... helps patients, caregivers, clinicians, insurers, policymakers and others, make better-informed decisions about their health and healthcare.
- “Given my personal characteristics, conditions, and preferences, what should I expect will happen to me?”
- “What are my options, and what are the potential benefits and harms of those options?”
- “What can I do to improve the outcomes that are most important to me?”
- “How can clinicians and the care delivery systems they work in help me make the best decisions about my health and health care?”

PCORI inclusivity
- Includes on Executive board, Advisory Panels, Research Review groups, Staff, etc
- Patients and families, healthcare providers (nurses, physicians, NP/PA, pharmacists, dentists, optometrists, therapists), Payers (insurance execs, medicaid/medicare), government policy experts, as well as researchers.
- From all states, races, genders
PCORI Addresses:

- Strategies for prevention, screening, diagnosis, treatment, or management of clinical conditions
- Methods to improve delivery of care
- Features of healthcare systems including facilities, insurance, newer arrangements like accountable care organizations, patient centered healthcare homes, etc
- Interventions to reduce or eliminate disparities in health and health care
- Health communication techniques

Types of PCOR/CER (1)

Pragmatic Clinical Studies

- Provides information that can be directly adopted by healthcare providers. Conducted in routine clinical settings, they are large and include different types of patients.
- Compares two or more alternatives for addressing prevention, diagnosis, treatment, or management of a disease or symptom; improving healthcare system–level approaches to managing care; or eliminating health or healthcare disparities.
- Address critical clinical choices faced by patients, involve broadly representative patient populations, and are large enough to determine differences across patient subgroups.

Types of PCOR/CER (2)

Observational Studies

- Interventions are part of normal clinical care.
- Useful when researchers can’t assign participants to treatment or control groups for practical or ethical reasons or when an effect is rare or slow to appear.
- Electronic health databases make it possible to conduct observational studies in large populations treated in standard clinical settings.
- Challenge is that the variability and complexity of patients and their circumstances, as well as the care they receive, often make it difficult to conclude whether a specific intervention is responsible for the observed outcomes.
- Uses statistical analytic tools aim to control the effects of confounding variables and obtain valid estimates of effects even in complex situations.
Types of PCOR/CER (3)

Infrastructure
- To improve our capacity to conduct patient-centered effectiveness research, by
- improving the pace, quality, and patient-centered nature of clinical research,
- PCORI is supporting the development of a large, representative, electronic, national clinical data network.
  - PCORnet, the National Patient-Centered Clinical Research Network
- Patients and other stakeholders are participating in the development and implementation of network activities.

PCOR pays particular attention to:
- Conditions that place a heavy burden on individuals, families, specific populations, and society
- Conditions that affect large numbers of people across a range of populations
- ALSO - Rare diseases, which are difficult to study (less than .07% of population (1:1500))
- Racial and ethnic minorities
- Older adults
- Low-income and rural populations
- Children

PCORI – Patient Centered

Patient Centered (Engagement)
- meaningful involvement of patients, caregivers, clinicians, and other healthcare stakeholders throughout the research process
- leads to research that is more patient centered, useful, and trustworthy and ultimately to greater use and uptake of research results by the patient and the providers
- requires a well-thought-out plan and all applications for PCORI funding must include an plan for engaging patient and stakeholder partners.
  - Patient partners include patients, family members, caregivers, and the organizations that are representative of them.
  - Stakeholder partners include clinicians, purchasers, payers, industry, hospitals and health systems, and policy makers.
PCORI – Patient Centered

• The Engagement Plan is designed to help applicants describe how input from patient and stakeholder partners will be incorporated throughout the research process.
  – Planning the Study,
  – Conducting the Study,
  – Disseminating the Study Results
• Applicants must include patient and stakeholder partners in all relevant sections of the application, such as the research design, biosketches, the budget, and the dissemination and implementation assessment.

Patient Engagement Awards

• A HealthyWomen project that will help support two events that will promote awareness and enhance interactions between PCORI and the women’s health research community.
• An Illinois Institute of Technology effort to develop a community-based participatory research curriculum and test how well it improves care for African Americans with serious mental illness.
• An initiative by the Bladder Cancer Advocacy Network to identify and prioritize research questions most pertinent to bladder cancer patients.
• The Coalition to Transform Advanced Care’s Summit on Advanced Illness Care: Driving Change through Leadership, Evidence, and Action, which aims to identify gaps in the care delivery process and explore how to overcome them.
• A Baystate Medical Center project to develop and convene a series of workshops on community-engaged comparative effectiveness research in Springfield, Mass., an urban community with a high rate of health problems and disparities.

Examples of currently funded projects

• Patient Engagement Awards
• Pragmatic Trials and CER
• Projects with Collaborative funding
• PCORnet
### Examples of PCORI Funded Research

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<th>Wisconsin</th>
<th>Illinois</th>
<th>Minnesota</th>
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<td>The Effectiveness of Peer-to-Peer Community Support to Promote Aging in Place</td>
<td>Improving the Effectiveness of Routine Surveillance Following Lung Cancer Resection</td>
<td>Measuring Patient Outcomes from High Tech Diagnostic Imaging Studies</td>
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<td>Integrated Care and Patient Navigators for Latinos with Serious Mental Illness</td>
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### PCORI with collaborating agencies

- Randomized Trial of a Multifactorial Fall Injury Prevention Strategy (National Institute on Aging)
- Comparing Options for Management: Patient-Centered Results for Uterine Fibroids (AHRQ)
- Pilot Project on Coordination of Care for Frail Elderly (John A. Hartford Foundation)

### PCORI Infrastructure

- Crucial healthcare decisions must be made while lacking key information about which preventive, diagnostic, or treatment approach would be best, given a patient’s preferences and circumstances;
- but, the nation’s capacity to conduct CER rapidly and efficiently remains extremely limited,
- therefore, PCORI has invested more than $100 million in the development of PCORnet.
- PCORnet fosters CER by establishing a resource of clinical data gathered in “real time” and in “real-world” settings, such as clinics.
- Data is collected and stored in standardized, interoperable formats under rigorous security
PCORnet

• requires that the patients, clinicians, and healthcare systems that provide the research data housed in each network be actively involved in the governance and use of the data.
• aims to advance the shift in clinical research from investigator-driven to patient-centered studies.
• reduces the time and effort required to start studies and build the necessary infrastructure to conduct them.
• enables studies to be conducted using real-time data drawn from real-world settings,
• increase the relevance of questions studied and the usefulness of the results.

PCORnet awards

• Clinical Data Research Networks
  – Children Hospital of Philadelphia
  – Oregon community Health Information Network
  – Kaiser Foundation Research Institute
  – Louisiana Public Health Institute
• Patient-Powered Research Networks
  – Accelerated Cure Project for Multiple Sclerosis
  – American sleep apnea Association
  – Epilepsy foundation
  – Crohn’s and Colitis foundation

How you can get involved in PCORI
http://www.pcori.org/content/

• Suggest Research Topics /suggest-patient-centered-research-question
• Participate in PCORI activities /participate-pcori-events
• Learn about Research networks in your area
• Work with a Group Applying for Research Grant
• Review Funding Applications /review-funding-applications
• Join a Research Advisory Group /join-advisory-panel
  – Assessment of Prevention, Diagnosis, and Treatment Options
  – Improving Healthcare Systems
  – Addressing Disparities
  – Patient Engagement
  – Clinical Trials
  – Rare Disease
  – Communication and Dissemination Research