Patient-Centered Comparative Effectiveness Research and Quality Improvement: Their Relationship in Transformative Research

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Goals of Presentation

• Patient-Centered CER: What it is and why it matters to you as a clinician and a researcher
• The relationship of CER and QI
• How Patient Centered CER is transforming the research process: theory, methods and practice
• Lessons learned: How might this change research and practice at your institution
Organization of Presentation

• PCORI
• CER and Patient Centered CER
• How is a CER study evaluated--How do we know one when we see one?
• Similarities and Differences between QI and CER
• Group Exercise
• Questions and Discussion
What is the Patient Centered Outcomes Research Institute (PCORI)?

- Authorized by the Patient Protection and Affordable Care Act of 2010 as a nonprofit, nongovernmental organization.
- Purpose: 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.”
- Funds comparative clinical effectiveness research (CER) that engages patients and other stakeholders throughout the research process
PCORI Statutory Prohibitions

• Cannot fund:
  
  ➢ Cost effectiveness analysis, including but not limited to analyses in the form of dollar-cost per quality adjusted life year to compare two or more alternatives
  
  ➢ Measures of the relative costs of care of two or more alternative approaches as the primary criteria for choosing the preferred alternative.
  
  ➢ Development of clinical guidelines
  
  ➢ Development of policy recommendations
What is Comparative Effectiveness Research?

- **Comparators**: Compares two or more real world options: diagnosis, prevention, treatment, health delivery. Each must be of proven efficacy (can be usual care, but not preferred). Can’t be novel or developmental.
- **Rigorous methods**: Often RCT, but can be observational study; must have well articulated design, powered to detect significant effects in primary outcomes
- **Pragmatic**: Conducted with real world populations in real life settings—not under controlled conditions
- **Looks at key subgroups**: Is the study powered to measure how the intervention works with different kinds of patients who have the disease or condition? (HTE)
What is *Patient-Centered CER*?

- Actively engages patients and key stakeholders throughout the research process.
- **Patients are partners** in research, not just “subjects”. Includes patients with lived experience.
- Researchers must prove that patients and caregivers find the research questions and outcomes important.
- Must **conduct research in real-life settings** that entail choices and options patients and clinicians will actually face.
- Includes diverse populations, especially hard to reach populations.
Patients are part of the Entire Research Process

- Topic Selection and Research Prioritization
- Evaluation
- Proposal Review; Design and Conduct of Research
- Dissemination and Implementation of Results
Research Prioritization Criteria for Patient-Centered CER

- Disease incidence, prevalence and burden (particularly chronic conditions)
- Potential for new evidence to improve health and quality of
- Technical Merit/Scientific Rigor
- Generalizability
- Sustainable
- Patient-centeredness
- Patient and stakeholder engagement
- Relevance to patients and clinicians
- Clear comparators
Research Prioritization: Criteria for Patient Centered CER: Usual Care

- Not preferred
- OK if it is a realistic choice faced by patients, but the clinical characteristics must be specified and justified (e.g., guideline).
- Must be accompanied by an explanation of how the care given in the usual care group will be measured in each individual patient and how appropriate inferences will be drawn from its inclusion.
QI and Patient-Centered CER: Key Similarities

- Focus on improvement in quality, access, care coordination, key patient outcomes
- Pragmatic-real life settings
- Impact of disease on population-what is the opportunity for improvement
- Use of validated measures to assess outcomes and quantify improvement (e.g., NQF measures)
- Involvement of patients and other stakeholders
- Variety of qualitative and quantitative methods
QI and Patient-Centered CER: Key Differences

- **Explicit and well-articulated comparators**: CER must have two or more comparators of proven efficacy.
- **Patient-centeredness**: CER must have patients with lived experience vet the research questions and the key outcomes; they must be rated as important by the patients in question.
- **Patient-Stakeholder Engagement**: CER must have them engaged as equal partners throughout the entire research process.
- **Methods**: CER must have robust design and use scientifically rigorous methods that produce statistically significant and clinically meaningful results generalizable to other healthcare settings.
- **QI is exempted from human subjects protections**.
- **Theoretical and conceptual models** are required to inform research.
Another View of the QI/Patient-Centered CER Relationship

• **QI provides a foundation and testing ground:** Conducts much of the developmental work that forms the basis for the comparators CER seeks. This includes measure development, particularly for key clinical and patient outcomes.

• **CER provides the head to head testing** that health systems and practitioners need to inform their recommendations to patients for treatments, particularly for subgroups, and to adopt better health system delivery models.
Proposed Relationship Between QI and Patient-Centered CER

QI

CER

Development of Efficacy Base for Comparators
Group Exercise
Patient and Stakeholder Engagement in PCORI Funded Research

• What is the effect of patient and stakeholder engagement in research on
  – Changes to research questions, processes, and design
  – Recruitment, retention, and study completion
  – Participants experience in the research
  – Study quality
  – To whom and how results are disseminated
  – User trust in and understanding of findings
  – Uptake of information in clinical decision-making
  – Impact on healthcare and health outcomes
Patients’ Influence on Study Design: Examples

- Investigator changed the study design: from randomizing individual parents and children to a cluster RCT in a telehealth intervention for children with behavioral and developmental health issues.

- Investigator changed the comparators: The Patient Advisory Board members asked for an enhanced version of a pediatric electronic asthma intervention to be the comparator, instead of the paper version versus the electronic version.
Serendipitous Impacts Upon Patients Who Participate: Examples

• **Increased knowledge and skills about research**
  - Patient partners complete HIPAA and human subjects trainings

• **Increased knowledge about or engagement in health/healthcare**
  - Preventive health care study: patient who feared preventive test was able to share fears and concerns with patient partner, who helped make an appointment

• **Personal Transformation**
  - Substance use study: Patient partner states that participating in study helps him feel he is making a contribution, which has helped him maintain recovery
  - Connecting those with serious mental illness through Wellness Center to community based services: Patient partners with SMI lead team meetings with researchers, take and distribute minutes. Patient: I’m not a token. I am asked for my input.”
Relevance of Changes to Clinicians and Researchers

- Transforming the way health services research is conducted by ensuring that as the IOM intended, care provided and the systems that deliver it are truly more patient centered by operationalizing what patient centered research and patient engagement in research means.

- Making the findings more applicable to real life choices that patients and clinicians face together, because the research focuses on currently available options and compares them in real health care settings.

- What can you take back to your practice and your research settings?
Thank you!

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Questions and Discussion