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Comparative Effectiveness Research and Patient Centered Outcomes Research in Public Health Settings: Design, Analysis, and Funding Considerations

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A changing paradigm for health research

- Research often fails to inform the choices faced by clinicians, patients, payors, policy-makers
- Need head-to-head comparisons of all relevant choice options and combinations
- Need evidence on effectiveness in real-world clinical & community settings
- Need to know whether/why interventions work for some and not for others (treatment heterogeneity)
- Need to determine value from the consumer’s perspective (patient-centered outcomes and costs)
CER Defined

- “Comparative effectiveness research is the generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor disease and improve the delivery of care.
- The purpose of CER is to assist consumers, clinicians, purchasers, and policy makers to make informed decisions that will improve health care at both the individual and population levels.”

-National Academy of Sciences
Institute of Medicine, 2009
Patient-Centered Outcomes Research (PCOR) helps people and their caregivers communicate and make informed health care decisions, allowing their voices to be heard in assessing the *value of health care options*. This research answers patient-centered questions such as:

- “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 healthcare?”
To answer these questions, PCOR:

- Assesses the benefits and harms of _preventive_, diagnostic, therapeutic, palliative, or _health delivery system interventions_ to inform decision making, highlighting comparisons and outcomes that matter to people;
- Is inclusive of an individual’s preferences, autonomy and needs, focusing on outcomes that people notice and care about such as _survival, function, symptoms, and health related quality of life_;
- Incorporates a wide _variety of settings and diversity of participants_ to address individual differences and barriers to implementation and dissemination; and
- Investigates optimizing outcomes while _addressing burden to individuals, availability of services, technology, and personnel_, and other stakeholder perspectives
Recent developmental history

- **2003 Medicare Modernization Act**: $30M annually for research to improve quality, effectiveness, efficiency
- **2007** federal legislation to expand CER passed House but failed Senate
- **2009 ARRA**: $1.1B to NIH and AHRQ for CER
  - **Federal Coordinating Council** for CER established
  - **IOM Top 100 Priority Topics** for CER identified
    - 50% involve health care delivery system
    - 33% address health care disparities
    - 20% address patient functional limitations or disabilities
- **2010 ACA**: scale up to $≈500M annually through Patient Centered Outcomes Research Institute
- **2011 ACA**: $10M for Public Health CER at CDC
PCOR Research Priorities

- Assessment of prevention, diagnosis, and treatment options
- Improving healthcare systems
- Communication and dissemination research
- Addressing health disparities
- Accelerating Patient-Centered Outcomes Research and Methodological Research
• Compare the effectiveness of two or more strategies for prevention, treatment, screening, diagnosis, or management that have not been adequately studied against alternative options. Topics are not limited to medical or surgical therapy and may include a range of strategies including self-care.

• Special emphasis is placed on studies conducted in typical clinical populations considering the full range of relevant patient-centered outcomes and possibilities that results may differ among patient groups based on patient characteristics (understood broadly as possibly including clinical, psychosocial, demographic, and other domains) or preferences.

• Compare the use of prognostication/risk-stratification tools with usual clinical approaches to treatment selection or administration.

• Compare the key determinants of the outcomes patients experience following treatment decisions, with attention to various patient factors, including demographic, biological, clinical, social, economic, and geographic factors that may influence the outcomes that follow a specific treatment.
• Research that compares alternative system-level approaches to supporting and improving patient access to care; receipt of appropriate evidence-based care; the quality, timeliness, and safety of the patient care experience; decision-making based on patients’ personal values; and self-care.

• Research that compares alternative approaches to models of care delivery or coordination of care across healthcare services or settings, including care for patients with complex, chronic, and/or multiple conditions, are of interest. The emphasis is on comparing approaches for their effect on patients and, when relevant, their caregivers, in ways that they experience and think are important.

• Research that compares alternative system-level approaches that aim to improve the efficiency of health care delivery to patient populations. These may include efforts to reduce the use of ineffective or wasteful care, to reduce redundant and duplicative care, to shorten waiting times, or enhance the timeliness and quality of communications during referrals and transitions in care.
• Compare alternative communication, dissemination, health literacy and/or implementation strategies that aim to improve patients’ health outcomes, by increasing patient, caregiver, and/or provider **awareness of health care options in clinical or community-based settings**.
• Compare the effectiveness of alternative approaches across a range of patient centered outcomes to increase or encourage effective patient, caregiver, or clinician **participation in care decisions and in shared decision making**.
• Compare alternative methods and tools to **elicit and include patient desired outcomes** in the health care decision making process.
• Compare alternative approaches, including **use of public health strategies or social media, for providing new information to patients, caregivers, or clinicians**, with attention to differences in effectiveness in different populations.
• Compare innovative approaches in the **use of existing electronic clinical data and other electronic modalities from the healthcare system** or from a network of systems to enhance clinical decision making by patients and providers.
**Disparities Research**

- Compare interventions to *reduce or eliminate disparities* in patient-centered outcomes, including health, health care, and patient-reported outcomes. For example, by accounting for possible differences at the patient, provider, or systems level, determine what interventions can be most effective for eliminating disparities in outcomes.

- Compare promising practices that address contextual factors such as *socioeconomic, demographic, or community factors* and their impact on patient-centered health outcomes.

- Compare benefits and risks of treatment, diagnostic, *prevention*, or service options across different patient populations, with attention to eliminating disparities.

- Research that compares strategies to overcome patient, provider, or *systems level barriers (e.g. language, culture, transportation, homelessness, unemployment, lack of family/caregiver support)* that may adversely affect patients and are relevant to their choices for preventive, diagnostic, and treatment strategies—as well as patient-centered outcomes.

- Compare and identifies best practices within various patient populations for *information sharing* about treatment outcomes and research.
Methods Research

- Identify optimal methods for engaging patients in the research process, and methods for evaluating the impact on research outcomes of patient engagement in the research process.
- Identify methods for conduct of systematic reviews of patient-centered comparative effectiveness research topics.
- Test methods for including patients and stakeholders in generating, selecting, and prioritizing topics for research.
- Test methods for including patients and stakeholders in the peer-review process.
- Improve the validity and/or efficiency of analytic methods for comparative effectiveness research (e.g., approaches for strengthening causal inference in observational and randomized studies; approaches to identifying and confirming heterogeneity in treatment effects).
- Determine the validity and efficiency of data sources commonly used in PCOR.
- Develop new patient-reported outcomes measures.
- Evaluate and compare strategies for training researchers, patients, and other stakeholders in PCOR methods.
Where Does Public Health Fit in CER/PCOR?

- Public health roles in community engagement, priority setting, preference assessment
- Public health roles in prevention delivery
- Use of public health strategies to improve health care systems
- Public health system roles in communication and dissemination
- Public health system roles in health information exchange, quality measurement, and reporting
- Public health system roles in disparities reduction
Valuing Prevention & Public Health

Methods in CER and PCOR

PCORI Draft Methodology Report

- Stakeholder engagement and patient centeredness
- Prioritizing research topics
- Choosing a study design
- Designing, conducting, and reporting results
Methods in CER and PCOR

- Prospective, **pragmatic trials** and “large simple trials”
- Advanced analytic methods used to strengthen internal validity and limit bias due to selection, confounding in observational studies
  - Propensity score and instrumental variables models
  - Explicit testing for **treatment heterogeneity**
  - Latent variable models for multiple interventions, multiple outcomes
  - Non-inferiority analyses
- Development and integration of large clinical and administrative **data sources and registries**
- Use of Bayesian models for synthesizing data from multiple studies, e.g. indirect treatment comparisons
Expanding Role of Pragmatic Trials

**Test**
1. Identify two or more policy interventions to compare (e.g. old vs new policy; different variations of a policy).
2. Determine the outcome that the policy is intended to influence and how it will be measured in the trial.
3. Decide on the randomisation unit: whether to randomise to intervention and control groups at the level of individuals, institutions (e.g. schools), or geographical areas (e.g. local authorities).
4. Determine how many units (people, institutions, or areas) are required for robust results.
5. Assign each unit to one of the policy interventions, using a robust randomisation method.
6. Introduce the policy interventions to the assigned groups.

**Learn**
7. Measure the results and determine the impact of the policy interventions.

**Adapt**
8. Adapt your policy intervention to reflect your findings.
9. Return to Step 1 to continually improve your understanding of what works.

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Estimating Treatment Heterogeneity

- Programs/Policies/Interventions may affect some people differently than others.
- Interventions may be moderated by community contextual factors, socioeconomic conditions, cultural and linguistic characteristics, individual health status and comorbidities.
- If you know and measure all relevant modifiers, traditional statistical methods can be used.
- Often, important modifiers may be unknown, others imperfectly measured.
- *Instrumental-variables* methods provide the most viable solution for estimating “person-centered” effects.
Estimating Treatment Heterogeneity

Surgery versus Active Surveillance

Absolute z-score for Survival PeTs: ○ > 1.96  ○ 1.645 - 1.96  ● < 1.645

Methods in CER and PCOR

PCORI Draft Methodology Report

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Example: Pragmatic Trials in PBRNs

Improving Cultural Competency of Public Health Workers

**Question of interest:** Can a health professions cultural competency training program be adapted to improve skills among local public health workers?

**Practice settings:** 56 local agencies

**Factors examined:**
- Knowledge and skills related to CLAS standards
- RE-AIM measures of success

**Study design:** random-assignment delayed intervention trial
Example: Estimating Treatment Heterogeneity in PBRNs

Effects of Medicaid Maternity Case Management Payment Model Change in North Carolina

- Policy change may affect some populations differently from others
- Women served by LHDs vs. other community providers
- Women in communities with more vs. less abundant community resources
- Women with comorbid conditions and higher-risk pregnancies
- Use Bayesian CER methods, propensity score estimation, instrumental-variables estimation for person-centered treatment (PET) effects
Example: Comparative efficiency of a delivery system innovation

Arkansas Community Connector Program uses public health-trained community health workers to identify and link elderly and disabled populations to community-based services and supports to avoid/delay need for institutional care.

Compare the CCP program to “usual practice” on Medicaid expenditures for elderly and disabled recipients eligible for long-term care services.

Determine whether the CCP program is cost-neutral to Medicaid after accounting for both Medicaid expenditures and program operating costs.

Felix HC, Mays GP, Stewart MK, Cottoms N, Olson M. Medicaid savings resulted when community health workers linked those with needs to home and community care. *Health Affairs*. 2011;30(7):1366-1374.
Methods: Comparison Group

Comparison Group: statistically matched on age, gender, race, eligibility category, enrollment duration, waiver enrollment, comorbidities, prior-year spending
Results of propensity score matching

Approximate a “statistically equivalent” control group that would be generated by random assignment. Each subject has an equivalent probability of being a CCP participant.
Estimates of Program Impact

Regression-Adjusted Spending Estimates

![Graph showing Medicaid spending per recipient over time for Comparison Group (dashed line) and CCP Participants (solid line). The graph displays spending trends from Pre-program to Year 3.]
CER/PCOR Funding Opportunities

- PCORI: third round deadlines in Spring 2013
- AHRQ: next round of awards scheduled for Spring 2013
- RWJF PHSSR awards program (current deadline Dec 18)
- RWJF PBRN Program (forthcoming funding 2013)
- Special RWJF/CTSA solicitation this winter
- NIH Common Fund: health economics program
- CMS Innovation Fund projects
- CDC Community Transformation Grant projects
The Robert Wood Johnson Foundation’s Public Health PBRN Program

- First cohort (December 2008 start-up)
- Second cohort (January 2010 start-up)
- Affiliate/Emerging PBRNs

Map of the United States indicating the distribution of the PBRN Program across different states.
Concluding Questions

- How can we help the public health community become aware of opportunities in PCOR and CER?

- How can we help the public health community position to be successful with PCOR and CER funding?
  - Meaningful stakeholder engagement
  - Rigorous research design and methods
  - Preliminary data
  - Multi-network studies, large simple trials
  - Collaborations with primary care PBRNs, CTSAs, etc

- How can we facilitate the productive dissemination and use of PCOR and CER evidence via the public health system?
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