Involving Stakeholders in Comparative Effectiveness Research and Patient-Centered Outcomes Research Approaches and Lessons Learned
Johns Hopkins University
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The Center for Medical Technology Policy (CMTP) is an independent, non-profit 501(c) (3) organization that seeks to advance health care innovation and effectiveness by improving the quality, relevance, and efficiency of health care research. CMTP works on methods, infrastructure and policy to support the conduct of comparative effectiveness research that generates information to assist patients, clinicians, and payers in making informed clinical and health policy decisions.
Overview

- Who are the stakeholders?
- What is stakeholder engagement?
- How can researchers start engaging stakeholders?
- What are some lessons learned about engaging stakeholders in CER?
Reasons evidence produced by current clinical research enterprise not translated into practice

- Differences between settings where research is conducted and where medicine is practiced
- Failure to report how treatment effects vary in individual patients and subgroups
- Underrepresentation of children, women, ethnic minorities, patients with comorbidities
- *Research priorities, study questions, endpoints, etc. defined by researchers and funders, not end users*
Stakeholder engagement is one proposed solution

- There is growing evidence that research is improved by involving stakeholders at key decision points.
- Particular emphasis on involving patient perspective.
- Nevertheless, the pathway forward is not entirely smooth.
Barriers to Involving Stakeholders in CER

- Confusing terminology
- Lack of shared understanding of what it means to “successfully” involve stakeholders in research
- Limited data regarding impact; systematic evaluation rare
- Timing, restrictions on number/availability of stakeholders
- Concerns that process will add time and costs to project plans
Addressing the Barriers

- Literature review
  - Biomedical
  - Social science
- Practical experience based on projects involving stakeholders
- Drafted definitions and conceptual model
- Review and revision by an expert panel
  - Patient and Consumer Advisory Council
  - NICE Patient and Public Involvement Program and Citizen Council in the UK
- Applied it to a complex multi-stakeholder project
Target stakeholder groups for CER
Deciding which stakeholder groups to involve in a project

- What topic(s) does the research address?
- What health care decision is the research meant to inform?
- Who are the decision makers responsible for these decisions?
- Who are the individuals and groups that are affected by these decisions?
Stakeholder Engagement

A process of actively soliciting the knowledge, experience, judgment and values of individuals selected to represent a broad range of direct interests in a particular issue, for the dual purposes of:

1) Creating a shared understanding;
2) Making relevant, transparent, and effective decisions.

Methods of combining evidence

**Process**
- Meta-criteria, Trust, Respect, Accountability, Legitimacy, Fairness, Competence
- Change in Knowledge/attitudes
- Change in CER project decisions (e.g. choice of interventions, study design, funding priorities)

**Outputs**
- More useful evidence for clinical and health policy decision making
- More efficient use of healthcare resources
- Improved health outcomes.

**Inputs**
- Types of evidence
  - Values
  - Research
  - Professional Experience
  - Patient and consumer knowledge and experience

**Methods**
- Methods of combining evidence
  - Quantitative
    - Questionnaires
    - Delphi method
    - Multi-Criteria Mapping
    - Value of Information modeling
  - Qualitative
    - Facilitated workshops/meetings
    - Stakeholder decision analysis

**Decision**
- Topic generation
- Research priorities
- Study designs
- Evidentiary thresholds for clinical and health policy decision making
- Implementation strategies

**Conceptual Model for Stakeholder Engagement in CER**
When to Involve Stakeholders in CER?

**Priority Setting**
- Major opportunity to exert influence
- Methods most well-described, but still evolving and lacking evaluation

**Study Design**
- Stakeholders should be involved here but may require additional preparation
- Skepticism from experts can be overcome

**Translation to Practice**
- High unmet need; reasons for slow or ineffective translation are multi-factorial
- Methods in development
Stakeholder Engagement Process

Recruitment
- Research
- Snowball sampling
- Vetting qualifications
- Communicate role and expectations
- COI

Preparation
- Provide background literature, case-studies, and meeting agenda
- Customized for stakeholder groups

Engagement
- In-person meetings
- Conference calls
- Online interactions
- Voting
- Iterative process

Evaluation
- Post-meeting interviews
- Surveys
- Pre-post or randomized designs ideal

Implementation
- Customized dissemination strategy enabled by stakeholder groups
- Publication of results – multiple channels

Outcomes
- Buy-in
- Shared understanding
- Trust
- Accountability

Define selection criteria; COI procedures

All stakeholders need prep

Multimodal channels needed. Possible to involve lay persons in technical discussions

Needs to be done routinely and published

Underutilized and Understudied. Describe stakeholder contributions in publications

Important to define both Process and CER outcomes at study outset
PCORI’s conceptualization of Patient-Centeredness

- Does proposed research compare effects of treatment options that matter to patients?
- Are these realistic treatment choices faced by patients or other decision makers?
- Does research focus on outcomes of interest to patients and their caregivers?
  - Health
  - Health-related QOL
  - Function
  - Symptoms
  - Shared decisions
  - Safety from medical harm
  - Survival
  - Satisfaction with care
Future Directions

- PCORI has identified three research gaps that will be the focus of future methods work:
  - What are the consequences of patient engagement in research on health decisions and clinical outcomes?
  - What are the specific consequences of patient engagement on the research process?
  - Which patient engagement methods are most effective, and for which populations?

- Trends we’ve observed
  - Payers role as less involved stakeholder group changing – increasing emphasis on resource utilization and costs
  - Greater use of quantitative methods, particularly for priority-setting
Summary

- Stakeholder engagement is essential for fulfilling the objectives of CER
  - Research funding needs to account for costs of implementing meaningful engagement activities
- The terminology and methods are being developed, worked out for CER
  - Methods need to be tailored to the research question
  - All stakeholders need to be familiar with the full process
- There is a growing body of examples of rigorous methods of SE being applied to CER
- Evaluation is critical for measuring impact and process improvement