Patient Centered Outcomes Research: An Overview

Navkiran K Shokar MA MD MPH
Associate Professor
Family and Community Medicine & Biomedical Sciences
Co-Center Director COE-Cancer
Vice Chair for Research FCM
TTUHSC-PLFSOM
Objectives

- Describe the development and importance of patient centered outcomes research (PCOR)

- Describe the PCOR Institute and its funding priorities

- Discuss the 5 challenge areas that will need to be addressed to effectively implement PCOR

- Explore potential solutions, identify areas for further research and development
CER became controversial politically.
Concerns about curbs on physician latitude and role of costs.
Patient Centered Outcomes Research (PCOR) is a national priority

- AHCPR (now AHRQ) ~ outcomes and effectiveness research
- American recovery and reinvestment act (ARRA) included $1.1 billion to encourage comparative effectiveness research.
- IOM Report advocating a national system to promote medical research to support better decision making by physicians and patients.
- The Affordable Care Act of 2010 authorized the Patient Centered Outcomes Research Institute (PCORI), as an independent not for profit organization.
PCORI

- A federal funding agency - set up as a non-profit corporation

- Established by law in 2010 as part of the ACA legislation

- Funded by the PCORI trust fund

- Expected to receive $3.5 billion to fund PCOR through September 2019

- http://www.pcori.org/
What is PCOR?

IOM definition

- generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care.

- The purpose of comparative effectiveness research 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...
What is PCOR

PCORI Definition

- Patient-centered outcomes research (PCOR) helps people and their caregivers communicate and make informed healthcare decisions, allowing their voices to be heard in assessing the value of healthcare options.
PCORI Priorities

- Assess the benefits and harms of preventive, diagnostic, therapeutic, palliative, or health delivery system features 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 (or may investigate) optimizing outcomes while addressing burden to individuals, availability of services, technology, and personnel, and other stakeholder perspectives.
Why do we need PCOR?

- Improve quality
  - *IOM:* 50% of interventions have inadequate evidence of effectiveness.
- Lack of clear guidance for best options for a patient’s specific situation (clinical, demographic).
- Information for payors/policymakers.
- Information for patients.
- Data on *relative effectiveness.*
Haven’t we been doing PCOR all along?

1. The question has been does this work? (not is this better than that)

2. RCTs are efficacy studies, i.e. done under ideal conditions that never actually exist in real world practice

3. Do not always include outcomes that matter to patients (or payors)
PCOR Steps Needed

1. Identify new and emerging clinical interventions.
2. Review and synthesize current medical research.
3. Identify gaps between existing medical research and the needs of clinical practice.
4. Promote and generate new scientific evidence and analytic tools.
5. Train and develop clinical researchers.
6. Translate and disseminate research findings to diverse stakeholders.
7. Reach out to stakeholders.
PCORI Funding Areas

1. Assessment of Prevention, Diagnosis and Treatment Options

2. Improving Health Care Systems

3. Communication and Dissemination Research

4. Addressing Disparities

5. Accelerating Patient-centered Outcomes Research and Methodological Research.
Current Paradigm

Observational

RCTs

Systematic reviews

Practice Guidelines
Future Paradigm

Better decision making information and tools for patients

Better decision making for providers

Systematic reviews

Practice Guidelines

Observational

RCT & PCTs

Future Paradigm
PCOR Challenges

- Improved methods
- Develop better tools for both patient and provider decision making
- Develop better implementation and dissemination
- Focus on clinical areas where effectiveness not established
- Focus on outcomes relevant to patients
Improved methods

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- Focus on clinical areas where effectiveness not established
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Develop better implementation and dissemination
Improved Methods:

What we need:

1. Compare multiple active treatments in real world settings
2. Focus experimental resources on the most promising approaches
3. Identify patient subgroups in which treatments are more or less effective
4. Introduce new treatments into the evaluation process quickly
5. Provide information to help patients, physicians and policymakers.
RCTS Improved Methods

RCTS the FUTURE:

a) Pragmatic trial designs

b) Increase operational efficiency

c) Improve analytic efficiency
RCTS: a) Pragmatic trial designs

- Clinically effective comparators.
- Common co-morbid conditions allowed
- Diverse demographic groups
- Providers from community settings (PBRNs)
- Patient centered primary and secondary outcomes
- New techniques to eliminate bias and confounding. Per protocol v intention to treat analysis

Table:

<table>
<thead>
<tr>
<th>Eligibility</th>
<th>Design Flexibility</th>
<th>F/u intensity</th>
<th>F/u Duration</th>
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<tbody>
<tr>
<td>Practitioner Expertise</td>
<td>Participant Compliance</td>
<td>Primary Analysis</td>
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PRECIS (Pragmatic-Explanatory Continuum Indicators)
<table>
<thead>
<tr>
<th>Pragmatic</th>
<th>Explanatory</th>
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<tr>
<td><strong>Pros</strong></td>
<td>If positive, it really works and you can implement the treatment just about everywhere</td>
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<tr>
<td></td>
<td>If negative, you can abandon the treatment (it won’t work anywhere)</td>
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<tr>
<td><strong>Cons</strong></td>
<td>If negative, you can’t distinguish a worthless treatment from an efficacious treatment that isn’t applied/accepted widely enough</td>
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<tr>
<td></td>
<td>If positive, you still don’t know whether it will work in usual health care conditions</td>
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RCTS: b) Increase Operational Efficiency

- RCTS are costly, lengthy and complex

- Comparisons of multiple effective treatments will require larger sample sizes to detect differences and results will be needed to be delivered more quickly.

- There are several ongoing initiatives to streamline the process (public/private) NIH CTSA, NCI IOM, FDAs CTTI (clinical trials transformative initiative)
RCTS: c) Increase analytic efficiency

- Real world RCTs = larger sample sizes: noise, effect sizes
- *Bayseian and adaptive* v Frequentist approaches: will allow more flexibility in trial design.
- Adaptive RCT designs: ~ add new interventions and drop ineffective ones. Only most relevant interventions are included at any time.
- Unequal random allocation: More patients - allocated to the most promising strategies as evidence accumulates. Tailor rx to clinical subgroups.
- Increasingly used in pharmaceutical protocols
Improved Methods

Observational research
• Definition: natural experiments
• Need to improve the observational research infrastructure.
• Develop data networks and more efficient data collection.
• Use of data collected for administrative purposes during health care delivery
  ▫ + generalisable
  ▫ - lack of randomization and data quality
• Increased use of EHRs
  ▫ Overcome technical barriers
  ▫ Analytic challenges
  ▫ Confidentiality
PCOR

Improved methods

Focus on clinical areas where effectiveness not established

Focus on outcomes relevant to patients

Develop better tools for both patient and provider decision making

Develop better implementation and dissemination
Identify Research priorities

• IOM prepared a portfolio of research priorities

• Solicited from stakeholders

• Reviewed and prioritized into 4 quartiles

PCOR Challenges

- Improved methods
- Focus on clinical areas where effectiveness not established
- Focus on outcomes relevant to patients
- Develop better tools for both patient and provider decision making
- Develop better implementation and dissemination
3. Choose Outcomes that matter: Patient Reported Outcomes (PROs)

- Standardised method of capturing patient data

- PROs: *any report of the status of a patient’s health condition that comes directly from the patient, without interpretation of the patient’s response by a clinician or anyone else*....

- E.g. Prostate cancer treatment decisions.

- **Future:** Linking PROs to admin claims data, and EHRS that link into systems such as Adverse Event Reporting System could impact safety and quality of care.
PROs

Potential constructs:

• Symptoms
• Functional status
• Health perceptions
• Health related quality of life (HRQoL)
• Satisfaction with care
• Access to care
• Perceived treatment benefit or harm
• Health behaviors
• Co-morbidities
• Treatment adherence
• Caregiver burden
PROs Considerations

- Health Profile Measures - Broad vs. disease specific e.g. WHO QOL, SF 36, SF12
  - SF 36
    - Physical functioning
    - Role-physical
    - General health
    - Bodily pain
    - Mental health
    - Vitality
    - Role mental
    - Social functioning
    - Summary scores
PROs

- Disease Specific, E.g. MOS-HIV, HAT QOL, functional assessment of cancer therapy-general
- Can do a combination of general and specific
- Look for reliable and validated measures
- Sensitivity to change over time, floor and ceiling effects
- Longitudinal - how to handle missing data
- Specify minimum important difference (MID)
- NIH Patient reported outcomes measurement information. PROMIS . http://www.nihpromis.org
PCOR Challenges

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Research Valley of Death

Takes 15 years for a research discovery to be used in clinical practice, if used at all
T3: Translation to Practice

- Usual process has been evidence synthesis and guideline development by authoritative entities,
  - United States Preventive Services Task Force
  - Medical Specialty Societies, e.g. American College of Cardiology, AGA, ADA, AHA, etc..

- However, evidence suggests that there is a large and growing gap between clinical research and practice in many areas of health care and public health
Communication, Implementation and Dissemination

• How best to engage patients/providers in the process of shared decision making

• Synthesize the evidence and develop and implement patient education tools of appropriate literacy level and language

• How to integrate this process into current clinical practice, *PCMH model*..

• Make information available to physicians at the point of care ~integrated into the EMR.
T3: Translation to practice

What are the barriers?

- Tension between efficacy and effectiveness
  - **Efficacy**: ability of an intervention to do more good than harm under ideal conditions (RCT)
  - **Effectiveness**: Ability of an intervention to do more good than harm in a real world setting

- Most studies are very tightly controlled, intensive, strict inclusion, exclusion criteria, etc..

- Lack of information about how they can be adapted to real world settings and still be beneficial

- The science and methods of **implementation science** are developing in order to better understand this process.
T3: Translation to practice

Dissemination research:

• Systematic study of how the targeted distribution of information and intervention materials to a specific public health audience can be successfully executed so that increased spread of knowledge about the evidence-based public health interventions achieves greater use and impact of the intervention.

• Examples of strategies:
  ▫ Social marketing
  ▫ Training/capacity building
  ▫ Linking systems
  ▫ Coalition building
  ▫ Media advocacy
  ▫ Agenda setting
T3: Translation to practice

Implementation research

• Studying the courses of action through which an idea, decision, rule, procedure, program or practice is put into use
• Public health programs
• Practice based research networks (PBRNS)
• Involves organisational change
• Mixed methods, community based participatory research approach
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Key References


- http://effectivehealthcare.ahrq.gov/index.cfm/what-is-comparative-effectiveness-research1/

Questions?