



UNIVERSITY *of* MARYLAND
SCHOOL OF PHARMACY

Patient-Centered Outcomes Research (PCOR)

A Framework for Meaningful Assessment of Value

C. Daniel Mullins, PhD

Professor, University of Maryland School of Pharmacy



Disclaimer: The views expressed in this presentation are solely those of the speaker and do not necessarily represent the views of the Patient-Centered Outcomes Research Institute (PCORI), its Board of Governors or Methodology Committee.

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Take-Away Points

- Patient-centered outcomes research (PCOR) allows the patient's voice to be heard
- Because PCOR provides more meaningful data, patients and their health care providers
 - Can make better treatment decisions
 - May be more adherent to evidence-based recommendations
 - May contribute to reductions in wasteful spending
- Value and return on investment (ROI) depends on
 - Perspective
 - What is included in "costs" of disease



Objectives:

- Describe methods for comparative-effectiveness research (CER)
- Describe methods for patient-centered outcomes research (PCOR)
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- Define the joint role for CEA and PCOR in formulary decision making



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Comparative Effectiveness Research (CER)

Definitions of Comparative Effectiveness Research



Comparative Effectiveness Research (CER)

- CER is the process of evaluating the impact of different options for treating a given medical condition for a particular set of patients



Comparative Effectiveness Research (CER)

CER is the 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.

-Institute of Medicine (IOM)



Comparative Effectiveness Research (CER)

- Active comparators (i.e. not a placebo comparator)
- Broad patient population
- Outcomes that are meaningful to patients
- Innovative Methods
 - Clinical trials
 - Registries
 - Electronic health records
 - Insurance claims datasets





Comparative Effectiveness Research (CER)

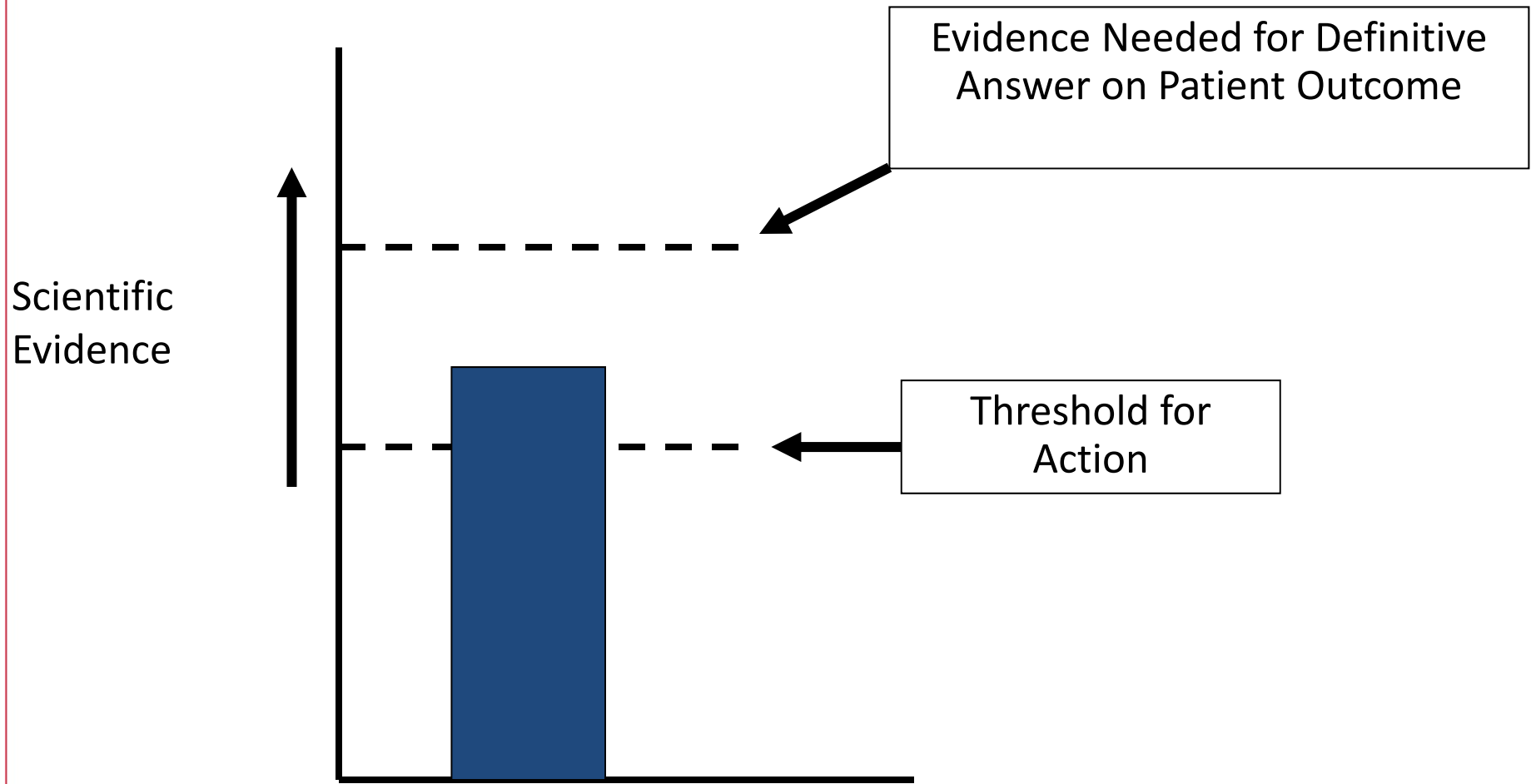
“What differentiates CE from conventional medical research is that it is demand-driven rather than investigator-driven. Demand is defined by the needs of decision makers, including public and private payers, clinicians, patients, and professionals.”



The Need for Additional Evidence

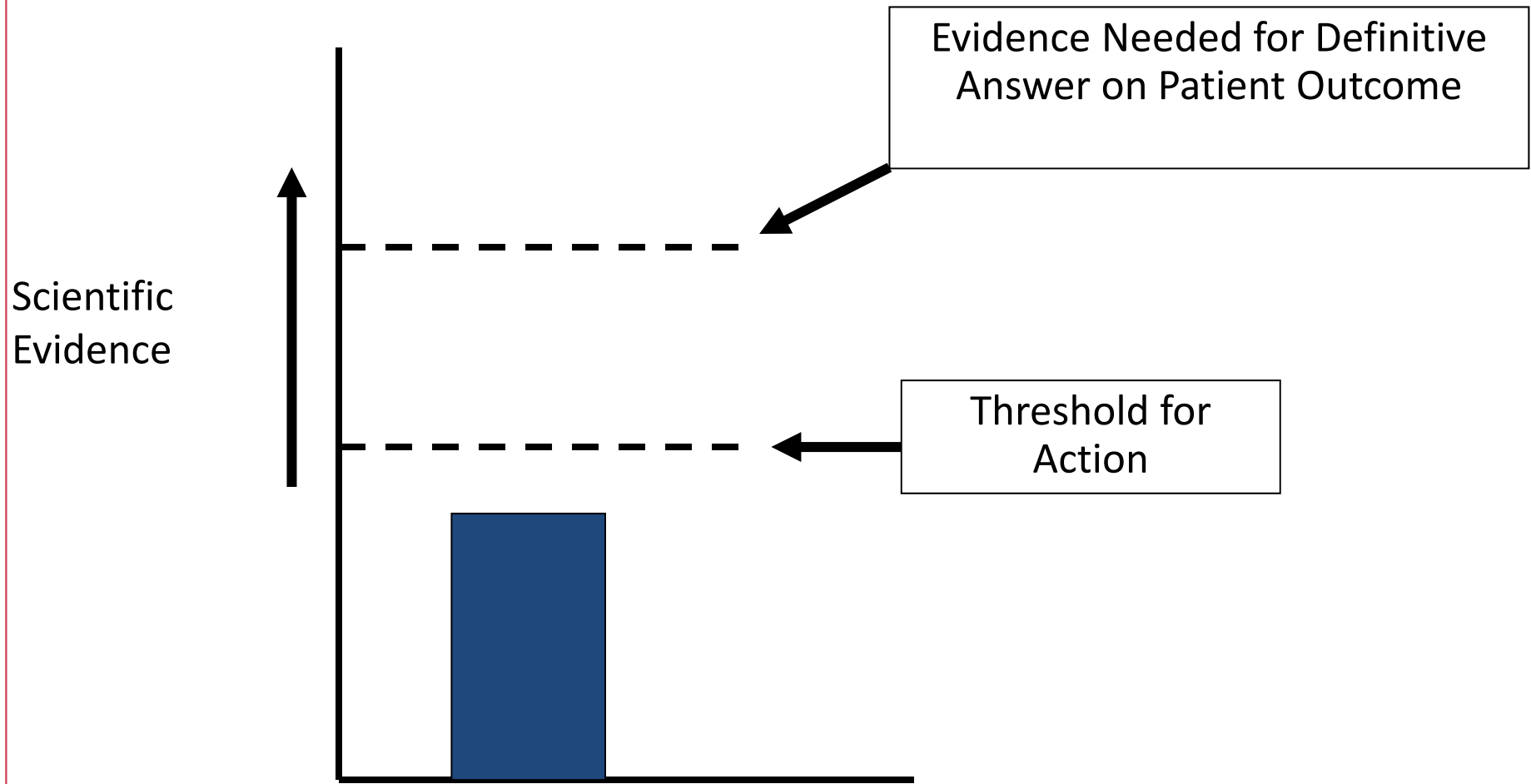


Best Case Situation: Threshold for Action vs. Definitive Answer



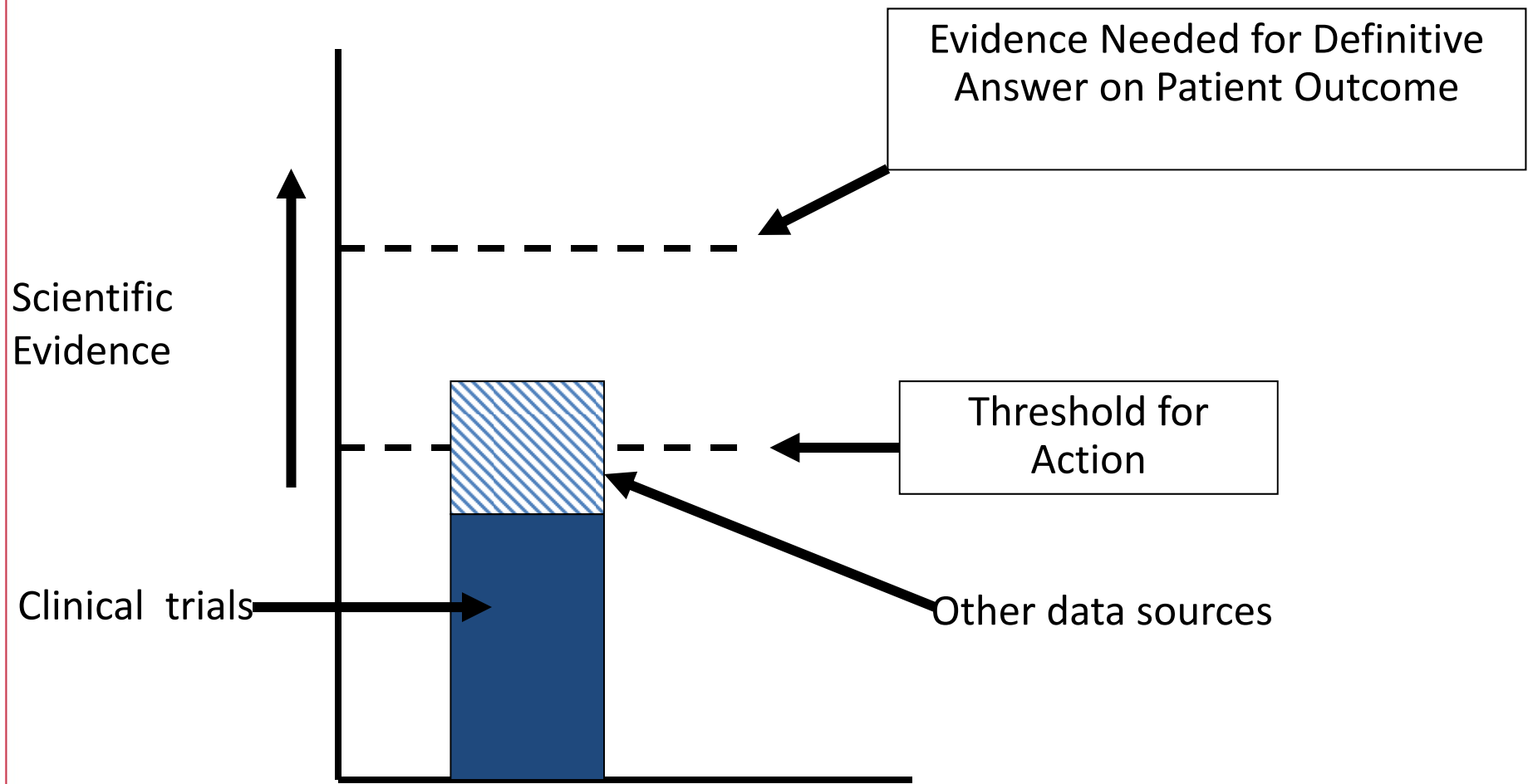


Worst Case Situation: Threshold for Action vs. Definitive Answer





CER Closes the Evidence Gap in order to get to the Threshold for Action





Filling the evidence gap

- It's important to fill the right gap/answer the right question
- Simultaneously “too little” and “too much” info



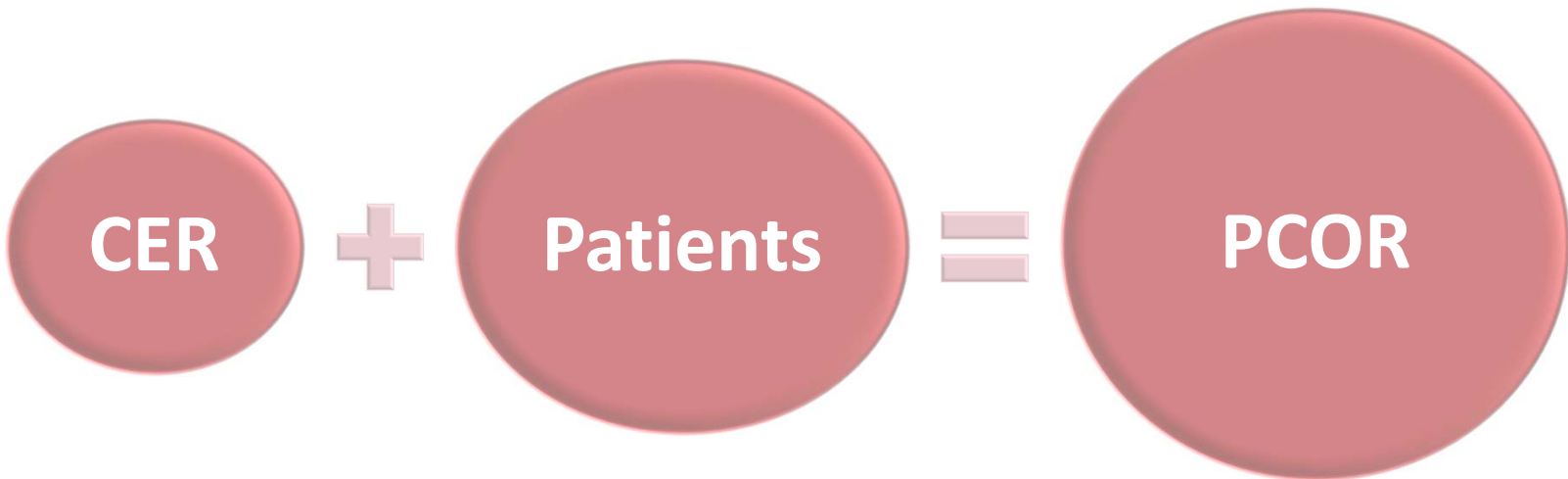


Patient-Centered Outcomes Research (PCOR)

Definitions of Patient-Centered Outcomes Research



Patient Centered Outcomes Research (PCOR)



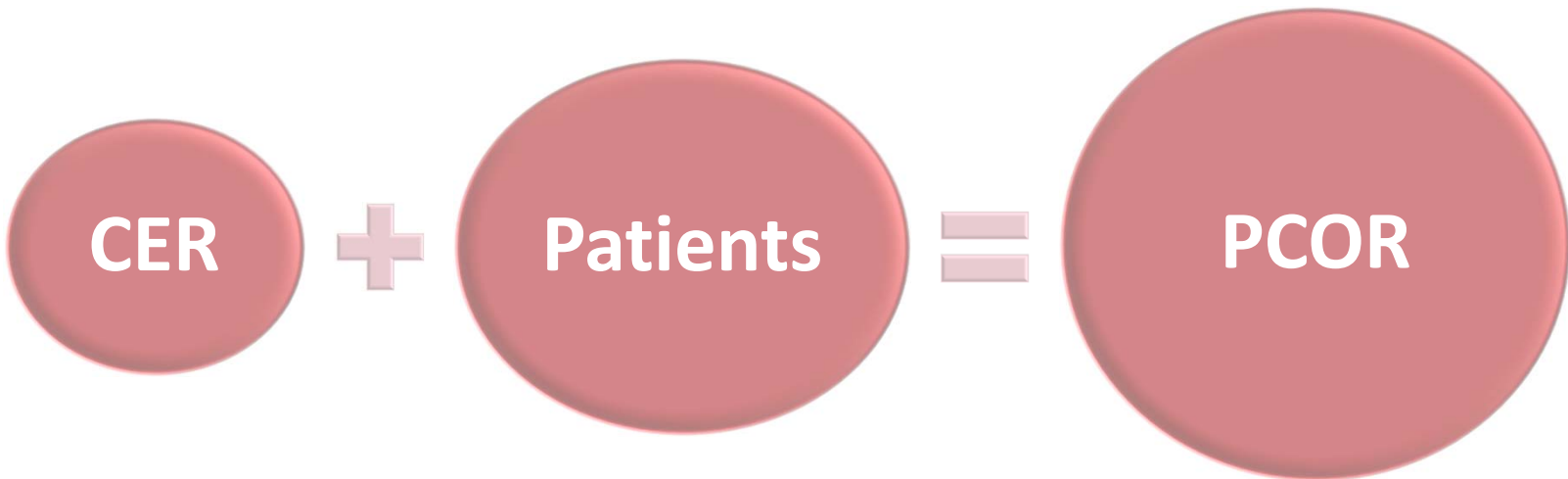
“PCOR involves the conduct and synthesis of research comparing the benefits and harms of different interventions and strategies to prevent, diagnose, treat and monitor health conditions, as well as the delivery of health care in ‘real world’ settings.”

- AHRQ PA-12-114

<http://grants.nih.gov/grants/guide/pa-files/pa-12-114.html>



Patient Centered Outcomes Research (PCOR)

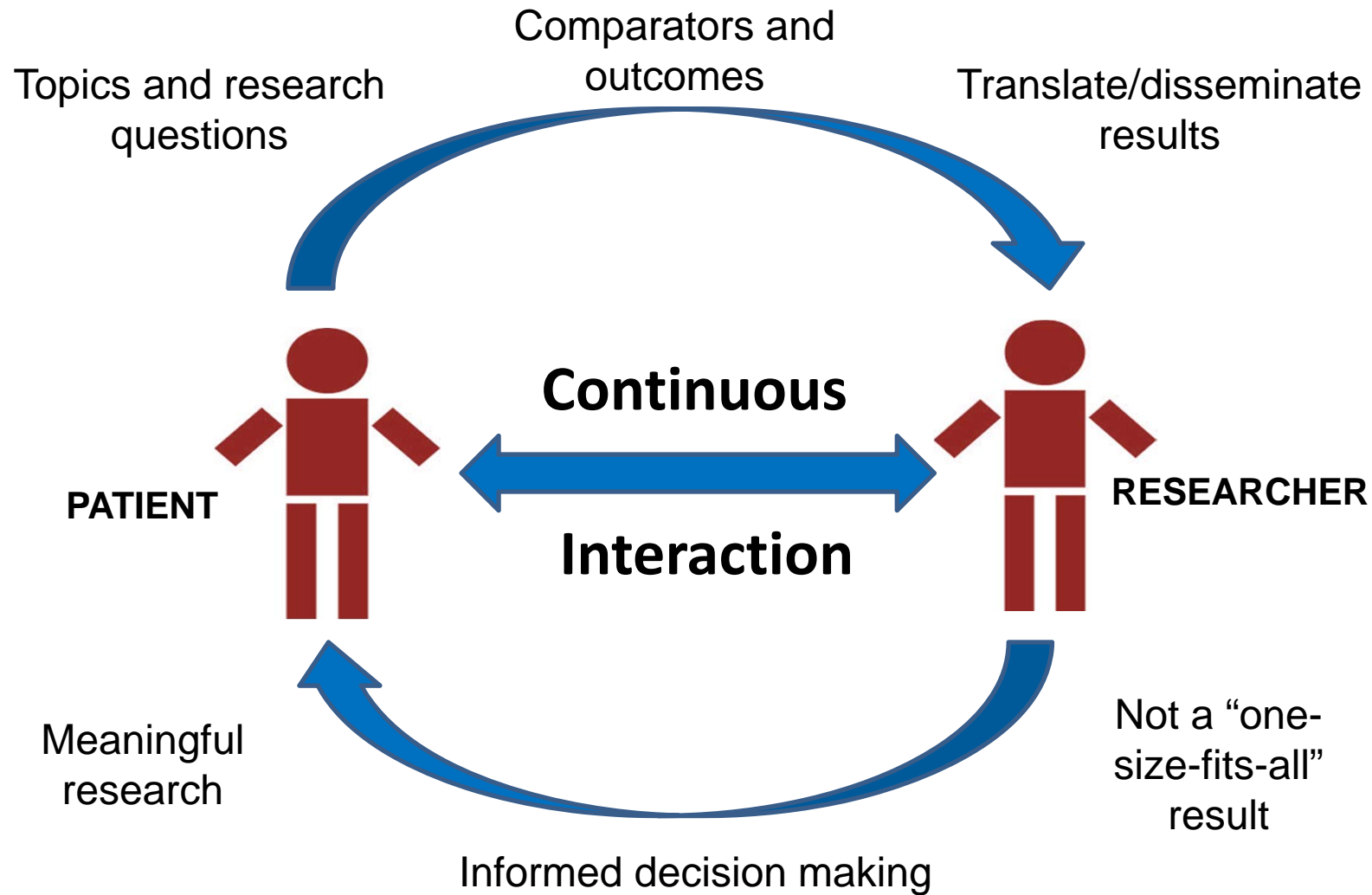


“Patient-Centered Outcomes Research (PCOR) helps people and their caregivers communicate and make informed health care decisions, allowing their voices to be heard in assessing the value of health care options. This research answers patient-centered questions such as:

1. ‘Given my personal characteristics, conditions and preferences, what should I expect will happen to me?’
2. ‘What are my options and what are the potential benefits and harms of those options?’ ...”



PCOR is a Win for Everyone





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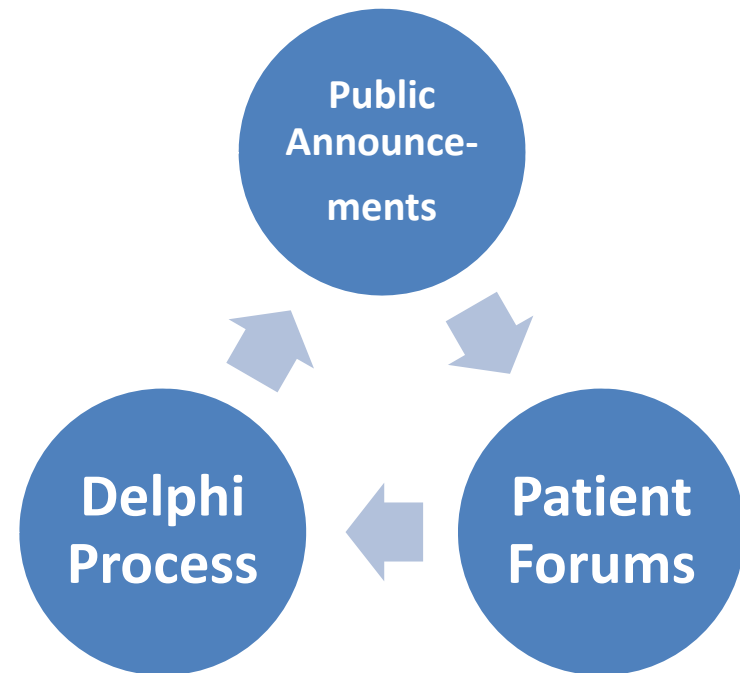


- Patient engagement at every step transforms CER-> PCOR
- Patient engagement is a continuous process
 - Patient engagement requires trust & respect
 - Pre-engagement is a critical component
- Patient engagement should involve diverse patients including hard-to-reach patients



10-Step Process for Conducting CER *

1. Topic Solicitation
2. Prioritization
3. Framing the Question

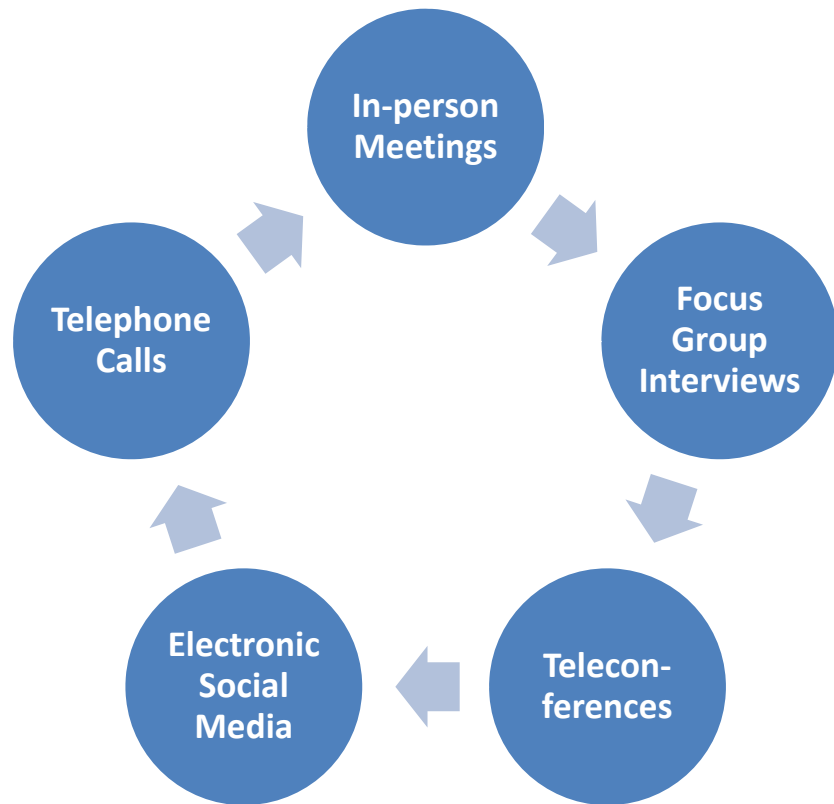


* Based on Mullins CD, Abdulhalim AM, Lavalley DC. Continuous Patient Engagement in Comparative Effectiveness Research. JAMA 2012; 307(15): 1587-8.



10-Step Process for Conducting CER *

4. Selection of Comparators and Outcomes
5. Creation of Conceptual Framework
6. Analysis Plan
7. Data Collection

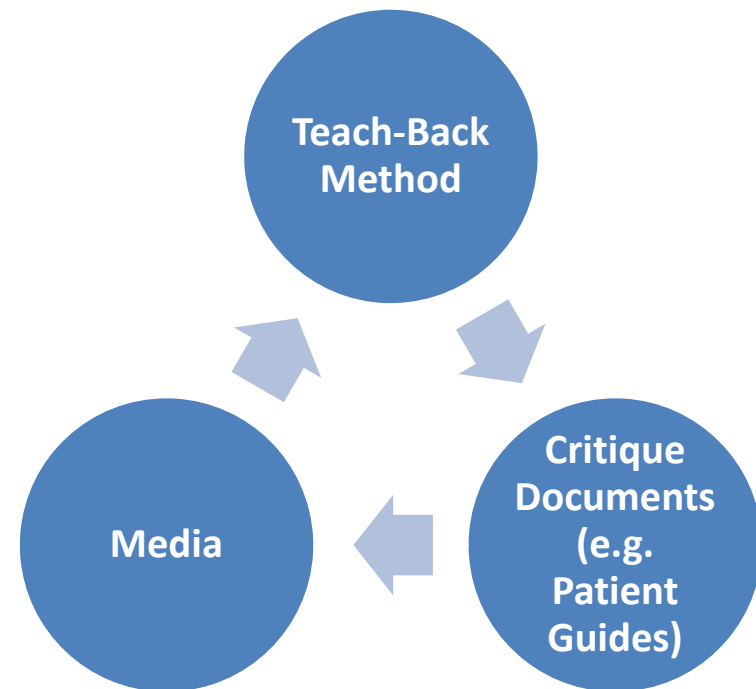


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10-Step Process for Conducting CER *

8. Reviewing & Interpreting Results
9. Translation
10. Dissemination



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Patient Engagement: Why?

- Makes PCOR meaningful
 - Framing the ***question*** so it is understood
 - Framing the ***answer*** so it is understood
 - Framing for ***cultural appropriateness***
 - Does meaningful imply significant or impactful?
- Helps with patient activation
 - Having the essential knowledge, skills and confidence for managing one's own health and healthcare*



Patient Engagement: Why?

- Public health could be improved
 - Patients have unique insights
 - Priority setting is important
 - Pragmatic considerations
- It shows an honest commitment to PCOR



Patient Engagement: How?



The journey begins by
meeting patients
where they are



Patient Engagement: How?

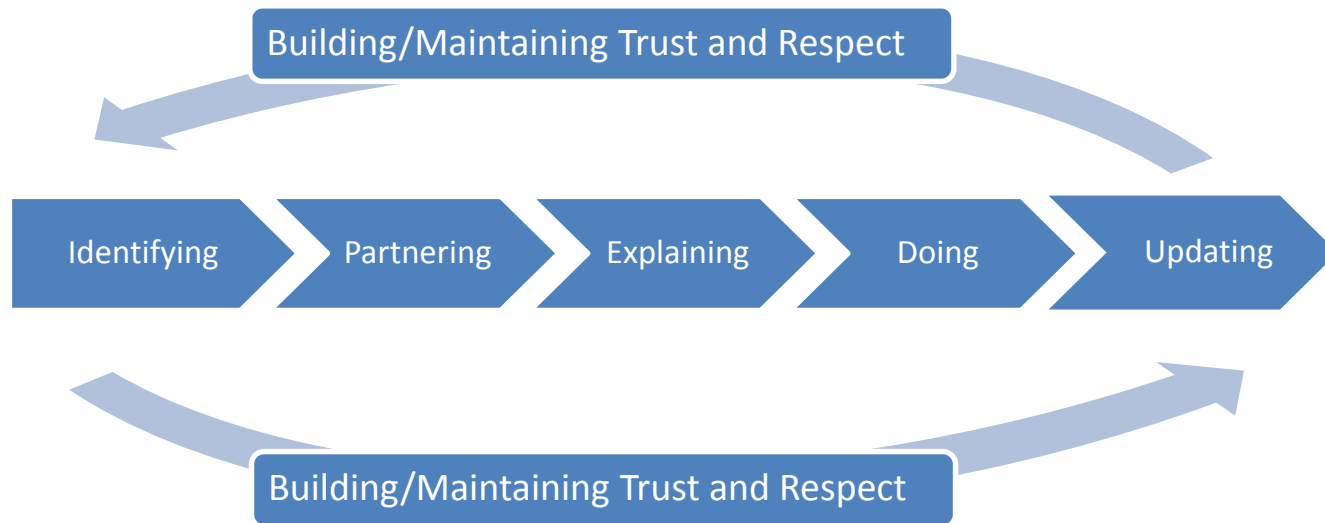


There are others along the journey who can help:

- Community Members
- Leaders
- Organizational Partners



Patient Engagement: How?





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The (Patient-Centered) Value Equation



Historically, productivity is typically not included (or used for calculating cost sharing) in formulary decision making



The (Patient-Centered) Value Equation

- Indirect costs often half the total cost of illness
- If indirect costs are considered, the “value” of reducing amenable morbidity is twice the amount when indirect costs are added to direct medical costs savings
- From a cost-effectiveness viewpoint, the incremental cost-effectiveness ratio is cut in half



The (Patient-Centered) Value Equation

- Personalized medicine
 - Tailored therapy
 - Biomarkers often do not exist
 - Other patient-level factors
 - What is important to patients
 - Adherence and downstream costs



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Goals of an Evidence-Based Approach

- Better Health Care
 - Health care coverage
 - Access to care
- Better Quality
 - Pay-for-performance
 - Integrated care models
 - Improve health outcomes
 - Appropriate drug therapy (evidence-based therapy)
- Improved Efficiency
 - Identify wasteful spending in the system
 - Decrease unnecessary ED and hospitalizations
 - Eliminate unnecessary or inappropriate medications



How PCOR Informs Evidence-Based Medicine

- PCOR answers relevant questions
 - Head-to-head comparisons not comparisons to placebo
 - “Hard outcomes” rather than intermediate endpoints
- PCOR provides evidence on
 - Effectiveness
 - Routine clinical practice
 - Broader patient populations



Health Technology Assessment (HTA)

– Definitions

- *Health technology assessment (HTA)* is a multidisciplinary process that summarizes information about the medical, social, economic, and ethical issues related to the use of health technology in a systematic, transparent, unbiased, robust manner
- Its aim is to inform the formulation of safe, effective, health policies that are patient focused and seek to achieve best value



Health Technology Assessment (HTA)

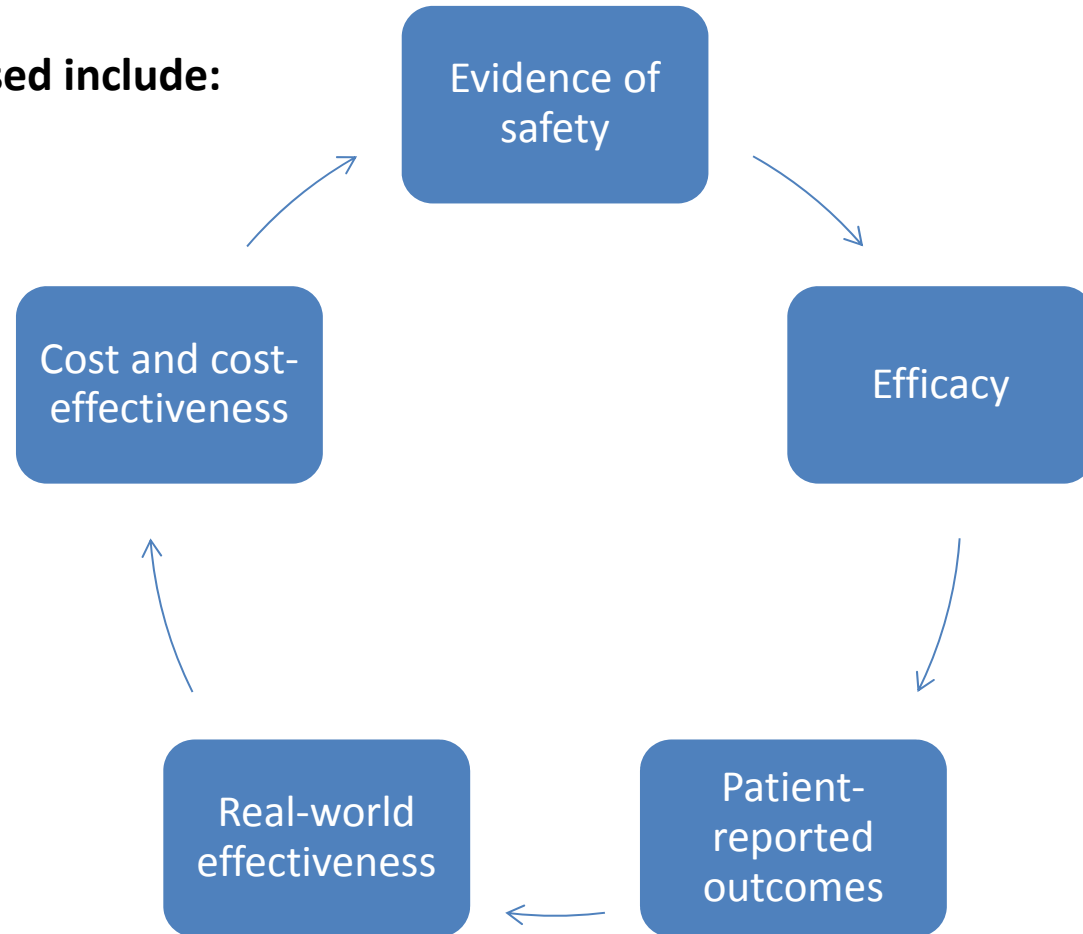
“A form of policy research that examines short- and long-term consequences of the application of a health-care technology.”

(O’Donnell et al., 2009)



Health Technology Assessment (HTA)

Properties assessed include:



Health Care Cost, Quality, and Outcomes: ISPOR Book of Terms. Lawrenceville, NJ: International Society for Pharmacoeconomics & Outcomes Research, 2003



Health Technology Assessment (HTA)

HTA also assesses the impact of the following:





Health Technology Assessment (HTA)

The scientific
methods and
data must be:

- Transparent
- Credible
- Consistently applied



Health Technology Assessment (HTA)

Quality

- The aggregate of quality ratings for individual studies, predicated on the extent to which bias was minimized.

Quantity

- Magnitude of effect, numbers of studies, and sample size or power.

Consistency

- For any given topic, the extent to which similar findings are reported using similar and different study designs.



CER/PCOR

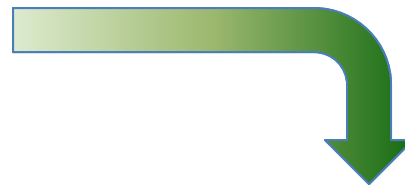
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CER/PCOR + Pharmacoeconomics

- Active comparators (i.e. not a placebo comparator)
- Broad patient population
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Potential for reduction
in wasteful spending



Summary

- Market incentives historically are misaligned
 - Potential for underuse or overuse of drugs
 - Potential for high economic barriers to appropriate use, particularly when focus is only on direct medical costs
- PCOR helps to assure that formulary and coverage decisions consider what is important to patients
 - What do patients value?
 - What are my options and what are the potential benefits and harms of those options?
- Value-based purchasing depends on
 - Value to whom
 - Cost to whom
 - Which costs are considered