

# Who We Are and What We Do: An Overview of PCORI

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**Evelyn P. Whitlock, MD, MPH**

Chief Science Officer

March 7, 2016



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

# About Us

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- An independent research institute authorized by Congress in 2010
  - Governed by a 21-member Board of Governors representing the entire healthcare community
- Funder of comparative clinical effectiveness research (CER)
  - We engage patients and other stakeholders throughout the research process
- Seek to answer real-world questions about what works best for patients
  - Ideally considering their individual circumstances and concerns



# Our Broad and Complex Mandate

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“The purpose of the Institute is to **assist patients, clinicians, purchasers, and policy-makers in making informed health decisions** by advancing the quality and relevance of evidence concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed **through research and evidence synthesis...**

**... and the dissemination of research findings** with respect to the relative health outcomes, clinical effectiveness, and appropriateness of the medical treatments, services...”

--from PCORI's authorizing legislation



# Our Mission and Strategic Goals

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PCORI helps people make [informed healthcare decisions](#), and improves healthcare delivery and outcomes, by producing and promoting [high-integrity, evidence-based information](#) that comes from research guided by patients, caregivers, and the broader healthcare community.

## Our Strategic Goals:



Increase quantity, quality, and timeliness of useful, trustworthy research information available to support health decisions



Speed the implementation and use of patient-centered outcomes research evidence

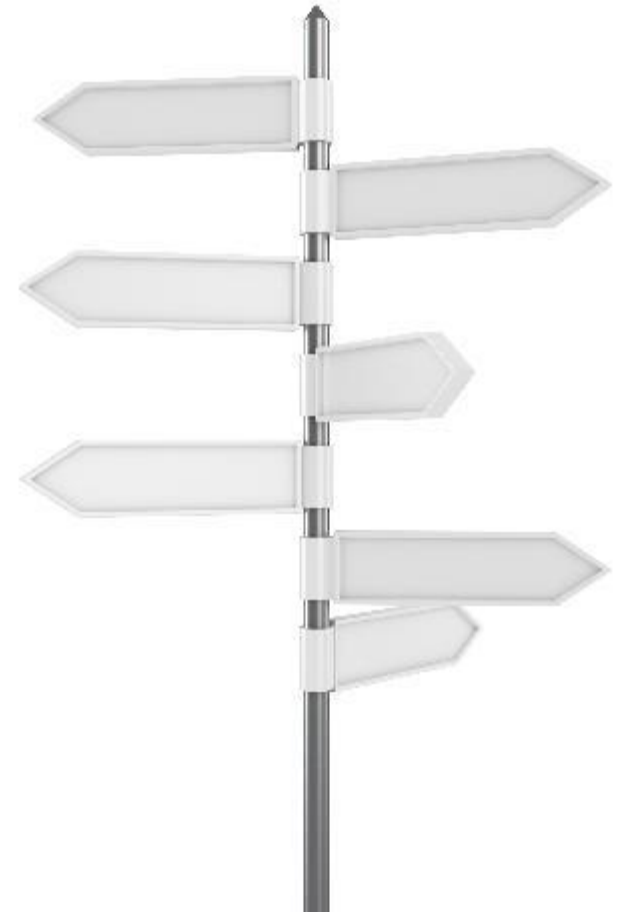


Influence research funded by others to be more patient-centered



# Why Is Our Work Needed?

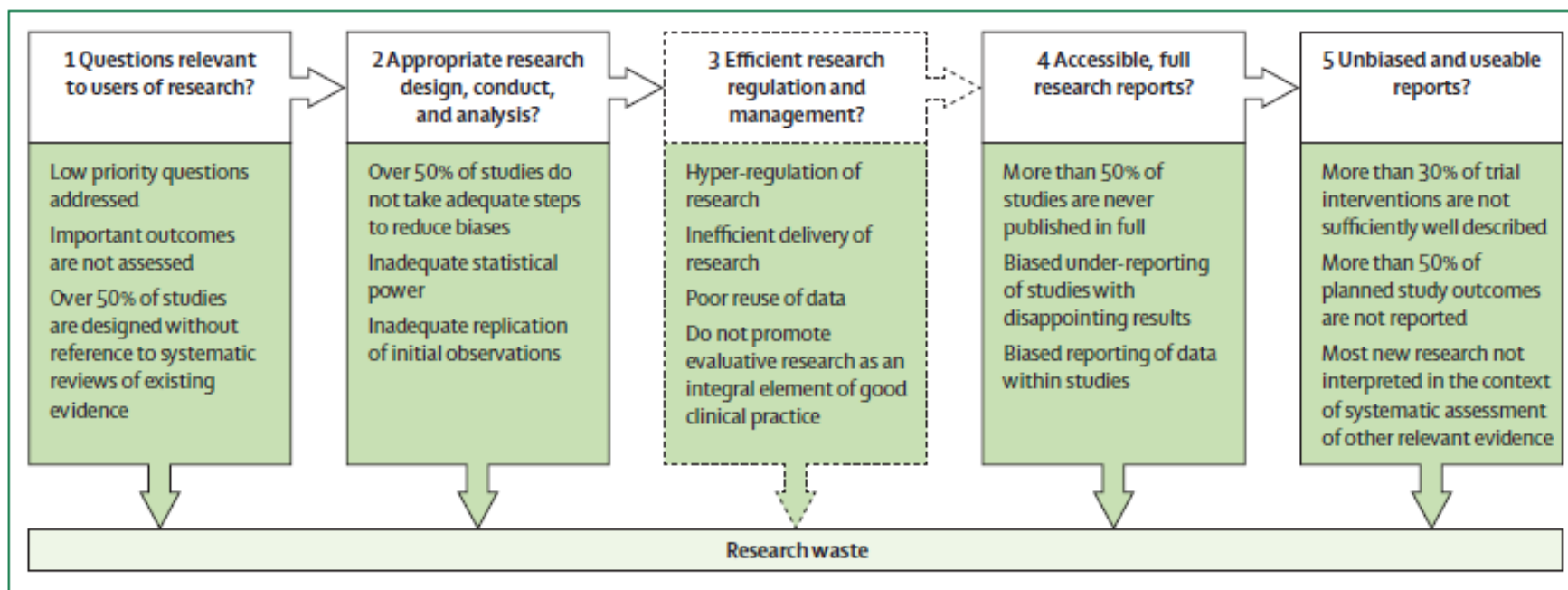
- For all the advances it produces, traditional healthcare research has not answered many common and important questions patients face as they make health care decisions.
- Most research is designed by researchers rather than those who will use the findings.
- People want to know which preventive, diagnostic, or treatment option is best for them—and what are the tradeoffs.
- Patients and their clinicians need information they can understand and use when making health care decisions.



# Increasing Value and Reducing Waste

- 2009 estimate: As much as 85% of research funding is avoidably wasted

## Stages in research production that lead to waste. Moher et al.



THE LANCET

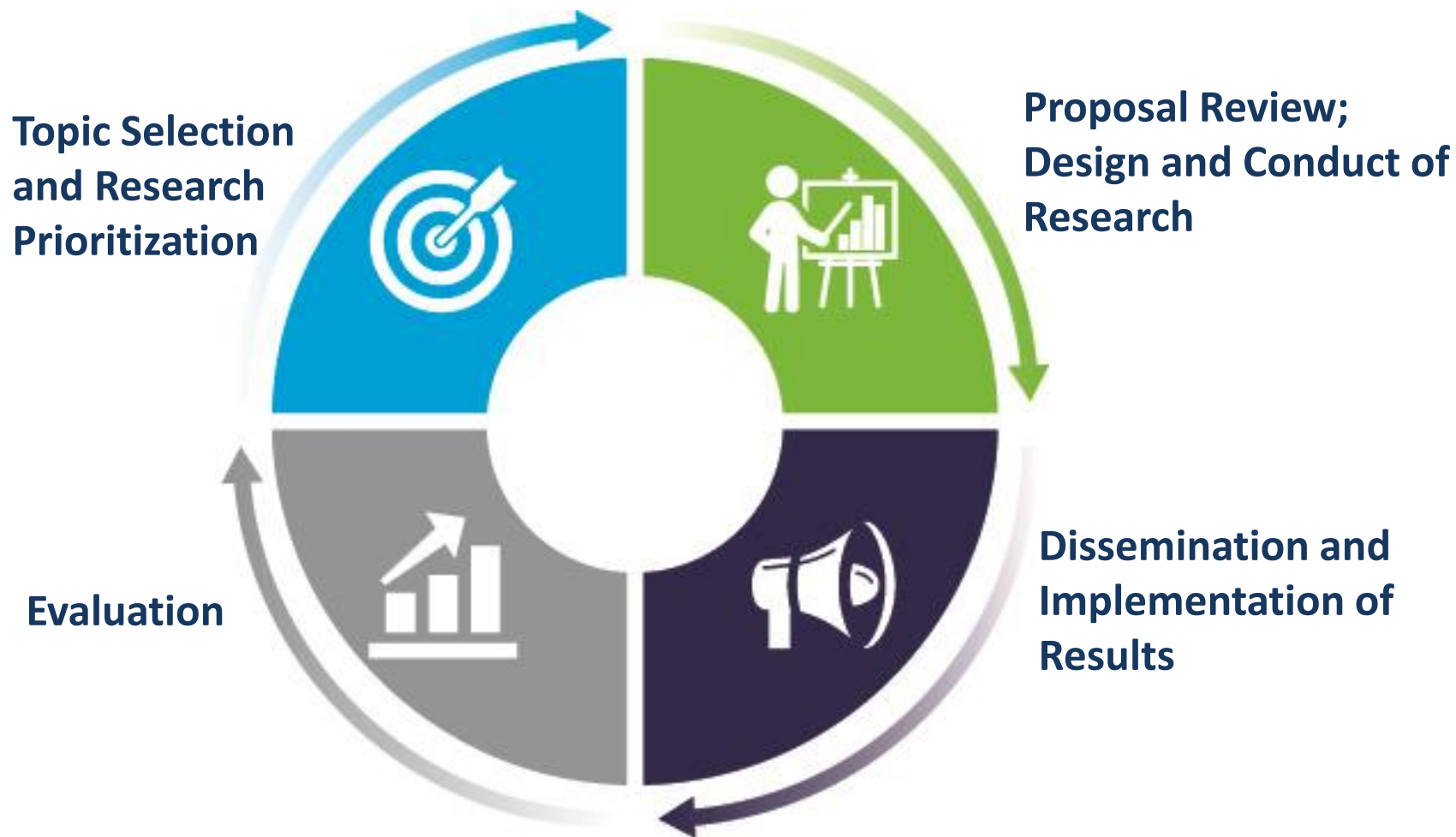
Avoidable waste in the production and reporting of research evidence. Chalmers I, Glasziou, P. *Lancet* 2009; **374**: 86-89.

Increasing value and reducing waste in biomedical research: who's listening? Moher D, et al. *Lancet* 2015; **Online**: Sept.28



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# Engagement as a Path to Useful, High-Quality Research



# Does Engagement Make a Difference?

**A systematic review\* provides the first international evidence of the impact of patient and public involvement on research on health and social-care research.**

- Literature search from 1995-2009 identified 66 studies
- Analysis showed patient and public involvement enhanced quality and appropriateness of research
- Impacts were described for all stages of research
- But authors note the evidence base on impact of engagement still needs significant enhancement



*\*Health Expectations 2014; 17(5): 637–650.*



# We Fund Research That...

## Meets these criteria:

### 1: Potential for the study to fill critical gaps and generate actionable evidence

Addresses a *clinical uncertainty or decisional dilemma* experienced by patients and other stakeholders

### 2: Potential for the study to be adopted into clinical practice and improve delivery of care

Has the potential to lead to *improvements in clinical practice and patient outcomes*

### 3: Scientific merit (research design, analysis and outcomes)

Has a research design of sufficient technical merit to ensure that the *study goals will be met*



### 4: Patient-centeredness

Focuses on improving *patient-centered outcomes* and employs a *patient-centered research design*

### 5: Patient and stakeholder engagement

Includes patients and other stakeholders as *partners throughout the entire research process*



# Our Growing Portfolio: Highest Concentration of Funded Projects

## By number of projects



## By amount awarded (in millions)



# Thank You

**Evelyn P. Whitlock, MD, MPH**

Chief Science Officer



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# **Prioritizing Comparative Effectiveness Research Questions: PCORI Stakeholder Workshops**

Background and plan – March 7, 2016

**Harold C. Sox, M.D.**

Director of Research Portfolio Development



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# Background on PCORI



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# PCORI's Mission Defined

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## “(c) PURPOSE

—The purpose of the Institute is to assist patients, clinicians, purchasers, and policy-makers in making informed health decisions by advancing the quality and relevance of evidence...”

Patient Protection and Affordable Care Act (PPACA): Subtitle D of Title VI - Sec. 6301. (2010)



# Comparative Effectiveness Research

- Representative study populations and clinicians
- Head-to-head comparisons of *specific* interventions
- Outcomes that matter to patients.
- Individualized decision-making: matching the intervention to the patient



# Development of Research Topics at PCORI

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“(d) DUTIES

— “(1) IDENTIFYING RESEARCH PRIORITIES AND ESTABLISHING RESEARCH PROJECT AGENDA

— “(A) IDENTIFYING RESEARCH PRIORITIES.—The Institute shall identify national priorities for research, taking into account factors of disease incidence, prevalence, and burden in the United States (with emphasis on chronic conditions), gaps in evidence in terms of clinical outcomes, practice.....”

“(B) ESTABLISHING RESEARCH PROJECT AGENDA

—The Institute shall establish and update a research project agenda for research to address the priorities identified under subparagraph (A).....

PPACA: Section 6301 (2010)



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PPACA: Section 6301 (2010)

**PCORI’s interpretation of the law:**

**PCORI should develop a list of research questions**



# Funding Streams at PCORI

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- **“Broad” Funding Announcement:**
  - Topics chosen by the investigator
- **Pragmatic Clinical Studies Funding Announcement:**
  - Topics chosen by PCORI and its stakeholders
- **Targeted Funding Announcement:**
  - Topics chosen by PCORI and its stakeholders



# Funding Streams at PCORI

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- **“Broad” Funding Announcement**
  - Investigator-initiated; up to \$2M and 3 years
  - Based on the 5 broad national priorities
- **Pragmatic Clinical Studies Funding Announcement:**
  - Lists ~25 PCORI High Priority Topics. Choose one or propose a topic; up to \$10M over 3-5 years.
  - 3 cycles per year; observational or randomized;
- **Targeted Funding Announcement:**
  - Lists one topic chosen by PCORI; may have multiple research questions; funding varies
    - (HCV: up to \$50M; four research questions).



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# Development of Research Topics at PCORI



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# Research Questions

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- In most funding agencies, the investigator chooses the research question.
  - Investigator-initiated research
- PCORI chooses the topics for its funding streams with the largest awards (Targeted and Pragmatic Clinical Studies)
  - Sponsor-initiated research
- The process of developing research questions is therefore a critical activity at PCORI.



# Stakeholder-informed Topic Development

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Nominations from stakeholders



Priority-setting by multi-stakeholder Advisory Panels



Research question refinement by multi-stakeholder panels

Oversight at each step by a multi-stakeholder Board of Governors committee



# Pathway to a Funding Announcement



# Priority Setting Criteria



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# Priority Setting Criteria

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- Patient-centeredness
- Burden of illness
- Evidence gaps
- What do guidelines say?
- Ongoing studies
- Likelihood of implementation in practice
- Likely durability of research results
- Proposed research questions



# Patient-Centeredness: The parameters of the study should matter to patients

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- **Populations:**
  - Patients and clinicians who are representative of daily practice
- **Interventions:** difficult choices that occur in day-to-day care
  - This means treatments that are in daily use, not novel, untested treatments
  - Two active, well-defined interventions that patients must decide between in real-life.
- **Comparator:**
  - Also a well-defined intervention in common use.
  - Must justify “usual care” as a comparator and measure the care each patient receives.
- **Outcomes:** patient-reported outcomes
  - Day-to-day function, disease-specific, mortality



# Priority Setting Criteria

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# Burden of Illness

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- Prevalence
- Mortality
- Disability
- Cost to society



# Priority Setting Criteria

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# Evidence Gap

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- Good evidence is lacking about information that is needed to make a fully informed decision.



# The Search for Evidence Gaps

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- How to find something that is important because it isn't there?
- Start with a systematic review
  - Its purpose is to summarize all of the evidence, so if something is missing, it should be evident.
  - To find an evidence gap, you need to be sure that the systematic review has investigated all of the information.



# Other Hints About Evidence Gaps

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- Systematic reviews
  - “The evidence is weak or of low quality.”
  - “The studies are few in number.”
  - “The studies are all small.”
  - “Among the studies, there is no consistent pattern to the results.”



# Priority Setting Criteria

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# Reasons for Studying Practice Guidelines

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- As a framework for the proposed research How does the research fit into the care of the patient?
- To identify evidence gaps:
  - A: strong evidence that benefits exceed harms
  - B: lesser but still solid evidence that benefits exceed harms
  - C: a toss-up
  - D: good evidence suggests that harms outweigh benefits.
  - I: The evidence is insufficient to make a recommendation



# Priority Setting Criteria

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# Ongoing Studies

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- PCORI does not want to fund research that someone is already funding.
- Staff review [clinicaltrials.gov](https://clinicaltrials.gov) for:
  - A heterogeneous collection of studies
  - Small studies
  - Limited outcome measures
  - Not head-to-head comparisons of active treatments



# Priority Setting Criteria

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- Patient-centeredness
- Burden of illness
- Evidence gaps
- What do guidelines say?
- Ongoing studies
- **Likelihood of implementation in practice**
- Likely durability of research results
- Proposed research questions



# Likelihood of Implementation in Practice

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- Are clinicians desperate for better information about which alternatives are effective?
- Will results from the study help patients and their families make choices about their health care?
- Are there practice guidelines with “insufficient evidence?”  
Could this study fill the evidence gap?
- Do the study aims align with stakeholders’ priorities?
- Do key professional organizations endorse the study goals?



# Priority Setting Criteria

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- Patient-centeredness
- Burden of illness
- Evidence gaps
- What do guidelines say?
- Ongoing studies
- Likelihood of implementation in practice
- Likely durability of research results
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# Durability of Research Findings

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- Will the results of the study be relevant when the study ends?
  - Is the field fast-moving? Cancer therapy, precision medicine, treatment of HCV hepatitis.
  - Is the field slow moving? Chronic pain, low back pain.



# How to Describe a Research Question: PICOTS

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- Population
- Intervention
- Comparator
- Outcomes
- Time of observation
- Setting



# The Plan for Today



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# The Plan for Today

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- **Step 1: Discuss the questions submitted by the group**
  - Narrow the field of questions to a manageable number for in-depth discussion (5 to 8).
  - In-depth discussion
    - PICOTS
    - PCORI criteria
    - Topic brief
- **Step 2: Refine the top 2-3 research questions**
  - PICOTS
  - PCORI criteria

*More details in the individual work groups.*



# Additional Discussion Points

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- Should the study be a randomized trial or an observational study?
- Is the research question answerable by a trial?
  - Is it feasible to assemble a study cohort typical of patients facing a decision between the interventions specified by the research question?
  - Will many patients be willing to be randomized?
- Treatment response heterogeneity: Is there reason to expect that a subgroup of patient might respond differently than the average patient to the intervention(s).



# What Happens Next?

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Prioritized questions and deliberations from workshop will be shared with PCORI staff leadership and PCORI Science Oversight Committee.

PCORI Board of Governors will decide whether to issue a funding announcement by May 2016.



# Thank You

**Hal C. Sox, M.D.**

Director of Research Portfolio Development



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