

# About PCORI

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PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

# About PCORI

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- An independent research institute authorized by Congress in 2010. Governed by a 21-member Board representing the entire healthcare community.
- Funds comparative clinical effectiveness research (CER) that engages patients and other stakeholders throughout the research process.
- Seeks answers to real-world questions about what works best for patients based on their 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

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PCORI helps people make informed health care decisions, and improves health care delivery and outcomes, by producing and promoting high integrity, evidence-based information that comes from **research guided by patients, caregivers and the broader health care community.**



# Our Strategic Goals

**Increase Quantity, Quality, Usefulness, and Timeliness of Research Information**



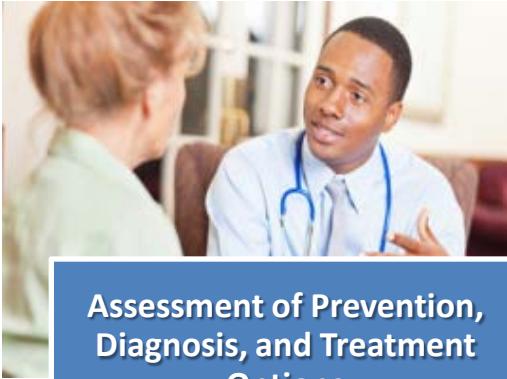
**Speed the Implementation and Use of Evidence**

**Use of**

**Influence Research Funded by Others**



# The Research We Fund is Guided by Our National Priorities for Research



Assessment of Prevention,  
Diagnosis, and Treatment  
Options



Improving  
Healthcare Systems



Communication &  
Dissemination  
Research



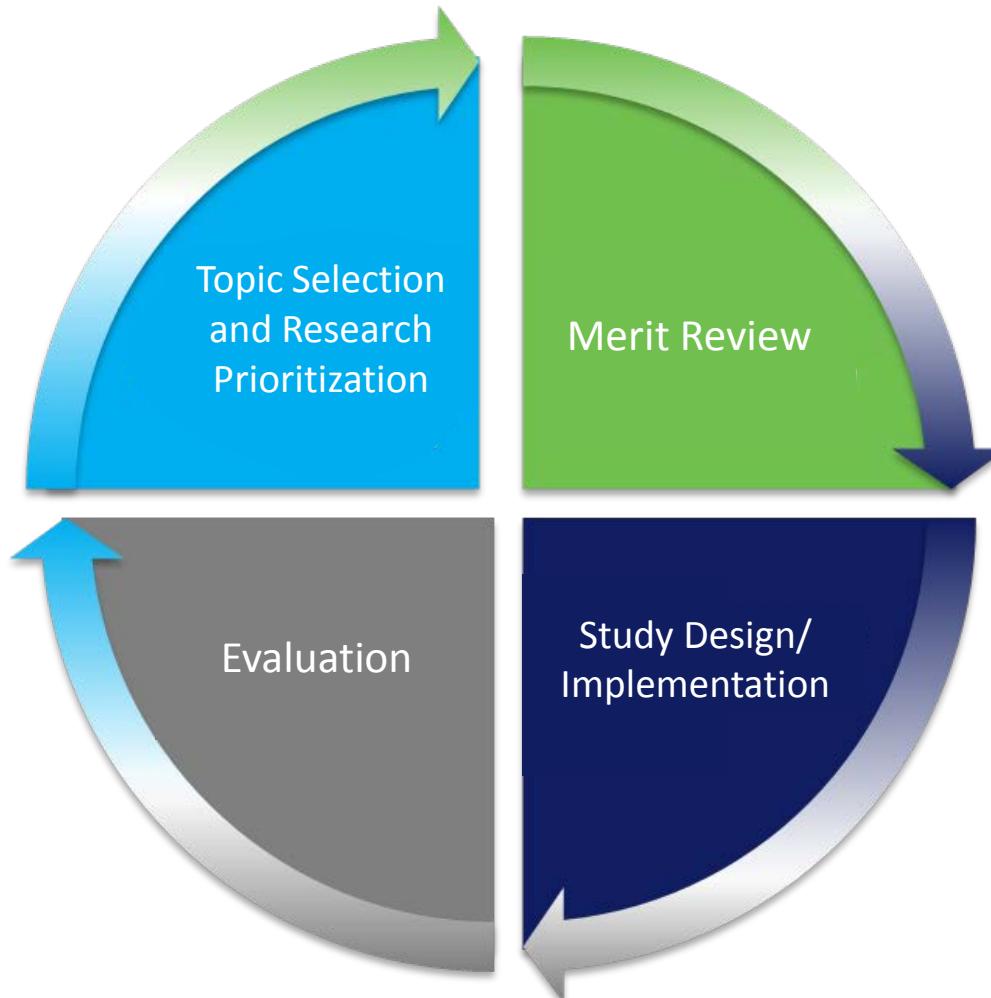
Addressing  
Disparities



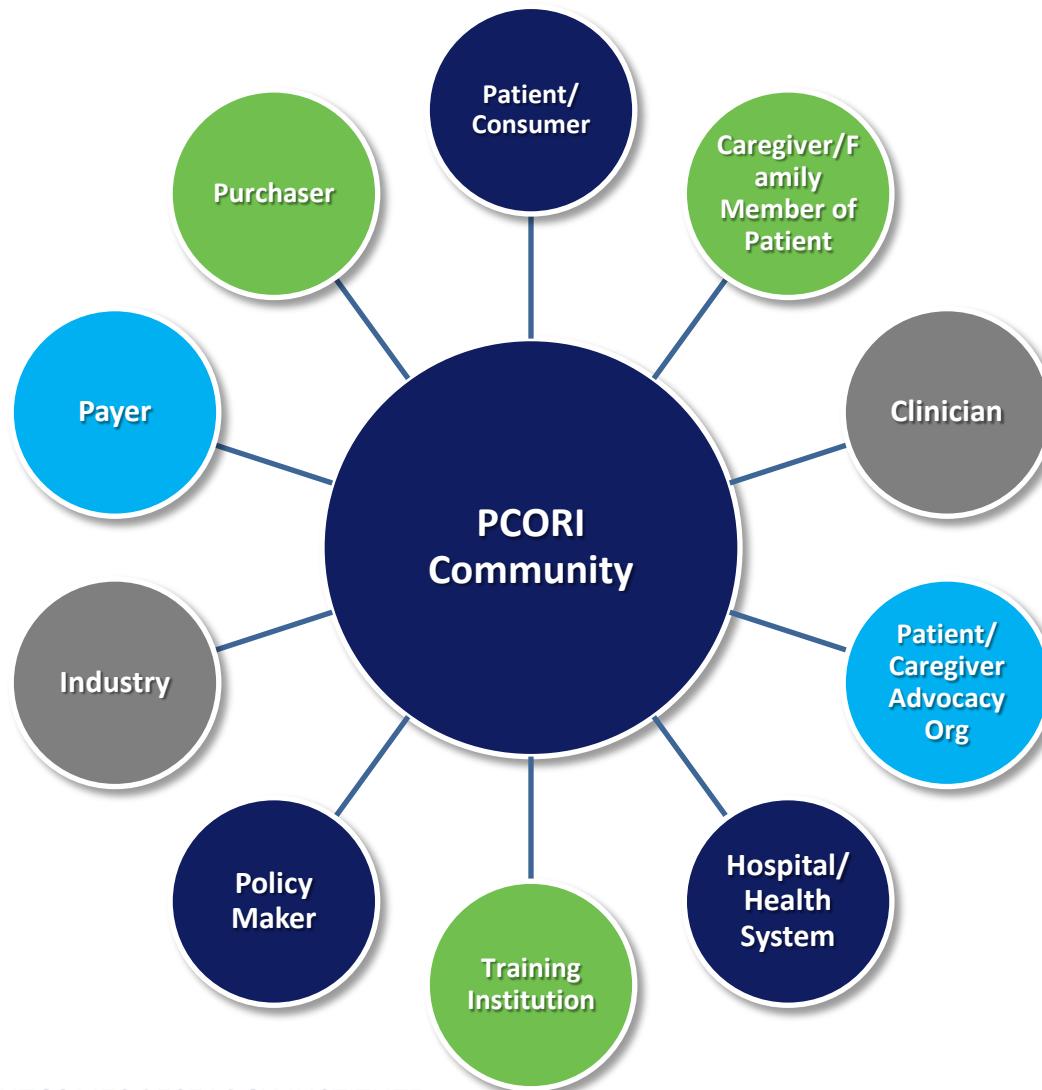
Accelerating PCOR  
and Methodological  
Research



# We Engage Patients and Other Stakeholders at Every Step

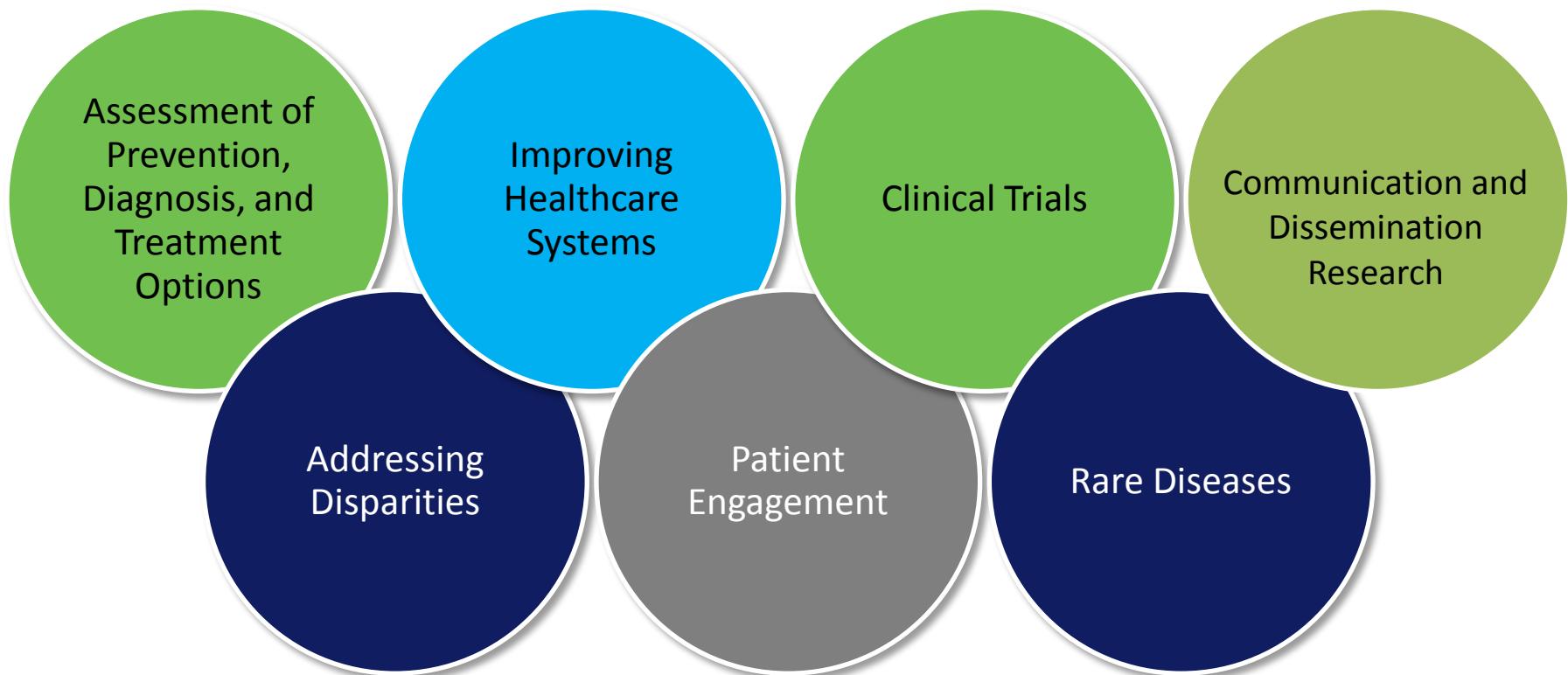


# Who Are Our Stakeholders?



# Our Advisory Panels

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# Learn More

[www.pcori.org](http://www.pcori.org)

[info@pcori.org](mailto:info@pcori.org)



# Prioritizing Comparative Effectiveness Research Questions: PCORI Stakeholder Workshops

Background and plan – June 9, 2015

**Harold C. Sox, MD**  
Director of Research Portfolio Development



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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

## “(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)



# Development of Research Topics at PCORI

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## “(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)

**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

- 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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- 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



# PCORI Topic Briefs Address these Priority-Setting Criteria



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# 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



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# Burden of Illness

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- Prevalence
- Mortality
- Disability
- Cost to society



# Priority-Setting Criteria

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- Patient-centeredness
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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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- Patient-centeredness
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- **Ongoing studies**
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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
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- What do guidelines say?
- Ongoing studies
- Likelihood of implementation in practice
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# Likelihood of Implementation in Practice

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- Are clinicians desperate for better information about which alternatives are effective?
- 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
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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: Rank the questions in order of priority**
  - Vote → a few research questions
- **Step 3: Refine the top 2-3 research questions**
  - PICOTS
  - PCORI criteria

*More details in the individual work groups.*



# What Happens Next?

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Prioritized questions and deliberations from workshop will be shared with PCORI leadership.

Determination regarding funding announcements on specified topics made by PCORI Board of Governors by August 2015.



# Thank You

**Hal C. Sox, MD**

Director of Research Portfolio Development



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