



PCORI's Hepatitis C Workshop

*Arlington, Virginia
October 17, 2014*

Patient-Centered Outcomes Research Institute



Welcome and Purpose of Workshop

Joe V. Selby, MD, MPH, Executive Director, PCORI

Patient-Centered Outcomes Research Institute

PCORI's Mandate

“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 *Patient Protection and Affordable Care Act*



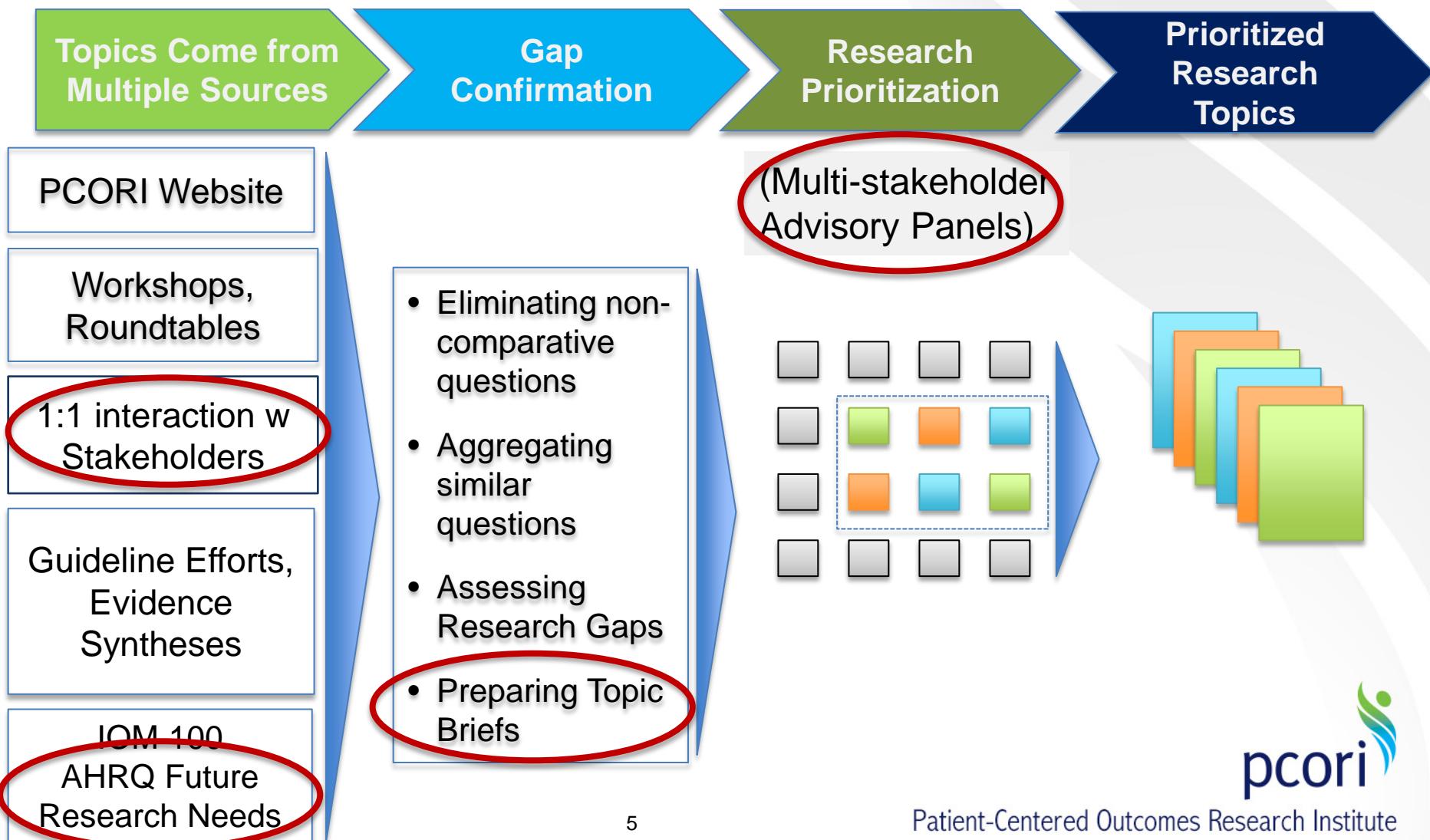
Identifying Research Priorities

“(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 variations and health disparities in terms of delivery and outcomes of care,.....”

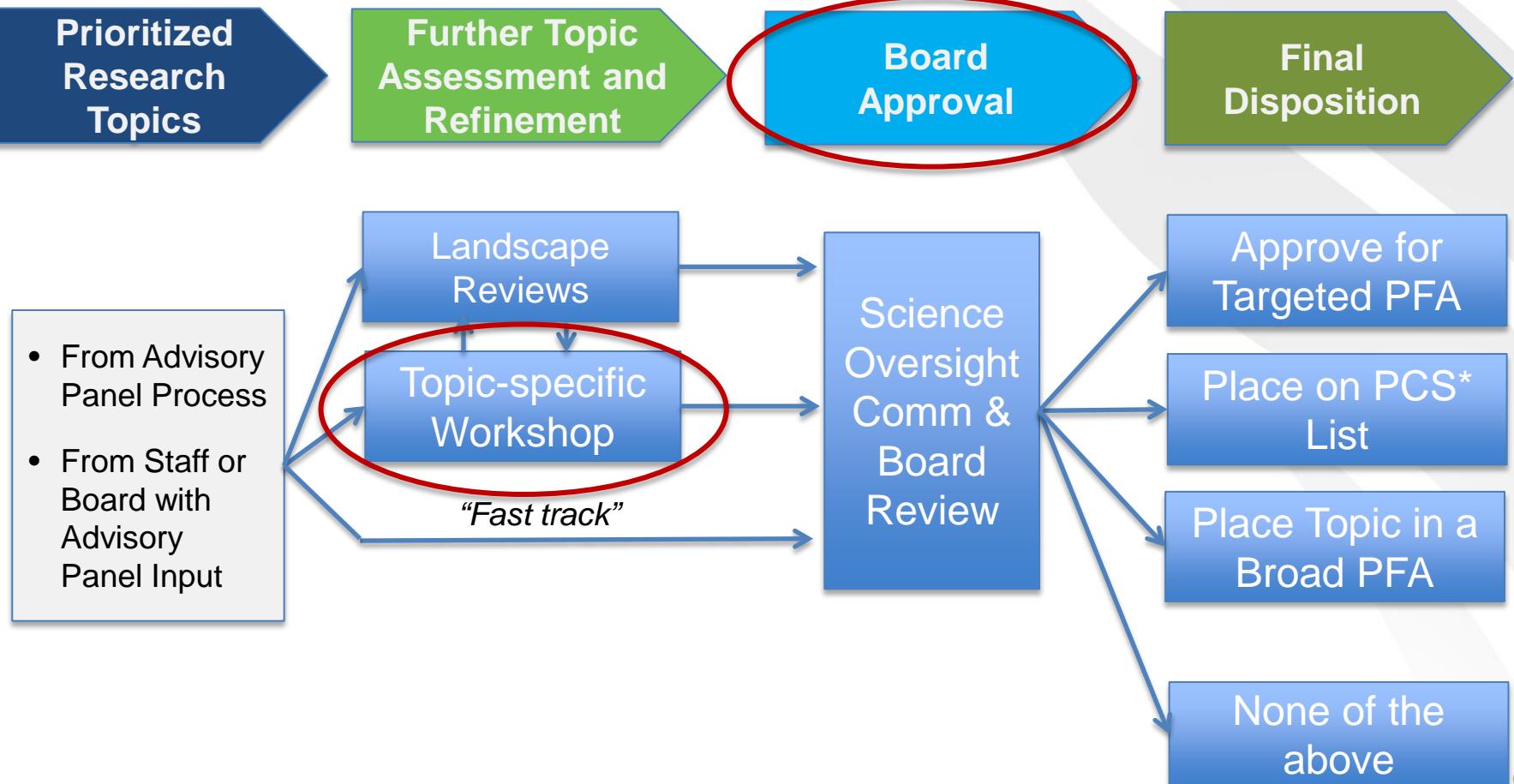
-- from *Patient Protection and Affordable Care Act*



PCORI's Research Prioritization Process



Post-Prioritization Process: Board Review and Final Disposition of Topic



*Pragmatic Clinical Study

Previous PCORI Workshops

- Treatment options for uterine fibroids
- Approaches to improving outcomes for African-American and Hispanic/Latino patients with severe asthma
- Prevention of injurious falls in high-risk elderly patients
- Treatment options for obesity in primary care
- Approaches to improving control for hypertension in African-American and rural patients
- Treatment options for patients with chronic low back pain
- Transitions in care

PCORI Relies on Engagement in Setting its Research Agenda, Conducting Research, and Disseminating Findings



What Is Comparative Clinical Effectiveness Research?

- Compares two or more potentially available options for prevention, diagnosis, or treatment (can include “usual care”)
- Considers range of clinical outcomes relevant to patients
- Does not consider cost comparisons or cost-effectiveness analysis
- Conducted in real-world populations and settings
- Attends to differences in effectiveness and preferences across patient subgroups
- Often requires randomized trial design

What Is Patient-Centered Research?

Compares options for prevention, diagnosis, or treatment that matter to patients; and that represent realistic choices faced by patients or other decision makers

Includes the range of clinical outcomes that are of interest to patients and their caregivers, including health, health-related quality of life, function, symptoms, safety from medical harm, survival, and satisfaction with care

Questions for this Workshop

Are there patient-centered comparative clinical effectiveness research questions that PCORI should pursue?

If so, how would this multi-stakeholder workshop prioritize these questions in terms of importance to patients?

Questions Submitted by Attendees – Four Large Buckets

- Care delivery
- Screening and diagnostic tests
- Head-to-head comparisons of new therapies
- Patient subpopulations, timing of treatment

Final Reminders

- Webinar/teleconference and archiving this workshop
- This workshop is advisory!
- PCORI's interest in collaborative funding of research



Introductions and Agenda

Bryan Luce, PhD, MBA, Chief Science Officer, PCORI

David Hickam, MD, MPH, Program Director, Clinical Effectiveness Research, PCORI

Patient-Centered Outcomes Research Institute

Agenda

Time	Agenda Item	Speaker(s)
9:00 – 10:00 AM	Introductions and agenda	Bryan Luce David Hickam
10:00 – 10:15 AM	Instructions for breakout sessions	Hal Sox
10:15 – 10:30 AM	Break	N/A
10:30 – 12:30 PM	Breakout sessions – Discussion and ranking of CER questions	N/A
12:30 – 1:00 PM	Lunch	N/A
1:00 – 2:00 PM	Breakout sessions – Discussion and ranking of CER questions (cont.)	N/A
2:00 – 2:30 PM	Break	N/A
2:30 – 4:00 PM	Plenary session: Review and discussion of prioritized CER questions	Eric Bass
4:00 – 4:30 PM	Ranking of CER questions	N/A
4:30 – 5:00 PM	Closing remarks	Joe Selby

Question for this Working Group

Are there patient-centered comparative clinical effectiveness research questions in the area of hepatitis C screening, diagnosis, and treatment that this group advises PCORI to support?

A Brief History of Hepatitis C

- 1970s and 1980s: rising incidence of “non-A non-B” hepatitis and idiopathic chronic hepatitis
- 1989: confirmation of the hepatitis C virus
- 1990s: clinical trials of interferon/ribavirin and recognition of differences in viral genotypes
- 2002: FDA approves ribavirin and pegylated interferon alpha 2a

Changing Landscape of Antiviral Therapy

- 2011: FDA approves telaprevir and boceprevir for use in “triple therapy”
 - Increased rates of SVR from 50% to 70%
- 2013: FDA approves simeprevir and sofosbuvir
 - Both are used in triple therapy for genotype 1
 - Sofosbuvir/ribavirin double therapy for genotypes 2 and 3
- October 2014: FDA approves combination of sofosbuvir and ledipasvir for genotype 1

Clinical Impact of Hepatitis C

Estimated prevalence

- US: 3 million
- Worldwide: 150 million

Primary mode of transmission is via blood

- Locus in IV drug users

Introduction of broad-based screening

- Uncertainty about follow-up and timing of treatment



Variable Clinical Course of Hepatitis C

- Acute infection can resolve without treatment
- About 80% of those exposed develop chronic infection
- Asymptomatic in the majority
- About 1/3 develop chronic liver disease if untreated
- Accounts for about 1/4 of all cases of cirrhosis and liver cancer
- No evidence about the effect of antiviral treatment on long-term outcomes



Instructions for Breakout Sessions

Harold Sox, MD, Senior Adviser, PCORI

Patient-Centered Outcomes Research Institute

Breakout Sessions

Care delivery

- Gillian D. Sanders, PhD

Screening and diagnostic tests

- John Wong, MD

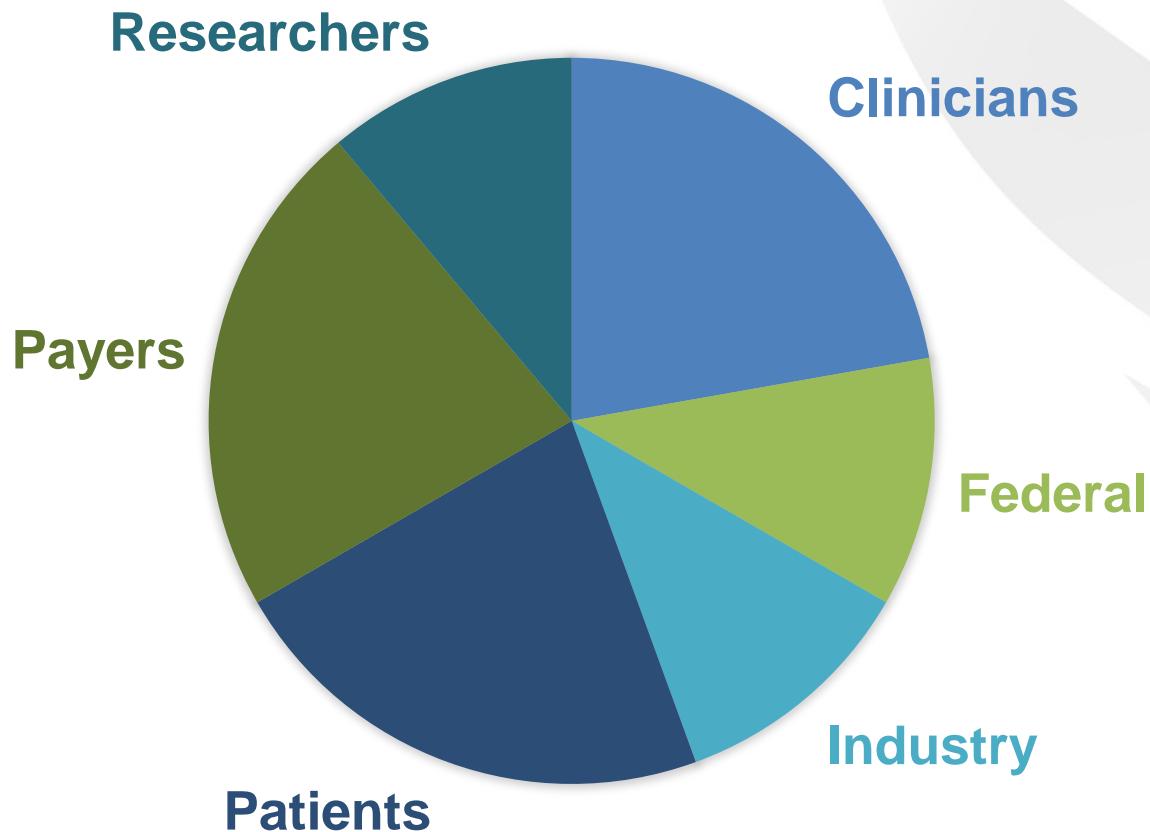
Head-to-head trials

- Camilla Graham, MD

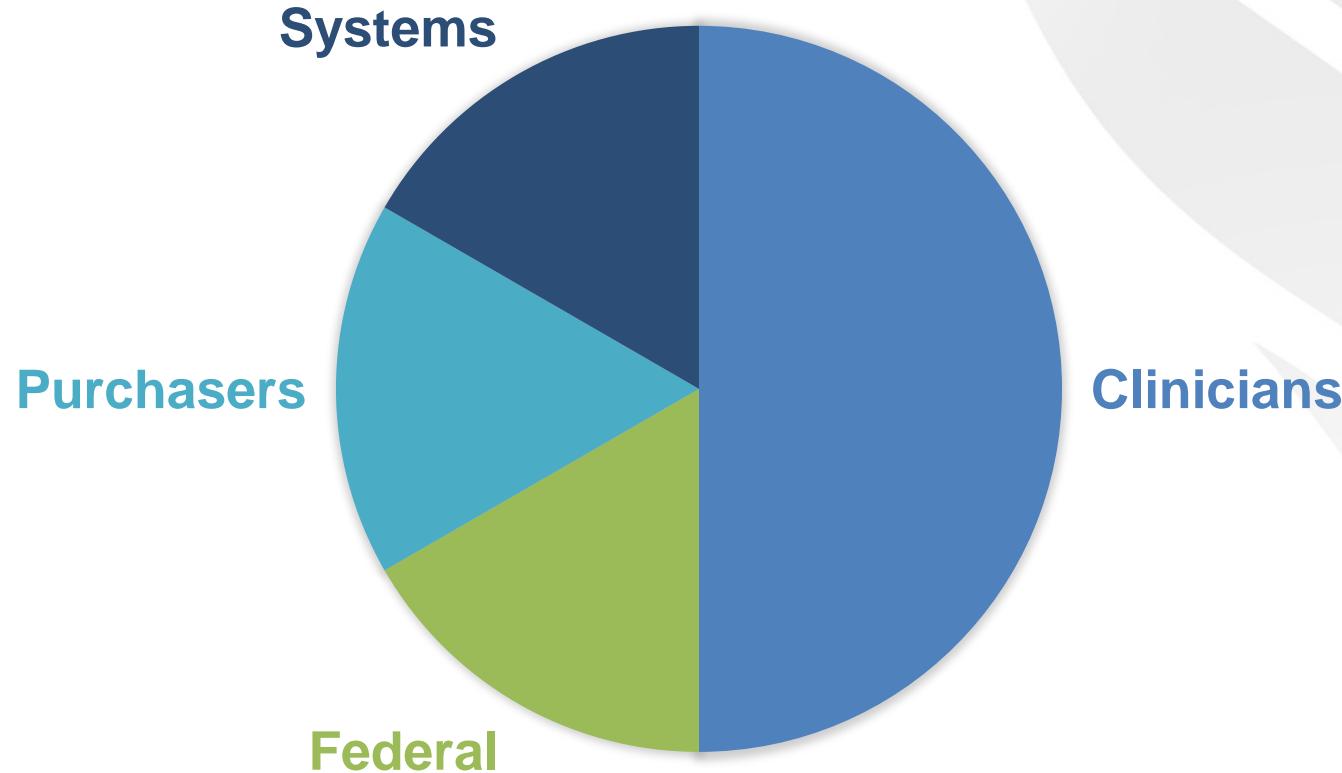
Patient populations and timing of treatment

- Martha Gerrity, MD, MPH, PhD

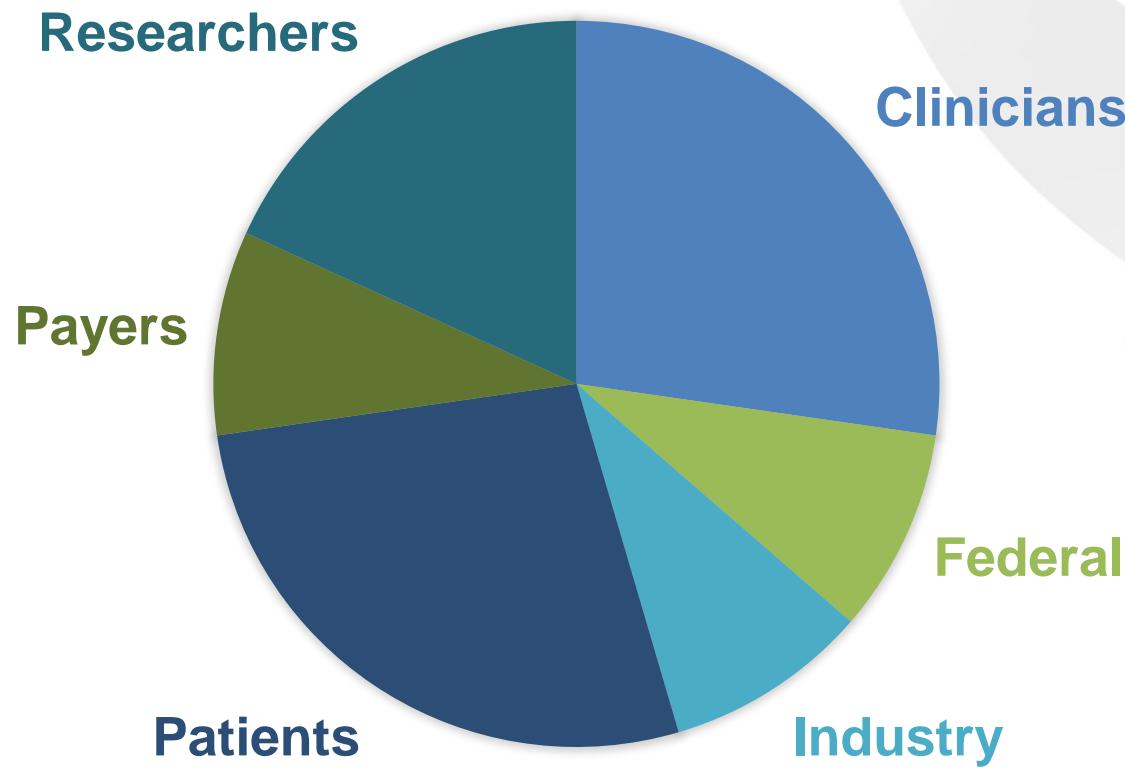
Care Delivery Breakout – Stakeholder Groups



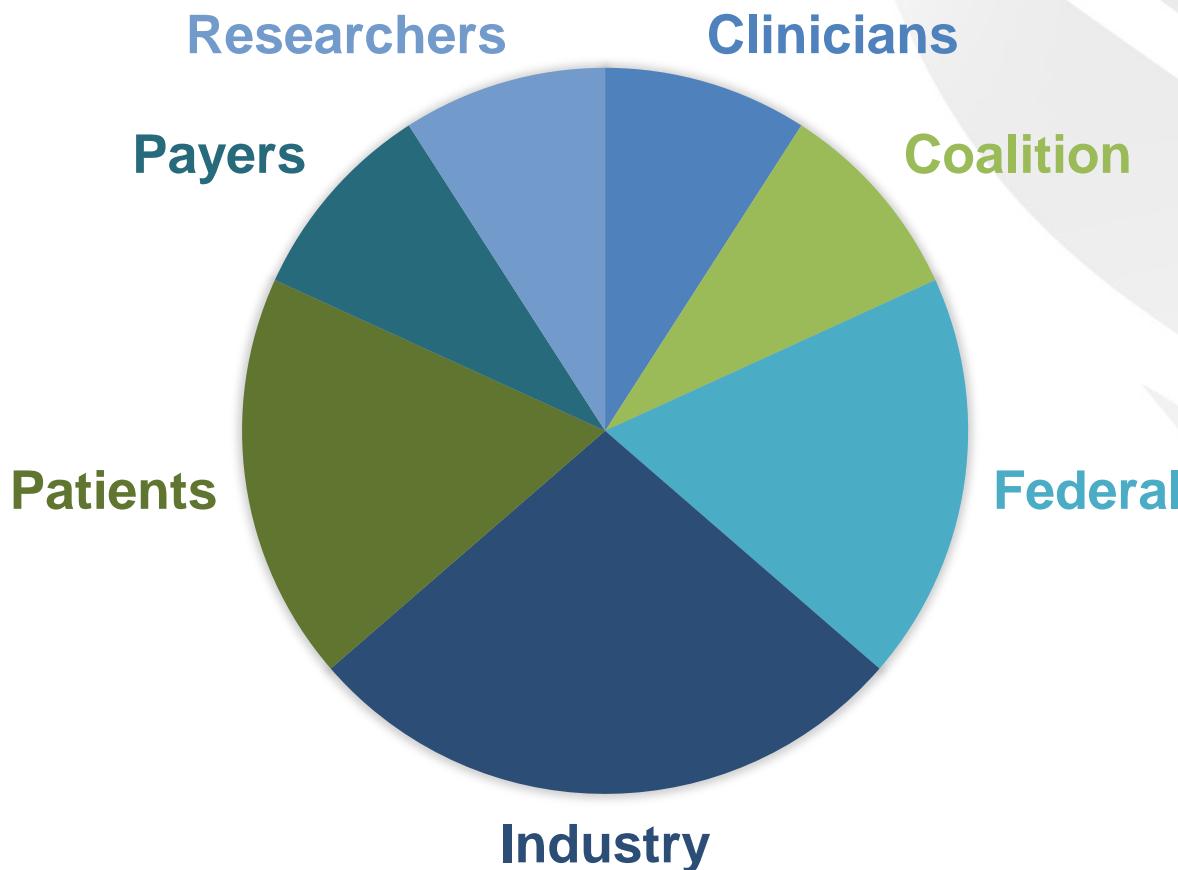
Screening and Diagnosis Tests Breakout – Stakeholder Groups



Head-to-Head Trials – Stakeholder Groups



Patient Populations and Timing of Treatment – Stakeholder Groups



Roles

- Leader
- PCORI staff moderator
- Flip-chart/slide note taker
- Note taker

Time

- 3 hours total
- About 30 min/CER question = 2 hours
- 30 min for ranking: Pareto principle
 - 4 self-sticking dots/person
 - Flip-charts

Key CER Question Elements to Be Discussed

- Target condition
- Target population
- Compared interventions
- Proposed outcome measures (including patient-centered outcomes)
- Study design
- Feasibility of doing the study as outlined. Potential problems.
- Possible results and how they might alter practice or policy.
- Feasibility of scaling up the intervention to national-level adoption





Break

10:15 – 10:30 AM

Patient-Centered Outcomes Research Institute