



2013 PIPC Annual Forum

Changing the Culture of Research: From CER Prioritization to Dissemination

*November 5, 2013
12:00 pm – 2:00 pm*

*Reserve Officers Association
Washington D.C.*

Panelists

- **Honorable Tony Coelho**, Chairman, PIPC (Moderator)
- **Dr. Bryan Luce**, Chief Science Officer, PCORI
- **Dr. Hal Sox**, Senior Advisor to PCORI
- **Mr. Marc Boutin**, Executive Vice President and COO, National Health Council
- **Dr. Darius Tandon**, Co-Chair, PCORI Advisory Panel on Patient Engagement
- **Dr. Tanisha Carino**, Executive Vice President, Avalere



The Honorable Tony Coelho

Chairman, PIPC

We all need good information to make good decisions about our health, and our health care. Comparative clinical effectiveness research can help us make good health care choices. But it is important to make sure this research isn't misused to make these decisions for us. The Partnership to Improve Patient Care exists to ensure that comparative clinical effectiveness research is focused on giving providers and patients the information they need, improving health care quality and supporting continued medical progress.



Hal Sox, MD

Senior Advisor to PCORI

“PCORI should plan its research agenda strategically, so that it addresses research questions that comparative effectiveness research could answer quickly and decisively.”

- Dr. Hal Sox, *Health Affairs*, 2012

Targeted research: priority-setting at PCORI

Harold C. Sox
Professor of Medicine
Geisel School of Medicine
Senior Advisor, PCORI

Conflicts of Interest

Dr. Sox is a part-time paid consultant to PCORI. He is not representing PCORI at this Forum.

No other conflicts.

Two approaches to funding research

- Investigator-initiated research
- Sponsor-initiated research (targeted funding)

Investigator-initiated research

- Agency announces a funding opportunity.
- Researchers who are expert in the field propose research that is based on their scientific interests.
- Agency peer reviewers rank the topics in order of scientific interest.
- The highest ranked proposals get funded.
- Who sets research priorities?
 - The investigators
 - The peer reviewers

Research programs at PCORI

- **Investigator-initiated projects**
 - Comparative Effectiveness Research
 - Improving Healthcare Systems
 - Communication and Dissemination Research
 - Addressing Disparities
 - Accelerating Patient-Centered Outcomes Research and Methodological Research
- Targeted Research

PCORI Clinical Effectiveness Program slate of funded projects

Cycle/Submission date	Cycle I	Cycle II	Cycle III	Aug 2013
Approx. award date	January 2013	July 2013	September 2013	January 2014
Maximum budget/yr (direct costs)	\$500K	\$500K	\$500K	\$500K
Max project period	3 years	3 years	3 years	3 years
Funds available	\$32M	\$34M	\$48M	\$32M
# review criteria	8	8	8	5
# awards made	9	22	24	TBD
Total funding	\$53.1M		\$45.7M	TBD

Courtesy of PCORI

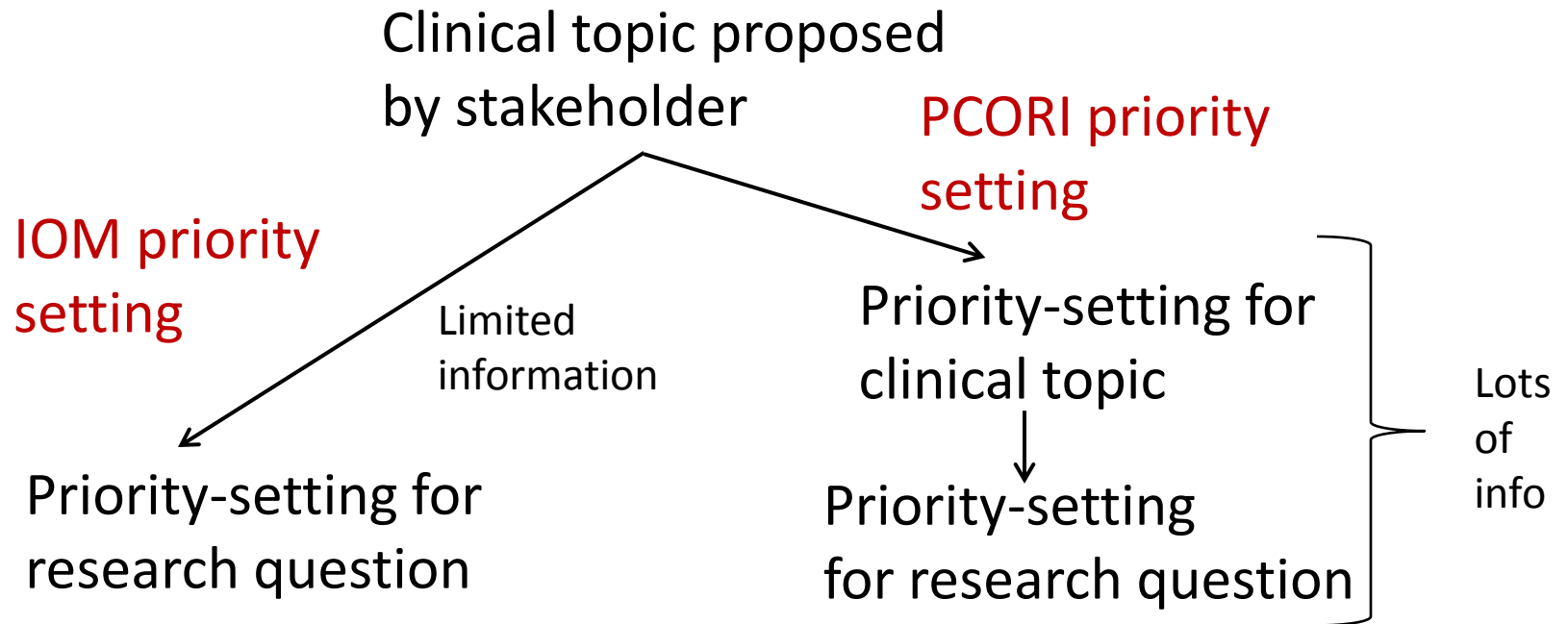
Research programs at PCORI

- **Investigator-initiated projects**
 - Assessment of Prevention, Diagnosis, and Treatment Options
 - Improving Healthcare Systems
 - Communication and Dissemination Research
 - Addressing Disparities
 - Accelerating Patient-Centered Outcomes Research and Methodological Research
- **Targeted Research**

Sponsor-initiated research

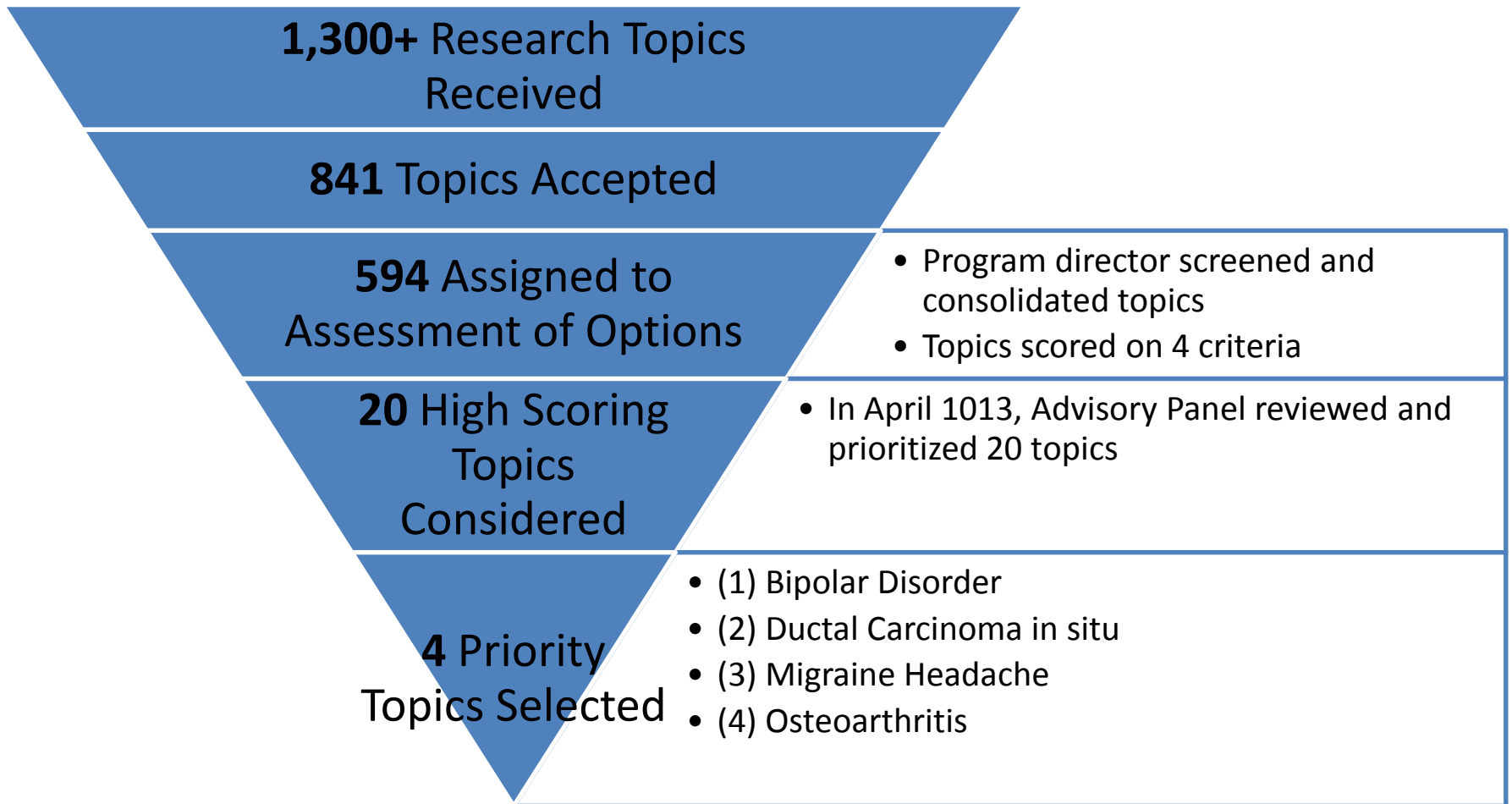
- The sponsor sets priorities for research questions to address.
 - PCORI Advisory Panels set priorities.
- The sponsor develops a research project agenda—>issues a RFA.
 - A package of research projects within a budget.
 - Expert advice on study design → RFA specifies the project design
 - Peer review → award a contract to perform the research.

Priority-setting: IOM and PCORI



Research question = clinical topic + intervention + outcome measure

PCORI: Topic Prioritization for CER



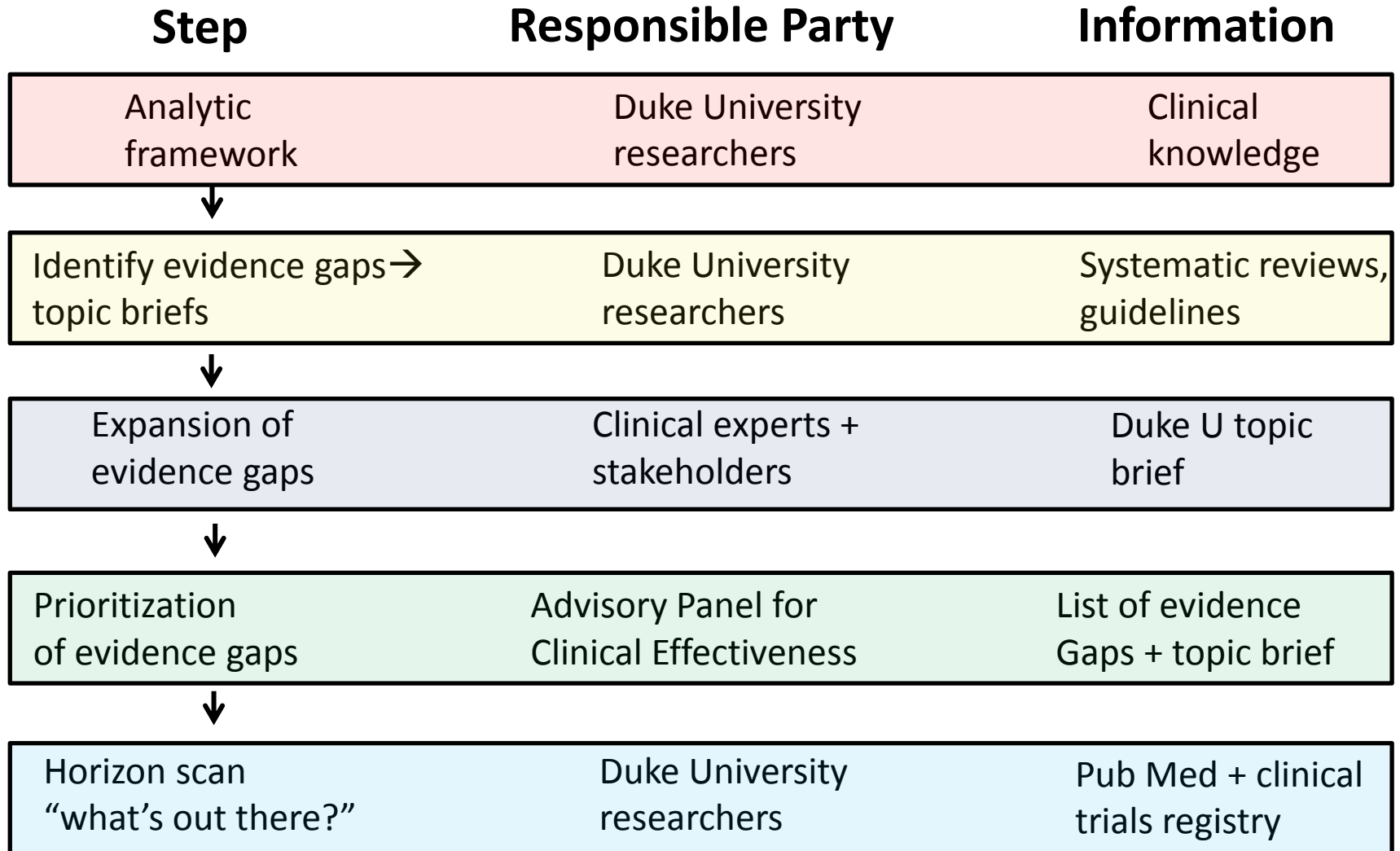
4 criteria used to score topics

- **1) Patient-centeredness**
- **2) Burden.**
- **3) Potential for improving healthcare practice.**
- **4) Timeliness.**

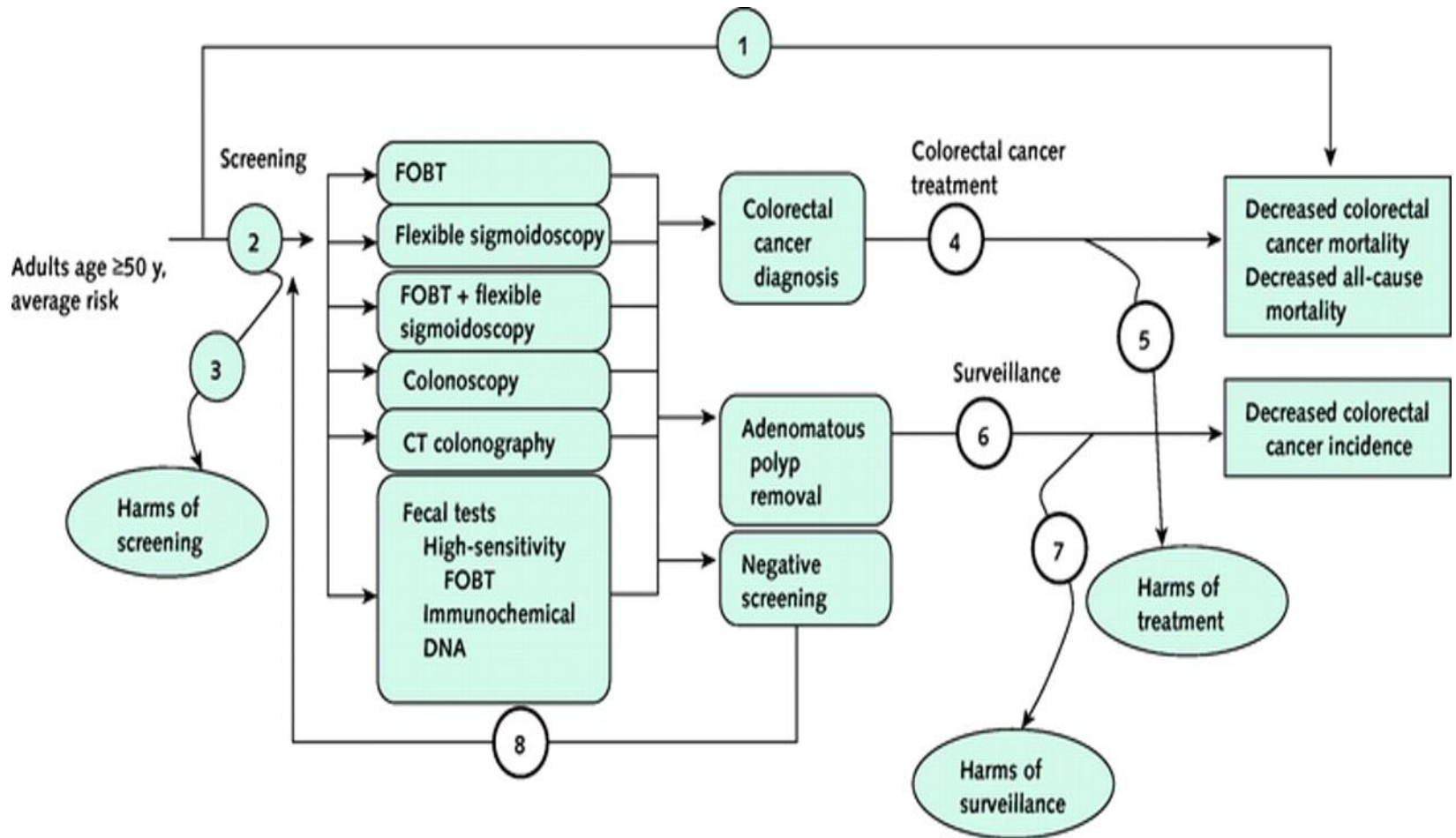
From priority topic to research question: an overview

1. Identifying Known Evidence Gaps
2. Creation of Expert Stakeholder Group
3. Expansion of Evidence Gaps
4. Analytic Framework
5. Stakeholder Prioritization
6. Horizon Scan
7. Study Design Considerations

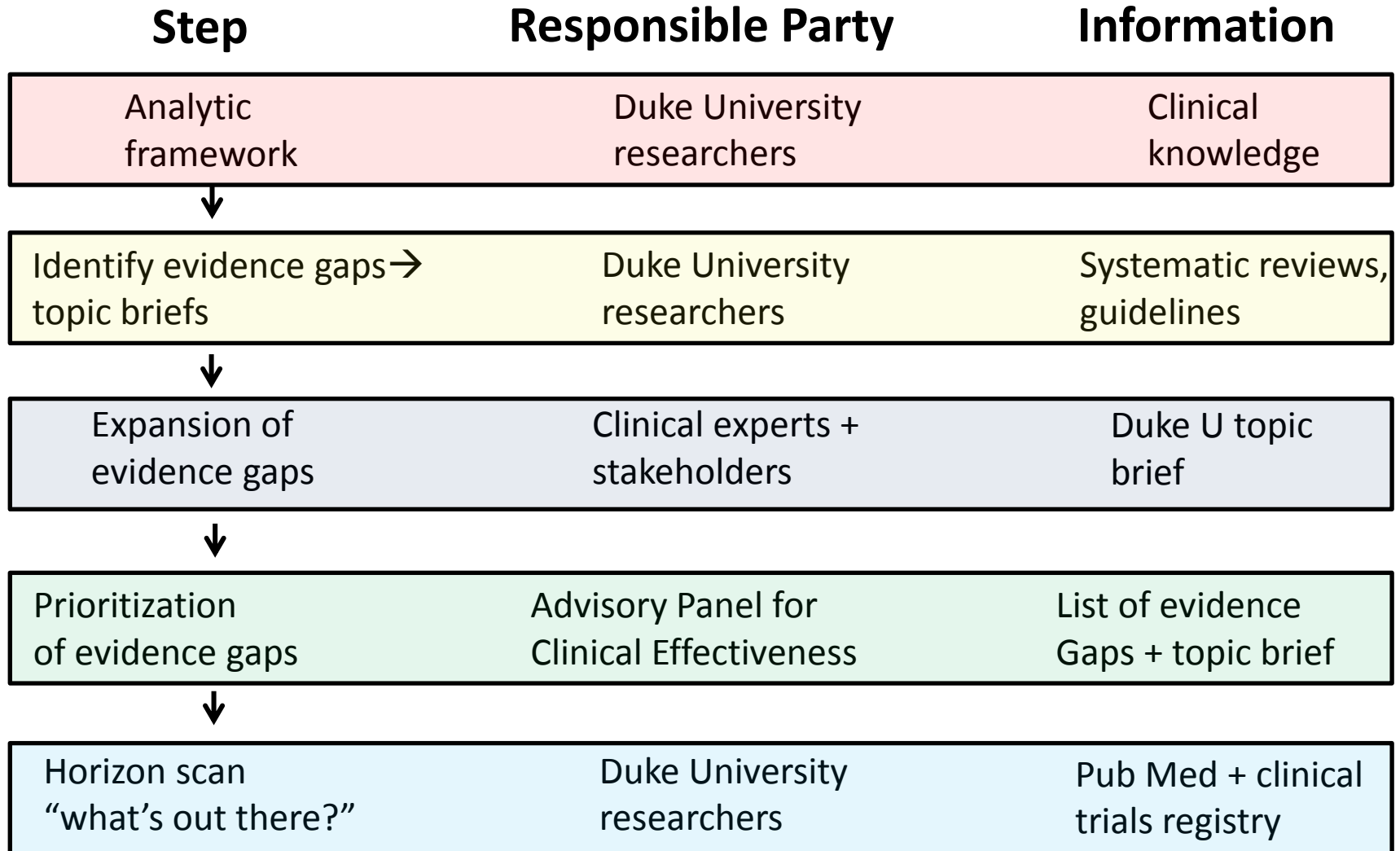
From a high priority clinical topic to key research questions



Analytic framework and key questions (KQs)



From a high priority clinical topic to key research questions



From disease to research question: an overview

1. Identifying Known Evidence Gaps
2. Creation of Expert Stakeholder Group
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Bryan Luce, PhD, MBA

Chief Science Officer, PCORI

The Patient-Centered Outcomes Research Institute (PCORI) is an independent, non-profit organization authorized by Congress in 2010. Its mission is to fund research that will provide patients, their caregivers and clinicians with the evidence-based information needed to make better-informed health care decisions. PCORI is committed to continuously seeking input from a broad range of stakeholders to guide its work.

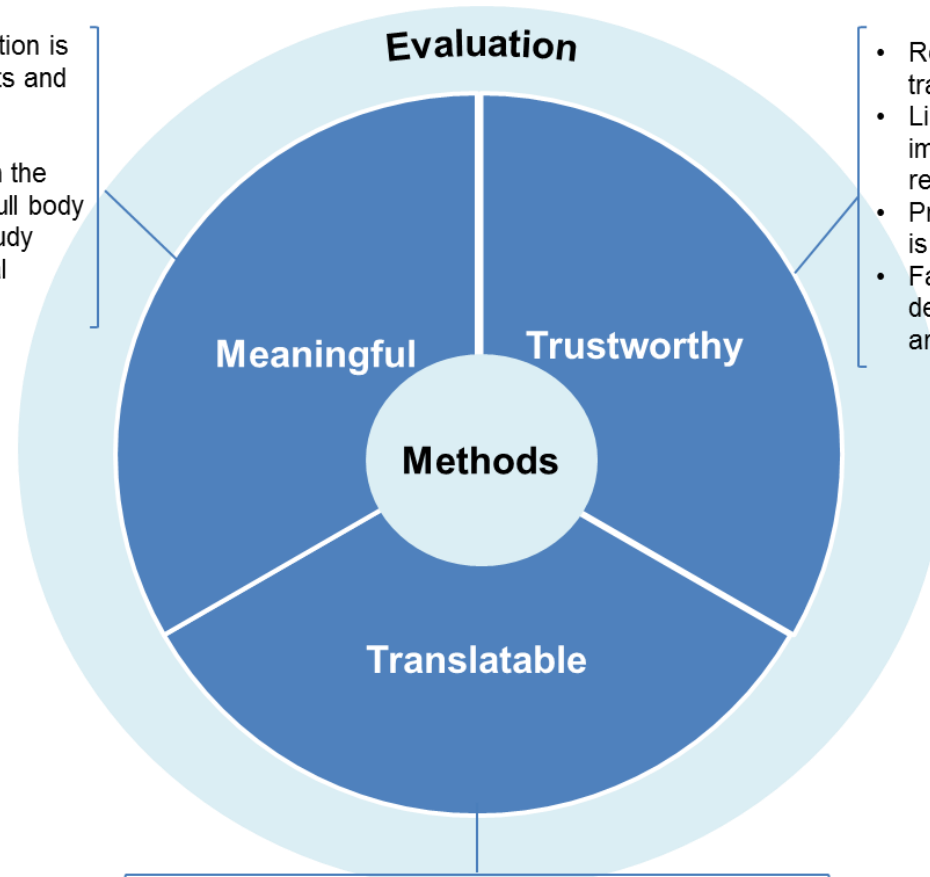
Marc Boutin

Executive VP/COO, National Health Council

“However, a study with high methodological quality is not necessarily one that patients and providers will find usable for their decision making.”

- Dr. Marc Boutin, *White Paper on Evaluating the Usability of CER, 2013*

- Research question is one that patients and providers want answered
- When placed in the context of the full body of evidence, study expands clinical understanding



- Research methods are transparent
- Limitations and implications for future research are discussed
- Presentation of study data is balanced
- Factors that led to the development of the study are disclosed

Establishing CER Usability Criteria:

- Study provides clarity on the extent to which findings can be applied in individual contexts

Darius Tandon, PhD

Co-Chair, PCORI Patient Engagement Panel

According to its charter, the Patient Engagement Panel will:

- Advise on processes to identify research topics and priorities that are important to patients;
- Advise on all aspects of stakeholder review of applications for PCORI funding;
- Provide general recommendations to PCORI and externally on the conduct of patient-centered research;
- Advise on methods to evaluate the impact of patient engagement in research;
- Assist and advise PCORI on communications, outreach, and dissemination of research findings; and
- Provide advice on other questions and areas of interest that may arise relevant to PCORI's mission and work.



Dr. Tanisha Carino, PhD.

Executive Vice President, Avalere

Tanisha Carino oversees Avalere's strategic advisory and research services for the nation's leading life sciences companies. For close to a decade, Tanisha has worked with senior leadership in Fortune 500 companies to establish organizational goals and align their internal functions to capture opportunities and mitigate challenges related to evolving regulatory, evidence, and commercial trends.



Trends in Comparative Effectiveness Research (CER)

Tanisha Carino, Ph.D.
Executive Vice President, Avalere Health

November 5, 2013
avalerehealth.net

The Current Healthcare Environment Has Driven Demand for More Evidence

Rising Costs

- Costs of new treatments are rising
- Businesses find it harder to pay for healthcare
- Patients are paying more out of pocket

Misaligned Payment

- Payment is based on volume, not quality
- Payment system comes with perverse incentives

Variable Treatment

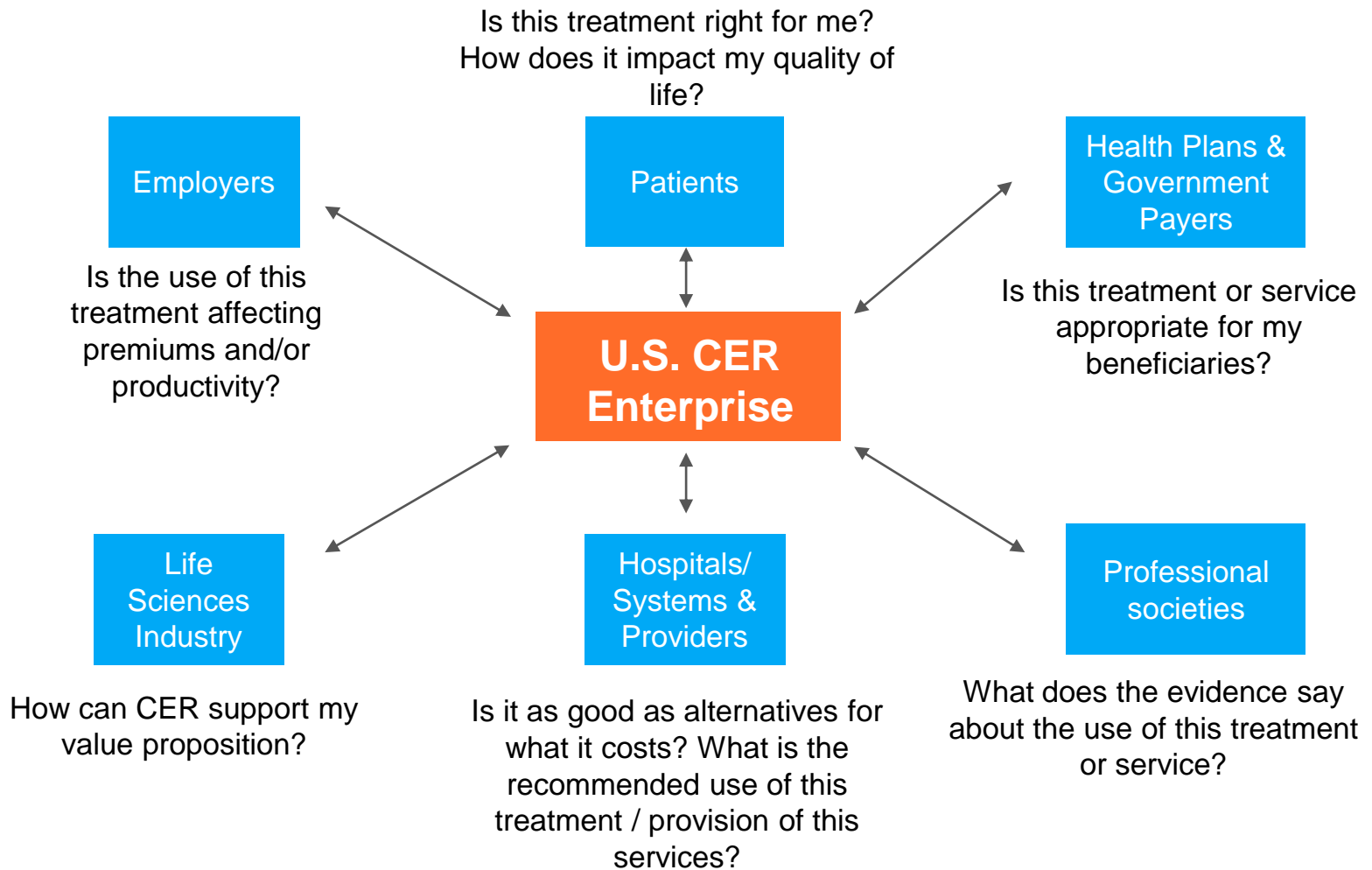
- Insufficient data on what works best
- Treatment varies; providers are not always following best practices
- Quality is suboptimal (preventable errors, safety concerns, overuse)

Lack of Information

- Minimal information about quality and cost of healthcare services
- Incomplete data at point of care to support good decision making
- Lack of data infrastructure to gather/share information



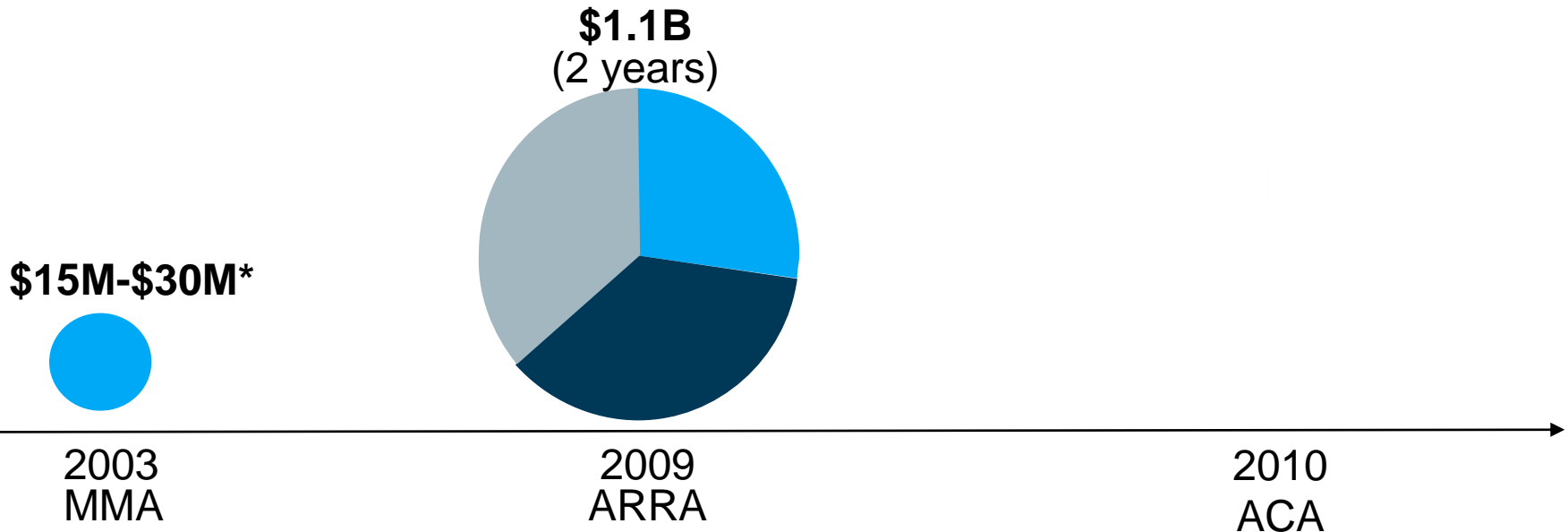
A Variety of Stakeholders Aim to Use CER to Answer Distinct Questions



The Federal Government's Investments in CER Have Grown Over the Years

- Agency for Healthcare Research and Quality (AHRQ)
- Office of the Secretary, Health and Human Services
- National Institutes of Health
- Patient-Centered Outcomes Research Institute

~\$4-5B**
(10 years)



*AHRQ CER budget was initially \$15M and has since been increased to \$30M

**2010 – 2012: appropriations of \$10M, \$50M, and \$150M respectively; mix of public and private funds thereafter

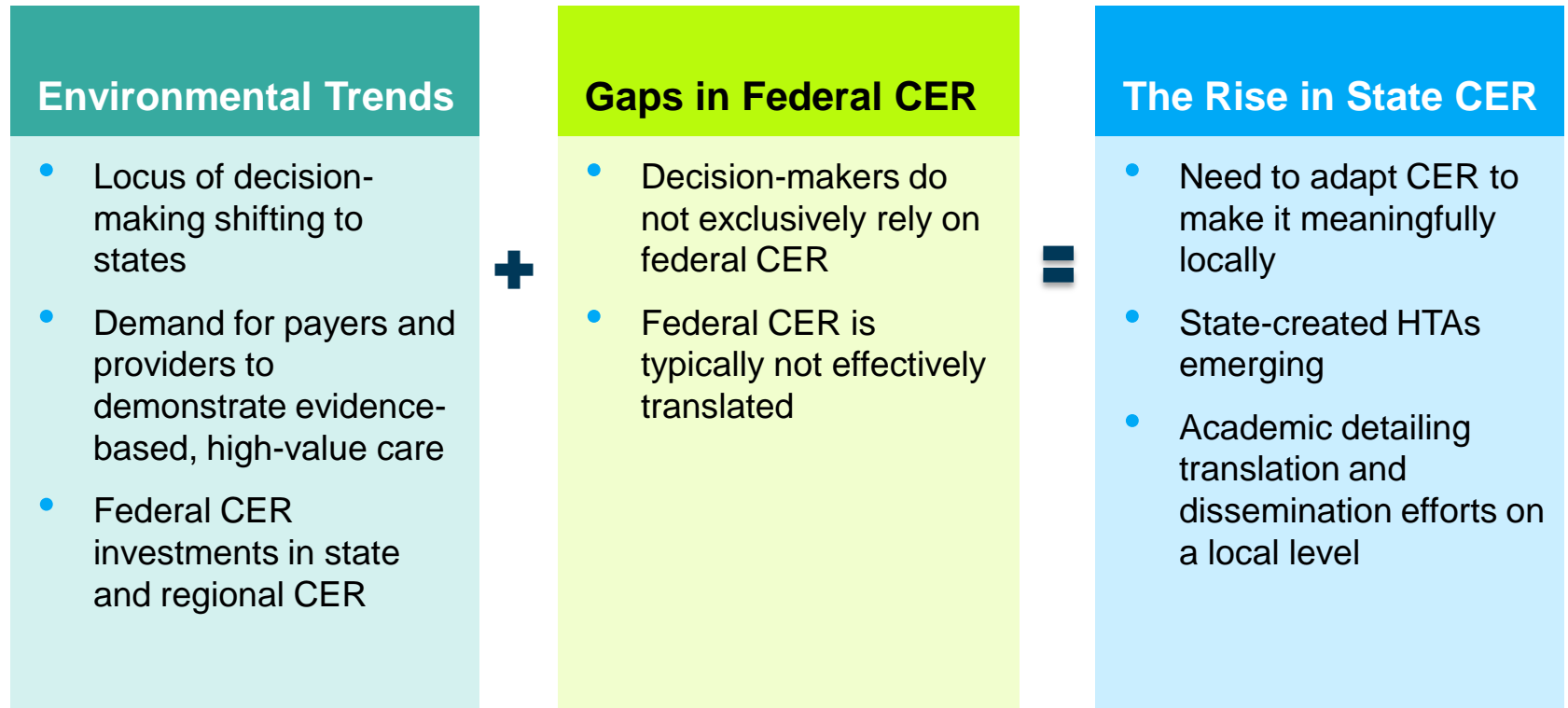
MMA: Medicare Modernization Act; ARRA: American Recovery and Reinvestment Act of 2009; ACA: Patient Protection and Affordable Care Act of 2010;

HHS: Health and Human Services ; NIH: National Institutes of Health; PCORI: Patient-Centered Outcomes Research Institute

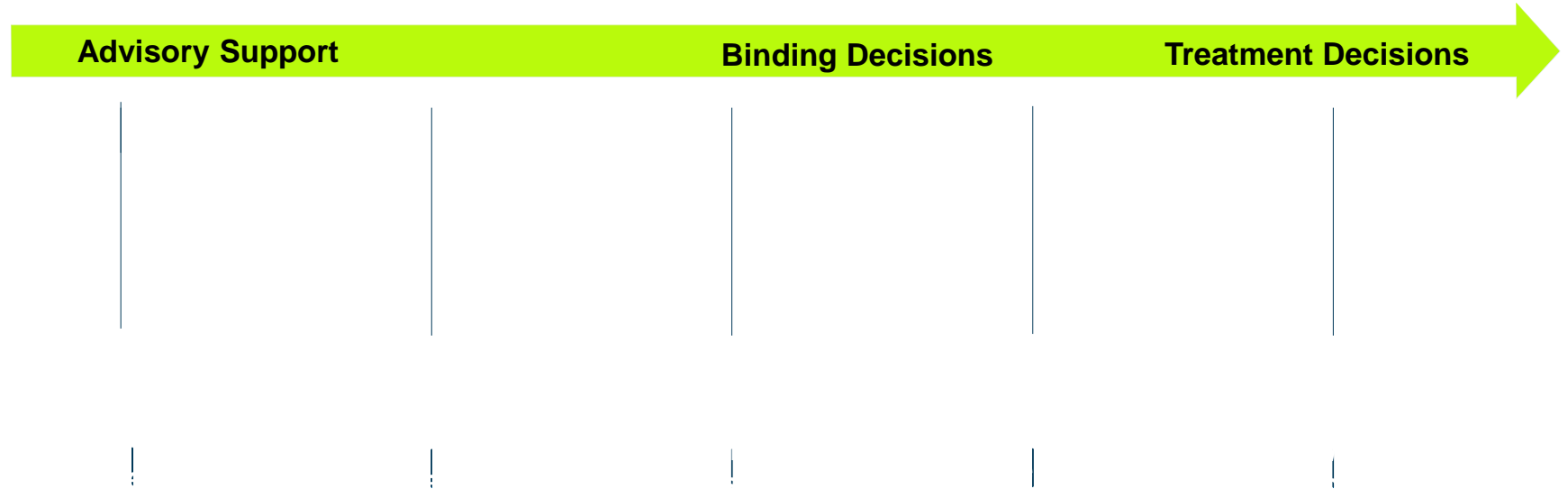


At the State Level, CER is Growing in Use As Well

DESPITE THE PROLIFERATION OF FEDERAL CER, ENVIRONMENTAL TRENDS AND GAPS IN THE EVIDENCE HAVE LED TO THE RISE IN STATE-LEVEL CER



A Number of Organizations Are Already Using CER to Make Decisions



NIH: National Institutes of Health
USPSTF: United States Preventive Services Task Force
NQF: National Quality Forum
NCQA: National Committee for Quality Assurance

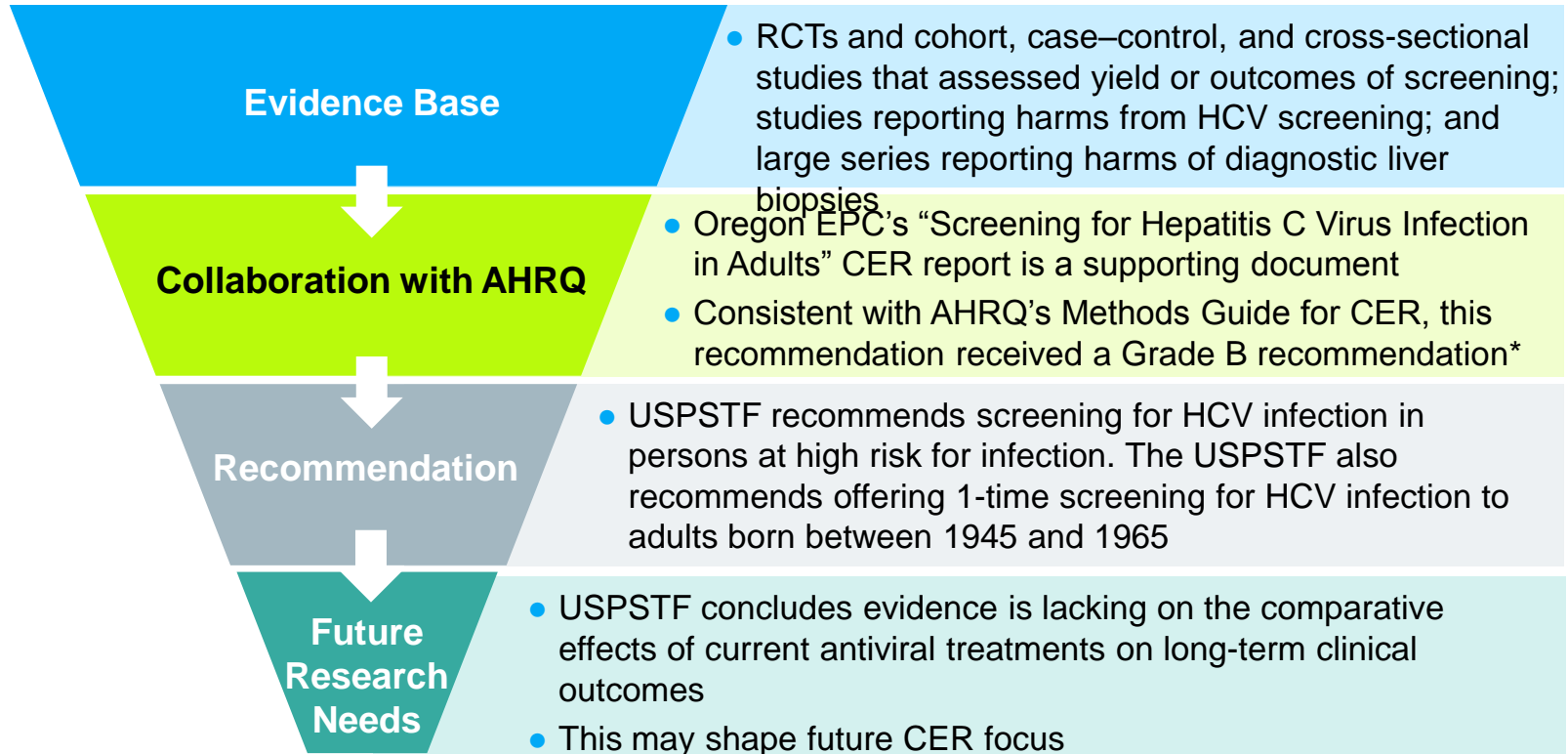
CMS: Centers for Medicare & Medicaid Services
FDA: U.S. Food and Drug Administration



Spotlight on USPSTF

USPSTF FORMALIZES EPC-GENERATED CER IN ITS CLINICAL RECOMMENDATIONS

CASE STUDY: HEPATITIS C VIRUS (HCV) SCREENING RECOMMENDATION, JUNE 2013



Given USPSTF's positive recommendation, it is likely that CMS will expand Medicare coverage for HCV screening in adults through the NCD it initiated on September 5

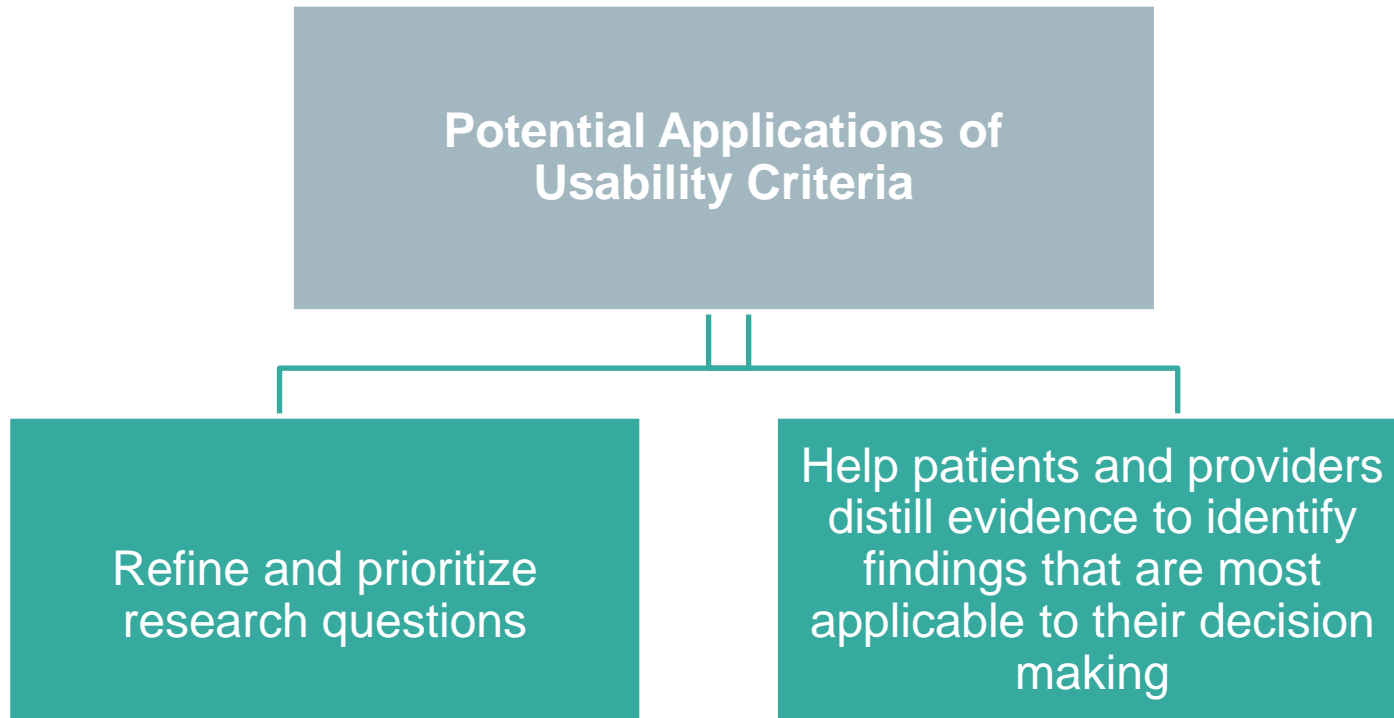
*Under the Affordable Care Act, Medicare and private payers are required to cover services recommended by the USPSTF with a grade of A or B with no cost-sharing

EPC: Evidence-based Practice Center
CMS: Centers for Medicare & Medicaid Services
NCD: National Coverage Determination



As CER Investments Grow, Focus is Growing on Usability of CER

THE NATIONAL HEALTH COUNCIL IS LEADING AN EFFORT TO CREATE CRITERIA THAT COULD BE APPLIED TO EVALUATING CER USABILITY



Key Takeaways

1

- CER-informed clinical recommendations increasingly impact patient access

2

- Monitoring state-level CER activities will be important to understanding the evidence-base for local decision-making

3

- As the number of entities that communicate information expands, consistency in communication will grow in importance





Questions?

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