

# About Us

- An independent research institute authorized by Congress in 2010, and reauthorized in 2019, governed by a 23-member Board of Governors representing the entire healthcare community
- Funds comparative clinical effectiveness research (CER)
- Engages patients and other stakeholders throughout the research process
- Seeks answers to real-world questions about what best meets the needs of the individual patient

MORE THAN  
**\$2.8B**  
AWARDED

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MORE THAN  
**1,700**  
RESEARCH AND  
RELATED PROJECTS

# We Fund Comparative Clinical Effectiveness Research



- CER compares benefits and harms of different methods to prevent, diagnose, treat, and monitor a clinical condition or improve care delivery
- Informs a specific clinical or policy decision
- Measures benefits in real-world populations
- Describes results in subgroups
- Helps patients, clinicians, purchasers, and policy makers make informed decisions

*Note: We do not fund cost-effectiveness research*

# We Fund Patient-Centered Outcomes Research



- Considers patients' needs and preferences, and the outcomes most important to them
- Investigates what works, for whom, under what circumstances
- Helps patients and other healthcare stakeholders make better-informed decisions about health and healthcare options



# PCORI's Engagement Approach



- In developing research questions that address gaps in evidence,
  - AHRQ and NIH are frequent informants or collaborators
  - USPSTF and guideline developers keep us informed about their evidence needs
  - Patients and stakeholders inform our choice of topics, interventions, and the outcomes that matter most to them.
- If you build it, they will come
  - PCORI asked guideline-developing organizations to nominate topics for systematic reviews to support clinical guidelines
    - 35 nominations from 19 organizations

# Charge to the NAM Committee on Evidence Gaps in Clinical Prevention



- Outline how research gaps are described in the current USPSTF portfolio.
- Propose a taxonomy for describing research gaps and apply it to a sample of existing USPSTF evidence gaps/I statements.
- Characterize the types of research studies needed to inform different types of evidence gaps, considering both existing USPSTF methods and innovative new methodologies.
- **Propose ways for prevention research funders and recommendation statement developers, including NIH and the USPSTF, to enhance partnerships to accelerate research to close important gaps in prevention**

Source: the NAM Committee on Evidence Gaps in Clinical Prevention

# Funding



- A funder's priorities
- A funder's research agenda

# PCORI's National Priorities: 2012



- PCORI's enabling legislation charged it with establishing National Priorities and a Research Agenda to **guide it in funding comparative CER.**
- The PCORI Board of Governors approved the National Priorities following robust, nationwide stakeholder engagement and a formal public comment period
  - Assessment of Prevention, Diagnosis, and Treatment Options
  - Improving Healthcare Systems
  - Addressing Disparities
  - Communication and Dissemination Research
  - Accelerating Patient-Centered Outcomes Research and Methodological Research



# PCORI's National Priorities: 2021



- Newly reauthorized, PCORI is refreshing its National Priorities and Research Agenda during 2021
- PCORI's Board of Governors is ensuring...
  - Robust, nationwide engagement with stakeholders
    - Clinicians, payers, purchasers, systems, researchers, and patient advocates, caregivers, and patients
    - A survey of the national health/health care priorities of diverse organizations (e.g., NAM, CMS, NIH, regional healthcare systems)
    - Discussions with health/health care leaders
  - A formal public comment period on our draft National Priorities
- Then, we will tackle the specifics of our Research Agenda.

# PCORI's 2019 Reauthorization

## New Considerations in Planning a Research Agenda



- PCORI's reauthorizing legislation directed us to ensure that our funding strategy includes attentiveness to two key areas:
  - Maternal mortality
  - Intellectual and developmental disabilities
- In developing its research agenda, PCORI must also
  - Reflect a balance of short- and long-term priorities
  - Be responsive to changes in our nation's priorities, medical evidence, and healthcare treatments
    - Two prime examples: COVID-19, eliminating disparities

# Priority-Setting Criteria for Research Questions



- Do research questions and outcomes reflect what is important to patients and caregivers (patient-centeredness)?
- Burden of illness
- What do guidelines/systematic reviews say about evidence gaps?
- Are similar studies ongoing?
- Likelihood of widespread uptake of results in practice
- Likely durability of research results (stable vs. fast-changing technology)
- Likelihood of recruiting patients (equipoise) and reaching them for follow-up.

# A PCORI Case in Point



- **Urinary Incontinence**

- Non-surgical interventions were identified as a priority topic by multiple stakeholder organizations representing patients and clinicians
- PCORI identified an existing systematic review that identified evidence.
  - Collaborating with AHRQ, we funded, a stakeholder-driven update of the review.
- PCORI is discussing a funding announcement to address remaining evidence gaps and intends to issue a funding announcement for implementation of new evidence

# In conclusion

- Relationships matter for funders, especially with end-users.
- Systematic reviews are the major, but not necessarily the only, source of evidence gaps.
- Research questions should reflect user-level considerations and input.

# Contact Information

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# Appendix

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# Key Criteria for PCORI Research



## 1. Potential for the study to fill critical gaps in evidence

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

## 2. Potential for the study findings 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. Investigator(s) and environment

- The proposed project has a team with appropriate investigators (e.g. qualifications and experience), as well as an environment with sufficient capacity (e.g. resources, facilities, and equipment)

## 5. Patient-centeredness

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

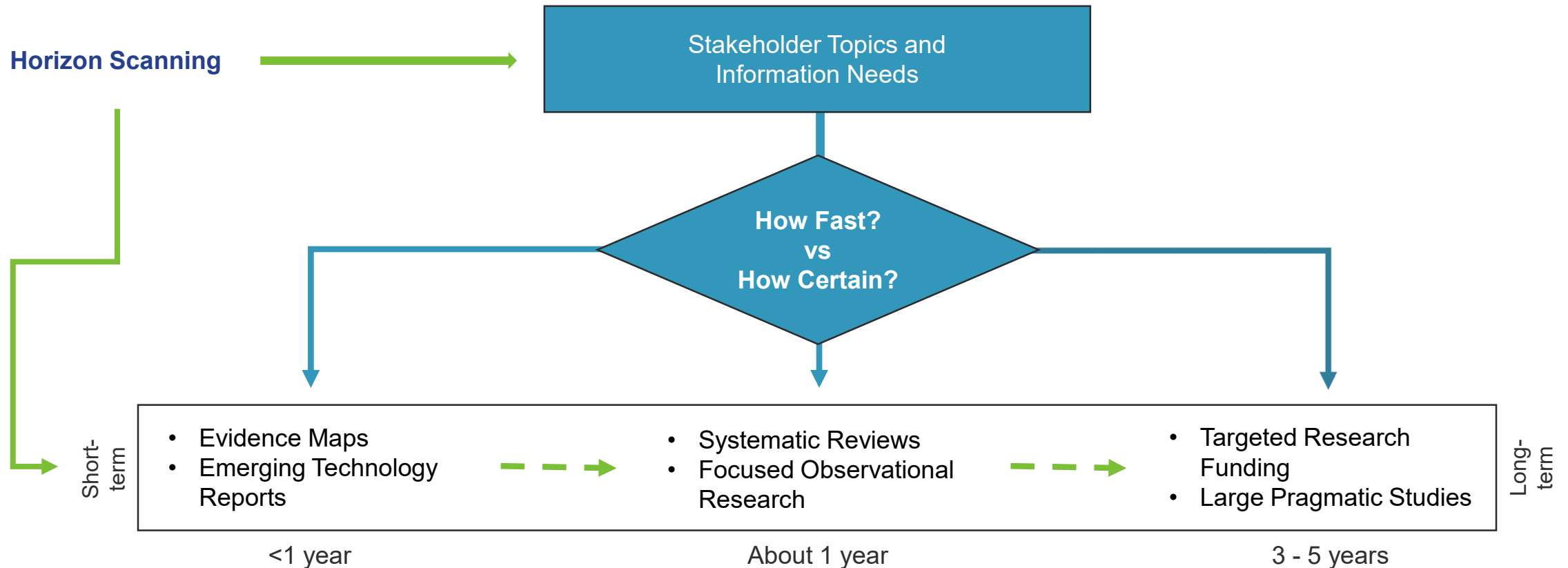
## 6. Patient and stakeholder engagement

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



# Continued Evolution in Evidence Development

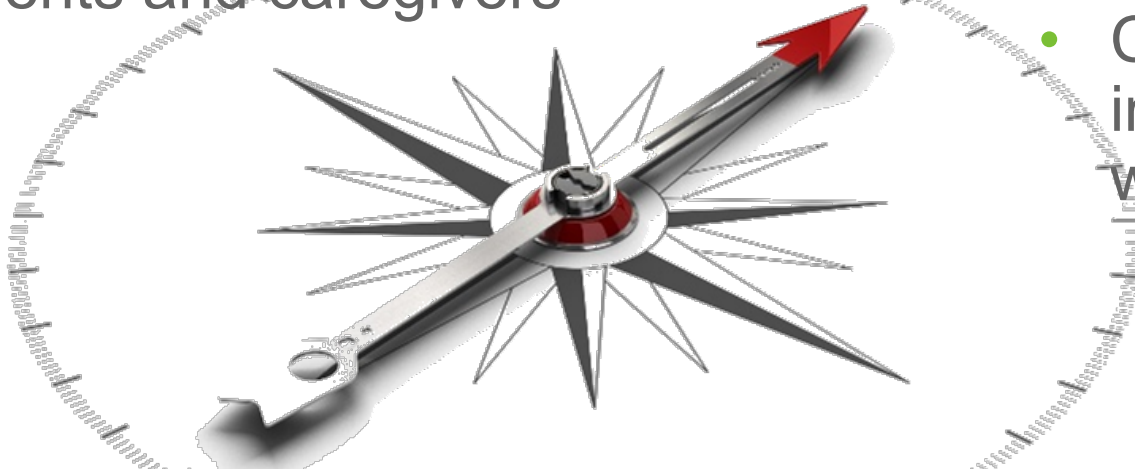
- Providing a range of evidence products to meet decision maker needs



# What we mean by...

## Patient-Centeredness

- The project aims to answer questions or examine outcomes that matter to patients within the context of patient preferences
- Research questions and outcomes should reflect what is important to patients and caregivers



## Patient and Stakeholder Engagement

- Patients are partners in research, not just “subjects”
- Active and meaningful engagement between scientists, patients, and other stakeholders
- Community, patient, and caregiver involvement already in existence or a well-thought-out plan