

What is patient-centered outcomes research? An introduction to PCORI

Kim Bailey
Senior Program Officer
Clinical Effectiveness and Decision Science

SIDM Diagnostic Researcher Workshop
November 9, 2019

Goals for Presentation

- 1) Introduce PCORI and PCORI's mission
- 2) Describe CER and the research that PCORI funds
- 3) Discuss the importance of patient and stakeholder engagement in research
- 4) Discuss how to design a patient-centered study
- 5) Answer any questions that you have

1.

Who PCORI Is and What We Do

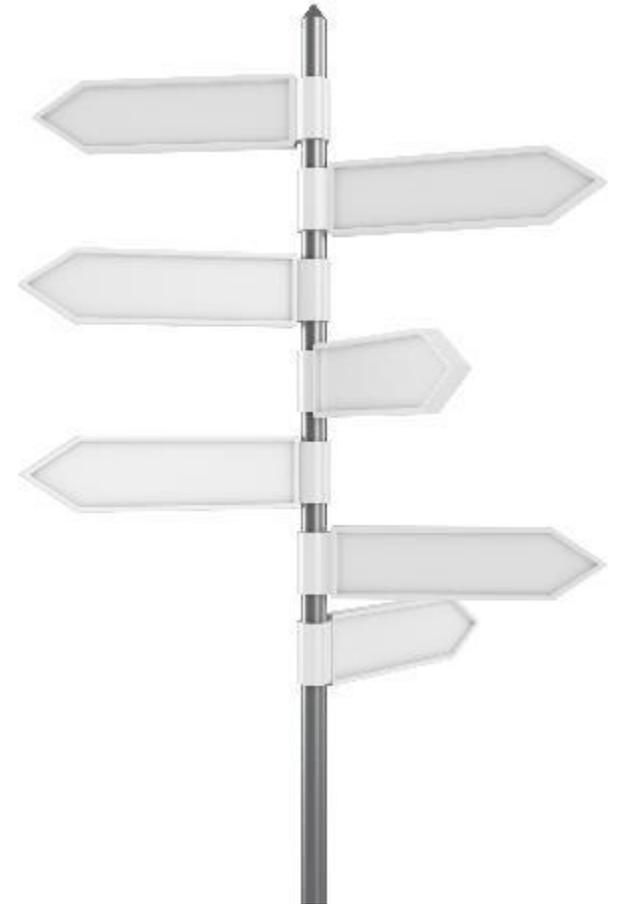
About Us

- An independent research institute authorized by Congress in 2010 and governed by a 21-member Board of Governors 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



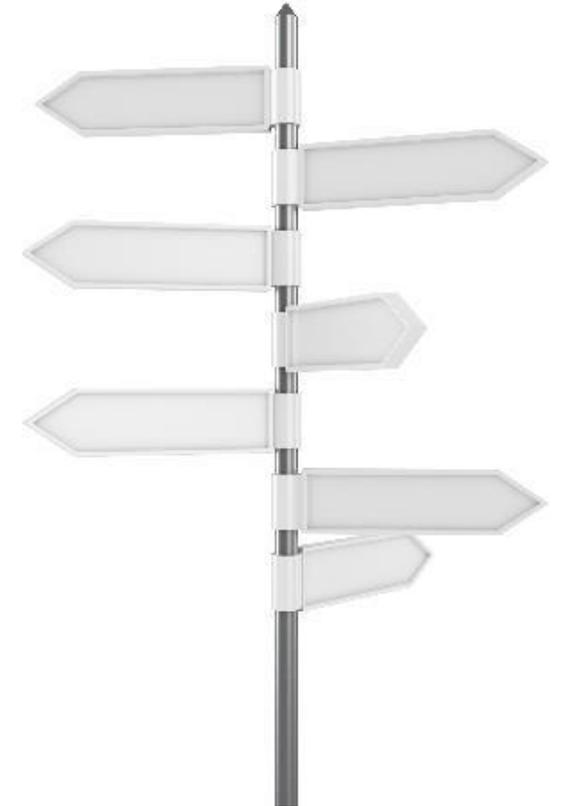
Why Is Our Work Needed?

- For all the advances it produces, traditional healthcare research has not answered many questions patients face
- People want to know which preventive, diagnostic, or treatment option is best for them
- Patients and their clinicians need information they can understand and use



How Is Our Work Different?

- We fund research on which care options work, for whom, under which circumstances
- We focus on answering questions most important to patients and those who care for them
- We aim to produce evidence that can be easily applied in real-world settings
- We engage patients, caregivers, clinicians, insurers, employers, and other stakeholders throughout the research process
- This makes it more likely we'll get the research questions right and the study results will be useful and taken up in practice



Our Mission and Strategic Goals



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

Who Are Our Stakeholders?



2.

Our Research Focus

Focus on comparative clinical effectiveness research (CER)



- **CER includes:**
 - Studies that compare health outcomes and the clinical effectiveness, risks, and benefits of two or more approaches to healthcare
- **All applicants should:**
 - Explain how the research is comparative
 - Name the comparators
 - State why the comparisons are important to decision-makers

Strong CER studies address important evidence gaps

- Assess the evidence about available options and their important outcomes
 - Systematic reviews
 - Evidence gaps that are important to decision makers
- Design a study that can feasibly close the evidence gap
 - If the gap is not important, the research will not be useful

Research we do not fund

- Based on PCORI's authorizing legislation, PCORI is not permitted to fund studies of cost-effectiveness analysis (CEA).

Examples of CEA

- Research that conducts a formal CEA in the form of dollar-cost per quality-adjusted life-year (including non-adjusted life-years) to compare two or more alternatives
- Research that directly compares the overall costs of care between two or more alternative approaches as the criterion for choosing the preferred alternative

PCORI's National Priorities for Research

National Priority	Description of Research Focus
Assessment of Prevention, Diagnosis, and Treatment Options	Research comparing the harms and benefits of alternative interventions to determine which work best for individual patients
Improving Healthcare Systems	Research comparing different health system-level approaches for improving access and care
Communication and Dissemination Research	Research comparing different ways of providing information produced by CER
Addressing Disparities	Research comparing interventions to reduce or eliminate disparities in health and health care
Accelerating PCOR and Methodological Research	Infrastructure and methodological development to support CER

We Fund Research That...



Meets these criteria:

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

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



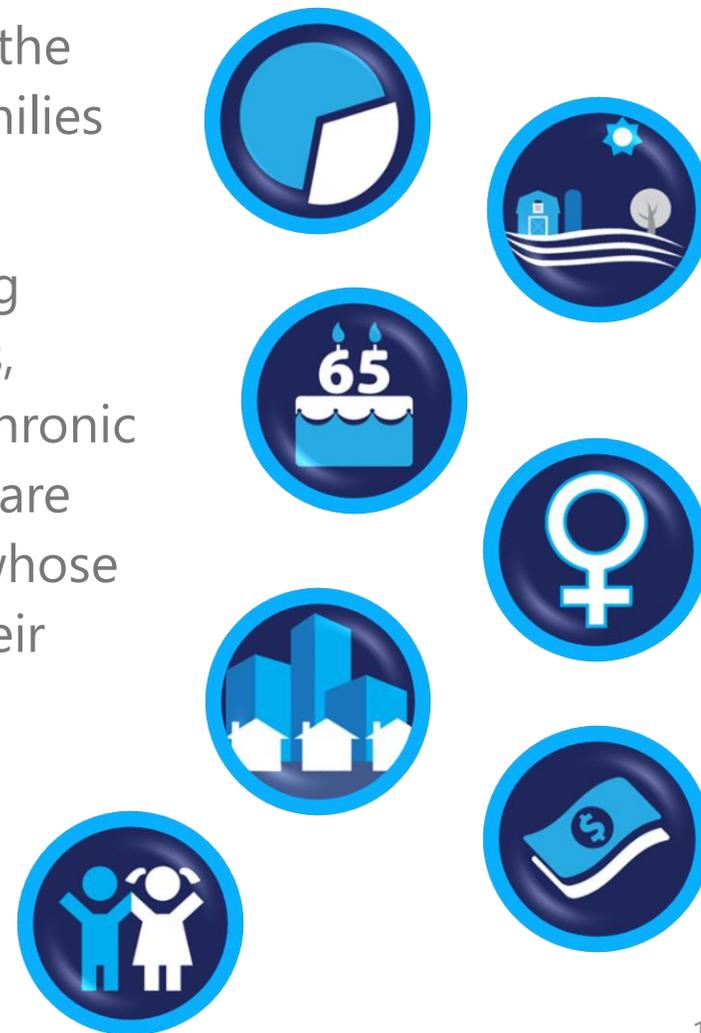
“Patient and stakeholder engagement”

- 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

Pays Particular Attention to Specific Populations

- Racial and ethnic minorities
- Older adults
- Low-income
- Residents of rural areas
- Women
- Children
- Patients with low health literacy/numeracy and limited English proficiency
- Lesbian, gay, bisexual, transgender (LGBT) persons

- Veterans and members of the armed forces and their families
- Individuals with special healthcare needs, including individuals with disabilities, individuals with multiple chronic diseases, individuals with rare diseases, and individuals whose genetic makeup affects their medical outcomes



We Also Work to Improve Research Methodology

In any study, methods matter. That's why we've developed methodology standards that patient-centered CER should follow, at a minimum.

Methodology Standards: 16 Broad Categories

- Formulating Research Questions
- Patient-Centeredness
- Data Integrity and Rigorous Analyses
- Preventing/Handling Missing Data
- Heterogeneity of Treatment Effects
- Data Registries
- Complex Interventions
- Mixed Methods Research
- Data Networks
- Causal Inference
- Adaptive and Bayesian Trial Designs
- Studies of Medical Tests
- Systematic Reviews
- Research Designs Using Clusters
- Qualitative Methods
- Individual Participant-Level Data Meta-Analysis

Snapshot of Funded Projects

Number of funded awards:

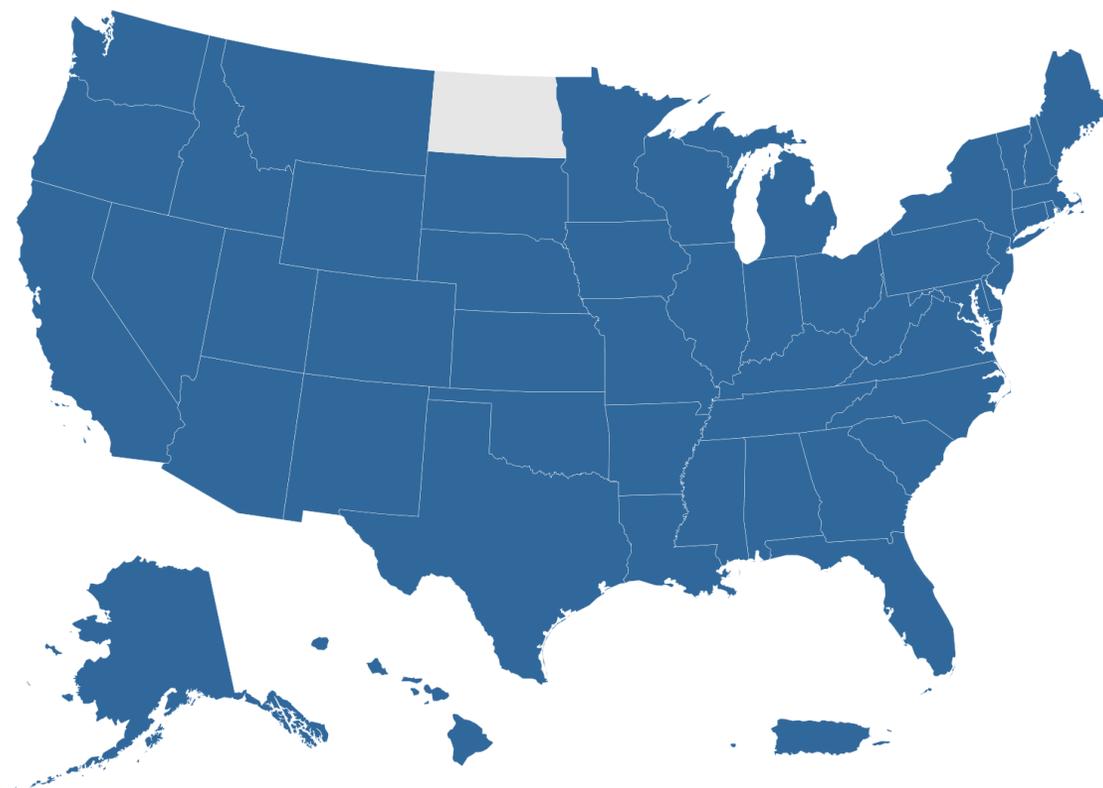
More than 1,400

Amount awarded:

More than \$2.4 billion

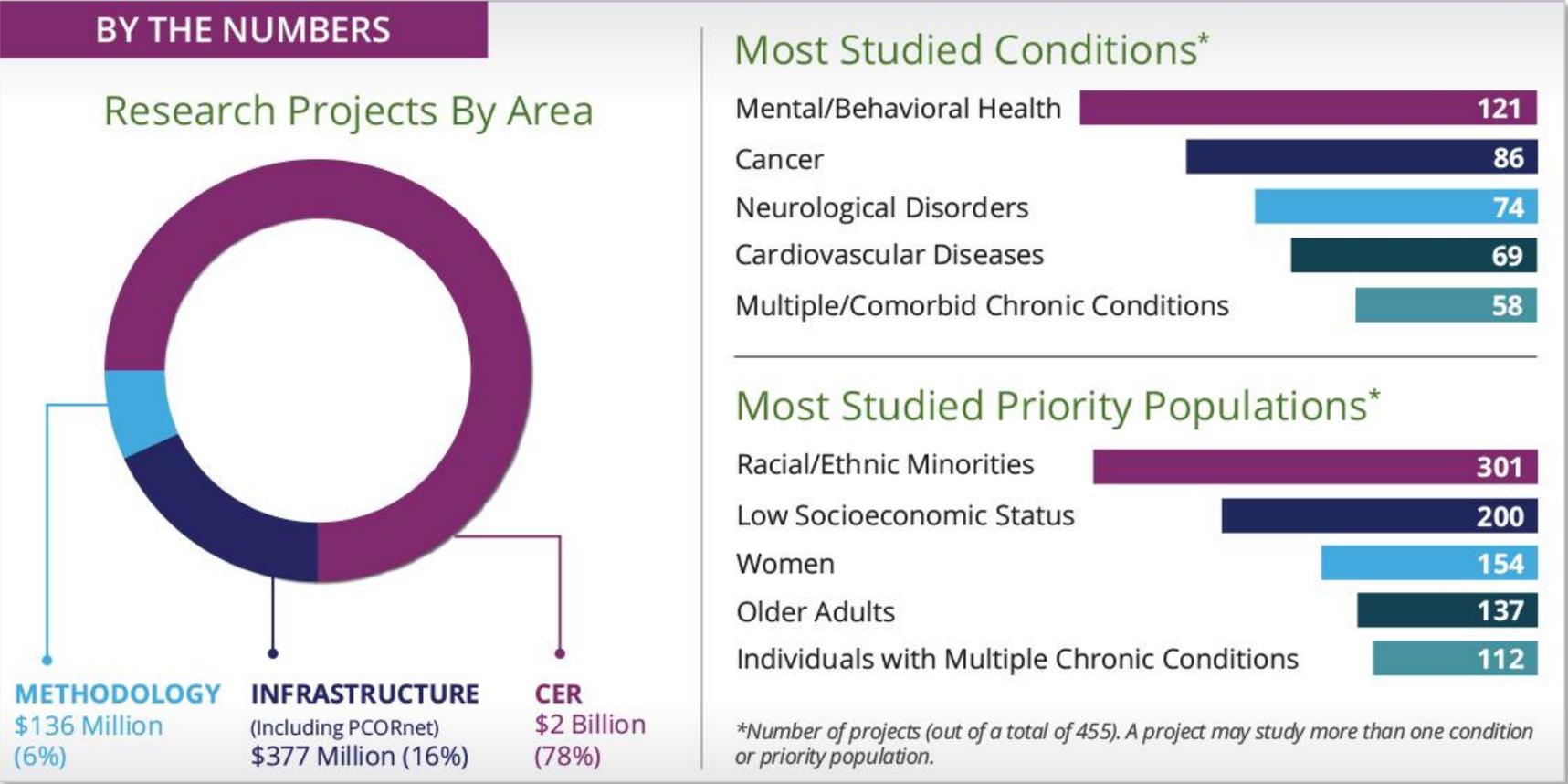
Number of states where we are funding projects:

49 (plus the District of Columbia and Puerto Rico)



AS OF MAY 2019
All awards

About Our Research Portfolio



AS OF MAY 2019

Pragmatic Clinical Study Awards



PCORI HAS AWARDED

\$493 MILLION TO FUND **43**

PRAGMATIC CLINICAL STUDIES

Including projects studying:

AS OF MAY 2019



Cancer



Mental/
Behavioral Health



Trauma/Injury



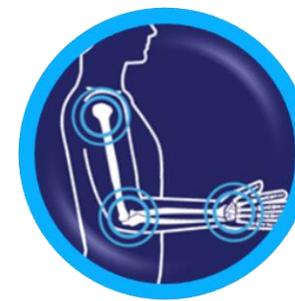
Cardiovascular
Diseases



Digestive
Diseases



Infectious
Diseases



Musculoskeletal
Diseases



Respiratory
Diseases

3.

Better Research Through Engagement

Why Engage?

- To influence research to be patient-centered, relevant, and useful
 - To establish trust and a sense of legitimacy in research findings
 - To encourage successful uptake and use of research results



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.

Engagement as a Path to Useful, High-Quality Research

**Topic Selection
and Research
Prioritization**



**Proposal Review;
Design and Conduct
of Research**

Evaluation

**Dissemination and
Implementation of
Results**

Our Engagement Rubric— A Valuable Resource



Provides practical guidance to applicants, merit reviewers, awardees, and engagement/program officers on effective engagement in research

- **Planning the Study:** How patient and stakeholder partners will participate in study planning and design
- **Conducting the Study:** How patient and stakeholder partners will participate in the conduct of the study
- **Disseminating the Study Results:** How patient and stakeholder partners will be involved in plans to disseminate study findings and ensure that findings are communicated in understandable, usable ways
- **PCOR Engagement Principles:** Reciprocal relationships, co-learning, partnership, trust, transparency, honesty

4.

Designing a patient-centered
outcomes research study

PCORI Funds Comparative Clinical Effectiveness Research that:



- 1) Compares two or more **commonly used options** (treatment or ways of delivering care) that work for prevention, diagnosis, or treatment
- 2) Fills **evidence gaps** that are important to patients and other key stakeholders
- 3) Is conducted in **real-world** populations and settings
- 4) Considers **outcomes relevant to patients**
- 5) **Engages patients and other stakeholders** in the study design and conduct

What is Clinical Evidence?



- **Clinical Evidence**: Valid data about the outcomes experienced by patients or providers who receive specific medical or health systems interventions
 - The population is well-defined
 - The clinical interventions are well-defined
 - This information describes the most important outcomes (both benefits and harms) associated with specific clinical interventions

Identifying an Evidence Gap

Examine the choices people make about the options for treating or managing a disease

Consider how compelling it is to make a choice among these options

Assess the evidence about available options and their important outcomes

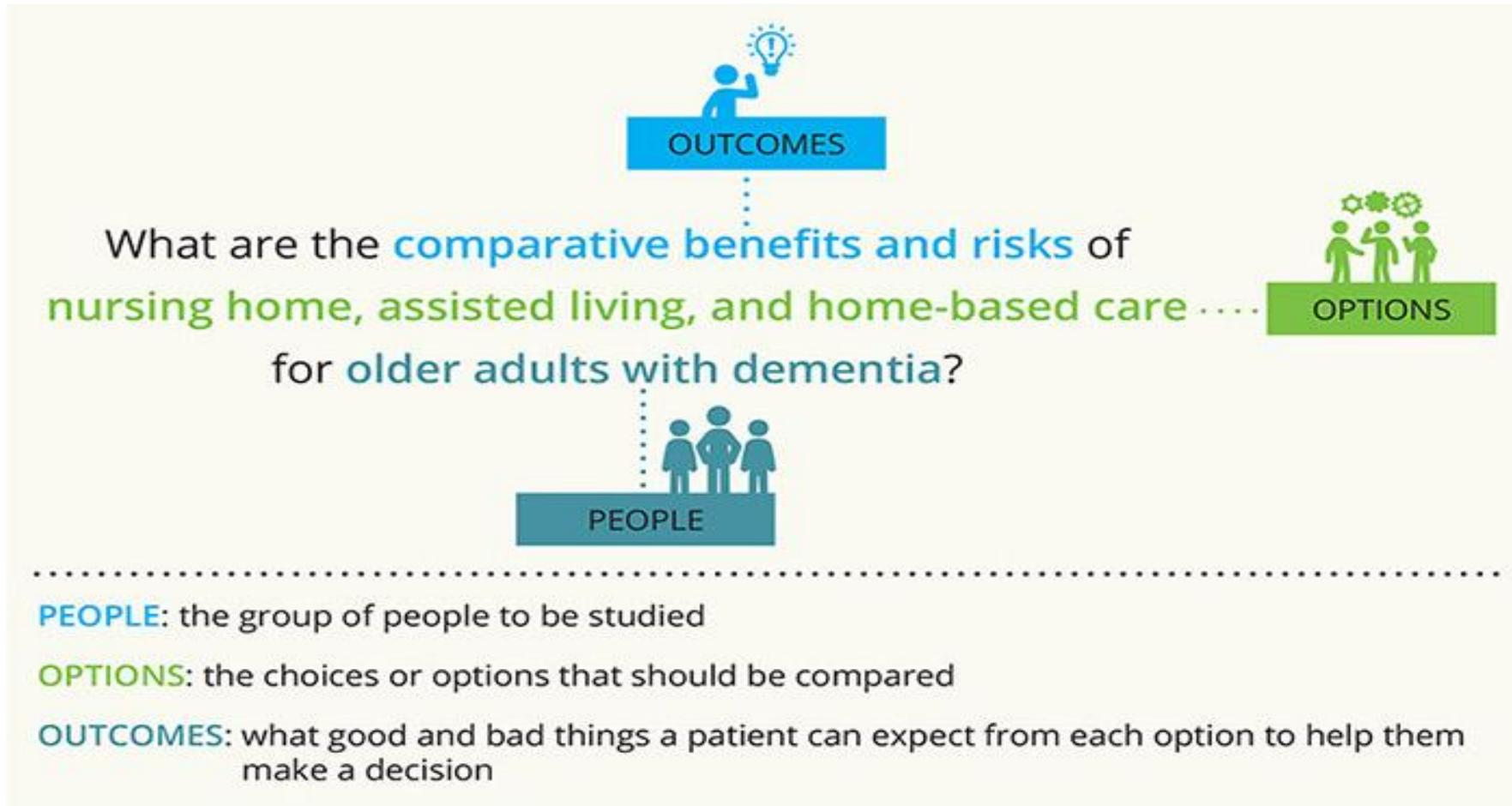
Formulate a research question and design a research project comparing the benefits and harms associated with each option

Formulating a Patient-Centered CER Question: The PICOTS Framework



- **P: Patients** – Who will be in your study?
- **I: Intervention** – What are the characteristics of the interventions you are studying?
- **C: Comparators** – What are the characteristics of the other intervention you are studying?
- **O: Outcomes** – What are the outcomes that are most important to patients and other stakeholders that you plan to measure?
- **T: Timing** – How long will you follow the individuals in your study?
- **S: Settings** – Where is your intervention delivered (e.g. clinic, hospital, etc.)?

Defining Your Question: People, Options, Outcomes



Contact Information

Kim Bailey, Senior Program Officer, Clinical Effectiveness and Decision Science

 202.827.7700

 kbailey@pcori.org

 www.pcori.org

 @pcori

 /PCORInstitute

 PCORI

 /pcori

Thank You!

New Engagement Award: Special Project Funding Announcements

- **Accelerating the Adoption of Tools and Resources**
 - Funding for projects that give organizations and community groups the opportunity to scale up or adopt meaningful engagement tools and processes with more communities, stakeholders, and patients with the intent to further build
- **Community Convening Around Patient-Centered Outcomes Research**
 - Funding for multi-stakeholder convenings that include patients, researchers, clinicians, purchasers, payers, health system leaders, and other stakeholders. These convenings will have a focus on, and commitment to, supporting collaboration around PCOR/CER.

Awards up to \$100,000 total costs

Awards up to 1 year in duration

LOI deadline 11/15 at 5 pm EST

Engagement Award Funding Opportunities



**Up to
\$300,000
2 years**

Dissemination Initiative

Objective: Actively disseminate PCORI-funded research findings

**Up to
\$50,000
1 year**

Conference Support

Objective: Convene stakeholders and communicate PCORI-funded research finding to a targeted end-user audience

**Up to
\$250,000
2 years**

Capacity Building

Objective: Develop infrastructure and partnerships for dissemination and implementation of PCORI-funded research findings