



Supporting Research that Matters to Patients

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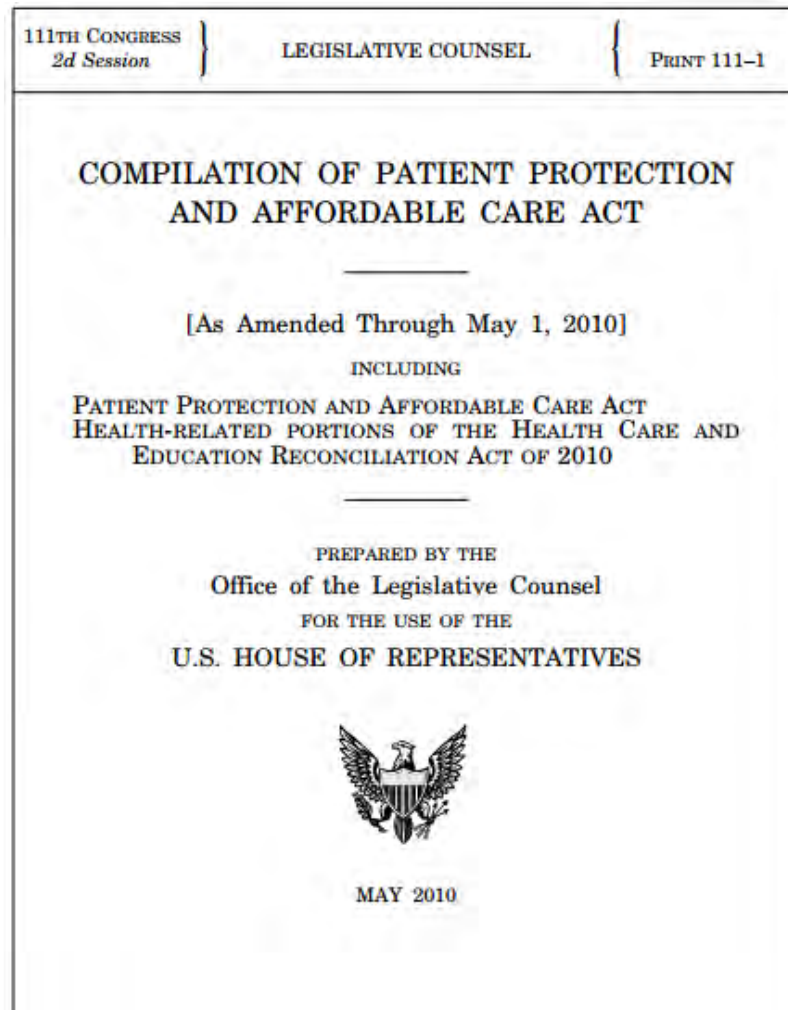
May 13, 2014

Patient-Centered Outcomes Research Institute

Overview

- To introduce you to PCORI
- To describe the research it supports and how it differs from that of other research funding agencies
 - Emphasis on comparative effectiveness research (CER)
 - Merit Review criteria
- To provide some pointers about research applications to PCORI
- To answer your questions

Patient Protection and Affordable Care Act



A lesser-known provision

Subtitle D—Patient-Centered Outcomes Research

SEC. 6301. PATIENT-CENTERED OUTCOMES RESEARCH.

(a) IN GENERAL.—Title XI of the Social Security Act (42 U.S.C. 1301 et seq.) is amended by adding at the end the following new part:

“PART D—COMPARATIVE CLINICAL EFFECTIVENESS RESEARCH

“COMPARATIVE CLINICAL EFFECTIVENESS RESEARCH

“SEC. 1181. (a) DEFINITIONS.—In this section:

“(1) BOARD.—The term ‘Board’ means the Board of Governors established under subsection (f).

“(2) COMPARATIVE CLINICAL EFFECTIVENESS RESEARCH; RESEARCH.—

“(A) IN GENERAL.—The terms ‘comparative clinical effectiveness research’ and ‘research’ mean research evaluating and comparing health outcomes and the clinical

42 USC 1320e.

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 . . .”



Source: Affordable Care Act. Subtitle D—Patient-Centered Outcomes Research Institute PUBLIC LAW

5 111–148—MAR. 23, 2010



Patient-Centered Outcomes Research Institute

PCORI's Mandate

“... 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 . . .”



Source: Affordable Care Act. Subtitle D—Patient-Centered Outcomes Research Institute PUBLIC LAW

6 111–148—MAR. 23, 2010



Patient-Centered Outcomes Research Institute

PCORI's Mandate

“ . . . that considers variations in patient subpopulations, and the dissemination of research findings with respect to the **relative health outcomes, clinical effectiveness**, and appropriateness of the medical treatments . . . ”



PCORI National Priorities for Research



Assessment of Prevention, Diagnosis, and Treatment Options



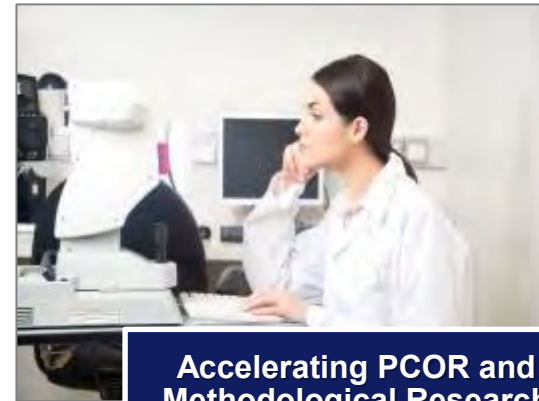
Improving Healthcare Systems



Communication & Dissemination Research



Addressing Disparities



Accelerating PCOR and Methodological Research

Comparative Clinical Effectiveness Research

Subtitle D—Patient-Centered Outcomes Research

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What is Comparative Effectiveness Research?

- Comparative effectiveness research is the conduct and synthesis of systematic research comparing different interventions and strategies to prevent, diagnose, treat and monitor health conditions.



Source: Federal Coordinating Council for Comparative Effectiveness Research, June 2009.

What is Patient Centered Outcomes Research?

Patient-Centered Outcomes Research (PCOR) is a type of comparative effectiveness research that helps people and their caregivers communicate and make informed health care decisions.

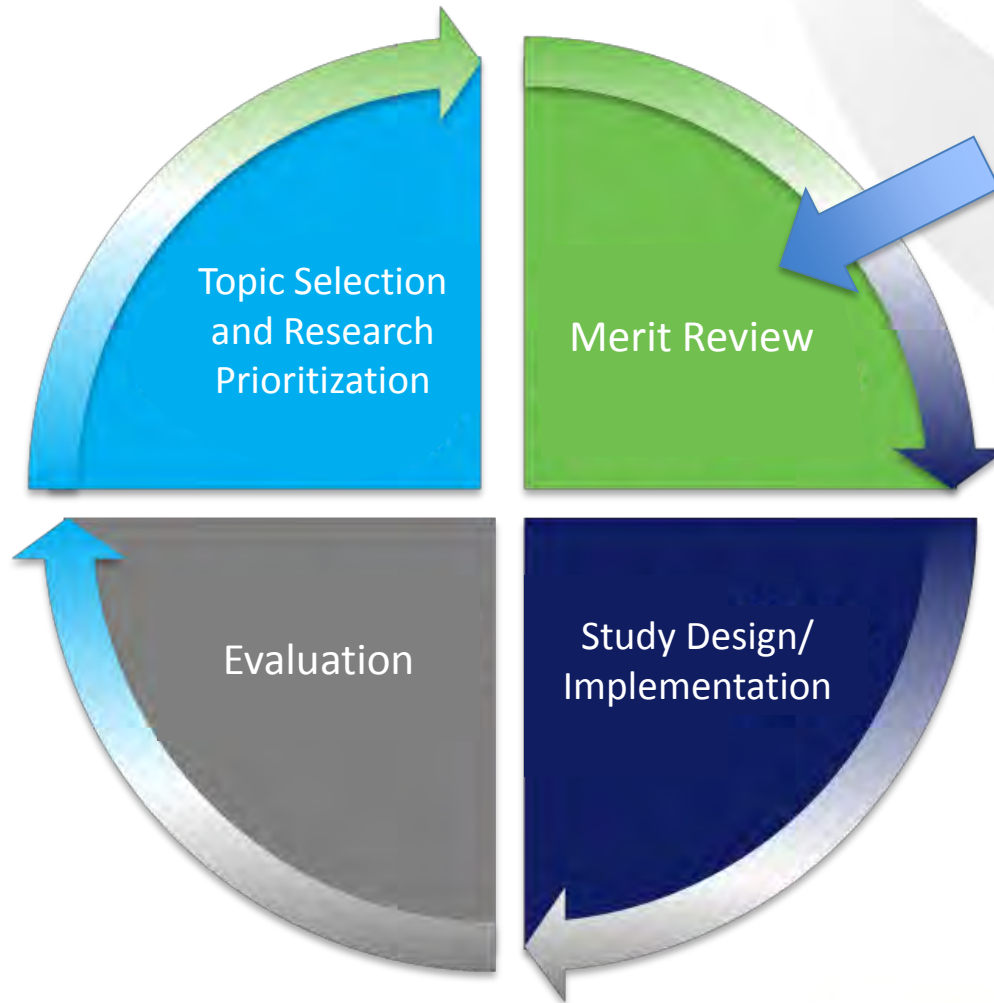
PCOR:

- Actively engages patients and key stakeholders throughout the research process.
- Compares important clinical management options.
- Evaluates the outcomes that are most important to patients.
- Addresses implementation of the research findings in clinical care environments.

PCORI Merit Review Criteria

- Impact of the condition on health of individuals and populations
- Potential for improving care and outcomes
- Technical merit
- Patient-centeredness
- Patient and stakeholder engagement

We engage stakeholders at every step



Applications are reviewed by 2 scientists, 1 patient, and 1 stakeholder

Criterion 1. Impact of the condition on health of individuals and populations

- Is the condition or disease associated with a significant burden in the US population, in terms of prevalence, mortality, morbidity, individual suffering, or loss of productivity?
- Alternatively, does the condition or disease impose a significant burden on a smaller number of people who have a rare disease?
- Does the proposal include a particular emphasis on patients with one or more chronic conditions?

Criterion 2. Potential for improving care and outcomes

- Does the research question address a critical gap in current knowledge as noted in systematic reviews, guideline development efforts, or previous research prioritizations?
- Has it been identified as important by patient, caregiver, or clinician groups?
- Do wide variations in practice patterns suggest current clinical uncertainty?
- Is the research novel or innovative in its methods or approach, in the population being studied, or in the intervention being evaluated in ways that make it likely to improve care?
- Do preliminary studies indicate potential for a sizeable benefit of the intervention relative to current practice? How likely is it that positive findings could be disseminated and implemented quickly, resulting in improvements in practice and patient outcomes?

Criterion 3. Technical Merit

- A clear research plan with rigorous methods that demonstrates adherence to PCORI's Methodology Standards
- A realistic timeline that includes specific scientific and engagement milestones
- A research team with the necessary expertise and an appropriate organizational structure
- A research environment sufficient to support the conduct of the work with appropriate resources
- A diverse study population with respect to age, gender, race, ethnicity, and clinical status, as appropriate for the proposed research

Criterion 4. Patient-centeredness

- Is the research focused on questions that affect outcomes of interest to patients and their caregivers?
- Does the research address one or more of the key questions mentioned in PCORI's definition of patient-centered outcomes research?

Criterion 5. Patient and stakeholder engagement

- Are patients and other stakeholders engaged in:
 - Formulating research questions
 - Defining essential characteristics of study participants, comparators, and outcomes
 - Identifying and selecting outcomes that the population of interest notices and cares about (e.g., survival, function, symptoms, health-related quality of life) and that inform decision making relevant to the research topic
 - Monitoring study conduct and progress
 - Designing/suggesting plans for dissemination and implementation activities
- Are the roles and the decision making authority of all research partners clearly stated?
- Does the proposal demonstrate the principles of reciprocal relationships, co-learning, partnership, trust, transparency, and honesty?

PCORI Methodology Report



<http://www.pcori.org/assets/2013/11/PCORI-Methodology-Report.pdf>



Patient-Centered Outcomes Research Institute

Research must adhere to PCORI's Methodology Standards

47 standards are grouped into 11 categories, including:

1. Formulating Research Questions
2. Patient-Centeredness
3. Data Integrity and Rigorous Analyses
4. Preventing/Handling Missing Data
5. Heterogeneity of Treatment Effects
6. Data Networks
7. Data Registries
8. Adaptive and Bayesian Trial Designs
9. Causal Inference
10. Studies of Diagnostic Tests
11. Systematic Reviews

Methodology Standards

APPENDIX A: PCORI METHODOLOGY STANDARDS

Cross-Cutting Standards for PCOR

1: Standards for Formulating Research Questions

RQ-1 Identify gaps in evidence

Gap analysis and systematic reviews should be used to support the need for a proposed study. If a systematic review is not available, a systematic review should be performed using accepted standards in the field (see standard SR-1), or a strong rationale should be presented for proceeding without a systematic review. In the case where a systematic review is not possible, the methods used to review the literature should be explained and justified.

RQ-2 Develop a formal study protocol

Studies should include a formal protocol specifying at least one purpose for which the data were collected (e.g., effectiveness, safety, natural history of disease, quality improvement); data sources and linkage plans, if any; data feasibility and quality, measure(s) of effect; and use of any standardized data dictionaries (nationally or internationally accepted).

RQ-3 Identify specific populations and health decision(s) affected by the research

To produce information that is meaningful and useful to people when making specific health decisions, research proposals and protocols should describe: 1) the specific health decision the research is intended to inform; 2) the specific population for whom the health decision is pertinent; and 3) how study results will inform the health decision.

RQ-4 Identify and assess participant subgroups

In designing studies, researchers should identify participant subgroups of interest and, where feasible, design the study with adequate precision and power to reach conclusions specific to these subgroups. In addition, subgroup information should be reported for later systematic reviews.

Learn more about our portfolio

The screenshot shows the PCORI website homepage. At the top is a dark blue navigation bar with links for Home, Subscribe, Contact Us, and Search. Below this is the PCORI logo and the text "Patient-Centered Outcomes Research Institute". A secondary navigation bar contains links for About Us, Research We Support, Funding Opportunities (highlighted with a red box), Meetings & Events, Get Involved, News Room, and Blog. The main content area features a large image of a keyboard with a yellow envelope icon. Below the image is a call to action: "Keep Informed About PCORI Funding Announcements and Opportunities to Get Involved" with a "Join Our Mailing List" link. To the right, a "More News From PCORI" section lists three articles: "PCORI Board Approves 10 Members of New Advisory Panel on Clinical Trials", "PCORI Board Approves 13 Members of New Advisory Panel on Rare Disease", and "PCORI App Challenge Seeks 'Matchmaking Tools'".

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About Us | Research We Support | **Funding Opportunities** | Meetings & Events | Get Involved | News Room | Blog

Keep Informed About PCORI Funding Announcements and Opportunities to Get Involved
[Join Our Mailing List](#)

More News From PCORI

PCORI Board Approves 10 Members of New Advisory Panel on Clinical Trials
Members will provide expertise on the design and implementation of randomized clinical trials. [more](#) →

PCORI Board Approves 13 Members of New Advisory Panel on Rare Disease
Panelists will apply their expertise to advise on rare disease research priorities and community engagement. [more](#) →

PCORI App Challenge Seeks "Matchmaking Tools"
Challenge offers \$150,000 in prizes to spur development of tools to build research partnerships. [more](#) →

pcori.org

Learn more about our portfolio

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About Us | Research We Support | **Funding Opportunities** | Meetings & Events | Get Involved | News Room | Blog

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- Eugene Washington PCORI Engagement Awards
- PCORnet: The National Patient-Centered Clinical Research Network
- Other Programmatic Awards and Funding
- The PCORI Challenge
- Applicant Training

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LEARN MORE ▶

From PCORI

- ...ves 10 Members of New Advisory Panel
...e expertise on the design and randomized clinical trials. [more →](#)
- PCORI Board Approves 13 Members of New Advisory Panel on Rare Disease**
Panelists will apply their expertise to advise on rare disease research priorities and community engagement. [more →](#)
- PCORI App Challenge Seeks "Matchmaking Tools"**
Challenge offers \$150,000 in prizes to spur development of tools to build research partnerships. [more →](#)

Learn More About Our Portfolio



PCORI Funding Awards

Most of our primary research funding is awarded through PCORI Funding Announcements (PFAs), Research and Research Agenda. We also issue "targeted" announcements on specific high-priority areas. We have also funded a series of Pilot Projects to study methods for engaging stakeholders in research. In addition to our first Pipeline to Proposal awards; these are not research awards but are designed to better understand the research process.

All proposed projects, including requested budgets and project periods, are approved subject to a formal award contract.

VIEW FUNDING AWARDS: Use the map below to explore our awards; scroll down or use the filter (by Funding Announcement) Zoom in on the map for greater geographic detail or use the drop-down menus to filter awards to certain institutions or in the same general location.

Download Complete List of Awards (PDF):

Pilot Projects | Cycle I | Cycle II | Cycle III/Inaugural Improving Methods | August 2013 Cycle

Filter by

State/Province PCORI Research Priority Area

- All - Assessment of Prevention, Diagnosis, and Treatment Options

Funding Announcement Project Summary

Assessment of Prevention, Diagnosis, and Treatment Options Go Reset

Year Awarded	Project Title	Organization	State
2013	Sustainable Methods, Algorithms, and Research Tools for Delivering Optimal Care Study (SMART DOCS)	Stanford University School of Medicine	California
2013	Randomized Controlled Trial of a Patient Activation Tool in Pediatric Appendicitis	Research Institute Nationwide Children's Hospital	Ohio
2013	Treatment Preference and Patient Centered Prostate Cancer	University of Pennsylvania	Pennsylvania
2013	Comparative Effectiveness of a Decision Aid for Therapeutic Options in Sickle Cell Disease	University of Pittsburgh at Pittsburgh	Pennsylvania
2013	Patient Valued Comparative Effectiveness of Corticosteroids versus Anti-TNF Alpha Therapy for Inflammatory Bowel Disease	University of Pennsylvania	Pennsylvania
2013	Generating Critical Patient-Centered Information for Decision Making in Localized Prostate Cancer	Vanderbilt University	Tennessee
2013	Long Term Outcomes of Lumbar Epidural Steroid Injections for Spinal Stenosis	University of Washington	Washington
2013	Patient Participation Program for Pulmonary Fibrosis: Assessing the Effects of Supplemental Oxygen	National Jewish Health	Colorado
2013	Developmental Trajectories of Impairments, Health, and Participation of Children with Cerebral Palsy	University of Washington	Washington
2013	Researching the Effectiveness of a Decision-Support Tool for Adult Consumers with	Family Service Agency of San	California

pcori.org/pfaawards

Coming Soon – a new PCORI website

- Enhanced search and filter functions for awards database
- Ability to search/filter by conditions, populations, methods, stakeholder partners, and more
- Improved data visualization options
- Ability to display query results in chart, graph, and animated formats

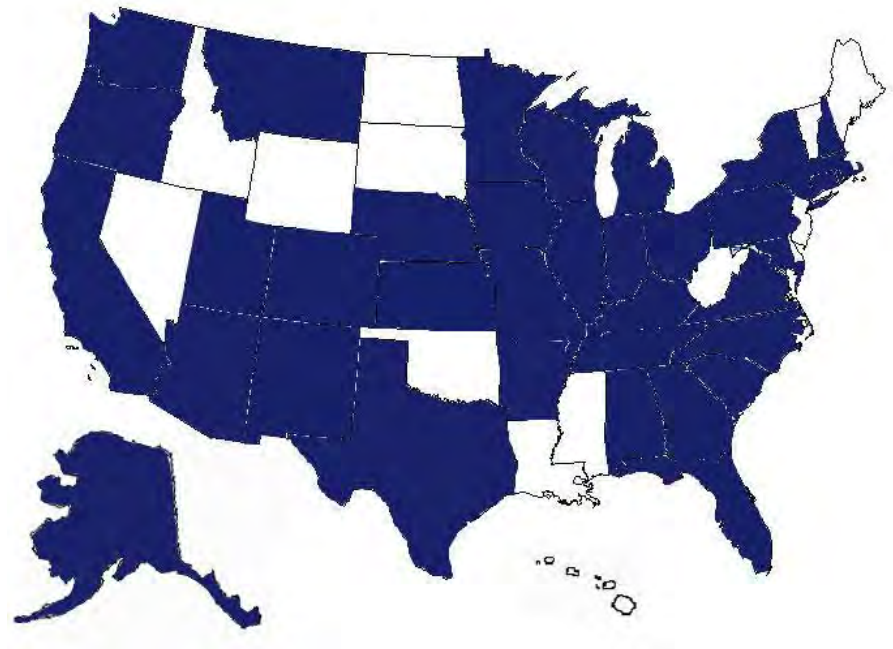
PCORI's Research Portfolio

Total number of research projects awarded : **279**

Total funds committed : **\$464.4 million**

Number of states where we are funding research:

37 states (including District of Columbia and Quebec, Canada)



The National Patient-Centered Clinical Research Network (PCORnet)

A “network of networks” to conduct large-scale, efficient CER.

- **Clinical Data Research Networks (n=11)** - System-based networks, such as hospital systems
- **Patient-Powered Research Networks (n=18)** - Patients with a single condition form a research network and participate in research.
- **A Coordinating Center** - Provides technical and logistical assistance under the direction of the Steering Committee and PCORI Staff.



Currently PCORI Funding Announcements

Fall 2014 Cycle

“Broad” PFAs (maximum total funding in this round):

- Assessment of Prevention, Diagnosis, and Treatment Options (\$32M)
- Improving Healthcare Systems (\$16M)
- Communication and Dissemination Research (\$8M)
- Addressing disparities (\$8M)
- Improving Methods for Conducting Patient-Centered Outcomes Research (\$12M)

LOIs due 9/5/14 → Applications due 11/4/14

Current Funding Announcement: Pragmatic Clinical Studies and Large Simple Trials to Evaluate Patient-Centered Outcomes

Objectives:

- Address critical clinical and health-related comparative effectiveness questions faced by patients, their caregivers, and their clinicians
- Test novel methodological approaches to seek answers to clinical questions in real-world environments efficiently and in a rigorous manner

PCORI seeks to fund:

- Pragmatic clinical trials
- Large simple trials
- Large scale observational studies

Available Funds and Duration:

- A total of \$90 million (direct + indirect) for this cycle
- Up to \$10 million in total direct costs per project
- Projects should be completed within 5 years

LOIs due 6/27/2014 → Application due 11/4/2014



Examples of high priority questions



- Treatment strategies for symptomatic osteoarthritis
 - Compare different nonsurgical therapies (pharmacotherapy, injections, physical therapy/exercise, weight loss alone and in combination with other therapies, complementary medicine alternatives)
- Treatment options for patients with multiple sclerosis
 - Compare management options for modifying disease progression. These might include FDA-approved disease modifying agents, behavioral interventions including exercise and physical therapy, and complementary medicine alternatives.
- PCORI-specified topics – see <http://www.pcori.org/assets/2014/02/PCORI-PFA-2014-Spring-Pragmatic-Studies.pdf>

Eugene Washington PCORI Engagement Awards

Awards of up to \$250,000 to provide “wrap-around” support and enhance impact of PCORI’s research initiatives.

Objectives

- Engage new groups who have not previously been involved with PCORI
- Develop new mechanisms for disseminating research findings
- Promote research done differently by supporting engagement and partnering in the conduct and usage of comparative effectiveness research

Three Types of Awards

- Knowledge
- Training and Development
- Dissemination and Implementation

Some common issues encountered in PCORI applications

- “Mind the gap!”
- Make sure you have a comparator.
- Address both benefits and harms.
- Compare existing clinical strategies.
- Describe “usual care.”
- What is “Patient-centeredness” vs. “Patient Engagement”?
- Include preliminary data.

“Mind the gap!”

- Document that there is a significant gap based on evidence reviews.
 - Identify important areas in which the new project will provide evidence that fills a well-defined evidence gap.
 - Lack of evidence is not a sufficient criterion.
- PCORI wants to fund impactful studies and avoid unnecessary redundancy.



Make sure you have a comparator

- Comparative effectiveness research is the conduct and synthesis of systematic research comparing different interventions and strategies to prevent, diagnose, treat and monitor health conditions. (FCCER)
- PCORI does not support natural history studies or efforts to create prediction models.



Address both benefits and harms

- “Comparative Effectiveness Research is the direct comparison of existing healthcare interventions to determine which interventions work best for which patients and which interventions pose the greatest benefits and harms.” (Definition from the PCORI funding guidelines)



Source: PFA application guidelines for Winter 2014

Compare existing clinical strategies

- Strategies being compared should each have known efficacy and/or be in use in clinical practice.
- PCORI funding is not the mechanism for determining if something works.



What is “Patient-centeredness” vs. “Patient Engagement”?

- **Patient-Centeredness** describes the degree to which the project answers questions or examines outcomes that matter to patients and caregivers.
- **Patient and (stakeholder) Engagement** describes the involvement of patients, stakeholders and scientists in all aspects of the research – from conception to execution to dissemination and implementation and *should facilitate dissemination*.

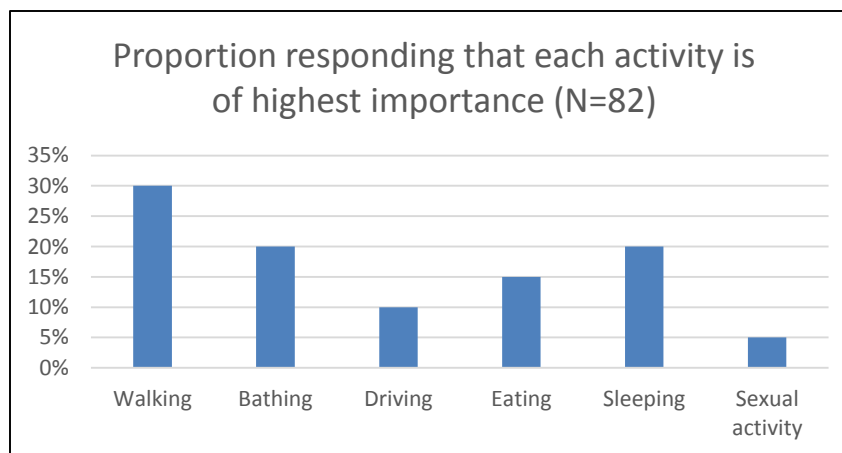
Describe “usual care”

- Usual care may be an appropriate comparator if it can be well-described.



Include preliminary data

- Include preliminary data necessary in your application to provide justification for your proposed project.
- Proposing to collect pilot data in your project suggests that a clinical strategy is “not ready for prime time” or that an important aspect of the study design is unknown.



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