



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options April 19-20, 2013 Meeting Summary

Overview

On April 19-20, 2013, PCORI convened the Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options in Alexandria, Virginia, for a two-day training and working session to help PCORI identify and prioritize research questions for potential funding. The panel included 21 members representing caregivers, patient-caregiver advocates, clinicians, researchers, organizational providers, and representatives from payers, industry, and purchasers.

Prior to the meeting, panelists received an orientation to PCORI's research prioritization process, describing the application of five criteria by which research topics should be assessed for their suitability and priority for PCORI funding, and research briefs describing the problem and state of current knowledge for twenty potential research topics (see Box 1, next page).

Over the course of the two-day meeting, panelists engaged in an open conversation about the merits of the research topics, sharing opinions about whether and why certain topics should be prioritized and funded by PCORI, and then ranked the topics using Expert Choice® and SurveyGizmo® software. By the end of the meeting, the panel reached consensus on the highest priority topics to submit to PCORI's Board of Governors for consideration for potential funding:

1. Bipolar Disorder
2. Ductal Carcinoma in situ
3. Migraine Headache
4. Osteoarthritis.

Related Information

- [About PCORI's Advisory Panels](#)
- [Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options](#)
- [Orientation to PCORI's Research Prioritization](#)
- [Assessment of Prevention, Diagnosis, and Treatment Options Research Briefs](#)

The Patient-Centered Outcomes Research Institute (PCORI) is an independent organization created to help people make informed healthcare decisions.

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Background

The [Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options](#)¹ was opened by PCORI Program Director David Hickam with an overview of how the research topics were nominated and winnowed down to the [20 topics referred to the panel for prioritization](#)² (see Box 1).

Box 1: Topics for Research Prioritization

Topic 1: Attention Deficit Hyperactivity Disorder (ADHD)	Topic 11: Generalized Anxiety Disorder (GAD)
Topic 2: Bipolar Disorder	Topic 12: Hearing loss
Topic 3: Hip fracture	Topic 13: Chronic Kidney Disease
Topic 4: Carotid Artery Disease	Topic 14: Treatments for liver cancer
Topic 5: Cerebral Adrenoleukodystrophy (ALD)	Topic 15: Macular Degeneration
Topic 6: Coronary Artery Disease	Topic 16: Melanoma
Topic 7: Ductal Carcinoma	Topic 17: Migraine headache
Topic 8: Gestational Diabetes	Topic 18: Multiple Sclerosis
Topic 9: Eczema	Topic 19: Obstructive Sleep Apnea
Topic 10: Epilepsy	Topic 20: Osteoarthritis

Discussion

Over the course of the two-day meeting, panel members discussed the research topics presented in the briefs with respect to the PCORI topic criteria. Key discussion points included:

Developing research tools and methods needed for comparative effectiveness research (CER). In narrowing down the topics to those of highest priority for PCORI, panelists realized that the final decisions on funding and study design will need to be made in consultation with the PCORI Methodology Committee. Depending on how PCORI interpreted its mandate, adjunctive non-CER research might or might not be fundable.

Topics related to other panels: healthcare systems, disparities, patient engagement. The panel members recognized that several of the research topics for their consideration also were relevant for the other advisory panels meeting concurrently. In its discussions of specific topics, the panel frequently focused on PCORI's role in disseminating information and educating patients because, for patients to be able to make optimal decisions, they need information on the benefits and harms of the available options.

¹ Available at pcori.org/get-involved/pcori-advisory-panels/advisory-panel-on-assessment-of-prevention-diagnosis-and-treatment-options

² Available at pcori.org/assets/PCORI-Assessment-of-Options-Topic-Brief-All-0418131.pdf

Shared decision making/personalized medicine. Across many topics, panel members noted that research to facilitate shared decision making was a “natural” for PCORI funding. In particular, such research should consider how patient choices and preferences would be reflected in the development of new research questions and hypotheses.

Chronic illness management. A recurring theme across many topics was effective management of chronic illness. Panel members viewed many topics as rooted in one or more chronic conditions that require attention over time and opined that a study in this area will bring value across topics.

Taking the topic “upstream” to a preventive intervention. Another recurring theme in the panel’s deliberations was attention to the precursors of the condition or disease presented in the research topic briefs. PCORI has already released a public funding announcement in obesity research.

Head-to-head trials and appropriate comparators. Panel members addressed important opportunities for PCORI to fund head-to-head trials that other funding agencies and commercial entities were unlikely to support. In particular, post-licensing trials of pharmaceuticals, trials involving devices and surgical procedures and techniques, and medical versus surgical treatment strategies are understudied.

Clarity and interpretation of PCORI’s criteria for prioritization of research topics. Panel members explicitly addressed PCORI’s five criteria for reviewing topics in their initial presentations and discussion of each one. They agreed on the following definition: “Potential for PCORI study to make a difference, to add to what we already know, and lend itself to CER research.”

Rare diseases. PCORI is committed to include studies of patients with rare conditions, as well as those with more common illnesses. Given the difficulty of considering rare diseases in the same context as highly prevalent conditions, the panel suggested that PCORI find another approach for prioritizing rare diseases.

Action

Using Expert Choice® and SurveyGizmo® software, the panel ranked the topics. The top four topics were ranked in the following order and will be recommended to PCORI’s Board of Governors as funding priorities:

1. **Bipolar Disorder**—Compare the effectiveness of medication regimens for adolescents and young adults with bipolar disorder.
2. **Ductal Carcinoma in situ**—Compare the effectiveness of management strategies for ductal carcinoma in situ among women who had this diagnosis made after undergoing screening mammography.

3. **Migraine Headache**—Compare the effectiveness of treatment strategies for adults with episodic and chronic migraine headaches.
4. **Osteoarthritis**—Compare the effectiveness of alternative strategies for stabilizing symptoms in people with osteoarthritis.

Next Steps

The advisory panel discussed issues that will need to be addressed at future meetings and teleconferences. Panel members suggested that this advisory panel hold a joint meeting with, or consult with, PCORI's Methodology Committee to better understand the kinds of studies that PCORI is able to fund and the Methodology Committee's own research agenda. The panel agreed to hold at least one conference call before the next meeting. Finally, Dr. Hickam invited nominations for the advisory panel chairperson.

—*Meeting summary prepared by David Hickam and Stanley Ip. Posted June 11, 2013 and available on [PCORI's website](#).*³

³ Available at pcori.org/get-involved/pcori-advisory-panels/advisory-panel-on-assessment-of-prevention-diagnosis-and-treatment-options