



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options Meeting Summary

June 2014

Overview

On April 28 and 29, 2014, the 21-member Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options convened in Alexandria, Virginia. The panel includes caregivers, patient-caregiver advocates, clinicians, researchers, organizational providers, and representatives from payers, industry and purchasers.

Before the meeting, panel members received topic briefs. After extensive discussion, panelists prioritized 16 clinical effectiveness research (CER) topics according to [PCORI's review criteria](#). The highest ranked topics: biologics for treatment of inflammatory bowel disease, treatment strategies for atrial fibrillation, and major depressive disorder.

The panel also prioritized six topics that [PCORnet](#) is considering for an interventional, individual-level clinical trial intended to explore how the national network might be used in future research. The highest ranked of those topics: optimal maintenance aspirin dose for patients with coronary artery disease, role of spacers (add-ons to inhalers) in asthma, and optimal second-line treatment for glycemic control in type 2 diabetes

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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Prioritization of Clinical Effectiveness Research Topics

After discussion, the panel prioritized 16 new research topics ([Group 1](#) and [Group 2](#)) for the Clinical Effectiveness Research (CER) program. The topics are listed below, in priority order, with key points from the panel's discussion.

1. Biologics for treatment of inflammatory bowel disease

Inflammatory bowel disease is an important clinical problem that has a significant impact on functioning and quality of life, affects a young population, and is increasing in incidence. Several types of treatments are used to reduce daily symptoms and acute exacerbations. An AHRQ future-research report identified areas for CER related to these treatments. Studies are needed that would compare step-up (progressive intensification of treatment as indicated) versus top-down (early introduction of intensive treatments) approaches. Other areas for CER include types of biologic treatments (e.g., injectable biologics versus infusion therapies), strategies for induction and maintenance treatments, and alternative treatment strategies, such as diet and lifestyle choices. In addition, CER could evaluate treatment doses, how to dose treatments over time, and shared decision making.

2. Treatment strategies for atrial fibrillation

There are many types of treatments that can reduce the risk of fatigue, shortness of breath, stroke, and early death from atrial fibrillation, a chronic heart-rhythm problem. Treatment choices and technologies are evolving quickly, and there's much uncertainty. The Institute of Medicine identified atrial fibrillation as an important topic for further study, and AHRQ recently conducted a systematic review. There are currently 60 studies listed in clinicaltrials.gov, including a large randomized, controlled trial (CABANA) being conducted by NIH that compares one catheter ablation therapy to physician-selected medication therapy. While this study has been slow in its progression due to difficulties with patient enrollment, it is expected to produce new evidence. The panel discussed options for additional research, such as randomized, controlled trials comparing different types of catheter ablation therapy, observational studies to examine subgroups and various treatments, treatment sequencing, and whether treatment effects vary by patient age.

3. Major depressive disorder

Major depressive disorder causes significant distress and interferes with a person's basic functioning. As a cause of disability, major depressive disorder ranks second of all diseases and injuries in the United States. There are many treatments and screening options available, and there have been many head-to-head comparisons. However, there is still a need for CER to clarify how to best match patient subgroups to the various types of treatment. The panel noted that further refinement and development of this topic may be necessary.

4. Mindfulness-based interventions

Mindfulness-based interventions are centered on daily meditation practice designed to improve coping skills. Although meditation is not frequently used to reduce symptoms of depression, anxiety, and chronic pain, there is moderate evidence—although the state of research is poor—that it can do so with little harm. Meditation is a relatively low-cost intervention that could improve outcomes for a large number of people. Research could determine whether meditation adds value to other treatments, such as pharmaceuticals, and determine the best populations to use meditation.

5. Management strategies for community-dwelling individuals with dementia

People with dementia often require a high level of caretaking, and various treatments have been proposed to reduce the caregiving burden. With limited pharmacological treatments for dementia, there are many gaps in the research, no practice guidelines, variation in outcomes from pharmacological treatments, and no quality measures. Also, there is no evidence on how to prevent or delay symptoms. CER could address the following: implementation of case-manager versus behavioral intervention; different family support models; and different models of pharmacological care, caregiver interventions, reducing caregiver burden, and behavioral interventions.

6. Renal replacement therapies

Chronic kidney disease is an increasingly prevalent condition, is associated with other conditions, and puts a huge economic burden on the healthcare system. There are two primary dialysis modes for people who require that treatment to stay alive but limited data about differences in outcomes. A randomized controlled trial is underway in China to compare the two treatments. Similar studies in the United States may be difficult to initiate because of strong patient and provider preferences. Additional research could clarify which symptoms and outcomes are particularly important to patients.

7. Behavioral interventions for posttraumatic stress disorder

Symptoms vary among patients with posttraumatic stress disorder (PTSD) because other psychological conditions can affect the symptoms. Because posttraumatic stress disorder (PTSD) is a risk factor for suicide, it is an important clinical problem to study. The stigma associated with PTSD may introduce barriers to patients receiving useful medical interventions. Research conducted on many of the available PTSD treatments has provided some evidence of the efficacy of treatment, and the findings have been incorporated into treatment guidelines. PTSD is an active research area within the Veterans Administration. Additional CER could examine which patients respond best to which type of treatment.

8. Treatment strategies for intermittent claudication

Intermittent claudication is defined as leg pain that occurs with exertion. It is common among patients with peripheral artery disease and can result in reduced functional capacity and quality of life, as well as increased morbidity and mortality. Treatment options include exercise, lifestyle modification,

medications, and surgical and other therapies. Increasingly prevalent, the condition affects a large portion of the population. IOM has identified intermittent claudication as a top priority for CER. There have been limited studies directly comparing current pharmacologic treatments. The panel suggested that PCORI fund CER on the impact of lifestyle changes and non-pharmacologic treatments and that studies include patient-centered outcomes, such as the effect of the disease on activities of daily living.

9. Nonsurgical treatment for cervical disc and neck pain

Cervical disc and neck pain is common, troublesome, and potentially debilitating. Treatment options for neck pain depend on its cause and timing, and providers tend to choose treatments within their specialty areas. The panel noted the need for better definitions of neck pain, pain severity, and patient subgroups. There are many high-cost therapy options, and the panel suggested a study of whether low-cost options are just as beneficial. Additional work may be needed (e.g., a systematic review) to further refine this topic.

10. Prevention and care in periodontal disease

Periodontal disease is the leading cause of tooth loss in older adults and contributes to chronic inflammation and other chronic conditions that affect general health. Few studies have provided evidence on the treatment options, which include medical management (e.g., antibiotics) and surgery. Prevention strategies, such as improving oral hygiene, might be a better focus of new research.

11. Treatment strategies for primary open-angle glaucoma

In open-angle glaucoma, there is a slow clogging of the drainage channels for aqueous humor in the eye. Treatment is focused on lowering intraocular pressure, the only known modifiable risk factor. Open-angle glaucoma is the leading cause of blindness in African Americans and Hispanics. African Americans are six to eight times as likely to become blind from glaucoma. There is no efficient means of screening and detection of glaucoma. Currently, there is limited insight into how to match the various treatments to patient subgroups. Much of the current research is focused on intermediary outcomes (e.g., intraocular pressure) rather than visual functioning. However, tracking visual functioning requires very long-term studies (e.g., more than 10 years).

12. Eye diseases

Common causes for vision loss are age-related macular degeneration and diabetic retinopathy. Many of the same treatments are used for these two diseases. New treatments have replaced older ones, but comparisons have not been done. However, such research may become quickly outdated as even newer drugs and technologies are introduced to the market. Studies of prevention may be a better focus for future research, such as how lifestyle (e.g., diet, smoking) affect long-term outcomes.

13. Imaging technologies in cancer

Imaging techniques, such as positron emission tomography (PET), are often used for surveillance following the initial treatment of cancer to detect early tumor recurrence and determine whether and where the tumor has spread. However, such post-treatment surveillance is controversial. It assumes that early identification of disease progression and early treatment will increase survival, and there is a concern about harms of overtreatment. Another issues with PET scans are inequalities in access to the care, including uneven geographical availability of machines. The panel suggested that basic research on sensitivity and specificity of the PET scan may be required before CER comparing the PET with simpler imaging modalities is worthwhile.

14. Strategies for detecting mild cognitive impairment

Mild cognitive impairment (MCI) does not interfere with daily activities, but it can worsen and eventually lead to dementia. There is a high rate of MCI in minority populations. Early diagnosis may permit the use of treatments to slow progression of the condition. Several different diagnostic tests have been proposed. New research could focus on screening and diagnosis efforts. Progressive functional decline and quality of life are major issues with the condition. More research needs to be conducted to determine whether it is beneficial to start drug treatment early; if a patient is stigmatized by having a brain condition, early diagnosis may be harmful.

15. Managing serious emotional disorders in children and teens

Various underlying psychiatric diagnoses lead to serious emotional disorders in children and teens. The overall goal of treatment is to improve symptoms, avoid institutionalization, and prepare the young person for life as an adult. The two approaches to treatment are residential care and wraparound treatments, a coordinated and collaborative effort between the young person and his or her family and important resources such as the school system and other community services (e.g., care managers, psychiatrists, pediatricians, psychologists, speech therapists). No systematic reviews have examined whether wraparound treatment is as effective as residential treatment for children and teens who need a high level of mental health services, and there are no good standards for defining the content of wraparound services. CER could compare traditional care models with this approach and examine required levels of training and determine which services are most important for care. The panel noted that caregiver burden adds another layer of complexity.

16. Concussion management

Concussion is a prevalent form of traumatic brain injury (TBI). Better definitions of injury severity, as well as treatment options, would be useful prior to conducting additional comparative research.

Prioritization of PCORnet Research Topics

To explore how PCORnet might be used in future research, it plans to support an interventional, individual-level clinical trial intended to generate evidence that is directly meaningful to patients, clinicians, and health systems. Potential study topics for this first trial were solicited from the networks that make up PCORnet. [Six of the submitted research topics](#) were deemed operationally feasible. After discussion, the panel prioritized the topics as listed below. Key discussion points are described.

1. Optimal maintenance aspirin dose for patients with coronary artery disease

Despite widespread use of aspirin in patients with coronary artery disease, the optimal dose is still debated. PCORnet access to the large numbers of data records necessary to conduct a trial to determine the question of relative efficacy and safety -of high- and low-dose aspirin. Panelists discussed policy significance, study sample size, and length of follow-up

2. Role of spacers in asthma

Difficulty in properly administering medication for asthma is a barrier to disease management. Spacers—add-ons to inhalers that help patients receive the appropriate dose—are often misused. A PCORnet study might implement an educational intervention to improve adherence to proper medication use, and consequently patient outcomes. Panel members suggested that such a study would be easy to implement, provide outcomes quickly, and have low-risk design.

3. Optimal second-line treatment for glycemic control in type 2 diabetes

Diabetes represents a leading cause of hospital admissions and healthcare system costs. Metformin is considered the gold-standard, first-line treatment for type 2 diabetes. Though there are many competing agents for second-line treatment, guidelines are fractured and treatment patterns vary considerably. NIH has sponsored a seven-year study (called GRADE), currently in recruitment, comparing glycemic control among four classes of second-line treatment. PCORnet may be able to augment GRADE findings with real-world results, though the design and implementation of such a study may be complex.

4. Stroke prevention in nonvalvular atrial fibrillation

Real-world comparative effectiveness of the newer anticoagulants to prevent stroke among people with nonvalvular atrial fibrillation has not been well-studied. The newer drugs are underprescribed and may carry different health risks than their older counterparts, such as warfarin. The panel discussed how to determine the risk-benefit profile of the newer drugs, compared to each other, in a pragmatic setting where adherence is of significant concern.

5. Interventions to maximize and maintain weight loss after bariatric surgery

Many patients do not maintain weight loss after bariatric surgery. Practitioners have few options with proven results to recommend, resulting in low patient follow-up and ultimately weight gain. The proposed study would compare the effects of three web-based behavioral interventions on weight loss maintenance in patients after bariatric surgery. Panel members discussed the possibility of testing more intensive interventions and noted that success may depend on frequency of use of the behavioral tools.

6. Mindfulness-based weight reduction using a simple web-based training

Obesity is one of the strongest challenges in medicine today. Nonsurgical and non-pharmacologic approaches are needed. Mindfulness practice is being increasingly used to allow people to overcome uncomfortable sensations, for example teaching ways to handle stress without eating. The study, using a web-based video to teach techniques of mindfulness aimed at combatting overeating, would measure weight loss. Panelists discussed concerns over the design and impact of this particular approach and noted a need for further development of proposals in this area.

Next Steps

Prioritization recommendations from this Advisory Panel and the PCORnet member networks, in the case of the PCORnet research topics, will be taken to the PCORI Board of Governors for review. The next Advisory Panel meeting will be held in Fall 2014 in Washington, DC.