



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

Overview

On September 12, 2014, the 21-member Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options convened in Washington, DC for their fifth in-person meeting. The panel includes caregivers, patient-caregiver advocates, clinicians, researchers, organizational providers, and representatives from payers, industry, and purchasers.

The panelists met to prioritize 10 new Clinical Effectiveness Research topics against [PCORI's Topic Brief and Advisory Panel review criteria](#), which include patient-centeredness, impact of the condition on the health of individuals and populations, assessment of current options, likelihood of implementation in practice, and durability of information.

The panel was provided with briefs for each topic prior to the meeting. After extensive discussion of each topic, panelists prioritized the 10 topics against PCORI's research criteria and selected a subset of topics for further consideration to be included in future PCORI Funding Announcements (PFAs).

CER Topics for Research Prioritization Reviewed at September 12, 2014 Meeting

Topic 1: Anesthesia options for orthopedic procedures
Topic 2: Exercise and physical therapy for tendinopathies
Topic 3: Treatment options for acute venous thromboembolism
Topic 4: Implantable cardioverter-defibrillators (ICD) in elderly
Topic 5: Imaging tests for the evaluation of cognitive decline
Topic 6: Statin therapy for prevention of atherosclerotic disease
Topic 7: Genetic testing for rare diseases among children
Topic 8: Treatment options for Sjögren's syndrome
Topic 9: Eye disease
Topic 10: Mindfulness-based interventions

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 Topics

After discussion, the panel prioritized [10 new research topics](#) for the Clinical Effectiveness Research program. Key discussion points are described below each topic.

1. Anesthesia Options for Orthopedic Procedures

In orthopedic procedures, there are two major categories of anesthesia application: general anesthesia applied through inhalation or intravenous injection, and regional anesthesia performed as a single injection or with a catheter. There were 41.4 million inpatient knee, hip, and shoulder surgeries performed in the US in 2010. An estimated 724 anesthesia complications occurred per 1,000 surgical discharges in the US in 2000. Due to the high volume of these cases in orthopedic procedures, the differential impact of anesthetic approaches on patient-centered outcomes (length of stay, operative time, readmissions, complications, and need for more post-discharge care) can have a high impact on society due to the high volume of these cases, especially in knee, hip, and shoulder surgeries. Systematic reviews reached inconsistent and conflicting conclusions. The few existing studies are of poor quality, lack patient-centered outcomes, or have limited data on follow-up/long-term outcomes.

Panelists agreed that the topic is patient-centered, would help with decision making for patients, impacts a large portion of population, is likely to be implemented in practice, and information would be durable. Clarification is needed on whether the focus is intraoperative management or post-operative pain control.

2. Exercise and Physical Therapy for Tendinopathies

Overuse injuries are common among physically active individuals, and account for 7 percent of all physician visits in the US. Tendinopathy is a syndrome characterized by pain, focal tenderness, and decreased strength within or around a tendon. Treatments include rest, medications for inflammation and pain, rehabilitation, and when rehabilitation is not successful, surgery. Rehabilitation activities aim to restore activity and function. There is little existing evidence or ongoing research comparing physical therapy with home-based therapy, and the comparative effectiveness of home exercise versus formal physical therapy is unknown. Panelists noted that tendinopathies and ankle sprains are different conditions, and tendinopathies are more common and chronic. The panel suggested that PCORI split these conditions into two topics. The type of treatment received is influenced by insurance, geographic location, and severity of injury. There is insufficient evidence on the optimal treatment for either ailment. Finally, the details about content of formal physical therapy and home exercise must be defined.

3. Treatment Options for Acute Venous Thromboembolism (VTE)

Venous thromboembolism (VTE) includes deep venous thrombosis (DVT) and pulmonary embolism (PE). A patient presenting with an episode of VTE needs to be treated rapidly to prevent complications, including death. Treatment options include the use of anticoagulants (e.g., warfarin, heparin) and inferior vena cava (IVC) filters, which are used for patients who present with a contraindication to anticoagulation. There have not been head-to-head trials that have compared IVC filters with anticoagulation for treatment of DVT, and patients are rarely asked for their preferences regarding anticoagulation, permanent filter placement, or retrievable filter placement. Panelists noted that putting a filter in early may cost more but reduce long-term cost over time, by reducing burdens associated with long-term anticoagulation. Filters are used as escalation of treatment (need to study the appropriateness of escalating the treatment beyond anticoagulants). Research is needed on

comorbidities, patient characteristics, prior incidents of VTE, and other risk factors; treatment is often based on this information. Observational studies may also be useful.

4. Implantable Cardioverter-Defibrillators (ICD) in Elderly

ICD is a device consisting of a generator and one or more leads capable of sensing a ventricular arrhythmia, which then delivers an electric shock to terminate the arrhythmia. Sudden cardiac death (SCD) accounts for approximately 300,000 deaths annually; three-quarters are caused by ventricular tachyarrhythmia, in which ICDs could play an important role. Available management options include antiarrhythmic drugs, ICD, and CRT-D. Over 500,000 Medicare beneficiaries are eligible for ICD therapy and over 140,000 ICDs are implanted annually in the US; the rate of ICD implantation in the US is five times as high as the rate in other Western countries. There is an interest in focusing on treatment heterogeneity in patients with different baseline risk for SCD, and comparative effectiveness of ICDs versus other treatment modalities in older patients and in patients with multiple comorbidities.

5. Imaging Tests for the Evaluation of Cognitive Decline

Neurocognitive decline refers to a broad category of brain diseases that cause long-term memory loss, inability to perform daily functions, and behavior changes. Provisional diagnosis can be made clinically, but only post-mortem examinations can confirm presence/absence of disease. Use of functional imaging has the potential to assist with the accurate diagnosis and characterization of the extent of cognitive decline. However, panelists shared concerns about the lack of standardization and accuracy of diagnostic modalities, poor test-retest reliability, and variation in the interpretation of MRIs. There are also potential ethical issues, including unclear benefits (e.g., preparation for end-of-life care) and harms (e.g., reduced functioning). Studies should differentiate between the different anatomic conditions that lead to dementia. Research could also look at how knowing the diagnosis improves or diminishes functioning in patients. It was noted that patients and caregivers may want early diagnosis even in the absence of available effective treatment. Research that resulted in more accurate diagnosis of neurocognitive decline could enhance clinicians' and patients' abilities to make plans to address issues that result from these diseases. However, basic science studies might be more appropriate than comparative effectiveness research at this time.

6. Statin Therapy for Prevention of Atherosclerotic Disease in Patients Age 70 or Older

Atherosclerosis is a disease in which plaque builds up inside the arteries, leading to serious problems including cardiovascular disease (CVD), heart attack, stroke, or even death. Greater utilization of statins by elderly individuals without previous history of CVD has the potential to prevent cardiovascular and cerebrovascular (stroke) events. However, current guidelines do not recommend initiating statin treatment in elderly individuals who have not had previous CVD. Statins have the potential for negative impacts on quality of life and other patient-related outcomes related to side effects. Alternative methods for the primary prevention of CVD resulting from atherosclerosis could result in significant individual and societal healthcare cost savings, and improvement in elderly individuals' quality of life. Primary prevention through alternative approaches such as diet modification, physical activity, and smoking cessation has had limited population-level effects compared to statin therapy. A targeted randomized controlled trial (RCT) studying statin-mediated primary prevention of atherosclerotic disease, specifically focusing on the elderly, could provide definitive evidence for or against the initiation of statin therapy in this age group.

7. Genetic Testing for Rare Diseases among Children

“Genetic testing” refers to tests seeking to confirm a particular diagnosis; these tests are performed when there is a suspicion for a genetic disorder based on screening results, family history, ethnicity, and physical findings. Early diagnosis of a genetic disease may direct a person toward available prevention, monitoring, and treatment options that can alter their disease course and prognosis. Assessment of the incidence and prevalence of some genetic diseases may be complicated by two issues: incomplete penetrance (patients may not develop the symptoms of a genetic disease even with inheritance of a disease-causing mutation) and variable expressivity, where a genetic disease may manifest with different symptoms in different patients. New research could contribute to achieving better patient-centered outcomes by clarifying the relative benefits and risks associated with genetic testing, and clarifying the impact and appropriateness.

8. Treatment Options for Sjögren’s Syndrome

Sjögren’s syndrome is a chronic autoimmune disease in which white blood cells attack moisture-producing glands. Hallmark symptoms include severe dry mouth and dry eye; however, this syndrome may cause additional symptoms such as dysfunction of the liver, kidneys, and gastrointestinal area. One recent systematic review reported a prevalence range of .21 percent to 2.7 percent for primary Sjögren’s syndrome. Currently, there is no known cure for this disease, and it can be resistant to treatment. Current treatment is limited to easing symptoms. There is little evidence that compares the benefits and harms of different types of treatments, or combinations of treatments, to manage the symptoms or fetal complications of Sjögren’s syndrome. A recent review of the literature showed that there are approximately 26 ongoing meta-analyses and RCTs, only five of which look at biologic agents. However, these are newer studies, and details are lacking. New research could significantly contribute to better patient-centered outcomes that target treatment-resistant symptoms such as fatigue and chronic pain, and use of therapies during pregnancy to reduce fetal complications.

9. Alternative Screening Options for Open-Angle Glaucoma

Open-angle glaucoma, the most common form of glaucoma, is characterized by progressive peripheral visual field loss (tunnel vision) and is usually associated with elevated pressure in the eye (intraocular pressure). In the US, 2.8 million people are living with open-angle glaucoma and the number will increase to 3.4 million by 2020. It is the leading cause of blindness; however, only 50 percent to 75 percent of people with glaucoma are aware they have it. Glaucoma is 6.6 times as frequent in African Americans. It has a major impact on patient quality of life. There is insufficient to limited evidence on the relative benefits and harms of screening for primary open-angle glaucoma in adults. Additionally, there is a lack of consensus on how and when to screen for glaucoma. New research could provide evidence to support several issues: clarify the role of early detection of glaucoma on patient-reported vision issues; provide a screening tool that optimizes patient-reported vision issues; describe how the effects of screening differ by key subgroups, optimal sequencing of screening tools, how and where screening should be conducted, and if screening is found to be effective, inform the methods for identifying and engaging patients early in the glaucoma disease process. Panelists agreed that there is a need for a screening technique that is portable and does not require dilation, which could be combined with other screening.

10. Treatment Options for Hepatitis C

Treatment for HCV is complicated and is dependent upon several factors: the current stage of liver disease, HCV genotype, extra-hepatic manifestations, anticipated adverse effects, patient preferences, and previous treatments attempted. Treatments currently available for HCV include IFN, Ribavirin-oral antiviral nucleoside analog, Simeprevir/Boceprevir (DAAs)-selective HCV protease inhibitors for genotype 1, and Sofosbuvir (DAA) nucleotide polymerase inhibitor (brand name Sovaldi) for genotypes 1-6. Newer regimens are much better tolerated than traditional IFN-based regimens, and could have a profound impact on patient-centered outcomes. Although treatment options continue to evolve, it is likely that new information regarding antiviral treatment and effects on patient-centered outcomes in different populations would remain relevant for years.

Next Steps

The next Advisory Panel meeting will be held in January 2015 in Washington, DC.