

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

March 2015

Overview

On January 13, 2015, the PCORI Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options convened in Washington, DC to review three previously prioritized Clinical Effectiveness Research topics.

The Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options is made up of 21 representatives of patients, caregivers, patient advocates, clinicians, researchers, industry, and policy makers. The panel was joined by PCORI leadership, staff, and research topic experts. The meeting was open to the public via teleconference, and slides and meeting materials were posted to the website in advance of the sessions.

The panel was provided with briefs for each topic prior to the meeting. After extensive discussion of each topic, panelists prioritized a subset of comparative effectiveness research questions for further consideration to be included in future PCORI Funding Announcements (PFAs).

CER Topics for Research Topic Refinement Reviewed at January 13, 2015 Meeting:

- Topic 1: Genetic Testing in Children in Whom a Rare Genetic Disease is Suspected
- Topic 2: Implantable Cardioverter Defibrillator Therapy in Older Patients
- Topic 3: Mindfulness-Based Interventions for the Treatment of Anxiety, Depression, and Pain

Related Information

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

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

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Introduction

The Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options reviewed three specific clinical research areas, with the input of topic experts, with the aim of formulating a subset of specific questions for further consideration as priority research areas. All three topics had been previously highly prioritized, but further topic refinement was needed. The areas discussed include genetic testing in children in whom a rare genetic disease is suspected, implantable cardioverter defibrillator therapy in older adults, and mindfulness-based interventions for the treatment of anxiety, depression, and pain.¹

Genetic Testing in Children in Whom a Rare Genetic Disease is Suspected

At its September 2014 meeting, the advisory panel met to prioritize 10 new Clinical Effectiveness Research topics. One of the topics discussed was “What Is the Comparative Effectiveness of Genetic Testing among Children in Whom a Rare Disease Is Suspected?” “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. Panelists prioritized this topic highly and noted that new research could contribute to achieving better patient-centered outcomes by clarifying the relative benefits and risks associated with genetic testing. More discussion was needed in order to better clarify the impact and appropriateness of such testing.

In January 2015, at the panel’s sixth face-to-face meeting, the panel was joined by the members of PCORI’s [Advisory Panel on Rare Disease](#) in order to engage in meaningful dialogue regarding how to best frame comparative effectiveness research questions for this topic. Panelists discussed current issues such as: 1) gaps in patient family experiences, providers’ strategies for supporting patients, shared decision making, and patient-centered outcomes of genetic decision making; 2) medical, legal, ethical issues inherent in “rare” disease genetic testing; 3) value and evidence of genetic testing to patients and providers 4) lack of standard reporting mechanism for testing results increased the amount of difficulty in interpretation and application of results; and 5) cost of testing, access to testing, and the window of treatment opportunity.

Topic Experts

- *Genetic Testing in Rare Disease*
 - Marshall Summar, MD
 - Uday Deshmukh, MD, MPH, CPE, FACP
- *ICDs in Older Patients*
 - Gillian D. Sanders-Schmidler, PhD
 - Sana Al-Khatib MD, MHS
- *Mindfulness-based Interventions*
 - Madhav Goyal, MD, MHS
 - Regina Dehen, ND, LAc

¹ Available at <http://www.pcori.org/sites/default/files/PCORI-APDTO-Advisory-Panel-Topic-Briefs-011315.pdf>

Panelists decided the topic needed further scoping and parameterizing. A small joint task group, comprised of members of the Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options and the Advisory Panel on Rare Disease, will be convened to create and refine questions.

Implantable Cardioverter Defibrillator Therapy in Older Patients

The implantable cardioverter defibrillator (ICD) is a battery-operated 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. It is the first-line treatment for the secondary prevention of sudden cardiac death (SCD) in patients with prior events due to suspected ventricular tachycardia (VT) and ventricular fibrillation (VF). Other treatments for VT/VF included anti-arrhythmic drugs, ablation by surgery or by catheter, and cardiac transplant.

ICD therapy in older patients was a topic that was initially discussed by the panelists during the September 2014 in-person meeting. Panelists were interested in focusing on treatment heterogeneity in patients with different baseline risk for sudden cardiac death (SCD), and comparative effectiveness of ICDs versus other treatment modalities in older patients and in patients with multiple comorbidities.

During the January 2015 in-person meeting, panelists discussed current gaps, such as: 1) lack of elderly representation in early trials for ICDs; 2) a need to look at outcomes beyond survival, such as inappropriate shocks, limitations on MRIs, end-of-life issues, and deactivation; 3) disparities on referrals in urban and rural areas based on age and race; 4) lack of sufficient risk and benefits data to address all important comorbidities; and 5) need more access to patient perspective concerning safety, preference, and effectiveness; and 6) the necessity of a decision model that addresses ICD versus no ICD.

Panelists prioritized a subset of questions but decided that there is a need for further scoping and parameterizing. A small joint task group, comprised of members of the Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options and PCORI Staff, will be convened to refine these questions.

Mindfulness-Based Interventions for the Treatment of Anxiety, Depression, and Pain

Mindfulness-based interventions are programs centered on daily mindfulness meditation practice designed to improve coping skills. Depression, anxiety, and chronic pain are often treated with pharmaceuticals, and meditation is not frequently used. However, there is moderate evidence that meditation can reduce symptoms with little harm. Initially prioritized during the April 2014 in-person meeting, the panelists noted that research could be useful to determine the best populations to use meditation in as an adjunctive approach to other treatments.

During the January 2015 in-person meeting, panelists agreed that mindfulness-based interventions hold promise, as they are inexpensive and safe. But research in this field is in its infancy, interventions are diverse, outcomes for anxiety, depression, and chronic pain are diverse, and designs of future studies need to be enhanced with rigor. Panelists discussed current issues, such as: 1) defining and measuring mindfulness; 2) training and sustainability of mindfulness-based interventions; 3) effectiveness of mindfulness-based interventions regarding mode of delivery, dose, frequency, and patient practice; 4) feasibility of randomization for a study that assessed behavioral versus medical interventions; and 5) misalignment between stakeholder goals and existing research concerning the use of mindfulness-based interventions.

Panelists prioritized a subset of questions but decided that there is a need for further scoping and parameterizing. A small joint task group, comprised of members of the Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options and PCORI Staff, will be convened to refine these questions.

Next Steps

- The Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment will work with the Advisory Panel on Rare Disease to form a subcommittee to shape specific comparative effectiveness research questions for:
 - Genetic Testing in Children in Whom a Rare Genetic Disease is Suspected
- The Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options will work with PCORI staff to form two separate subcommittees to sculpt specific comparative effectiveness research questions for:
 - Implantable Cardioverter Defibrillator Therapy in Older Patients
 - Mindfulness-Based Interventions for the Treatment of Anxiety, Depression, and Pain
- The panel will next convene for a series of webinars in April 2015 and the next in-person meeting will be held in July 2015.