



Welcome

**Please be seated by 9:50.
The teleconference will go live at 10:00.**

Patient-Centered Outcomes Research Institute



Assessment of Prevention, Diagnosis, and Treatment Options

Advisory Panel Meeting

April 28 – 29th, 2014

Patient-Centered Outcomes Research Institute

Welcome and Introductions: 10:00 am – 10:15 am



David Hickam, MD, MPH

Program Director
Clinical Effectiveness Research
PCORI

Housekeeping

- Today's teleconference is open to the public and is being recorded
 - Members of the public are invited to listen to this teleconference
 - Meeting materials can be found on the PCORI website
 - Comments may be submitted via email to advisorypanels@pcori.org; no public comment period is scheduled
- For those in the room, please remember to speak loudly and clearly into a microphone
- Where possible, we encourage you to avoid technical language in your discussion
- Reminder – please complete a new COI form

Welcome New Advisory Panel Member



Linda McNamara, RN, MBA

Healthcare Consultant, Author, Public Speaker
Representing: Patients, Caregivers, and Patient
Advocates

Advisory Panel Members



Duke Evidence Synthesis Group



Matthew J. Crowley, MD



Jennifer Gierisch, PhD

The Clinical Effectiveness Research Program Team



Diane Bild, MD, MPH



Jana-Lynn Louis, MPH



David Hickam, MD, MPH



Raymond Lockett



Julie McCormack, MA



Sandi Myers



Stanley Ip, MD



Hal Sox, MD



Patient-Centered Outcomes Research Institute

Background and Status of Previous Topics:

10:15 am – 10:45 am



David Hickam, MD, MPH

Program Director
Clinical Effectiveness Research
PCORI

Assessment of Prevention, Diagnosis and Treatment Options

Broad PFAs

4 cycles

- 65 projects totaling \$117M

Targeted PFAs

- Uterine Fibroids \$20M

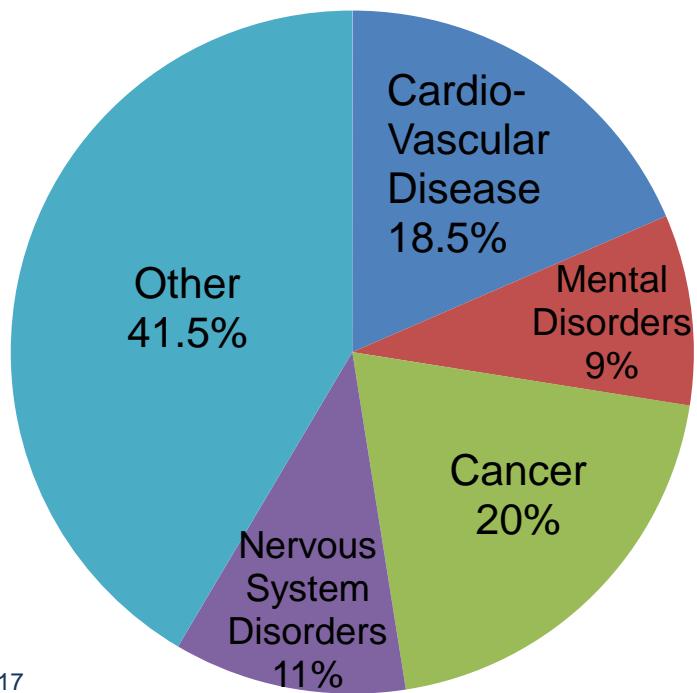
2014 Pipeline



Assessment of Prevention, Diagnosis and Treatment Options

Comparative studies of critical decisions that patients, their caregivers, and clinicians face with too little information.

By Primary Health Topic

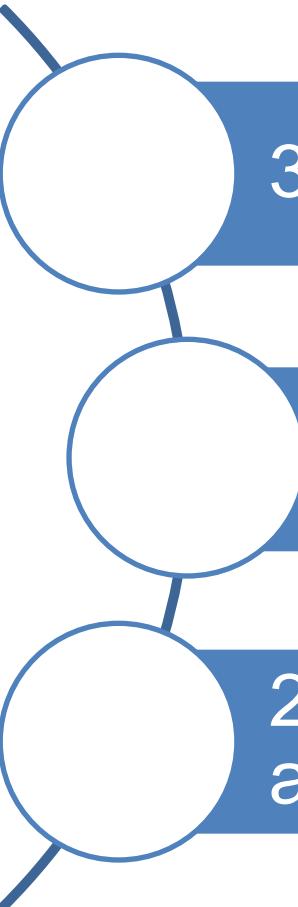


65 Awards
\$117 Million

How We Pick Research Questions to Study



Overview of CER Topics



34 Topics Reviewed by AO Advisory Panel*

9 Topics included in Pragmatic Studies Announcement

200+ LOIs received (20% invited to submit application)

*The multiple sclerosis and hearing loss topics were prioritized at the April 2013 and January 2014 meeting

Status of Prioritized CER Topics

April 2013 Ranking

- 1. **Ductal Carcinoma in Situ**
- 2. **Osteoarthritis**
- 3. **Migraine Headache**
- 4. **Bipolar Disorder**
- 5. Chronic Kidney Disease
- 6. Multiple Sclerosis*
- 7. Coronary Artery Disease
- Attention Deficit Hyperactivity Disorder
- Hip Fracture
- Carotid Artery Disease
- Cerebral Adrenoleukodystrophy
- Gestational Diabetes
- Eczema
- Epilepsy
- Generalized Anxiety Disorder
- Hearing Loss*
- Liver Cancer
- Macular Degeneration
- Melanoma
- Obstructive Sleep Apnea

January 2014 Ranking

- 1. **Lung Cancer**
- 2. **Opioid Substance Abuse**
- 3. **Autism Spectrum Disorder**
- 4. **Multiple Sclerosis***
- 5. **Proton Beam Therapy**
- 6. Pelvic Floor Mesh Implants
- 7. Biomarker Testing
- 8. Psoriasis
- 9. Hearing Loss*
- 10. Hypercholesterolemia
- 11. Robotic Surgery for Urologic and Gynecologic Cancers
- 12. Mesh for the Management of Inguinal and Abdominal Hernia
- 13. Pemphigus Vulgaris
- 14. Arrhythrogenic Right Ventricular Dysplasia

April 2014 – To Be Ranked

- Topic 1: Atrial fibrillation
- Topic 2: Intermittent claudication
- Topic 3: Posttraumatic stress disorder
- Topic 4: Inflammatory bowel disease
- Topic 5: Major depressive disorders
- Topic 6: Cervical disc and neck pain
- Topic 7: Renal replacement therapies
- Topic 8: Imaging Technologies in Cancer
- Topic 9: Eye disease
- Topic 10: Mindful-based interventions
- Topic 11: Concussion Management
- Topic 12: Mild cognitive impairment
- Topic 13: Dementia
- Topic 14: Glaucoma
- Topic 15: Periodontal disease
- Topic 16: Emotional disorders

*Prioritized at April 2013 and January 2014 meeting

Next Steps for Remaining Topics

 Revisit any old topics

 Pathways for funding

- Add to Pragmatic Studies funding announcement, or
- Further development of topic via FRP report (e.g. migraine headache, bipolar, etc...)

Meeting Objectives and Overview of the Agenda: 10:45 am – 11:00 am



Alvin I. Mushlin, MD, ScM

Chair, Panel on the Assessment of Options

Chairman, Department of Public Health, Weill Cornell Medical College; Public Health Physician-in-Chief, New York Presbyterian Hospital/Weill Cornell Medical Center



Margaret F. Clayton, RN, PhD

Co-chair, Panel on the Assessment of Options

Associate Professor, College of Nursing and Co-Director of the PhD Program, University of Utah



pcori

Meeting Objectives

- Prioritize 16 new clinical topics for the Clinical Effectiveness Research program area and select a subset of topics for further consideration as research priority areas
- Prioritize 6 PCORnet interventional study topics for the CER Methods and Infrastructure program area

Agenda Overview – Monday

Time	Agenda Item
11:00 – 11:15 am	Break (group picture)
11:15 – 11:30 am	Procedures for Reviewing Topics and Voting
11:30 – 12:15 pm	Review Clinical Effectiveness Research Topics 1 - 3
12:15 – 1:00 pm	Lunch
1:00 – 3:15 pm	Review Clinical Effectiveness Research Topics 4 - 12
3:15 – 3:30 pm	Break
3:30 – 4:45 pm	Review Clinical Effectiveness Research Topics 13 - 16
4:45 pm	Adjourn
5:30 pm	Reception
6:30 pm	Dinner

Agenda Overview – Tuesday

Time	Agenda Item
8:30 – 9:30 am	Review Clinical Effectiveness Research Topics
9:30 – 10:00 am	Voting on Clinical Effectiveness Research Topics
10:00 – 10:15 am	Break
10:15 – 10:30 am	Introduction to PCORnet Interventional Study Topics
10:30 – 12:00 pm	Review PCORnet Interventional Study Topics 1 - 6
12:00 – 12:15 pm	Voting on PCORnet Interventional Study Topics
12:15 - 1:00 pm	Lunch
1:00 – 1:30 pm	Voting Results
1:30 pm	Adjourn



Break
11:00 am – 11:15 am

***Group photo –
Convention Level Foyer***

Patient-Centered Outcomes Research Institute

Procedures for Reviewing CER Topics and Voting: 11:15 am – 11:30 am

- Today: Review 16 clinical effectiveness research topics
 - 15 minutes per topic
 - One panelist will give a brief overview of topic (denoted with *)
 - Reviewers will comment
 - Open for discussion to entire panel
- Tomorrow: Topic Ranking
 - Brief review of Clinical Effectiveness Research topics
 - SurveyGizmo Ranking
 - Results

Topic 1: Treatment strategies for atrial fibrillation

- Karen Chesbrough
- Priti Jhingran
- Mark Johnson
- Angela Smith*

Topic 2: Treatment strategies for intermittent claudication

- Kathie Insel
- Denise Kruzikas
- Cynthia Mulrow
- Daniel Wall*

Topic 3: Behavioral interventions for posttraumatic stress disorder

- Regina Dehen
- Bettye Green
- Ronald Means
- James Pantelas*



Lunch
12:15 – 1:00 pm

Upper Lobby Foyer

Patient-Centered Outcomes Research Institute

Topic 4: Biologics for treatment of inflammatory bowel disease

- Regina Dehen
- Linda McNamara*
- Alan Rosenberg
- Daniel Wall

Topic 5: Major depressive disorders

- Debra Madden*
- Ronald Means
- Bruce Monte
- Marcia Rupnow

Topic 6: Nonsurgical treatment for cervical disc and neck pain

- Karen Chesbrough*
- Mark Johnson
- Denise Kruzikas
- Alan Rosenberg

Topic 7: Renal replacement therapies

- Bettye Green
- Priti Jhingran
- Cynthia Mulrow*
- Angela Smith

Topic 8: Imaging technologies in cancer

- Bettye Green*
- Priti Jhingran
- Angela Smith
- Seema Sonnad

Topic 9: Eye disease

- Debra Madden
- Ronald Means
- Bruce Monte
- Seema Sonnad*

Topic 10: Mindfulness-based interventions

- Regina Dehen*
- Sara Hohly
- Linda McNamara
- Seema Sonnad

Topic 11: Concussion management

- Sara Hohly
- Linda McNamara
- Alan Rosenberg*
- Seema Sonnad

Topic 12: Strategies for detecting mild cognitive impairment

 Karen Chesbrough

 Kathie Insel*

 Alan Rosenberg

 Marcia Rupnow



Break

3:15 pm – 3:30 pm

Patient-Centered Outcomes Research Institute

Topic 13: Management strategies for community-dwelling individuals with dementia

- Priti Jhingran*
- Debra Madden
- Ronald Means
- Cynthia Mulrow

Topic 14: Treatment strategies for primary open-angle glaucoma

- Mark Johnson*
- Marcia Rupnow
- Angela Smith
- Daniel Wall

Topic 15: Prevention and care in periodontal disease

- Regina Dehen
- Sara Hohly*
- Denise Kruzikas
- Linda McNamara

Topic 16: Managing serious emotional disorders in children and teens

- Karen Chesbrough
- Ronald Means*
- Cynthia Mulrow
- James Pantelas

Announcements & Adjourn: 4:45 pm

Tonight

- Reception at 5:30 pm – Upper Lobby Foyer
- Dinner at 6:30 pm – Upper Lobby Foyer

Tomorrow

- Breakfast at 8:00 am – Upper Lobby Foyer
- Meeting will begin at 8:30 am, **please be seated by 8:25 am**



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Patient-Centered Outcomes Research Institute

Agenda Overview

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8:30 – 9:30 am	Review Clinical Effectiveness Research Topics
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1:30 pm	Adjourn

Review of CER Topics: 8:30 am – 9:30 am

- Voting on the CER topics will begin at 9:30 am
- Prior to voting, 16 CER topics that were discussed yesterday will be reviewed
 - High level overview of each topic
 - Approximately 3-4 minutes per topic

Topic 1: Treatment strategies for atrial fibrillation

Heart rhythm problem, can cause fatigue, shortness of breath, higher rates of stroke and earlier death. There are treatments that can reduce the risk of these complications.

Key points from discussion:

- Most common rhythm abnormality, high mortality rate, and high cost
- IOM identified as important topic
- AHRQ recently conducted systematic reviews
- 60 studies in clinicaltrials.gov
 - CABANA trial (n=2000) of one catheter therapy compared to physician selected medication therapy, slow accrual and difficulty randomizing patients, 5 year follow-up planned

Topic 1: Treatment strategies for atrial fibrillation, continued

- Many treatments, new CER would help patients make decisions
- Randomized study could compare different types of catheter ablation therapy
 - Require many arms, enrollment/randomization could be an issue
- Large observational database could examine subgroups and various treatments
- Challenge for research would be which comparators to use and when intervention should occur (how to sequence therapy would be a good area to study)
- Treatment decisions based on age of patient
 - Effects of treatments may vary by age groups
 - Catheter ablation is costly and used more with younger patients
- Quickly evolving field with uncertainty around choices and technologies

Topic 2: Treatment strategies for intermittent claudication

Leg pain that occurs with exertion, caused by peripheral arterial disease (PAD). Can result in poorer functional capacity, worse quality of life, and higher morbidity and mortality.

Key points from discussion:

- ⌚ Prevalence is on the rise
- ⌚ IOM identified this areas as top priority for CER
- ⌚ Few studies have compared current treatment options, head to head comparisons are needed
- ⌚ Often associated with comorbid conditions, so a multifactorial approach may be needed
- ⌚ Few studies include patient centered outcomes
- ⌚ Evaluation of non-pharmacologic treatments could be option for PCORI

Topic 3: Behavioral interventions for post-traumatic stress disorder (PTSD)

PTSD is a chronic psychological disorder that causes somatic, cognitive, affective, and behavioral symptoms. PTSD is caused by psychological trauma that occurred in the past.

Key points from discussion:

- High value in exploring the correlation of suicide, the 10th leading cause of death, to PTSD
- Mental health stigma, cost of care, and legal issues are significant barriers to care that adversely affect patient enrollment in studies
- There are several evidence-based treatments, both pharmacological and counseling options
- There are evidence-based guidelines, calling into question the need for more CER.
- Central issue is heterogeneity of treatment effects: matching patients with the best treatment choice.



Topic 4: Biologics for treatment of inflammatory bowel disease

Two primary disorders: Ulcerative colitis (bloody diarrhea and abdominal pain/cramping), and Crohn's disease (abdominal pain, diarrhea, nausea, vomiting, and sometimes fever and weight loss). The treatments are used to reduce daily symptoms and prevent acute exacerbations.

Key points from discussion:

- Incidence of IBD is on the rise
- Impacts younger population (diagnosis typically in late teens/early 20s)
- Significant impact on QOL and work performance
- Costly disease
- AHRQ future research report identified areas where CER is needed (e.g. comparison of treatment of biologic and immuno-modulator combination versus biologic alone)

Topic 4: Biologics for treatment of inflammatory bowel disease, continued

- Several types of medications are used for treatment (biologics are most advantageous because reduce inflammation in targeted fashion)
 - No universal agreement in timing of treatments (step up versus top down approach)
 - Research needed on dose (no clinical guidelines on changing doses)
 - Comparisons of various treatment modalities (e.g. injectables, infusion therapies)
 - Clarification of treatment strategies for induction vs maintenance.
- Comparisons with complementary/alternative treatment strategies such as diet and lifestyle choices should be addressed (especially for UC)
- Shared decision making research is also needed

Topic 5: Major depressive disorder

Characterized by a depressed mood, diminished interest or pleasure, sleeping problems and tiredness, and negative thoughts; causes significant distress and interferes with a person's basic functioning.

Key points from discussion:

- Ranks second of all diseases and injuries in the United States.
- Leading cause of work-related disability.
- People with major depressive disorder (MDD) will keep working, but how effective are they while working?
- Many different treatments and screening options but not good comparators.
- Need better clarity on how to match patients to the multiple types of treatment.
- This topic needs further scoping, stakeholder input, and a landscape review.

Topic 6: Nonsurgical treatment for cervical disc and neck pain

Common, bothersome, and potentially debilitating problem that results from degeneration of the structures of the cervical spine. Options for addressing neck pain depend greatly on its cause and chronicity.

Key points from discussion:

- Lack of a concise definition of neck pain
- No clear correlation between pain and why patients decide to seek care.
- Need better definition of patient sub-groups.
- Providers tend to choose treatments within their specialty areas.
- Many high cost therapy options; and it may be efficacious to study if low cost options are just as beneficial
- May be an opportunity for PCORI to conduct new systematic review to inform public



Topic 7: Renal replacement therapies

Chronic kidney disease (CKD) is the presence of kidney damage or decreased kidney function; end-stage renal disease is the final stage of CKD when renal replacement therapy must be initiated to preserve life.

Key points from discussion:

- Prevalent condition, associated with comorbid conditions, huge economic burden to health care system
- Two primary dialysis modes (PD versus HD)
 - Variations in cost and reimbursement
 - Limited data about differences in outcomes
 - In US, RCT may be difficult because patients want to choose dialysis type (trial in Netherlands failed because patients wanted to choose dialysis type).
 - Current trial in China comparing PD and HD.

Topic 7: Renal replacement therapies, continued

- Could independently evaluate treatments, but would need to include health literacy component
- Not much research on what symptoms are important to patients
- Comparing PD to HD, or trying to facilitate uptake of PD as option (true comparator or shared decision making study)

Topic 8: Imaging technologies in cancer

Imaging techniques (PET, CT or MRI) are used for surveillance following the initial treatment of cancer to determine if and where the tumor has spread (metastasized) and to detect early tumor recurrence.

Key points from discussion:

- Positron emission tomography (PET) is generally well accepted for initial diagnosis and staging of cancers but more controversial in post-treatment surveillance.
- We make an assumption in the U.S. that more post-treatment surveillance is better and early treatment is better. We also assume this will increase survival. May be harm of over-treatment.
- The other problem with PET scans are the geographical locations: the availability of machines and access to the care. Also the appropriate use of PET scans (pro vs. con). Efficacy trials, focusing on comparative accuracy of PET scans vs. other post-treatment methods (but what would be the gold standard?).

Topic 9: Eye disease

Common causes for vision loss are age-related macular degeneration and diabetic retinopathy; vision loss in general can lead to loss of functioning, depression, and falls.

Key points from discussion:

- Comparison of treatments which have replaced older options.
- Studies of prevention may be more fruitful: how lifestyle (i.e. diet, non-smoking, etc.) affects outcomes.
- Difficult issue of which drugs would be compared in a new head to head trial.

Topic 10: Mindfulness-based interventions

Programs centered on daily mindfulness meditation practice designed to improve coping skills. Based on the principle that the practices lead to living in way that is more conscious, deliberate, kind, and compassionate and less reactive, automatic, and judgmental.

Key points from discussion:

- Depression, anxiety, and chronic pain are often treated with pharmaceuticals, meditation is not frequently used, however, moderately strong evidence that meditation can reduce symptoms with little harm
- New research findings might not be readily accepted by practitioners
- Meditation is a life-long skill that can improve overall health
- Research could be useful to determine best populations to use meditation in, and does meditation add value to other treatments
- State of research in this area is poor, and meditation is a low cost intervention that could improve outcomes for a large number of people



Topic 11: Concussion management

A form of traumatic brain injury that may occur when the head hits an object, or a moving object strikes the head; can lead to a bad headache, changes in alertness, or loss of consciousness. Recovery occurs over a period of days or weeks.

Key points from discussion:

- 250,000 U.S. children were treated in 2009 for sports and recreation-related injuries that included a diagnosis of concussion or TBI.
- Preventative possibility: should helmets be required for all sports?
- Lack of well accepted methods for classifying the severity of the injury.
- The treatment options need to be better defined before comparative research can be done.

Topic 12: Strategies for detecting mild cognitive impairment

A state of cognitive function below normal, but not severe enough to be classified as dementia, and does not interfere with daily activities. Progressive over time and can lead to dementia. Several different tests have been proposed to detect the condition. Earlier diagnosis may permit use of treatments that slow its progression.

Key points from discussion:

- Progressive functional decline and quality of life are major issues.
- Is early diagnosis a benefit or a harm?
- Question as to whether drug treatment may be more beneficial if started earlier.
- Focus needed on screening/diagnosis efforts and how to train providers.
- High rate of MCI in minority populations (i.e. The Manhattan Study) so there may be a link to disparities.

Topic 13: Management strategies for community-dwelling individuals with dementia

A set of symptoms “associated with decline in memory or other thinking skills severe enough to reduce a person’s ability to perform everyday activities.” People with dementia often require a high level of caretaking, and various treatments have been proposed to reduce the caregiving burden.

Key points from discussion:

- Age is primary risk factor, prevalence increases with age, limited pharmacological treatments
- Gaps in research: no practice guidelines, outcomes are varied with pharmacological treatments, no quality measures, don’t know how to prevent or delay symptoms
- CER could address:
 - Implementation of case manager versus behavioral intervention
 - Different family support models
 - Different models of pharmacological care
 - Caregiver interventions and reducing caregiver burden
 - Behavioral interventions

Topic 14: Treatment strategies for primary open-angle glaucoma

A subtype of glaucoma (elevated pressure inside the eye) in which the drainage channels for aqueous humor in the front of the eye are open; treatment is focused on lowering intraocular pressure, the only known effective treatment.

Key points from discussion:

- It is the leading cause of blindness in African Americans and Hispanics. African Americans are 6 – 8 times more likely to become blind from glaucoma.
- Major issue is screening/detection of glaucoma.
- Limited current insight on how to match treatments to patient sub-groups.
- Intermediary outcomes (intraocular pressure) are most commonly studied outcomes, rather than vision loss. Examining vision loss may require very long studies.

Topic 15: Prevention and care of periodontal disease

Chronic infection of the hard and soft tissue supporting the teeth. Leading cause of tooth loss in older adults and contributes to the pathogenesis of chronic inflammation and other chronic conditions that affect general health. Treatments include medical management and surgery

Key points from discussion:

- ⌚ Periodontal disease has been linked to many medical complications, but not many studies have provided evidence on the treatment options.
- ⌚ Prevention (improving oral hygiene) might be a better focus of new research.

Topic 16: Managing serious emotional disorders in children and teens

Complex behavioral problems, arising from emotional disorders, in children and teenagers. Many possible treatments; goal is to improve symptoms, avoid institutionalization and prepare them for life as adults.

Key points from discussion:

- The central issue is whether wrap around treatments are as good as residential treatment for children and teens with a high level of need for mental health services.
- No systematic reviews have been conducted.
- No good standards for defining the content of wrap around services.
- The underlying psychiatric diagnoses are variable, but the patients have the common trait of being severely disturbed.
- CER could look at comparisons with traditional care models with this approach, levels of training, which services are most important for care
- Caregiver burden adds another layer of complexity



Voting on CER Topics: 9:30 am – 10:00 am

- SurveyGizmo link has been emailed to panelists
- Drag and drop ranking method to prioritize 16 CER topics
 - Topics listed on left-hand side of screen – drag to right-hand side of screen with highest priority topic placed at the top of the list
- Comments can be added at the end of the ranking
- Please provide your name and click “submit” when completed



Break
10:00 am – 10:15 am

Patient-Centered Outcomes Research Institute



Introducing PCORnet: The National Patient-Centered Clinical Research Network

Rachael Fleurence, PhD

April 2014

Patient-Centered Outcomes Research Institute

This slide presentation explains:

- Why PCORnet was created
- What PCORnet will do for research
- How it works
- Who is involved

Our national clinical research system is well-intentioned but flawed

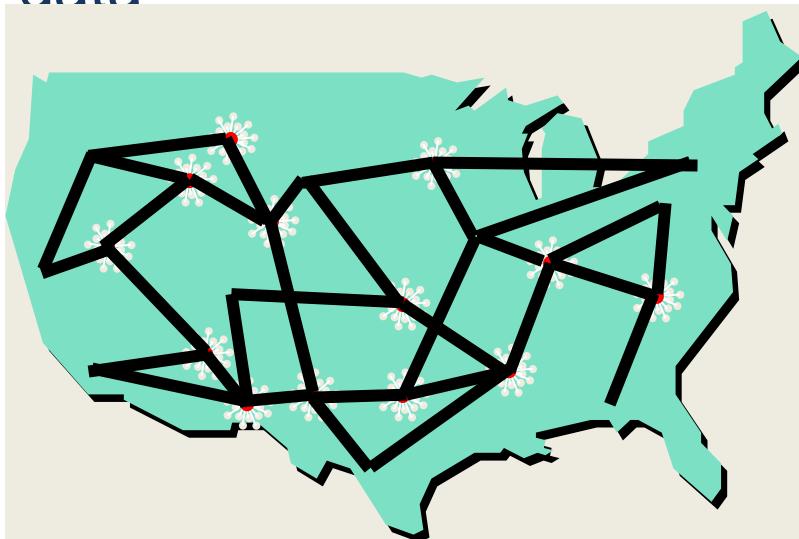
- High percentage of decisions not supported by evidence*
- Health outcomes and disparities are not improving
- Current system is great **except:**
 - Too slow
 - Too expensive
 - Unreliable
 - Doesn't answer questions that matter most to patients
 - Unattractive to clinicians & administrators

We are not generating the evidence we need to support the healthcare decisions that patients and their doctors have to make every day.

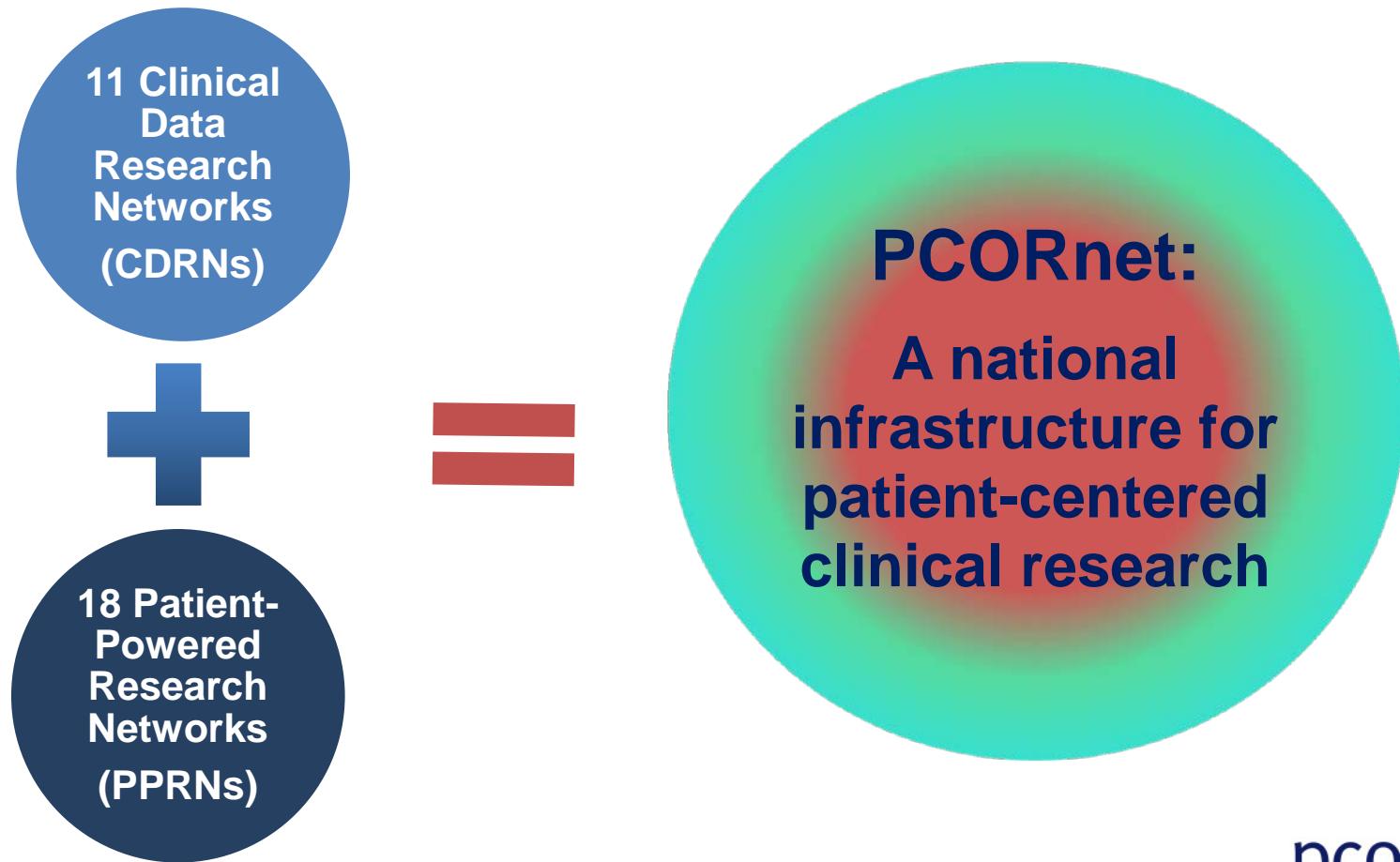
*Tricoci P et al. JAMA 2009;301:831-41.

Both researchers and funders now recognize the value in integrating clinical research networks

- Linking existing networks means clinical research can be conducted more effectively
- Ensures that patients, providers, and scientists form true “communities of research”
- Creates “interoperability” – networks can share sites and data



PCORnet embodies a “community of research” by uniting systems, patients & clinicians





What will PCORnet do for research?

Patient-Centered Outcomes Research Institute

PCORnet's goal



PCORnet seeks to improve the nation's capacity to conduct clinical research by creating a large, highly representative, national patient-centered network that supports more efficient clinical trials and observational studies.

PCORnet's vision

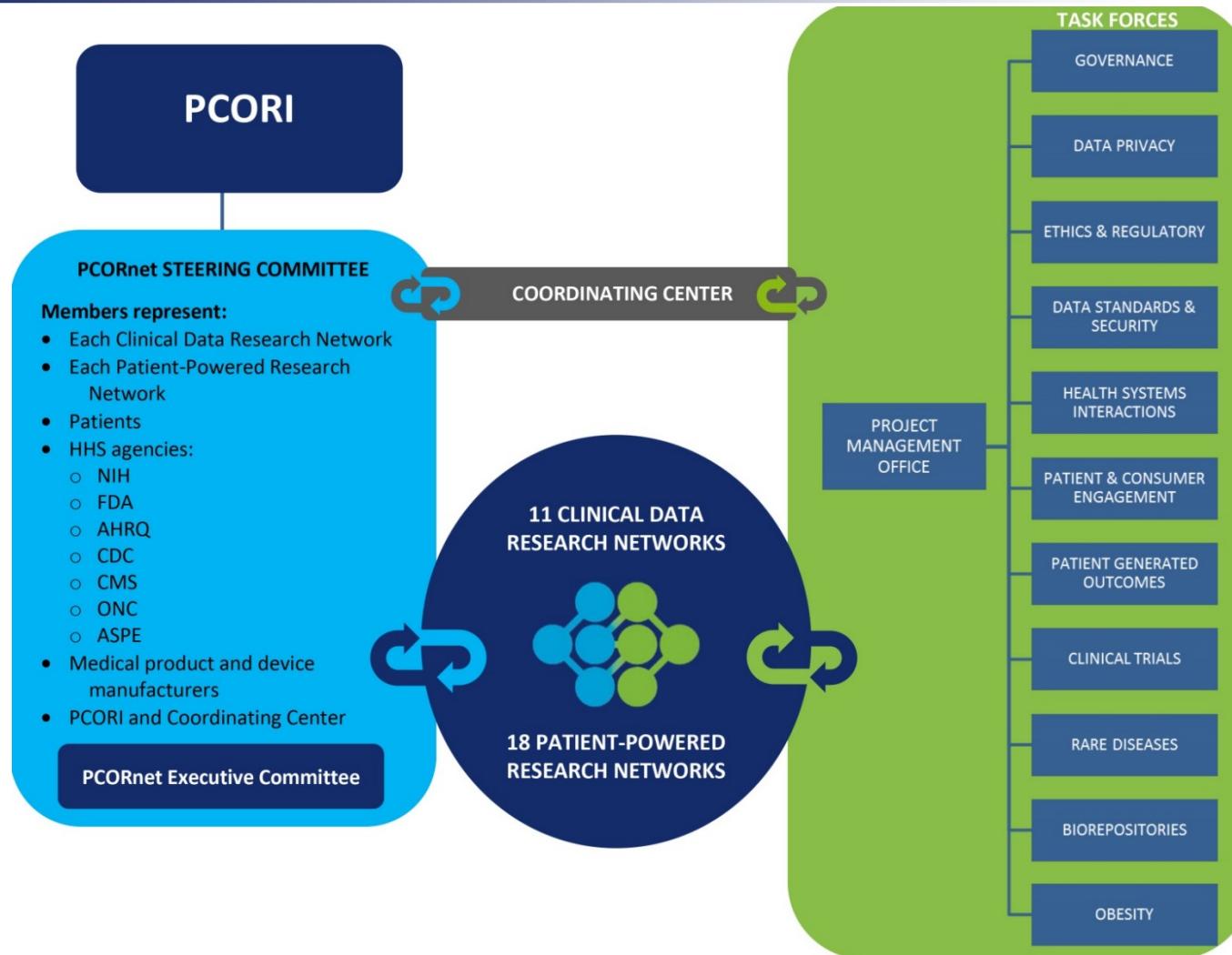
PCORnet will support widespread capability for the US healthcare system to learn from research, meaning that large-scale research can be conducted with greater speed and accuracy within real-world care delivery systems.



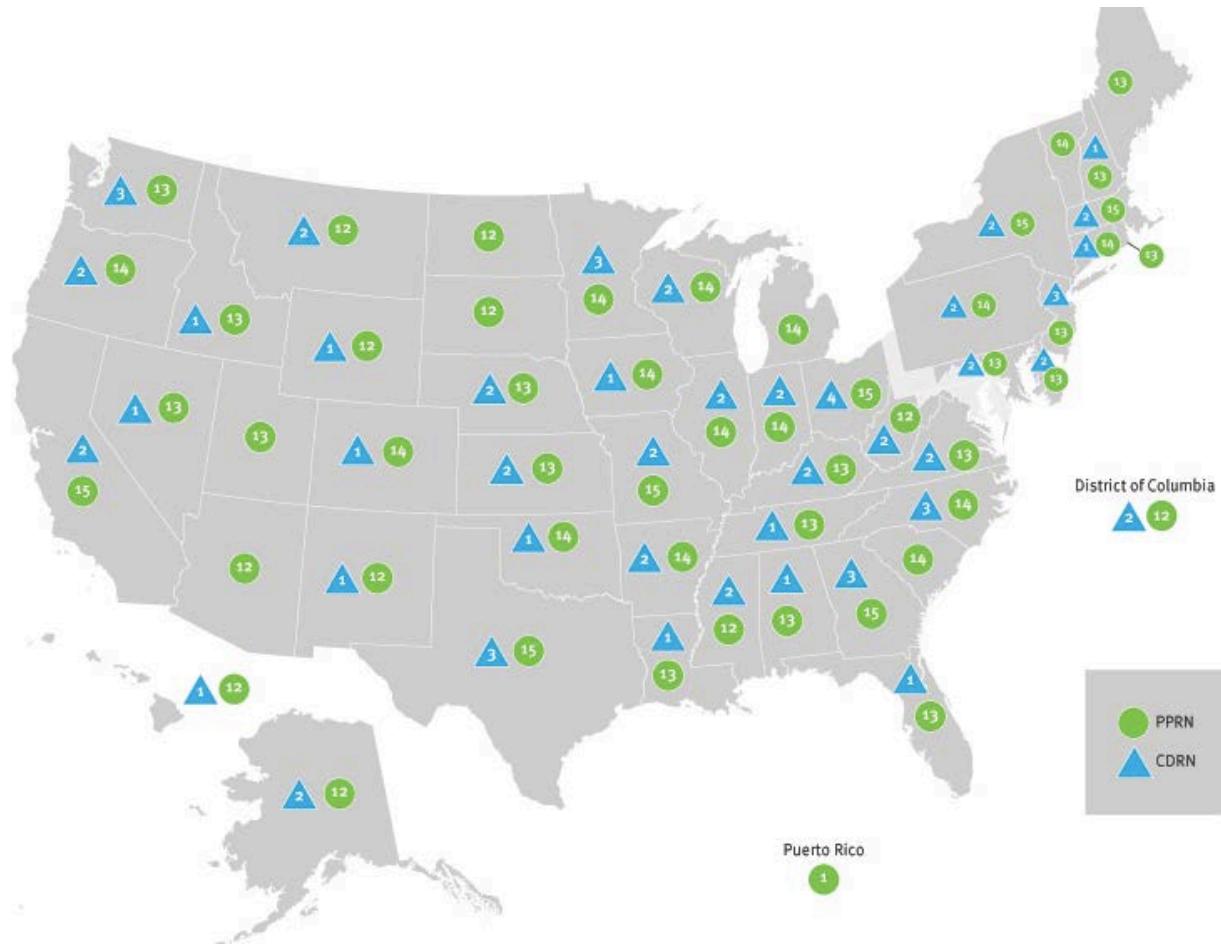
Overall objectives of PCORnet: achieving a single functional research network

- **Create** a secure national research resource that will enable teams of health researchers, patients, and their partners to work together on researching questions of shared interest.
- **Utilize** multiple rich data sources to support research, such as electronic health records, insurance claims data, and data reported directly by patients
- **Engage** patients, clinicians & health system leaders throughout the research cycle from idea generation to implementation
- **Support** observational and interventional research studies that compare how well different treatment options work for different people
- **Enable** external partners to collaborate with PCORI-funded networks
- **Sustain** PCORnet resources for a range of research activities supported by PCORI and other sponsors

PCORnet organizational structure



29 CDRN and PPRN awards were approved on December 17th by PCORI's Board of Governors



This map depicts the number of PCORI funded Patient-Powered or Clinical Data Research Networks that have coverage in each state.



CDRN Partners

Patient-Centered Outcomes Research Institute

Goals for Each Clinical Data Research Network (CDRN)

- Create a research-ready dataset of at least 1 million patients that is:
 - **Secure** and does not identify individual patients
 - **Comprehensive**, using data from EHRs to describe patients' care experience over time and in different care settings
- Involve patients, clinicians, and health system leaders in all aspects of creating and running the network
- Develop the ability to run a clinical trial in the participating systems that fits seamlessly into healthcare operations
- Identify at least 3 cohorts of patients who have a condition in common, and who can be characterized and surveyed

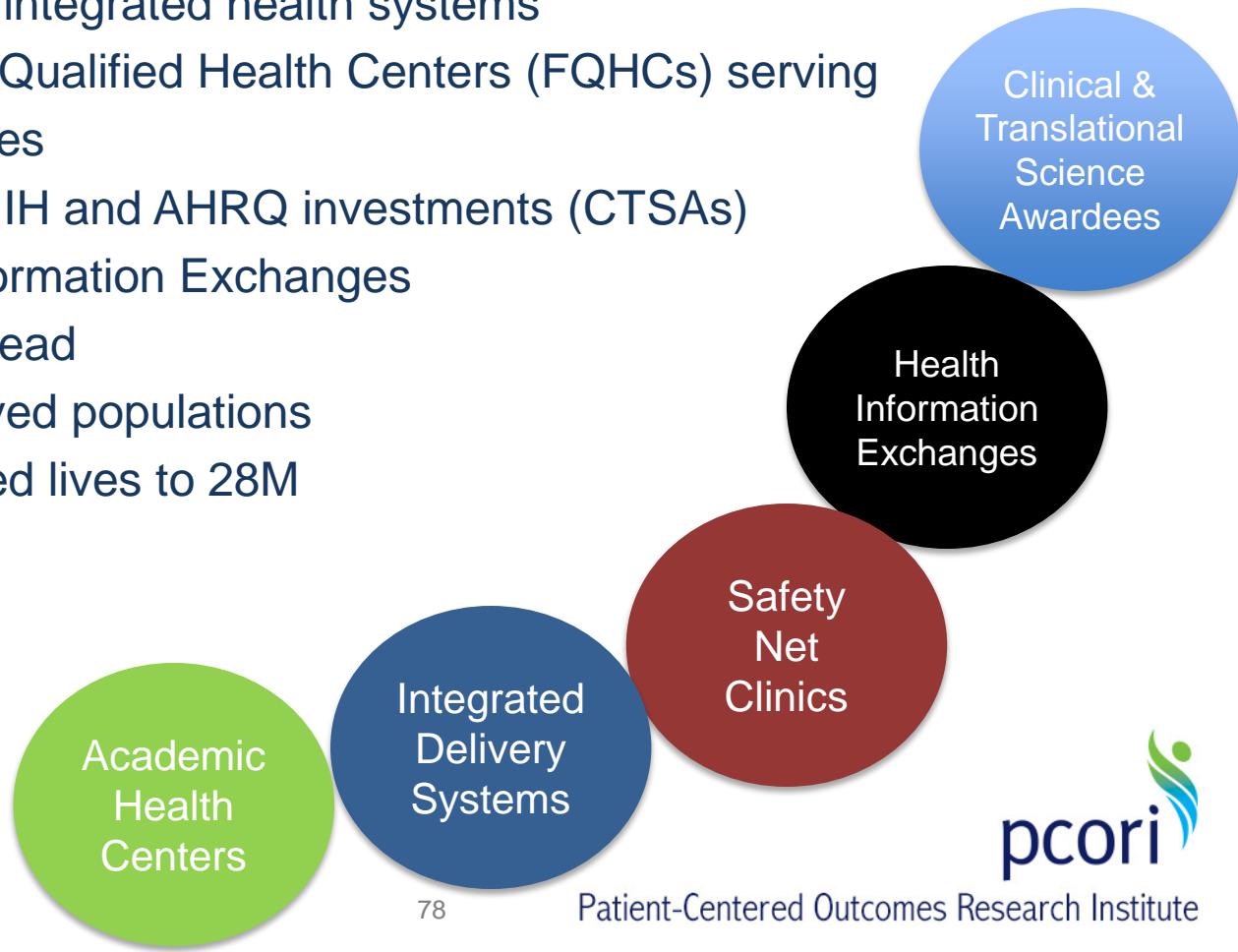


WiseGEEK



CDRN highlights

- Networks of academic health centers, hospitals & clinical practices
- Networks of non-profit integrated health systems
- Networks of Federally Qualified Health Centers (FQHCs) serving low-income communities
- Networks leveraging NIH and AHRQ investments (CTSAs)
- Inclusion of Health Information Exchanges
- Wide geographical spread
- Inclusion of under-served populations
- Range from 1M covered lives to 28M





PPRN Partners

Patient-Centered Outcomes Research Institute

Goals for each Patient-Powered Research Network (PPRN)

- Establish an activated patient population with a condition of interest (Size >50 patients for rare diseases; >50,000 for common conditions)
- Collect patient-reported data for ≥80% of patients in the network
- Involve patients in network governance
- Create standardized database suitable for sharing with other network members that can be used to respond to “queries” (ideas for possible research studies)



PPRN highlights

- Participating organizations and leadership teams include patients, advocacy groups, clinicians, academic centers, practice-based research networks
- Strong understanding of patient engagement
- Significant range of conditions and diseases
- Variety in populations represented (including pediatrics, under-served populations)
- 50% are focused on rare diseases
- Varying capabilities with respect to developing research data
- Several PPRNs have capacity to work with biospecimens

The PCORnet opportunity: making a real difference for patients and their families

Until now, we have been unable to answer many of the most important questions affecting health and healthcare

By combining the knowledge and insights of patients, caregivers, and researchers in a revolutionary network with carefully controlled access to rich sources of health data, we will be able to respond to patients' priorities and speed the creation of new knowledge to guide treatment on a national scale.

Early Opportunity to conduct a clinical trial within PCORNet

- Large, highly representative electronic data infrastructure to facilitate efficient research
 - Observational
 - Pragmatic randomized trials
- Officially launched Jan 2014 – early phase
- PCORI has identified a unique **early opportunity** to support an interventional **individual-level randomized clinical trial** that will inform future research studies in PCORnet

Process

Step 1

- Topic generation

Step 2

- Topic prioritization

Step 3

- PCORI Program Development Committee (PDC) & PCORI Board of Governors Approve Final Topic for the Clinical Trial

Step 4

- PCORI issues request Q2 2014

About the trial

- The trial should be characterized by **operational simplicity** and **clinical relevance**
- The trial will make extensive use of **EHR** to identify patients and report outcomes
- The study will complete in no more than **18 months** with a total cost of \$10 million
- PCORI has received **6 viable topics** from the network for prioritization



Prioritization

- Each topic author has submitted detailed topic briefs in a standard template
- Topics will be presented by Ad-board members assigned to review each brief
- Discussion of topics will follow utilizing the **5 PCORI prioritization criteria**, with an eye towards the technical limitations/goals of PCORnet
- A final **ranking 1-6** of each topic will be submitted by advisory panel members



The Topics

- What is the optimal second line treatment for glycemic control in type 2 diabetes?
- What is the role of spacers for treatment of Asthma?
- Comparative effectiveness of anticoagulants for atrial fibrillation
- Randomized clinical trial to determine optimal maintenance aspirin dose for patients with coronary artery disease
- Comparative effectiveness of interventions to maximize and maintain weight loss after bariatric surgery
- Mindfulness-based weight reduction using a simple web-based training



Prioritization Criteria

Patient-Centeredness: Is the proposed research focused on questions and outcomes of specific interest to patients, their caregivers, and clinicians?

Impact of the Condition on the Health of Individuals and Populations: Is the condition or disease associated with a significant burden in the US population? Burden of disease may be measured by how many people have the disease (prevalence); how many new cases occur every year (incidence); and other measures such as mortality, morbidity, individual suffering, and loss of productivity. PCORI is also interested in rare diseases.

Assessment of Current Options: What current guidance is available on the topic, and is there ongoing research? How does this help determine whether further research in this area would be valuable?

Likelihood of Implementation in Practice: Would new information generated by this research be likely to have an impact in practice? Research is valuable when it leads to the use of more beneficial treatments or interventions. Providing new evidence by itself does not ensure that the results of research will be used in clinical practice. Several factors may influence uptake in practice, including the knowledge and behaviors of health professionals, the acceptability to patients and to health systems.

Durability of Information: Would new information on this topic remain current for several years, or would it be rendered obsolete quickly by subsequent studies? Durability is generally associated with the rate at which new clinical evidence and/or better alternatives for patient management are emerging. Durability might be limited when there are rapid modifications to procedures and techniques. This commonly occurs in domains such as medical device development.

Feasibility of the clinical trial within health systems*. Does this trial meet the requisite technical, governance and regulatory constraints of the trial?

*new criterion added for the purposes of identifying a topic suitable for this particular opportunity

Prioritization

- Each topic author has submitted detailed topic briefs in a standard template
- Topics will be presented by Ad-board members assigned to review each brief
- Discussion of topics will follow utilizing the **5 PCORI prioritization criteria**, with an eye towards the technical limitations/goals of PCORnet
- A final **ranking 1-6** of each topic will be submitted by advisory panel members

Procedures for Reviewing PCORnet Topics and Voting

Review 6 PCORnet research topics

- 15 minutes per topic
- One panelist will give a brief overview of topic (denoted with *)
- Reviewers will comment
- Open for discussion to entire panel

Topic Ranking

- SurveyGizmo Ranking
- Results

Topic 1: Stroke prevention in nonvalvular atrial fibrillation

- Bettye Green
- Kathie Insel*
- Debra Madden
- Seema Sonnad

Topic 2: Role of spacers in asthma

- Bettye Green
- Priti Jhingran
- Alan Rosenberg*
- Daniel Wall

Topic 3: Optimal maintenance aspirin dose for patients with coronary artery disease

- Cynthia Mulrow*
- James Pantelas
- Marcia Rupnow
- Daniel Wall

Topic 4: Web-based training for weight reduction

- Sara Hohly
- Debra Madden
- Bruce Monte*
- Angela Smith

Topic 5: Interventions to maximize and maintain weight loss after bariatric surgery

- Karen Chesbrough
- Regina Dehen
- Mark Johnson*
- Linda McNamara

Topic 6: Optimal second line treatment for glycemic control in type 2 diabetes

- Kathie Insel
- Bruce Monte
- James Pantelas
- Marcia Rupnow*

Voting on PCORnet Interventional Study

Topics: 12:00 pm – 12:15 pm

- SurveyGizmo link has been emailed to panelists
- Drag and drop ranking method to prioritize 6 PCORnet topics
 - Topics listed on left-hand side of screen – drag to right-hand side of screen with highest priority topic placed at the top of the list
- Comments can be added at the end of the ranking
- Please provide your name and click “submit” when completed



Lunch
12:15 pm – 1:00 pm

Patient-Centered Outcomes Research Institute



Voting Results

1:00 pm – 1:30 pm

Patient-Centered Outcomes Research Institute

Ranking Results – CER Topics (n = 21)

Item	Total Score ¹	Overall Rank
Topic 4: Biologics for treatment of inflammatory bowel disease	264	1
Topic 1: Treatment strategies for atrial fibrillation	240	2
Topic 5: Major depressive disorders	218	3
Topic 10: Mindfulness-based interventions	215	4
Topic 13: Management strategies for community-dwelling individuals with dementia	199	5
Topic 7: Renal Replacement Therapies	198	6
Topic 3: Behavioral interventions for posttraumatic stress disorder	192	7
Topic 2: Treatment strategies for intermittent claudication	183	8

¹ Score is a weighted calculation. Items ranked first are valued higher than the following ranks, the score is the sum of all weighted rank counts.

Ranking Results – CER Topics (n = 21)

Item	Total Score ¹	Overall Rank
Topic 6: Nonsurgical treatment for cervical disc and neck pain	169	9
Topic 15: Prevention and care in periodontal disease	167	10
Topic 14: Treatment strategies for primary open-angle glaucoma	162	11
Topic 9: Eye disease	151	12
Topic 8: Imaging technologies in cancer	145	13
Topic 12: Strategies for detecting mild cognitive impairment	139	14
Topic 16: Managing serious emotional disorders in children and teens	120	15
Topic 11: Concussion management	94	16

¹ Score is a weighted calculation. Items ranked first are valued higher than the following ranks, the score is the sum of all weighted rank counts.

Ranking Results – PCORnet (n = 19)

Item	Total Score ¹	Overall Rank
Topic 3: Optimal maintenance aspirin dose for patients with coronary artery disease	100	1
Topic 2: Role of spacers in asthma	96	2
Topic 6: Optimal second line treatment for glycemic control in type 2 diabetes	66	3
Topic 1: Stroke prevention in nonvalvular atrial fibrillation	65	4
Topic 5: Interventions to maximize and maintain weight loss after bariatric surgery	44	5
Topic 4: Mindfulness-based weight reduction using a simple web-based training	28	6

¹ Score is a weighted calculation. Items ranked first are valued higher than the following ranks, the score is the sum of all weighted rank counts.

Next Steps and Adjourn: 1:30 pm

- Next in-person meeting tentatively scheduled for the fall in Washington, DC (date and location TBD)
- Reminder: Please complete the Post Event Survey



Thank you for your participation.

Patient-Centered Outcomes Research Institute