



**Welcome**

**Please be seated by 8:20 a.m.**  
**The teleconference will go live at 8:30 a.m.**

Patient-Centered Outcomes Research Institute



# **Assessment of Prevention, Diagnosis, and Treatment Options**

## **Advisory Panel Meeting**

**September 12, 2014**

Patient-Centered Outcomes Research Institute

# Welcome and Introductions



## 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](mailto: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

# Advisory Panel Members



*Not pictured: Sara Hohly, Denise Kruzikas*



# Clinical Effectiveness Research Team



Diane Bild, MD, MPH



Hal Sox, MD



David Hickam, MD, MPH



Stanley Ip, MD



Julie McCormack, MA



Sandi Myers



Jana-Lynn Louis, MPH



Katie Hughes, MA



Jackie Dillard

# Advisory Panel Chairs



## **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*

# Meeting Objectives

- Summarize the current Clinical Effectiveness Research portfolio
- Prioritize 10 new clinical topics for the Clinical Effectiveness Research program area and select a subset of topics for further consideration as priority research areas



# Agenda Overview

Time	Agenda Item
9 – 9:45 a.m.	CER Portfolio Presentation
9:45 – 10:15 a.m.	Background and Status of Previous Topics
10:15 – 10:30 a.m.	Procedures for Reviewing Topics and Voting
10:30 – 10:45 a.m.	Break
10:45 – 12 p.m.	Review Clinical Effectiveness Research Topics 1 - 5
12 – 1 p.m.	Lunch
1 – 2:15 p.m.	Review Clinical Effectiveness Research Topics 6 - 10
2:15 – 2:45 p.m.	Review and Voting CER Topics
2:45 – 3 p.m.	Break
3 – 3:15 p.m.	Voting Results
3:15 – 3:30 p.m.	Announcements and Next Steps
3:30 p.m.	Adjourn

# CER Portfolio Presentation



## **Diane Bild, MD, MPH**

Senior Program Officer  
Clinical Effectiveness Research  
PCORI



# Clinical Effectiveness Research Program Portfolio

*Diane Bild, MD, MPH  
David Hickam, MD, MPH  
CER Advisory Panel  
September 12, 2014*

Patient-Centered Outcomes Research Institute

# Outline

- Goals of the Program
- Basic portfolio description
- “Deeper dive”
  - Clinical trials
  - Observational studies
  - Specific examples
- Assessment
- Discussion

# PCORI's Mandate. . .

PUBLIC LAW 111-148—MAR. 23, 2010

“The purpose of the Institute is to assist patients, clinicians, purchasers, and policy-makers in making **informed health decisions** by advancing the quality and relevance of **evidence** concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be **prevented, diagnosed, treated, monitored, and managed** through research and evidence synthesis ...”



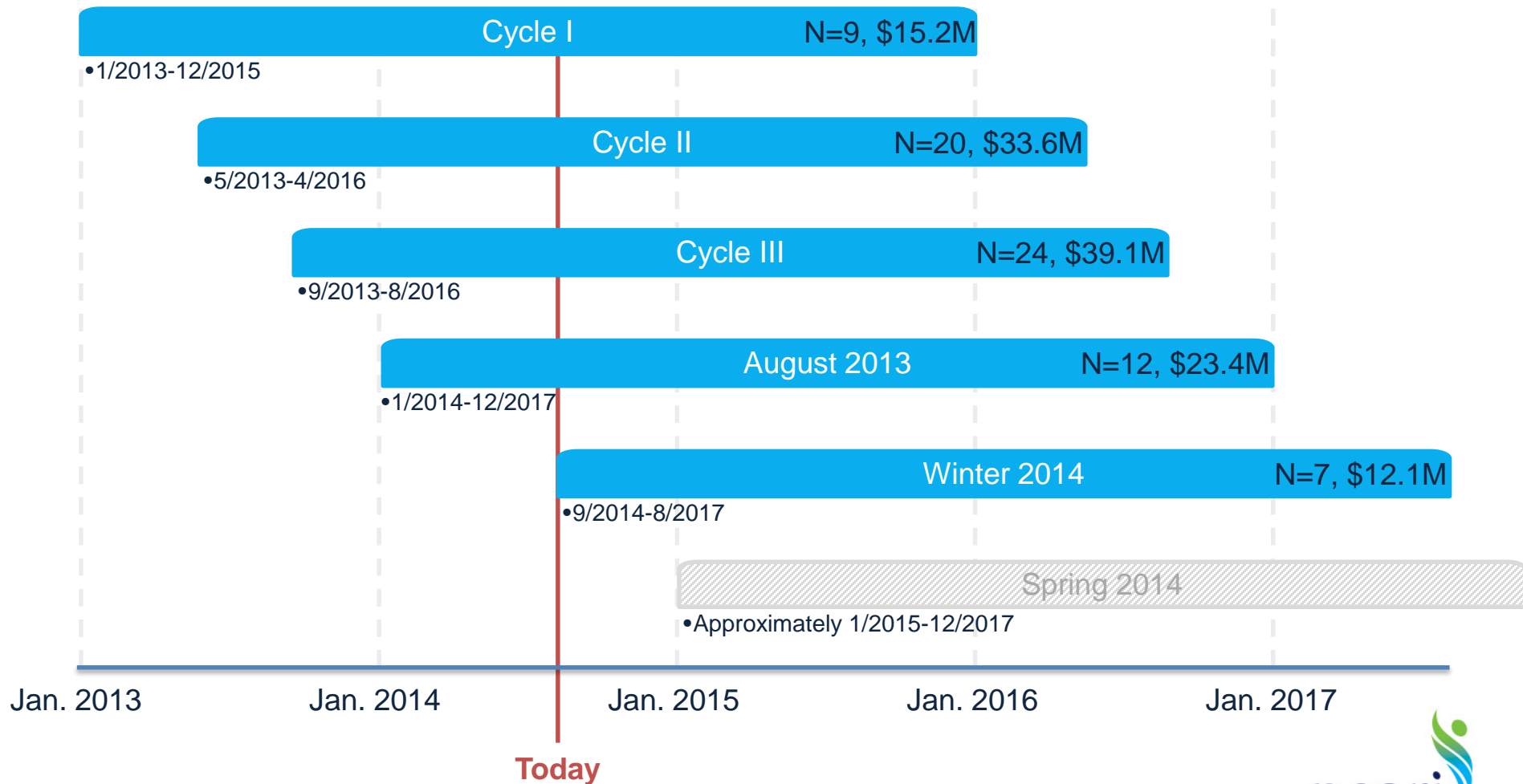
# Goals of APDITO Program\*

- “ . . . inform **critical decisions** that face patients and caregivers, clinicians, policy makers, and healthcare system leaders.
- . . . decisions must . . . be occurring now in the **absence of sound evidence** about the comparative effectiveness of alternative approaches.
- . . . substantial **potential that patients/caregivers will benefit** from the new knowledge . . .
- . . . provide insight about the comparative **benefits and harms** of the options and provide information about outcomes that are important to patients.”

# The Broad Research Program: Assessment of Prevention, Diagnosis, and Treatment Options

- Investigator-initiated research contracts
- No specified priority clinical topics
- Have completed 5 funding cycles (6<sup>th</sup> imminent)
- Limited size of projects
  - Budgets up to \$1.5 - \$2.0 million in direct costs
  - Project duration no greater than 3 years
- Required adherence to PCORI Methodology Standards
- Continuous improvement of the process of merit review

# APDТО Funded Projects Timeline



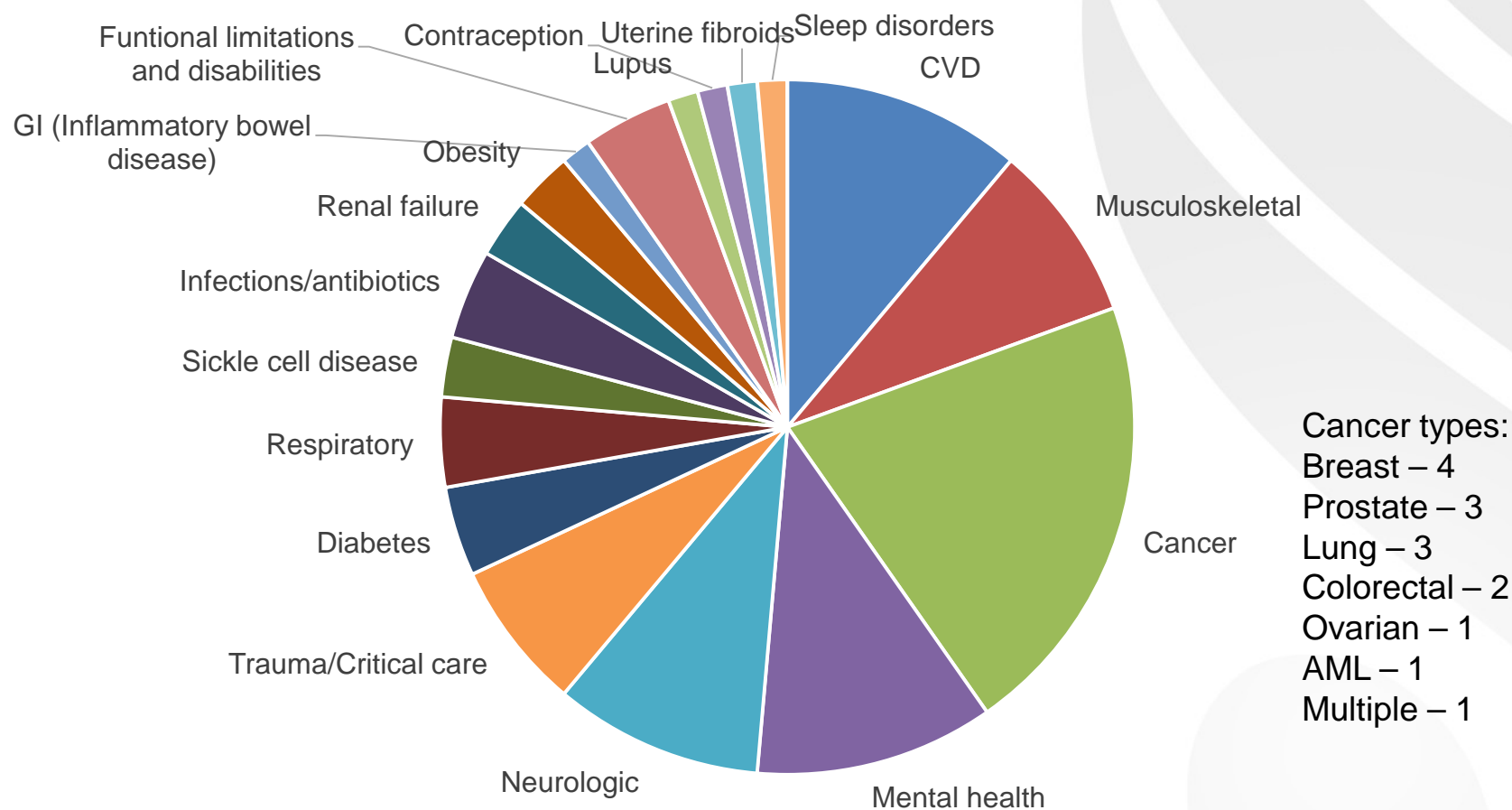
Note: This graphic represents earliest award start dates and projected project end dates for PCORI-funded APDТО projects.

# Study Characteristics

- 72 projects
- About  $\frac{3}{4}$  are in chronic disease and cancer
  - Not in this category: infectious disease, trauma, etc.
- 60% are clinical trials, most of moderate size
  - 37% compare medications or surgery
  - 19% are studies of decision aids
  - 44% are studies of self-care
- 40% are observational studies

*\*based Cycle I – Winter 2014*

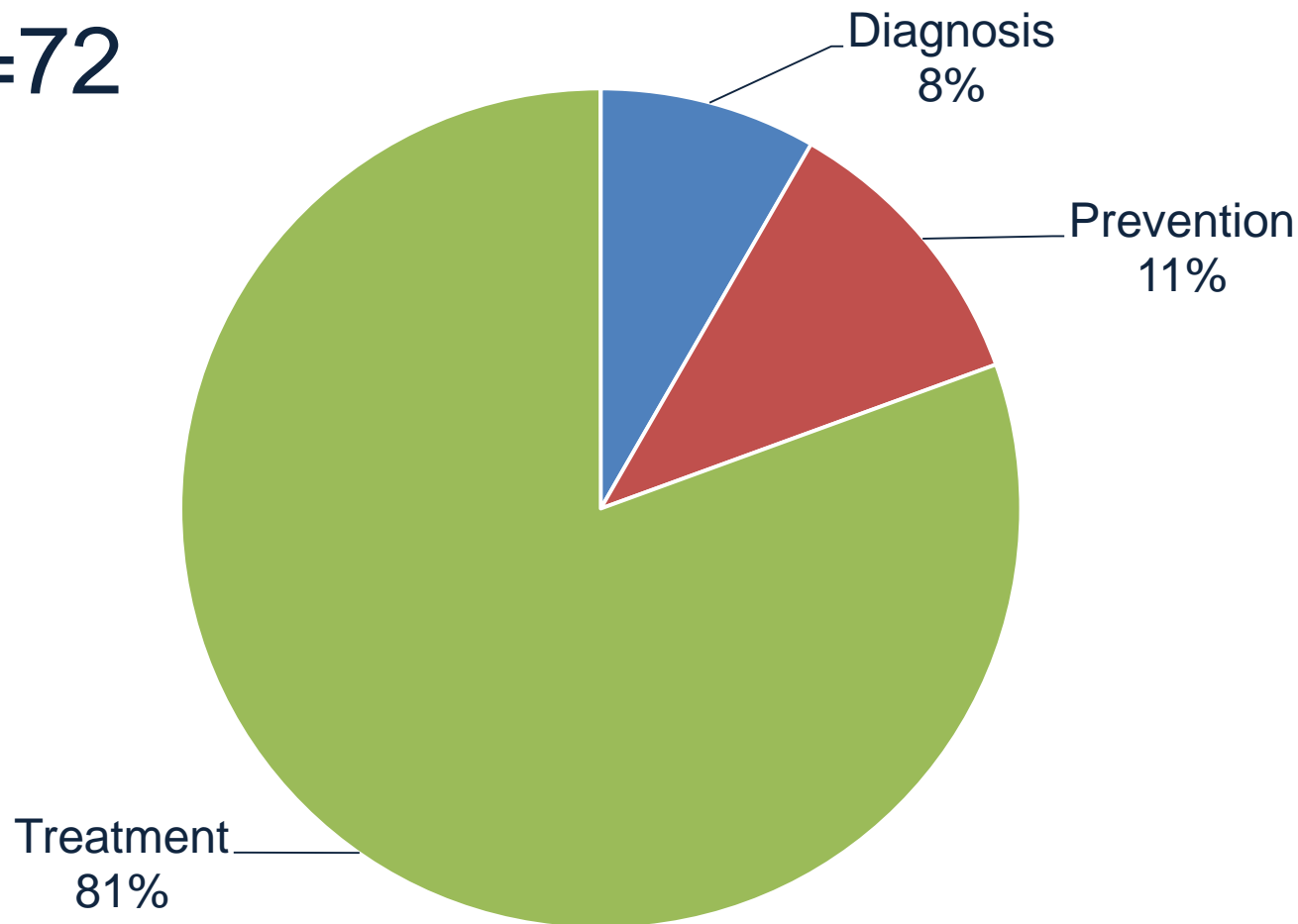
# Conditions studied in 72 APDTO Projects



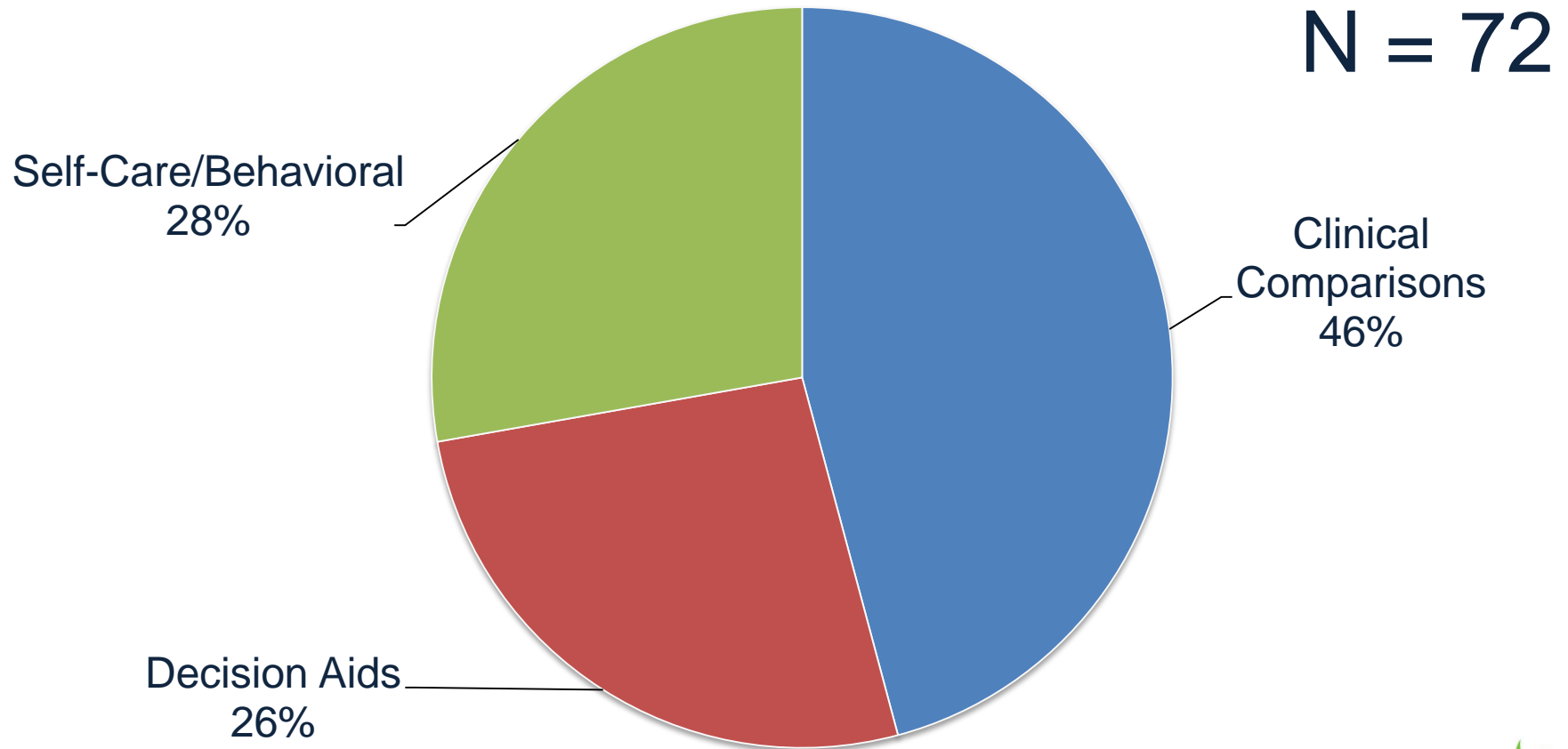


# Clinical Focus of Funded Projects

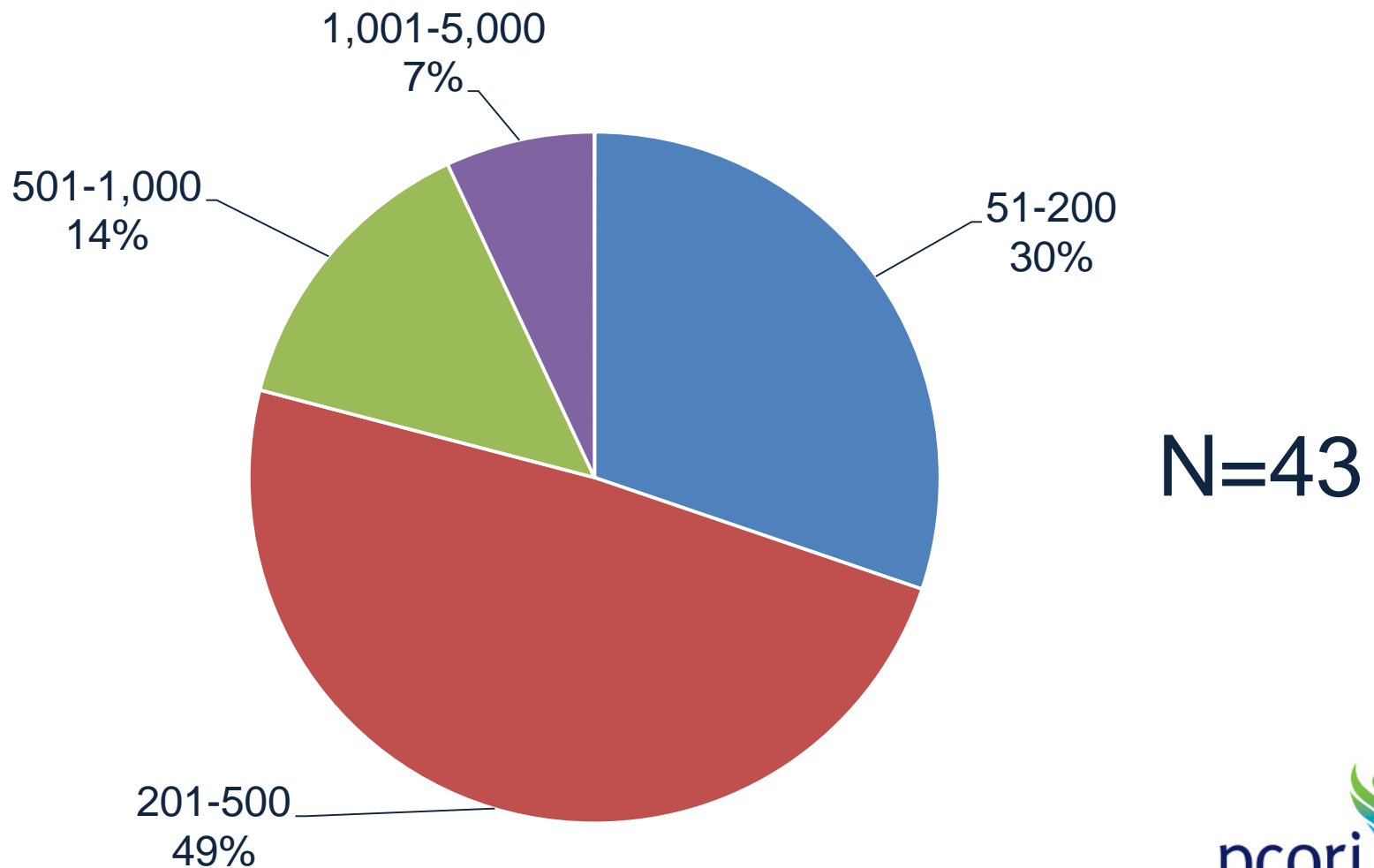
N=72



# Types of Interventions

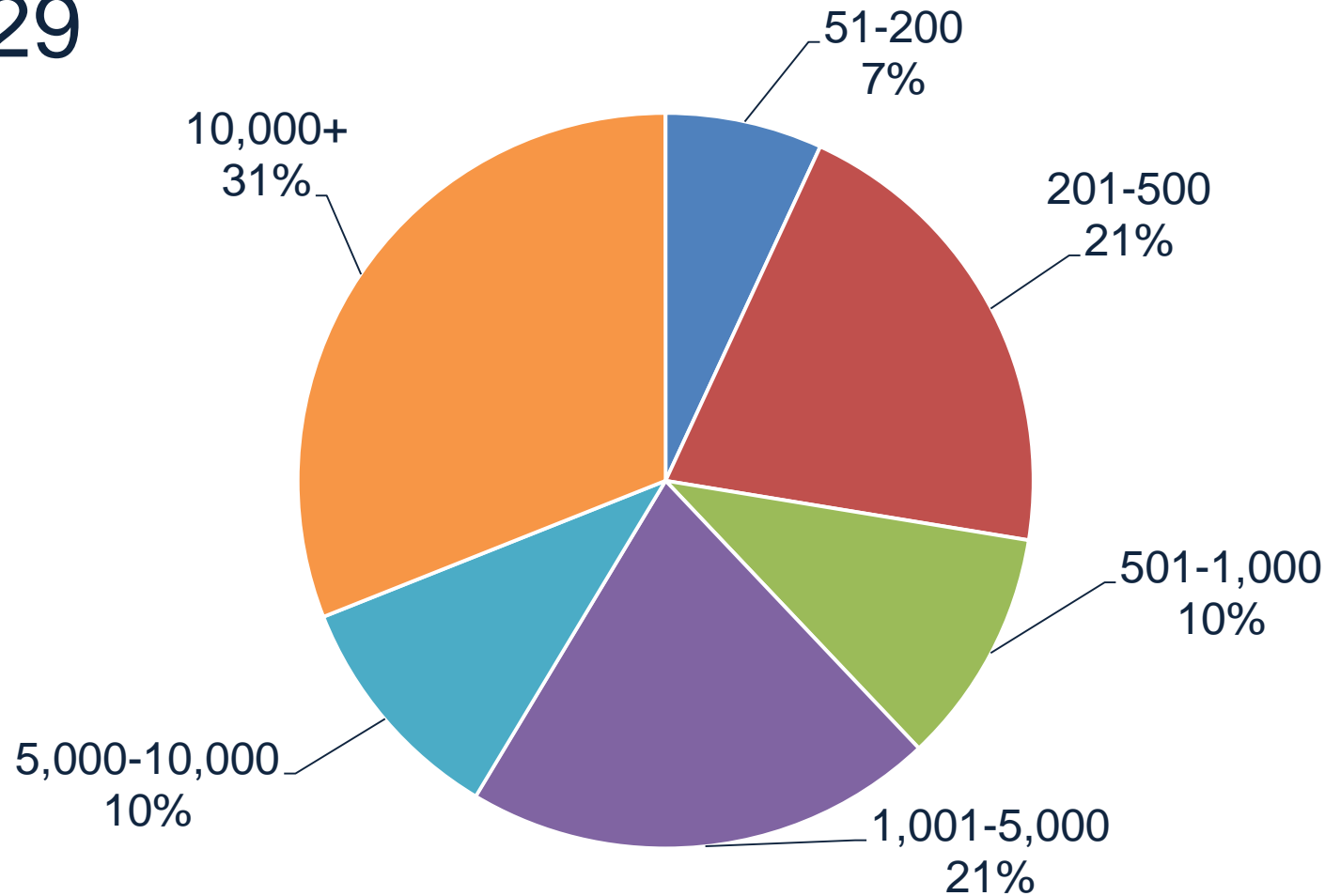


# Sample Sizes for Clinical Trials



# Sample Sizes for Observational Studies

N=29



# Trials Comparing Clinical Therapies

- Drugs for childhood epilepsy
- Drugs for neuropathic pain
- Surgical techniques for cervical disc disorders
- Manipulative and non-manipulative treatment for back pain
- Physical therapy regimens for knee arthritis
- Nicotine replacement regimens
- Weight loss programs
- Treatments to prevent dementia
- Counseling interventions in mental health (3 trials)



# **Trials of Interventions to Promote Self-Care**

- Management of symptoms in cancer patients
- Pain management
- Exercise in older adults
- Mobilization after back surgery
- Cardiovascular risk reduction
- Medication adherence
- Home oxygen adherence
- Home glucose monitoring

# **Trials of Interventions for Caregivers**

- Caregivers of patients receiving allogeneic stem cell transplants
- Caregivers of elderly patients with dementia
- Parents of children with severe injuries or critical illness (3 trials)

# Trials to Assess the Impact of Decision Aids

- Decision to obtain screening for lung cancer
- Treatment options for appendicitis
- Choosing methods for contraception
- Choosing treatments for back pain
- Treatment options for lupus
- Cancer treatment choices (4 trials)
- Guidance for use of diagnostic tests (3 trials)

# Initiatives Derived from Stakeholder-Based Clinical Priorities

- Opportunity to identify important evidence gaps
  - Nomination of clinical topics
  - Advisory panel
- Targeted initiative on treatment options for uterine fibroids (AHRQ partnership)
- Pragmatic Studies Announcement
  - PFA first released in January 2014
  - Competitive LOIs
  - Up to \$10M in direct costs and up to 5 years duration
  - Collaboration with Disparities and IHS Programs

# Priority Topics for the Pragmatic Studies Announcement

- Management of ductal carcinoma *in situ*
- Treatment strategies for osteoarthritis, including joint replacement
- Treatment strategies for adult patients with migraine headache
- Diagnosis and management of bipolar disorder
- Strategy for follow-up of incidentally discovered pulmonary nodules
- Treatment of opioid substance abuse



# Priority Topics for the Pragmatic Studies Announcement

- Treatment strategies for autism spectrum disorder
- Treatments for multiple sclerosis
- Proton therapy for breast, prostate, and lung cancer.
- Biological agents in Crohn's disease
- Medical versus invasive procedures for asymptomatic carotid artery disease
- Surgical options for hip fracture in the elderly
- Pelvic floor mesh implants

# Study on a Tool to Promote Informed Decision-Making on Lung Cancer Screening among Tobacco Users

## Promoting Informed Decisions about Lung Cancer Screening

- PI – Robert Volk, PhD
- University of Texas MD Anderson Cancer Center


## Compare 2 strategies for providing information to help people decide whether to pursue screening via CT scanning

## Will assess changes in intentions and receipt of screening

# Study on Diagnostic Methods for Detecting Recurrence among Women Previously Treated for Breast Cancer

## **Comparative Effectiveness of Surveillance Imaging Modalities in Breast Cancer Survivors**



- PI – Karen Wernli, PhD, MS
- Group Health Cooperative, Seattle, WA

 Data on 36,000 women with a history of breast cancer diagnosed from 2005 to 2012 in whom either surveillance mammograms or breast MRI examinations have been obtained in 7 diverse regions in the US

 Will assess breast cancer mortality, cancers missed, false positive rates, and biopsy rates

# Adaptive Clinical Trial to Compare Medications for Painful Peripheral Neuropathy

## Patient Assisted Intervention for Neuropathy: Comparison of Treatment in Real Life Situations

- PI – Richard Barohn, MD
  - University of Kansas Medical Center
-  Will enroll 400 patients in a prospective adaptive trial. Will compare four medications. Adaptive randomization will help to increase power to detect differences among the drugs.
-  Primary outcome is pain relief.

# Observations about the Portfolio of the PCORI CER (APDTO) Program

- Includes a wide variety of studies that compare clinical options across a broad range of clinical questions and with a broad set of intervention types
- Unique among US research funding organizations
  - Emphasis on comparative effectiveness
  - Emphasis on patient-centeredness

# Observations about the Portfolio of the PCORI CER (APDTO) Program (cont'd)

## Areas of study:

- Head-to-head comparisons of impactful clinical strategies
- Decision aids
- Interventions to promote self-care
- Interventions to reduce caregiver burden and stress
- Care management and transitional care interventions

# Plans

- Develop initiatives based on clinical priority topics that are based on patient and stakeholder input.
- Continued active portfolio management, including portfolio analysis and modification of PFAs to encourage more impactful proposals.
- Improve adherence to Methodology Standards.

# Background and Status of Previous Topics



## David Hickam, MD, MPH

Program Director  
Clinical Effectiveness Research  
PCORI



# Overview of CER Topics



48 Topics Reviewed by AO Advisory Panel

13 Topics included in Pragmatic Studies Announcement

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

# Status of Prioritized Topics

## April 2013 Ranking 20 Topics

- 4 topics into Pragmatic Trials PFA
- Ductal Carcinoma in Situ
  - Osteoarthritis
  - Migraine Headache
  - Bipolar Disorder

6 topics re-reviewed during Aug 2014 Webinar

## January 2014 Ranking 14 Topics

- 5 Topics into Pragmatic Trials PFA
- Multiple Sclerosis
  - Lung Cancer
  - Opioid Substance Abuse
  - Autism Spectrum Disorder
  - Proton Beam Therapy

5 topics re-reviewed during Aug 2014 Webinar

## April 2014 Ranking 16 Topics

- 1 Topic into Pragmatic Trials PFA
- Inflammatory Bowel Disease

4 topics re-reviewed during Aug 2014 Webinar

# August 2014 Webinar Results

Item	Score <sup>1</sup>	Overall Rank
<b>Topic 5: Carotid artery disease</b>	<b>186</b>	<b>1</b>
Topic 11: Nonsurgical treatment for cervical disc and neck pain	184	2
Topic 2: Coronary artery disease (CAD)	181	3
<b>Topic 7: Hip fracture</b>	<b>175</b>	<b>4</b>
<b>Topic 1: Assessment of benefits and harms of pelvic floor mesh implants</b>	<b>158</b>	<b>5</b>
Topic 4: Gestational diabetes	145	6
Topic 9: Concussion management	139	7
Topic 15: Treatment strategies for intermittent claudication	116	8

<sup>1</sup> 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.

Topics in **BLACK** included in Large Pragmatic Trials Announcement

# August 2014 Webinar Results (cont'd)

Item	Score <sup>1</sup>	Overall Rank
Topic 8: Eczema	111	9
Topic 6: Periodontal disease	98	10
Topic 3: Cerebral adrenoleukodystrophy (ALD)	89	11
Topic 13: Treatment options for pemphigus vulgaris	86	12
Topic 12: Treatment options for hypercholesterolemia	85	13
Topic 10: Management of arrhythmogenic right ventricular dysplasia (ARVD)	84	14
Topic 14: Treatment options involving mesh for management of inguinal and abdominal hernias	83	15

<sup>1</sup> 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.

# Procedures for Reviewing Topics and Voting

- Prior to voting, 10 CER topics will be reviewed
  - High-level overview of each topic
  - Approximately 15 minutes per topic
- SurveyGizmo link has been emailed to panelists
  - Drag and drop ranking method
  - 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**  
**10:30 a.m. – 10:45 a.m.**

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**Review Clinical Effectiveness  
Research Topics 1–5  
10:45 a.m. – 12 p.m.**

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# Topic 1: Anesthesia Options for Orthopedic Procedures

**Overview: (1) Angela Smith, (2) Marcia Rupnow**

- 🌐 **Background:** In orthopedic procedures usually performed in day surgery settings, general anesthesia is applied through inhalation or intravenous injection. Regional anesthesia can be added as an additional modality for pain control, but this increases the complexity and costs of the anesthesia.
  
- 🌐 **Key points from topic brief:**
  - 51.4 million inpatient knee, hip, and shoulder surgeries performed in U.S in 2010
  - An estimated 0.724 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 PCOs (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



# Topic 2: Exercise and Physical Therapy for Tendinopathies and Ankle Sprains

Overview: (1) Karen Chesbrough, (2) Seema Sonnad

- 🌐 **Background:** Tendinopathy is a syndrome characterized by pain, focal tenderness and decreased strength within or around a tendon; ankle sprains are acute injuries that cause damage to the lateral ligament structures. Treatments include rest, medications for inflammation and pain, and physical therapy delivered in a center or as a self-care strategy (home exercise). Physical therapy aims to restore activity and function; the comparative effectiveness of home exercise versus formal physical therapy is unknown
  
- 🌐 **Key points from topic brief:**
  - Overuse injuries are common among physically active individuals. Together they account for 7% of all physician visits in the U.S.
  - Ankle sprains account for up to 45% of all sports-related injuries
  - Little existing evidence or ongoing research comparing physical therapy with home-based therapy
  - Variation in care is unknown

# Topic 3: Treatment Options for Acute Venous Thromboembolism (VTE)

**Overview: (1) Mark Johnson, (2) James Pantelas**

- 🌐 **Background:** VTE includes deep venous thrombosis (DVT) and pulmonary embolism (PE); patients presenting with an episode of VTE need to be treated rapidly to prevent complications, including death. Anticoagulants and inferior vena cava (IVC) filters (umbrella-like devices placed in the large vein below the heart) can be used to prevent recurrent PE.
- 🌐 **Key points from topic brief:**
  - No head-to-head trials that have compared IVC filters with anticoagulation for treatment of DVT
  - Patients are rarely asked for their preferences regarding anticoagulation versus permanent filter placement versus retrievable filter placement; need research on patient preference

# Topic 4: Use of Implantable Cardioverter-Defibrillators (ICD) in Elderly People

Overview: (1) Ronald Means, (2) Linda McNamara

🌐 **Background:** 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. Its purpose is to prevent sudden cardiac death caused by arrhythmias.

🌐 **Key points from topic brief:**

- Sudden cardiac death (SCD) accounts for approximately 300,000 deaths annually; three quarters of SCDs are caused by ventricular tachyarrhythmias, 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 U.S.; The rate of ICD implantation in the U.S. is five times as high as the rate in other Western countries.
- Need for research on:
  - Treatment heterogeneity in patients with different baseline risk for SCD
  - CER of ICDs versus other treatment modalities in older patients ( $\geq 65$  years) and in patients with multiple comorbidities
  - Incorporate patient preferences in the decision to place ICD

# Topic 5: Imaging Tests for the Evaluation of Cognitive Decline

**Overview:** (1) Kathie Insel, (2) Debra Madden

- 🌐 **Background:** Broad category of brain diseases that cause long-term memory loss, inability to perform daily functions, and behavioral changes. Provisional diagnosis can be made clinically; structural imaging examines anatomical features; functional imaging measures changes in blood flow, metabolism, and receptor binding in order to identify abnormalities in brain tissue.
- 🌐 **Key points from topic brief:**
  - Use of functional imaging has the potential to assist with the accurate diagnosis and characterization of extent of brain disorders that cause cognitive decline.
  - 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.



**Lunch**  
**12 p.m. – 1 p.m.**

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**Review Clinical Effectiveness  
Research Topics 6–10  
1 p.m. – 2:15 p.m.**

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# Topic 6: Statin Therapy for Prevention of Atherosclerotic Disease

**Overview: (1) Denise Kruzikas, (2) Alan Rosenberg**

- 🌱 **Background:** 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. Lower cholesterol levels are associated with a lower risk of atherosclerosis. Statins are drugs that lower cholesterol levels, and they sometimes are continued for decades. As the overall burden of disease increases in older age, the value of statin therapy may diminish.
- 🌱 **Key points from topic brief:**
  - Greater utilization of statins by elderly individuals without previous history of CVD has the potential to prevent cardiovascular and cerebrovascular (stroke) events; however, there is not a consensus in guidelines on recommendations to initiate or continue 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 health care cost-savings, and improvement in elderly individuals' quality of life.
  - Primary prevention through alternative approaches such as diet modification, physical activity, and smoking cessation have had limited population-level effects compared to statin therapy.

# Topic 7: Genetic Testing for Rare Diseases in Children

Overview: (1) Bruce Monte, (2) Daniel Wall

- 🌐 **Background:** “Genetic testing” refers to tests seeking to confirm DNA mutations associated with a particular diagnosis – these tests are performed when there is a suspicion for a genetic disorder based on screening results, family history, ethnicity, or physical findings.
  
- 🌐 **Key points from topic brief:**
  - 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.
    - Variable expressivity – A genetic disease may manifest with different symptoms in different patients
  - Over \$5 billion was spent on genetic testing in 2010 in the U.S., and it is estimated that this figure will reach between \$15 billion and \$25 billion by 2021



# Topic 8: Treatment Options for Sjögren's Syndrome

**Overview: (1) Alan Rosenberg, (2) Sara Hohly**

- 🌐 **Background:** Sjögren's Syndrome is a chronic autoimmune disease in which people's white blood cells attack their moisture-producing glands; Onset of the disease usually begins when people are in their 40s or 50s; There is no cure for this disease, and it can be very resistant to treatment; Current treatment is limited to modalities for relieving symptoms.
- 🌐 **Key points from topic brief:**
  - One recent systematic review reported a prevalence range of 0.21% to 2.7% for primary Sjögren's Syndrome
  - 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
  - New research could significantly contribute to better patient-centered outcomes that target:
    - Treatment resistant symptoms such as fatigue and chronic pain
    - Use of therapies during pregnancy to reduce fetal complications

# Topic 9: Screening Options for Open-Angle Glaucoma

Overview: (1) Bettye Green, (2) Kathie Insel

- 🌐 **Background:** Open-angle glaucoma is the most common form and is characterized by progressive peripheral visual field loss (tunnel vision). Usually, though not always, it is associated with elevated pressure in the eye (intraocular pressure).
- 🌐 **Key points from topic brief:**
  - In the US, 2.8 million people are living with open-angle glaucoma, and the number will increase to 3.4 million by 2020.
  - Limited evidence on the relative benefits and harms of screening for primary open-angle glaucoma in adults. The available screening methods include: direct and indirect ophthalmoscopy; fundus photography or computerized imaging of the posterior pole, optic disc, or retinal nerve; pachymetry (corneal thickness measurement); perimetry; tonometry.
  - New research could provide evidence to support the following: The role of early detection of glaucoma on patient-reported vision issue; Screening tool that optimizes patient-reported vision issues; How the effects of screening differ by key subgroups; Optimal sequencing of screening tools; How and where screening should be conducted; Best provider to conduct screening test; If screening is found to be effective, methods for identifying and engaging patients early in the glaucoma disease process.

# Topic 10: Treatment Options for Hepatitis C Infection

**Overview: (1) Priti Jhingran, (2) Cynthia Mulrow**

- 🌐 **Background:** Hepatitis C is a virus that is carried by millions of people worldwide. In 20-30% of infected people, it can cause progressive liver disease. Several anti-viral drugs are available, but all have serious limitation related to cost, side effects, and strength of the evidence on efficacy. The decision of when/how to treat HCV is complicated and should consider the current stage of liver disease, HCV genotype, extra-hepatic manifestations, anticipated adverse effects, and patient preferences.
- 🌐 **Key points from topic brief:**
  - New research could contribute to achieving better patient-centered outcomes:
    - Newer regimens (e.g., sofosbuvir + simeprevir) are much better tolerated than traditional regimens, so if confirmed in real-world practice, could have a dramatic impact on patient-centered outcomes (e.g., quality of life, productivity).
    - CER further exploring longer term patient-centered outcomes (e.g., mortality, liver failure, HCC, hospitalization) is needed.
  - 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.



**Break**  
**2:45 p.m. – 2:55 p.m.**

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## **Review of CER Topics**

**2:55 p.m. – 3:00 p.m.**

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# Topic 1: Anesthesia Options for Orthopedic Procedures

## Key points from discussion:

- Choosing Wisely topic
- The few existing studies are of poor quality, lack patient centered outcomes, have limited data on follow-up/long term outcomes.
- Topic is patient-centered, would help with decision making for patients, impacts large portion of population, 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.

# Topic 2: Exercise and Physical Therapy for Tendinopathies

## Key points from discussion:

- Choosing Wisely topic
- Tendinopathies and sprains are common reason for visits in primary care settings.
- Tendinopathies and ankle sprains are different conditions, and tendinopathies are more common and chronic. Suggestion to split these conditions into two topics.
- 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.
- Must define the details about content of formal physical therapy and home-exercise.

# Topic 3: Treatment Options for Acute Venous Thromboembolism (VTE)

## Key points from discussion:

- Topic was nominated through Choosing Wisely based on concern of overuse of filters.
- Patients are rarely asked for their treatment preferences.
- Research is needed on comorbidities, patient characteristics, prior incidents of VTE, other risk factors; treatment is often based on this information.
- Putting a filter in early may cost more but reduce long-term cost over time, by reducing burdens associated with long-term anticoagulation.
- Observational studies may be useful.
- Filters are used as escalation of treatment; need to study the appropriateness of escalating the treatment beyond anticoagulants.



# Topic 4: Implantable Cardioverter-Defibrillators (ICD) in Patient Sub-Groups

## Key points from discussion:

- Choosing Wisely topic
- ICDs are used for patients with severe heart disease in all age groups.
- An ICD prevents deaths caused by an arrhythmia but does not otherwise alter the course of the heart disease.
- Need to identify subgroups of people who derive the greatest benefit.

# Topic 5: Imaging Tests for the Evaluation of Cognitive Decline

## Key points from discussion:

- This topic was nominated by the American Geriatric Society through Choosing Wisely.
- Studies should differentiate between the different anatomic conditions that lead to dementia.
- Concerns about the lack of standardization and accuracy of diagnostic modalities, poor test-retest reliability, and variation in the interpretation of MRIs.
- Patients and caregivers may want early diagnosis even in the absence of available effective treatment. Potential ethical issues: Unclear benefits (e.g. preparation for end-of-life care) and harms (e.g. reduced functioning).
  - Research could look at how knowing improves or diminishes functioning.

# Topic 6: Statin Therapy for Prevention of Atherosclerotic Disease

## Key points from discussion:

- Choosing Wisely topic
- Important topic related to heart disease, a leading cause of death, and will increase in relevance as population ages
- Evidence exists, but it is not focused on the elderly
- One clinical trial is underway in Australia, however, there is still an opportunity for PCORI either to play a role in defining future trial/future research need or funding an RCT with patient centered outcomes

# Topic 7: Genetic Testing for Rare Diseases among Children

## Key points from discussion:

- Topic from Rare Disease Advisory Panel
- Addresses critically important topic in health care system: the proliferation of genetic tests and the associated risks
- Because of different considerations in different patient populations, potential studies should look at how tests will be used specifically and assess the risks and benefits.
- Need to define the CER question and define relevant, patient-centered outcomes. How will confirmation of diagnosis change treatment decisions?

# Topic 8: Treatment Options for Sjögren's Syndrome

## Key points from discussion:

- Patient-nominated topic via PCORI's public nomination process.
- Relatively rare condition with critical importance on maternal fetal transmission complications.
- There is no cure. Treatment is often symptomatic and primarily for relief, and there is little evidence that compares the benefits and harms of different types of treatments.
- Approximately 26 ongoing meta-analyses and RCTs, only 5 of which look at biologic agents. However, these are newer studies, and details are lacking.
- New research could focus on:
  - Treatment-resistant symptoms such as chronic fatigue
  - Use of therapies during pregnancy to reduce fetal complications
  - Screening and diagnosis

# Topic 9: Alternative Screening Options for Open-Angle Glaucoma

## Key points from discussion:

- Leading cause of blindness, however, only 1/2 to 2/3 of people with glaucoma are aware they have it
- Lack of consensus on how and when to screen for glaucoma
- 6.6 times as frequent in African Americans
- Big impact on QOL
- Need for screening technique that is portable and does not require dilation, could be combined with other screening

# Topic 10: Treatment Options for Hepatitis C

## Key points from discussion:

- Nominated by payers.
- Panelists agree that this is a high priority topic, but lack of clarity regarding PCORI's role.
- Potential research could include:
  - Comparative effectiveness trials looking at duration of therapy using newer drug regimens.
  - Risk-stratification to look at the future development of complications in HCV patients by targeting a subset for treatment.
- PCORI cannot do cost-effectiveness, but can look at high cost and high prevalence conditions, particularly when there is a high cost to the individual in terms of suffering or dollars.



## **Voting and Voting Results**

**3:00 p.m. – 3:25 p.m.**

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# Ranking Results (N = 10)

Item	Score <sup>1</sup>	Overall Rank
Topic 10: Hepatitis C	176	1
Topic 9: Open-Angle Glaucoma	147	2
Topic 6: Statin Therapy for Atherosclerotic Disease	120	3
Topic 1: Regional vs. General Anesthesia for Orthopedic Procedures	110	4
Topic 7: Genetic Testing for Select Rare Diseases	108	5

<sup>1</sup> 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 (N = 10)

Item	Score <sup>1</sup>	Overall Rank
Topic 4: Implantable Cardiac Defibrillators (ICDs) in Elderly	101	6
Topic 3: Inferior Vena Cava Filters for Acute Venous Thromboembolism	92	7
Topic 2: Exercise and Physical Therapy for Tendinopathies	79	8
Topic 5: Cognitive Decline	56	9
Topic 8: Sjögren's Syndrome	56	10

<sup>1</sup> 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: 3:30 p.m.

- Next in-person meeting is scheduled for January 13, 2015 in Washington, DC.
- Reminder: Please complete the Post Event Survey by September 19, 2014.



**Thank you for your participation.**

Patient-Centered Outcomes Research Institute