



Welcome!

Please be seated by 8:20 am ET

The teleconference will go live at 8:30 am ET



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Assessment of Prevention, Diagnosis, and Treatment Options

Advisory Panel Meeting

May 25, 2017



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Welcome, Introductions, Overview of the Agenda, and Meeting Objectives

David Hickam, MD, MPH

Program Director, Clinical Effectiveness and Decision Science, PCORI

Stanley Ip, MD

Associate Director, Clinical Effectiveness and Decision Science, PCORI

Margaret F. Clayton, RN, PhD

*Chair, Panel on the Assessment of Options
Associate Professor, College of Nursing and
Co-Director of the PhD Program, University of Utah*



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Housekeeping

- Today's teleconference is open to the public and is being recorded
 - 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

Panel Member Introductions



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Agenda Overview

Time	Agenda Item
8:30 – 9:00 am	Welcome, Introduction, Overview of the Agenda and Meeting Objectives
9:00 – 10:30 am	Presentation of APDTO Portfolio
10:30 – 10:45 am	Break
10:45a – 12:15 pm	Topic Brief: Second-line therapies for Patients with Metastatic Colorectal Cancer
12:15 – 1:30 pm	Lunch and Recognition of Panel Members
1:30 – 2:30 pm	Shared Decision Making in the Emergency Department: The Chest Pain Choice Trial
2:30 – 3:15 pm	Dissemination and Implementation Program Updates
3:15 – 3:30 pm	Wrap up
3:30 pm	Adjourn

Meeting Objectives

- Update the APDTO Advisory Panel on future directions and next steps
- Provide an overview of the APDTO portfolio and solicit input on refinement of funding strategies
- Review new CER Topic: Second-line therapies for Patients with Metastatic Colorectal Cancer
- Provide an update on PCORI-initiated opportunities for dissemination and implementation

Status of CER Topics reviewed in November 2016

Topics
<i>Comparative Effectiveness of Treatments for Asymptomatic Bacteriuria including Watchful Waiting</i>
<i>Comparative Effectiveness of Treatment for Non-Muscle Invasive Bladder Cancer</i>
<i>Comparative Effectiveness of Treatments of Patients with Pancreatic Ductal Adenocarcinoma (PDAC) and its Subtypes</i>
<i>Comparative Effectiveness of Molecularly Directed Therapies in Patients with Lung, Pancreas, or Bladder Cancer</i>

PCORI's Programmatic Advisory Panels: Future Directions

- As PCORI evolves, we are seeking ways to increase our impact and improve the efficiency in which we operate
- The 2016 Science reorganization reflects PCORI's vision of how to align our national research priorities with programmatic functions and structure
 - Clinical Effectiveness and Decision Science
 - Healthcare Delivery and Disparities Research
- The PCORI Board of Governors will review the activities of the Advisory Panels
 - How to make best use of their perspectives to guide our research and dissemination activities



APDTO Advisory Panel: Next Steps

- Refocusing of programmatic Advisory Panels
 - All continuing APDTO Advisory Panel members will remain on the panel
 - Addition of new panel members
- Next Advisory Panel meeting will be in October 2017





Clinical Effectiveness Research

The evolution of PCORI's CER portfolio

**Assessment of Prevention Diagnosis and Treatment
Options Advisory Panel**

May 25, 2017

Kim Bailey, MS



Goals for Presentation

- Review PCORI's portfolio of clinical effectiveness research projects across the following portfolios
 - ✓ Assessment of Prevention, Diagnosis, and Treatment Options
 - ✓ Pragmatic Clinical Studies
 - ✓ Targeted Funding Announcements (Hepatitis C, Multiple Sclerosis, Opioids, Treatment Resistant Depression, New Oral Anticoagulants, and Low Back Pain)
- Discuss where the programs have been and how they have evolved over time
- Gather Advisory Panel's thoughts on how to refine what we are seeking and articulate this to the research community

Snapshot of Full PCORI Portfolio

Number of projects:

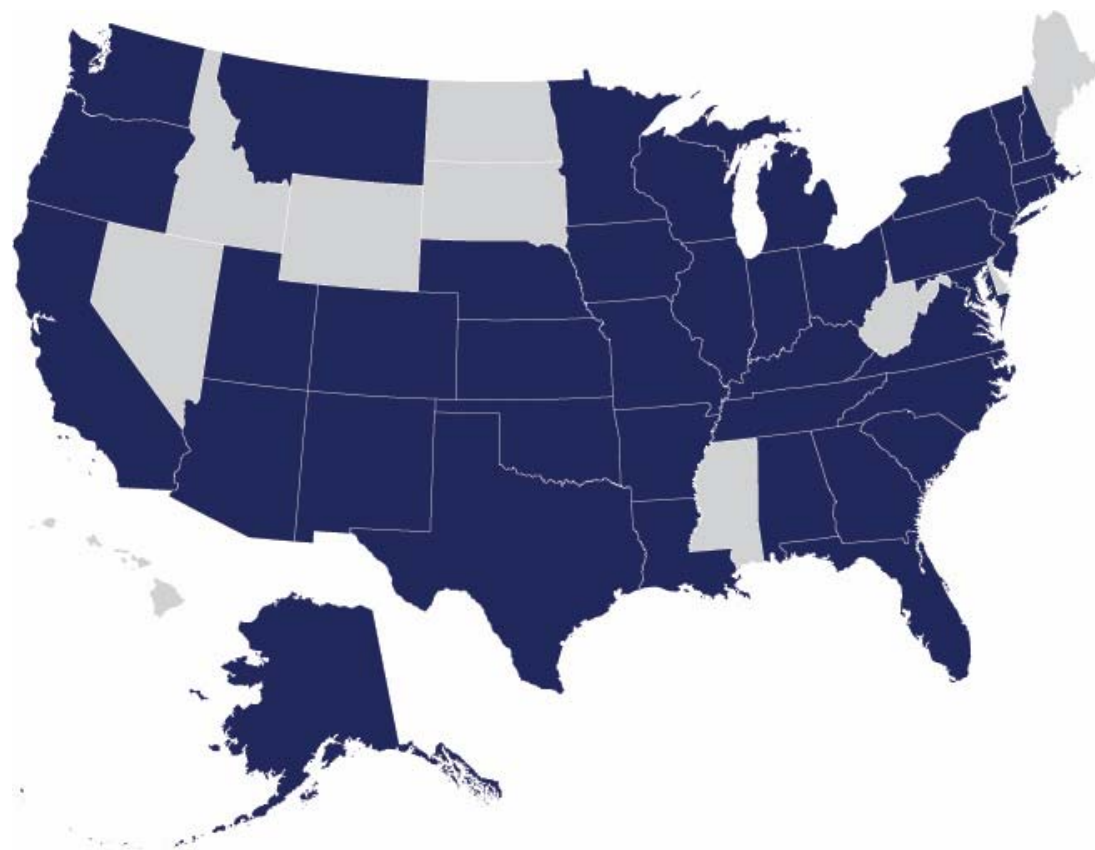
582

Amount awarded:

\$1.68 billion

Number of states where
we are funding research:

41 (plus the District of Columbia)



As of March 2017

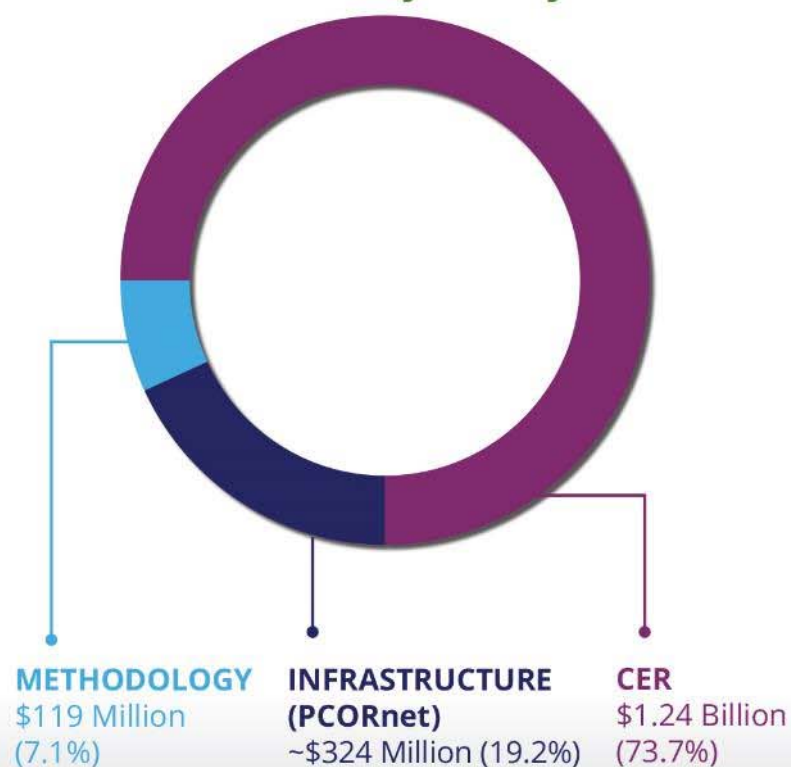


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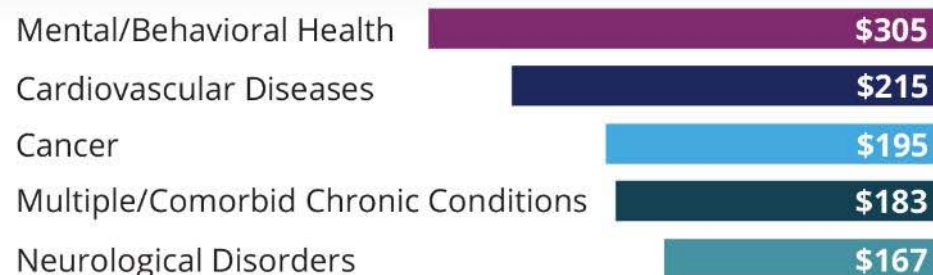
About Our Research Portfolio

BY THE NUMBERS

Research Projects By Area

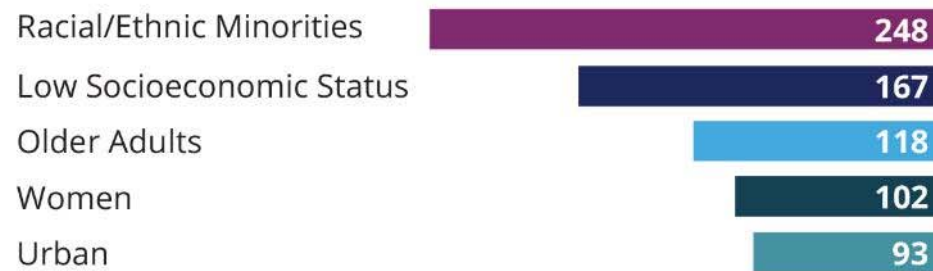


Most Studied Conditions*



*In millions. A project may study more than one condition.

Most Studied Populations of Interest**



**Number of projects (out of a total of 365). A project may study more than one population of interest.

Amounts in millions, as of March 2017

First Out of the Gate:

The Assessment of Prevention, Diagnosis, and Treatment Options (APDTO) Funding Announcement



APDTO

Purpose of Funding Announcement

- Goal of the program is to fund investigator-initiated research that
 - Compares the effectiveness of two or more strategies for prevention, treatment, screening, diagnosis, or management
 - Compares specific clinical services or strategies that are clearly defined and can be replicated in other clinical settings with minimal adaptations or changes
- Funding announcement does not support
 - Projects with the primary goal of developing and testing decision aids
 - Projects testing the use of lay personnel who perform ancillary services in healthcare settings

APDTO

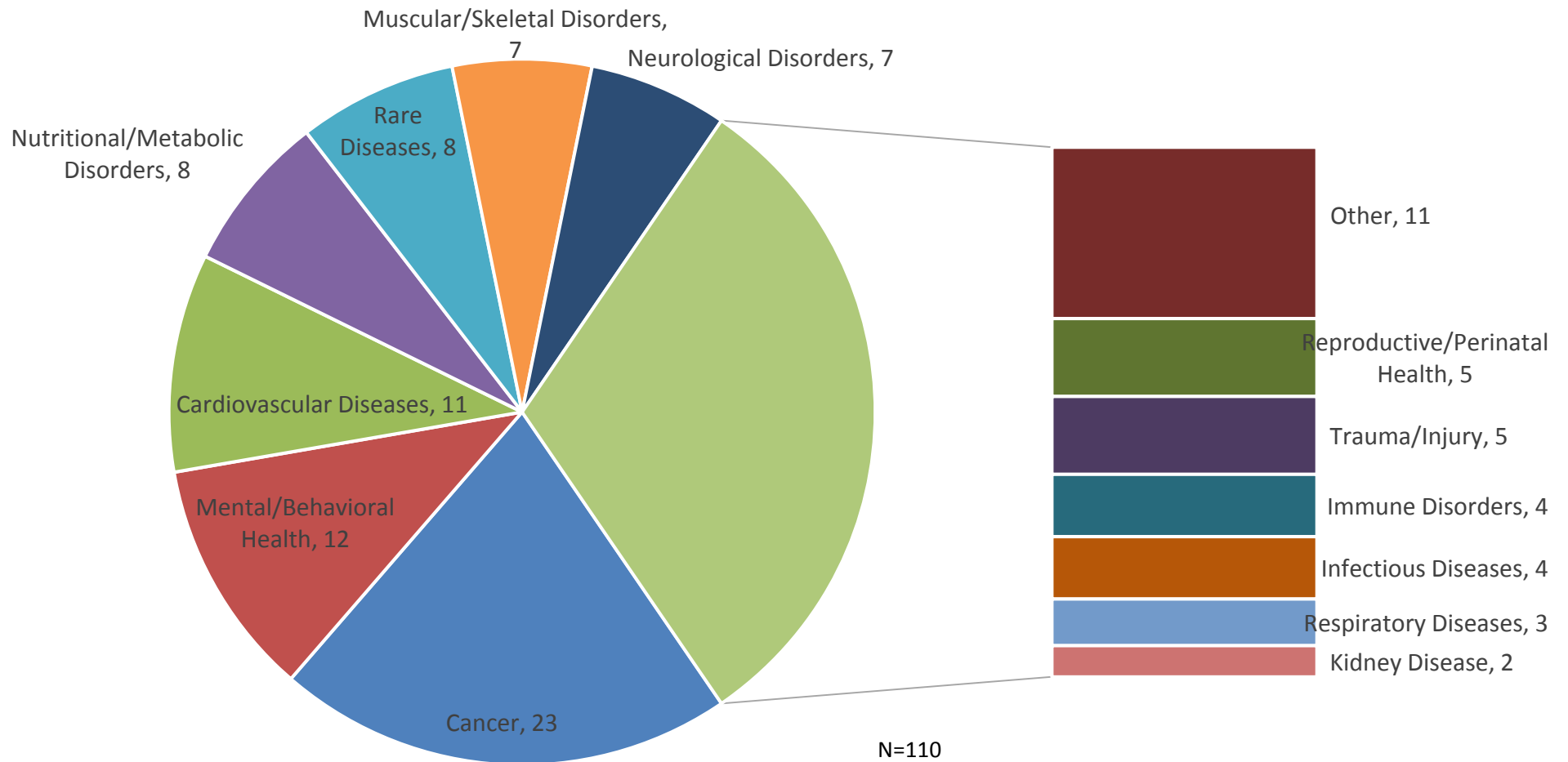
Program Overview

- Cycles: Cycle 1 2017 is the 12th release
- Funds Available: Up to \$32M per cycle; Up to \$2M in direct costs per project
- Duration: Typically 36 months; small handful contracted at outset for shorter or longer durations
- Projects Awarded: 114 through Cycle 1 2016
- Funds Awarded: Roughly \$220M through Cycle 1 2016
- Award amounts: ~\$700k– 6.7M in total costs
 - Median total costs of ~\$1.9M
- DFRRs submitted: 29 (25 approved as of 2/24/17)

APDTO

Current Portfolio: Clinical Conditions Under Investigation

Conditions Under Investigation by Awarded Projects

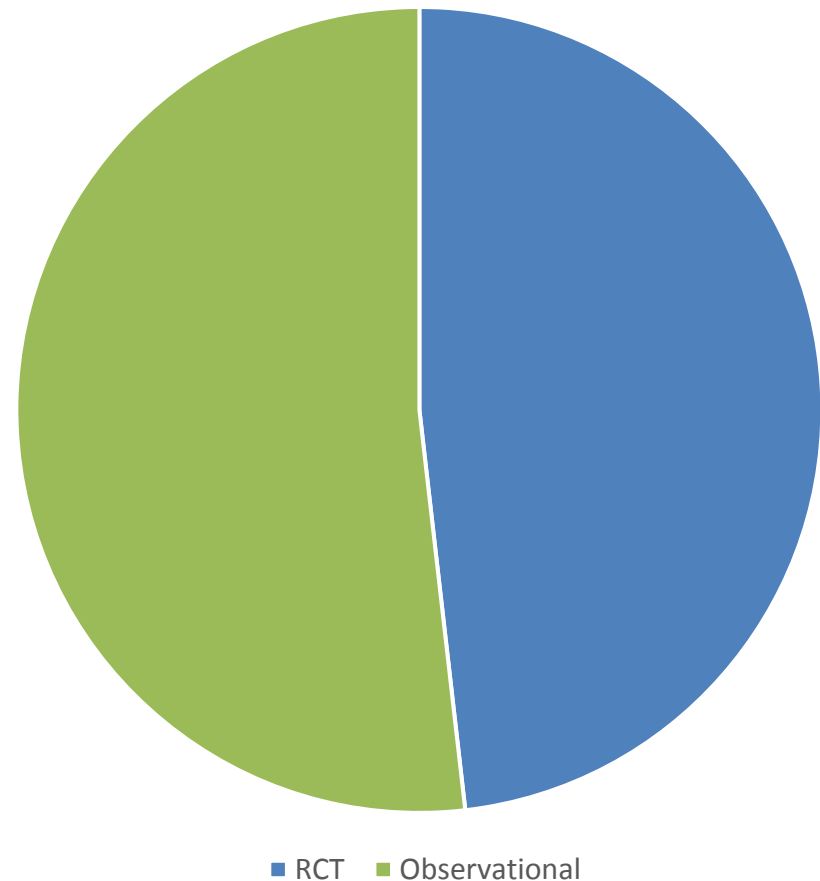


APDTO

Current Portfolio: Study Design

- Nearly even split between RCTs and observational designs
- For the RCTs, sample sizes range from 86 to 1,833 patients (Mean: 457; Median: 300)

Study Design of Awarded Projects

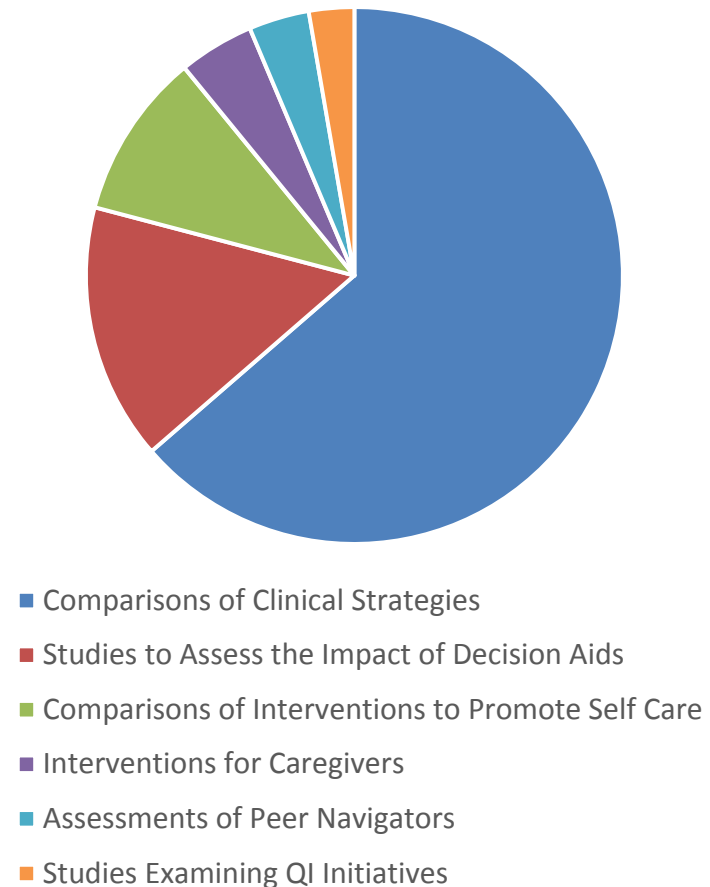


APDТО

Current Portfolio: Intervention Type

- Nearly 2/3 of the APDТО portfolio includes comparisons of clinical strategies
- The proportion of APDТО studies focused on comparisons of primary clinical strategies has increased in recent cycles
- Projects focused on QI efforts, assessments of decision aids, and assessments of the impact of peer navigators were awarded in early cycles

Intervention Type



Refining the Vision: The Pragmatic Clinical Studies Program



Pragmatic Clinical Studies

Background and Purpose

- Program launched in early 2014 to expand support of high-priority patient-centered comparative clinical effectiveness research
- Funding category was created in response to stakeholder feedback that many key health research questions require a greater investment and longer timeline than broad funding announcements allow
- Initiative emphasizes that we seek pragmatic studies appropriate for a specific high-priority question
- High-priority research questions may come from several sources:
 - IOM's Priorities for CER
 - AHRQ's Future Research Needs Projects
 - Topics recommended by patients and stakeholders through PCORI's topic prioritization process (PCORI Priority Topics)

Pragmatic Clinical Studies

Goals as Described in PFA

- Fund large pragmatic clinical trials, large simple trials, or large-scale observational studies that compare two or more alternatives for addressing:
 - Prevention, diagnosis, treatment, or management of a disease or symptom, or
 - Improving healthcare system-level approaches to managing care, or
 - Communicating or disseminating research results to patients, caregivers, or clinicians, or
 - Approaches to eliminate health disparities
- Must address critical clinical choices faced by patients, caregivers, clinicians, or delivery systems
- Must involve broadly representative patient populations and be large enough to provide precise estimates of hypothesized effectiveness differences
- Must support evaluation of potential differences in treatment effectiveness in patient subgroups

Pragmatic Clinical Studies

Timing and Budget

- Announcements appear two times per year (cycles 2 and 3)
- PCORI allots up to \$90 million dollars per funding cycle
 - Each project may request up to 10 million dollars in direct costs
- Maximum research project period is 5 years
- Beginning Cycle 1 2017, funding for PCS will be offered three times a year

Pragmatic Clinical Studies

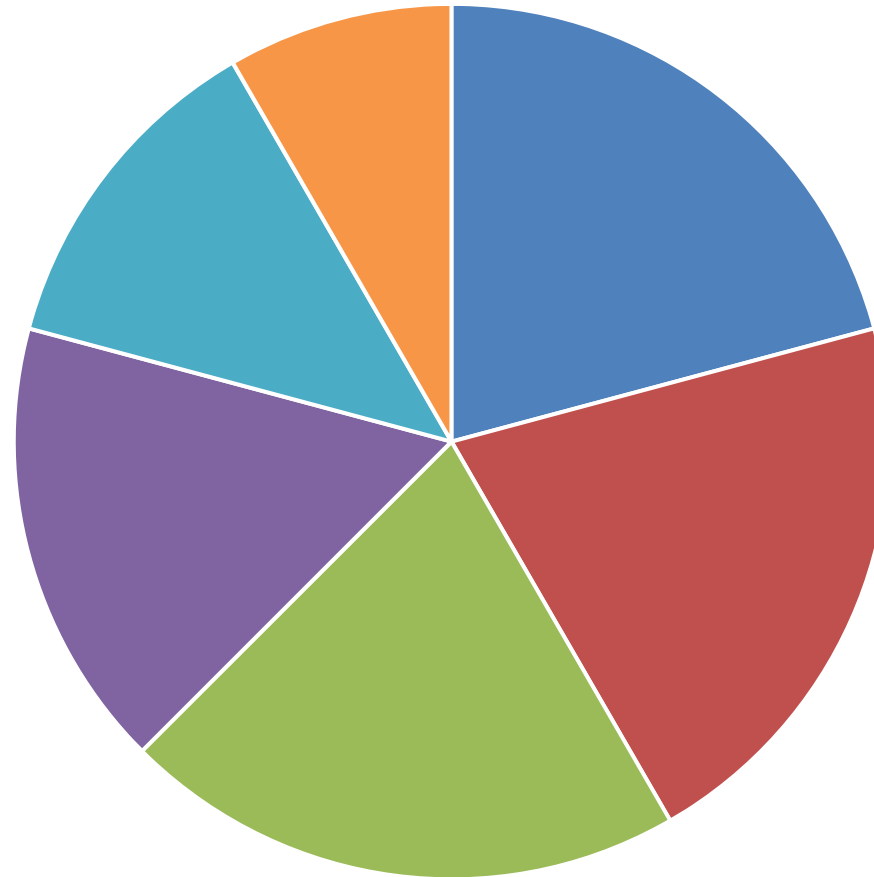
Current Portfolio: Overview

- The PCS PFA has been released 8 times (from Spring 2014 through Cycle 1 2017)
- As of mid-2017, there are 24 awarded projects in the portfolio, amounting to roughly \$280M to date
- Of the 24 studies, 15 are clinical comparisons managed by the CEDS team and 9 are health systems comparisons managed by the HDDR team
- Budget: \$7.5M – 18.5M in total costs
- Duration: 5 years to 7.5 years (includes peer review)
 - Earliest results will be available in 2020

Pragmatic Clinical Studies

Current Portfolio: Clinical Conditions Under Investigation

Conditions Under Investigation by Awarded Projects



■ Cancer ■ Mental/Behavioral Health ■ Other Conditions
■ Muscular/Skeletal Disorders ■ Cardiovascular Diseases ■ Respiratory Diseases

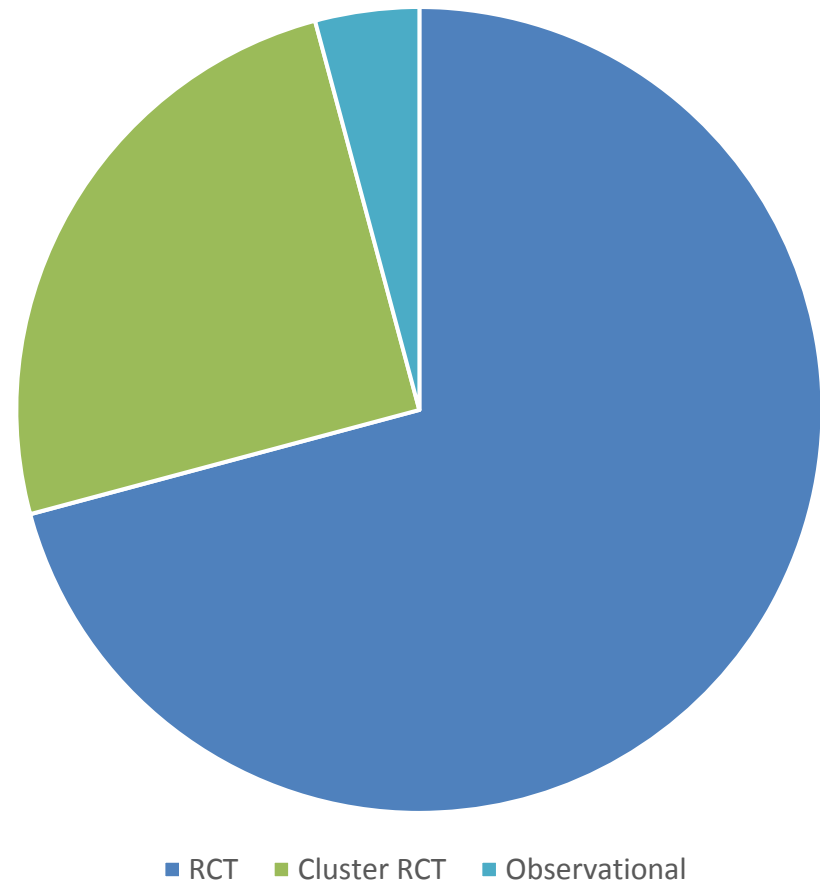


Pragmatic Clinical Studies

Current Portfolio: Characteristics of Awarded Projects

- Designs consist of mostly RCTs, with cluster RCTs and one observational study
- For the RCTs, sample sizes range from 500 to 65,000 patients
- The one observational study aims to review the scans of 1 million women (approximately 2.8 million scans)

Study Design of Awarded Projects



Pragmatic Clinical Studies

Cycle 1 2017 Priority Topic List

- **Treatment of community-acquired pneumonia**
- **Second-line treatments for non-muscle invasive bladder cancer**
- Screening, Brief Intervention, and Referral to Treatment for adolescent alcohol abuse
- **Surgical options for hip fracture**
- Multicomponent interventions to reduce initiation of tobacco use
- Teledelivery of interventions for anxiety and depression
- Integration of mental and behavioral health services into primary care
- **Treatment strategies for adult patients with migraine headache**
- **Treatment strategies for symptomatic osteoarthritis**
- Evidence-based models of perinatal care
- Preventing lower-extremity amputations in minority patients with diabetes
- Comprehensive support after NICU discharge
- Multidisciplinary rehabilitation for moderate to severe TBI
- Pharmacist- or nurse-led interventions to enhance management of chronic non-cancer pain in primary care
- Delivery models to prevent dental caries in children in underserved areas
- Strategies to integrate pharmacists or pharmacy services into patient care
- Strategies to prevent suicidality among adolescents
- Multimodal approaches for episodic back pain



APDTO AP-Reviewed Topics placed on PCS Priority List

Topics	Reviewed by Advisory Panel	Added to PCS Priority List
<i>Bipolar Disorder and Antipsychotic Use in Children, Adolescents and Young Adults</i>	April 2013	Spring 2014 PFA <i>Project Funded</i>
<i>Management Strategies for Ductal Carcinoma in Situ (DCIS)</i>	April 2013	Spring 2014 PFA <i>Project Funded</i>
<i>Treatment strategies for adults with frequent migraine headaches</i>	April 2013	Spring 2014 PFA <i>Project Funded</i>
<i>Treatment strategies for stabilization of symptoms from osteoarthritis</i>	April 2013	Spring 2014 PFA
<i>Treatment programs for recurring/remitting multiple sclerosis (MS)</i>	April 2013	Spring 2014 PFA <i>Became targeted PFA</i>
<i>Diagnostic modalities for identifying lung cancer in people with lung nodules.</i>	January 2014	Spring 2014 PFA <i>Project Funded</i>
<i>Medication regimens, intensive counseling, and combined modalities for treatment of opioid substance abuse</i>	January 2014	Spring 2014 PFA <i>Became targeted PFA</i>



APDTO AP-Reviewed Topics placed on PCS Priority List, continued

Topics	Reviewed by Advisory Panel	Added to PCS Priority List
<i>Proton Beam Therapy for Breast, Lung, and Prostate Cancer</i>	January 2014	Spring 2014 PFA Project Funded
<i>Treatment Options for Autism</i>	January 2014	Spring 2014 PFA Removed Cycle 1, 2017
<i>Strategies of introducing biologics into the treatment algorithm for inflammatory diseases, including Crohn's disease, ulcerative colitis, and rheumatoid arthritis</i>	April 2014	Fall 2014 PFA Project Funded
<i>Renal replacement therapies for patients of different ages, races, and ethnicities</i>	April 2014	Fall 2014 PFA Removed Cycle 1, 2017
<i>Medical and surgical treatment options of patients with asymptomatic carotid artery stenosis</i>	August 2014 (webinar)	Winter 2015 PFA Removed Cycle 3, 2015
<i>Surgical options for hip fracture in the elderly</i>	August 2014 (webinar)	Winter 2015 PFA Related Project Funded
<i>Benefits and Harms of Pelvic Floor Mesh Implants</i>	August 2014 (webinar)	Winter 2015 PFA Removed Cycle 1, 2017

APDTO AP-Reviewed Topics placed on PCS Priority List, continued

Topics	Reviewed by Advisory Panel	Added to PCS Priority List
<i>Narrow-spectrum antibiotics versus broad-spectrum antibiotics in the treatment of community-acquired pneumonia</i>	May 2015	Cycle 2, 2016 PFA
<i>Treatment for Non-Muscle Invasive Bladder Cancer</i>	November 2016	Cycle 1, 2017 PFA

Homing in: Targeted Funding Announcements



Targeted Funding Announcements

Background and Purpose

- Program launched in Spring 2015 in an effort to target funding toward topic areas of particular interest to PCORI's stakeholders
- Targeted funding announcements, including the specific research questions of interest, are developed in partnership with key stakeholders
- Successful proposals must be responsive to the questions defined in the targeted funding announcement

Targeted Funding Announcements

CER Targeted Announcements, to date

- Clinical management of hepatitis C (Spring 2015; 2 studies funded; 1 IHS)
- Treatment of multiple sclerosis (Cycle 3 2015; 4 studies funded*; 1 IHS)
- Management strategies for treatment-resistant depression (Cycle 3 2015; 3 studies funded)
- New oral anticoagulants (NOACs) in the extended treatment of VTE (Cycle 3 2015; 3 studies funded)
- Clinical strategies for managing and reducing long-term opioid use for chronic pain (Cycle 3 2015; 2 studies funded*)
- Comparison of surgical and nonsurgical options for management of nonspecific chronic low back pain (Cycle 2 2016, no studies funded, reissued Cycle 1 2017)

APDTO AP-Reviewed Topics in Targeted Funding Announcements

Topics	Reviewed by Advisory Panel	Targeted PFA
<i>Treatment strategies for hepatitis C</i>	September 2014	Spring 2015
<i>Treatment programs for recurring/remitting multiple sclerosis (MS)</i>	April 2013	Cycle 3, 2015 Cycle 3, 2016
<i>Modalities for treatment of opioid substance abuse</i>	January 2014	Cycle 3, 2015 Cycle 3, 2016

Targeted Funding Announcements

Current CER Targeted Portfolio: Overview

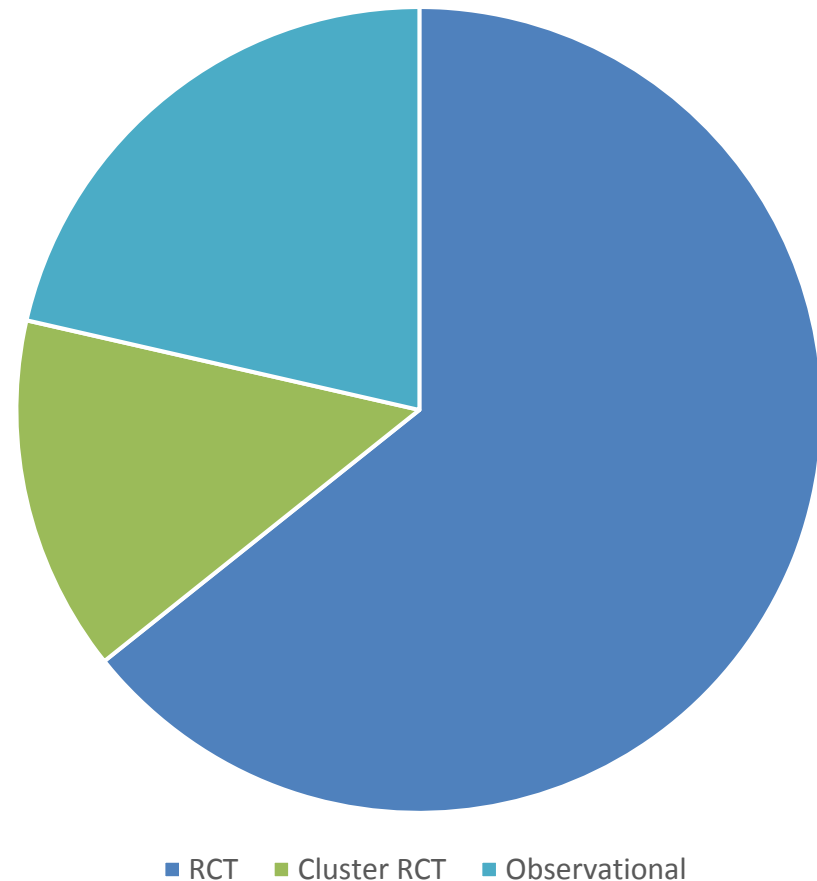
- As of May 2017, the CEDS team has managed the release of six targeted funding announcements
- 14 studies have been funded through these six funding announcements
- Of the 14 studies, 12 are managed in the CEDS program; 2 in the HDDR program
- Budget: \$2.0M – 15.0M in total costs
- Duration: Most are 5 year studies
 - Earliest results will be available in 2021

Targeted Funding Announcements

Current CER Portfolio: Characteristics of Awarded Projects

- Approximately 2/3 RCTs
- Two cluster RCTs managed by HDDR
- Three observational studies, all large with the ability to examine subgroups of interest
- For the RCTs, sample sizes 136 to 3,165 (median: 1,360)

Study Design of Awarded Projects



Conclusions

- PCORI has become increasingly targeted in the funding announcements that it issues
- Within funding announcements, PCORI has refined what we are seeking; trends in the funded portfolio reflect this refinement
- The APDTO advisory panel has been instrumental in helping PCORI define our direction and refine the scope of studies that we are seeking
- The development of the PCS priority topic list and targeted funding announcements have helped to highlight the areas of greatest interest and need to a diversity of stakeholders
- While we have funded studies responsive to many of the topics added to the PCS priority topic list and in the targeted funding announcements, studies have yet to be funded in a handful of topics

What's next?

Questions for discussion

- Now that we have looked at the evolution of PCORI's CER funding programs and have seen the progression from broader to more targeted, what are your views on whether we should become even more targeted in our funding announcements?
- How can we better articulate what we are seeking to the research community?
- Is the priority topic list working? Should we be concerned that we have been unable to attract studies in some priority topics?
- Does any of what was presented here surprise you?

BREAK

10:30 am – 10:45 am



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Topic Discussion:

Comparative Effectiveness of Second-Line Therapies for Patients with Metastatic Colorectal Cancer

Expert:

Brian Wilkinson, MA
ECRI Institute

PCORI Lead:

Sarah Daugherty, PhD, MPH



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Contributors

- ECRI Institute-Penn Medicine Evidence-Based Practice Center
 - Brian Wilkinson, M.A., ECRI Institute
 - Eileen Erinoff, M.S.L.I.S., ECRI Institute
 - Karen Schoelles, M.D., S.M., ECRI Institute
 - Bruce Giantonio, M.D., The Perelman School of Medicine of the University of Pennsylvania
 - Mark O'Hara, M.D., The Perelman School of Medicine of the University of Pennsylvania
 - Ursina Teitelbaum, M.D., The Perelman School of Medicine of the University of Pennsylvania



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Colorectal Cancer

- Approximately 135,000 cases of colorectal cancer will be diagnosed in the United States in 2017.
- Colorectal cancer is the second-leading cause of cancer-death in the United States: approximately 50,000 persons in the United States will die of colorectal cancer in 2017.



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Metastatic Colorectal Cancer

- The 5-year relative survival rate for patients with metastatic colorectal cancer is approximately 14%
- Approximately 30% of patients with colorectal cancer have metastatic disease at the time of initial diagnosis
- Additionally, approximately 50% of patients with colorectal cancer that was diagnosed at a loco-regional stage will develop metastatic disease



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Systemic Therapy for Metastatic CRC

- Chemotherapeutic Agents
 - 5-Fluorouracil
 - Capecitabine
 - Irinotecan
 - Oxaliplatin
- Targeted Agents
 - Antiangiogenic Drugs (Bevacizumab, Ramucirumab, Regorafenib, Ziv-Aflibercept)
 - Anti-EGFR Antibodies (Cetuximab, Panitumumab) for RAS mutation-negative disease only



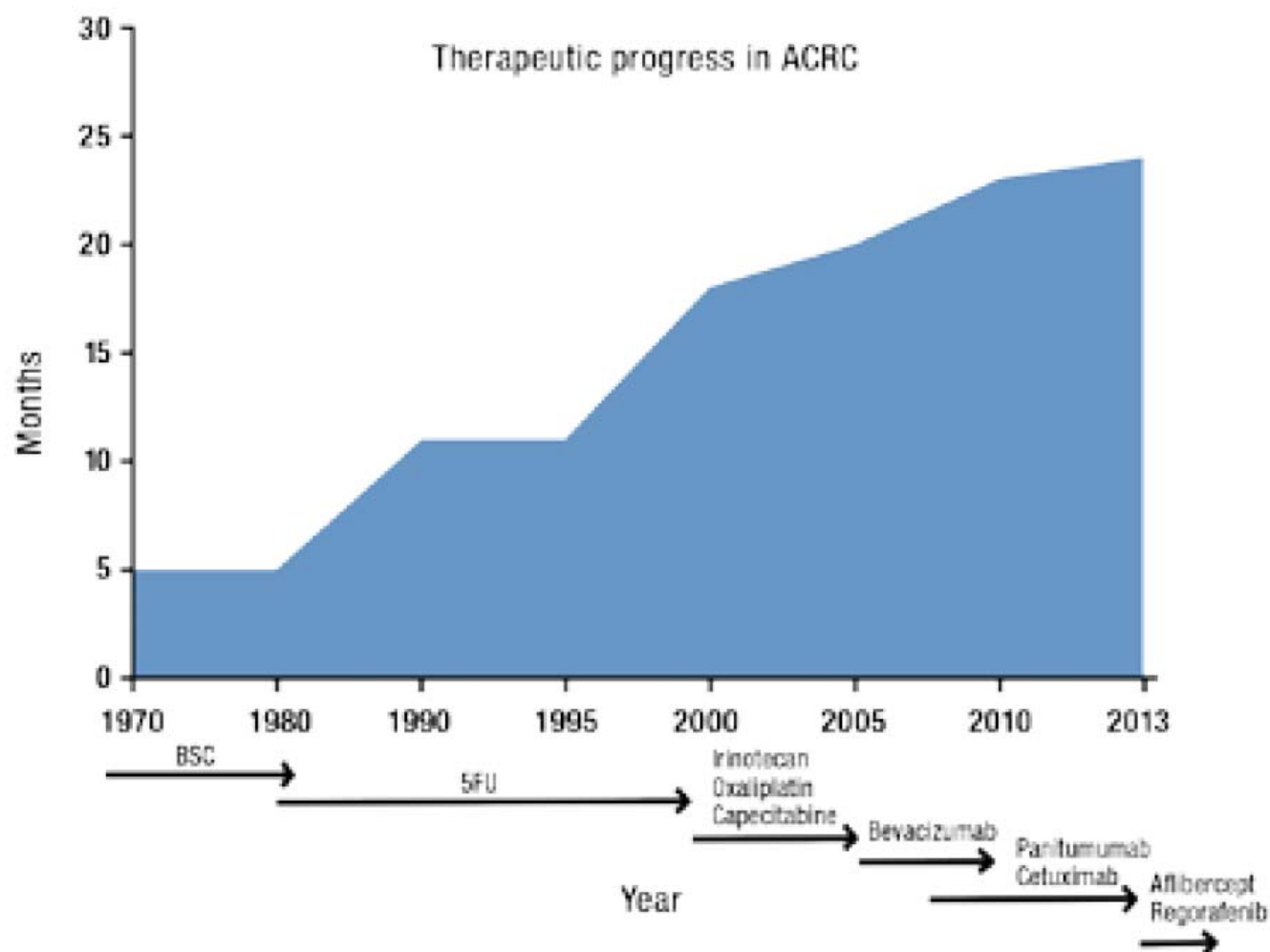
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Second-Line Treatment of Metastatic CRC

- Choice of second-line therapy dependent on treatment received in the first-line setting

1 st Line	FOLFOX + Bevacizumab	FOLFIRI + EGFR Antibody (RAS WT)	FOLFOXIRI
2 nd Line	FOLFIRI + Antiangiogenic	FOLFOX	Regorafenib
3 rd Line	Regorafenib	Trifluridine/ Tipiracil	Trifluridine/ Tipiracil

Progress In Treating Metastatic CRC



Evidence Base in 2nd Line Therapy for mCRC

- A 2017 systematic review by the Cochrane Collaboration summarized data from 34 randomized control trials in the second-line setting. Main conclusions:
 - I. Chemotherapy is more effective than best supportive care
 - II. Modern chemotherapy (e.g., FOLFOX, irinotecan) is more effective than outdated chemotherapy (e.g., 5-FU)
 - III. Irinotecan-based regimens (e.g., FOLFIRI) were more effective than irinotecan alone
 - IV. Targeted agents improve the efficacy of conventional chemotherapy

Evidence Base in 2nd Line Therapy for mCRC

- Cochrane systematic review identified several shortcomings of the data
 - Multiple RCTs testing the same regimens were rarely available for pooling and, therefore, the conclusions address more general questions (i.e., addition of any targeted agent to chemotherapy)
 - Many treatment options have not been studied in head-to-head RCTs, precluding a full ranking of these treatment options. Potential comparisons include:
 - Irinotecan vs. Irinotecan + Bevacizumab
 - FOLFIRI + Bevacizumab vs. FOLFIRI + Ramucirumab vs. FOLFIRI + Ziv-Aflibercept)

Evidence Base in 2nd Line Therapy for mCRC

- Cochrane systematic review identified the following as potential areas of future research
 - Other targeted agents, in particular targeted agents being used successfully against other tumor types should be investigated in the treatment of colorectal cancer
 - Identification of novel biomarkers capable of predicting response to treatment with a given anticancer agent should be pursued
 - Quality of life should be a mandatory outcome included in the design of future oncology clinical trials to formally investigate the balance between survival benefits and treatment-related toxicity



Recent Developments in mCRC - Immunotherapy

- Checkpoint inhibitors appear to have little to no efficacy in the majority of patients with CRC
- Checkpoint inhibitors have demonstrated promising initial results in the approximately 4% of patients with high levels of microsatellite instability (MSI-H)
- Potential research questions include:
 - What is the appropriate setting for use of checkpoint inhibitors in patients with MSI-H tumors?
 - Can use of immunotherapy be extended to MSI-stable patients?



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Recent Developments in mCRC - Sidedness

- Anatomic location of the primary tumor has implications for prognosis and efficacy of certain treatments
- EGFR antibodies may be less effective in right-sided tumors
- Data is largely from first-line setting – National Comprehensive Cancer Network Guidelines now indicates that first-line use of EGFR antibodies be restricted to patients with left-sided tumors
- Potential research questions include:
 - Can these observations can be extended to 2nd-line setting?
 - Do biomarker(s) exist for sidedness?
 - Should future trials stratify patients by primary tumor location?



Recent Developments in mCRC – Extended Testing for EGFR Antibody Eligibility

- Recent analyses have indicated that in addition to RAS mutations (i.e., KRAS and NRAS) other activating mutations (e.g., NRAS, BRAF, PIK3CA) may be negative predictors of EGFR antibody activity
- Potential research questions include:
 - Does extending genetic testing to RAS, NRAS, BRAF, PIK3CA improve patient outcomes?



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Recent Developments in mCRC – Liver-Directed Therapy

- Our searches identified two ongoing trials (NCT01483027 and NCT03069950) of liver-directed therapy used in combination with second-line chemotherapy
- The liver is the most common site for colorectal cancer metastases, and the progression of liver metastases contributes substantially to the morbidity and mortality associated with colorectal cancer
- Potential research questions include:
 - Can liver-directed therapies improve outcomes in appropriately selected patients?



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Recent Developments in mCRC – BRAF Inhibitors

- BRAF inhibitors have demonstrated efficacy in multiple tumor types harboring activating mutations in BRAF
- BRAF mutations are present in ~10% of patients with CRC and are associated with poor prognosis
- Single-agent BRAF inhibitor has not demonstrated efficacy in patients with BRAF mutation-positive mCRC
- Potential research questions include:
 - Whether alternative methods of targeting BRAF mutation-positive CRC can improve patient outcomes (e.g., combining BRAF inhibitors and anti-EGFR antibodies)



Conclusions I

- Colorectal cancer represents the second-leading cause of cancer-related death in the United States. Patients with metastatic colorectal cancer that has progressed following first-line systemic therapy have a median overall survival of approximately 1 year and this stage of the disease can also substantially impact quality of life due to symptoms from disease progression and accumulating treatment-related toxicity



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Conclusions II

- Systemic therapy is the standard of care in the second-line treatment of metastatic disease and multiple accepted treatment regimens are available. Few of the currently accepted treatment regimens have been compared to one another in randomized control trials and, therefore, questions remain regarding the appropriate sequencing of therapies and selection of therapy in the second-line treatment setting.



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Conclusions III

- In addition to questions regarding established therapies for treating colorectal cancer in the second-line setting, substantial interest exists in the development of new treatments for this disease. In particular, the success of immuno-oncology approaches to treating other solid tumors (e.g., lung cancer, melanoma) has created substantial interest using such an approach in colorectal cancer.



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Thank You

- Questions?



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Discussion Reminders

1. Consider the topic with respect to the following:
 - a) Patient-centeredness
 - b) Impact
 - c) Important evidence gap
 - d) Likelihood of implementation in clinical practice
 - e) Durability of information
2. Are there contextual issues that would hinder or facilitate the research?
3. How important is this topic for PCORI to pursue to fund CER?

source: <http://www.pcori.org/research-results/how-we-select-research-topics/generation-and-prioritization-topics-funding-4>



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LUNCH and Recognition of Panel Members

12:15 pm – 1:30 pm



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Recognition of Panel Members Completing Terms as of Spring 2017 Advisory Panel Meeting

Panel Member	Stakeholder Group
Margaret (Mardie) Clayton	Researchers
Regina Dehen	Clinicians
Bettye Green	Patients, Caregivers and Patient Advocates
Bruce Monte	Purchasers
Linda McNamara	Patients, Caregivers and Patient Advocates
James (Jim) Pantelas	Patients, Caregivers and Patient Advocates
Alan Rosenberg	Payers
Angela Smith	Clinicians
Daniel Wall	Patients, Caregivers and Patient Advocates



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**Thank you for your contributions to the APDTO
Advisory Panel!**

Shared Decision Making in the Emergency Department: The Chest Pain Choice Trial

*E Hess, J Hollander, J Schaffer, J Kline, C Torres, D Diercks, R Jones,
K Owen, Z Meisel, M Demers, A Leblanc, N Shah, J Inselman, J
Herrin, A Castaneda-Guarderas, V Montori*



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Disclosures

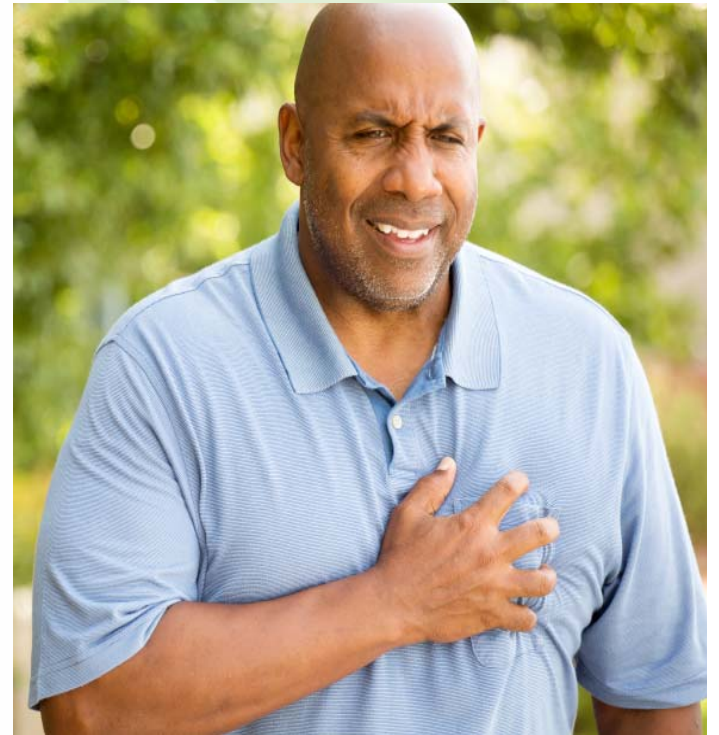
Funded by the Patient Centered Outcomes
Research Institute, contract 952



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Background

- Chest Pain 2nd most common complaint in US EDs
- 1.5% ACS missed
- Low risk patients frequently admitted for cardiac testing
- False positive test results, unnecessary procedures, ↑ cost



**Evidence synthesis
(ACS risk estimation tool)**

**Observations
clinical encounter**

Initial prototype

Designers
Study team
Patients
Clinicians
Stakeholders

Field testing

Modified prototype

Final Decision Aid

Evaluation (trial)



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What's Next?

Prepared for: _____

1 Your Chest Pain Diagnosis

Your initial test results are **NEGATIVE for a heart attack**. These included:

- **Blood tests** to look for an enzyme called troponin that is released when the heart muscle is damaged. Additional troponin tests may be done to monitor you for heart attack during your emergency visit.
- **An electrocardiogram** to check whether your heart is getting enough oxygen and blood.

The chest pain you are experiencing today may be a warning sign of a **FUTURE** heart attack.

2 What You Can Do

Examining your risk will help you and your clinician decide together whether or not you should have additional heart testing.

Additional tests¹ may include:

- **A stress test** which views blood flow to your heart at rest and under stress.
- **A coronary CT angiogram** which takes pictures of the arteries in your heart to check for a blockage in the flow of blood.

¹Stress test options include nuclear stress testing, ultrasound stress testing, or exercise ECG (electrocardiogram) stress testing. Nuclear stress testing and coronary CT angiography include exposure to radiation which has been shown to be related to increased cancer risk over a lifetime. Your doctor can help you explore which option may be best for you.

3 Your Personal Risk Evaluation

Your risk of having a heart or pre-heart attack within the next 45 days can be determined by comparing you to people with similar factors² who also came to the Emergency Department with chest pain.

4 Would you prefer to have additional heart testing during this emergency visit or decide later during an outpatient appointment?

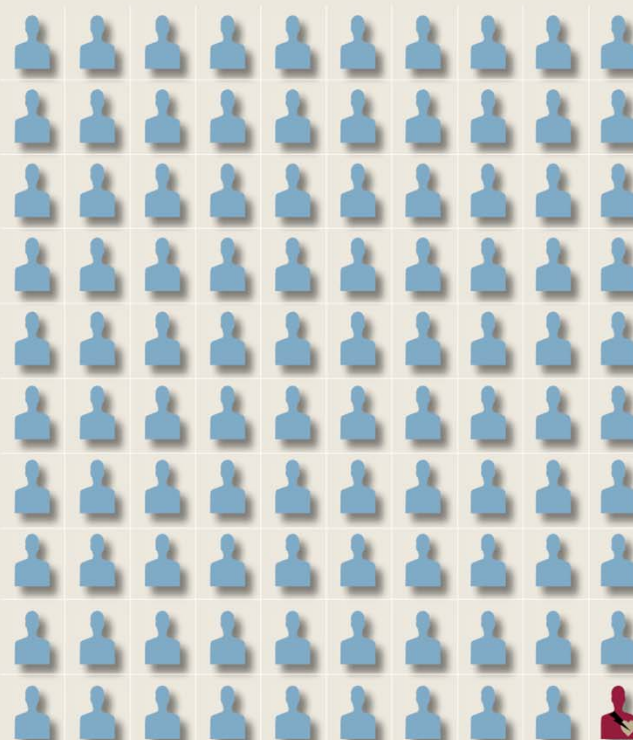
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• Findings on electrocardiograms (electronic tracings of the heart)
• Initial cardiac troponin result

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Prepared for: _____

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2 What You Can Do

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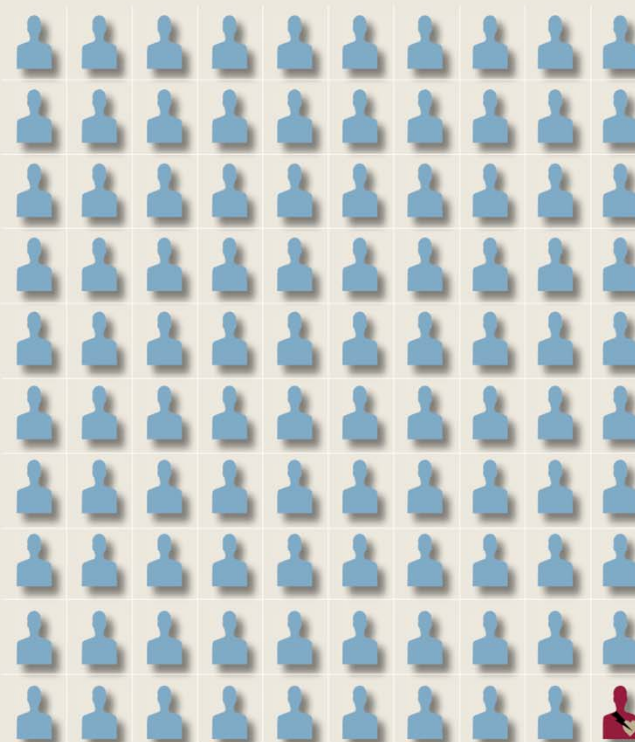
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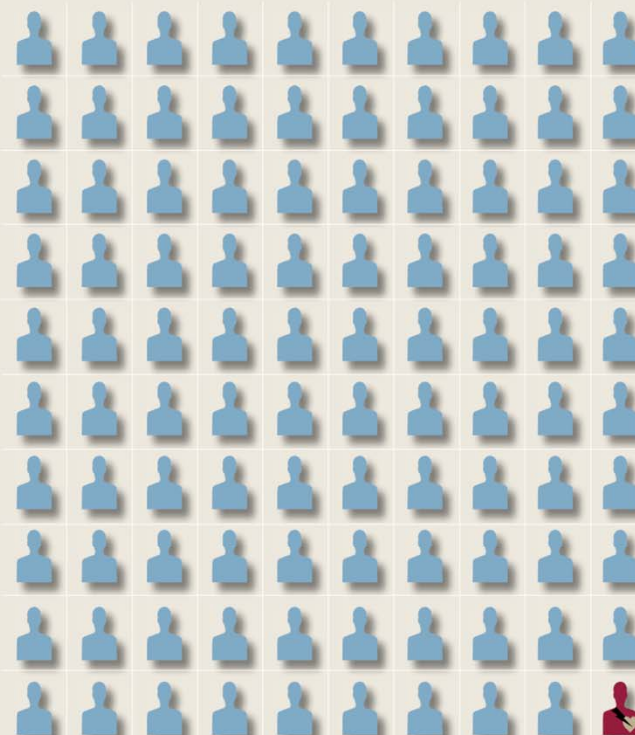


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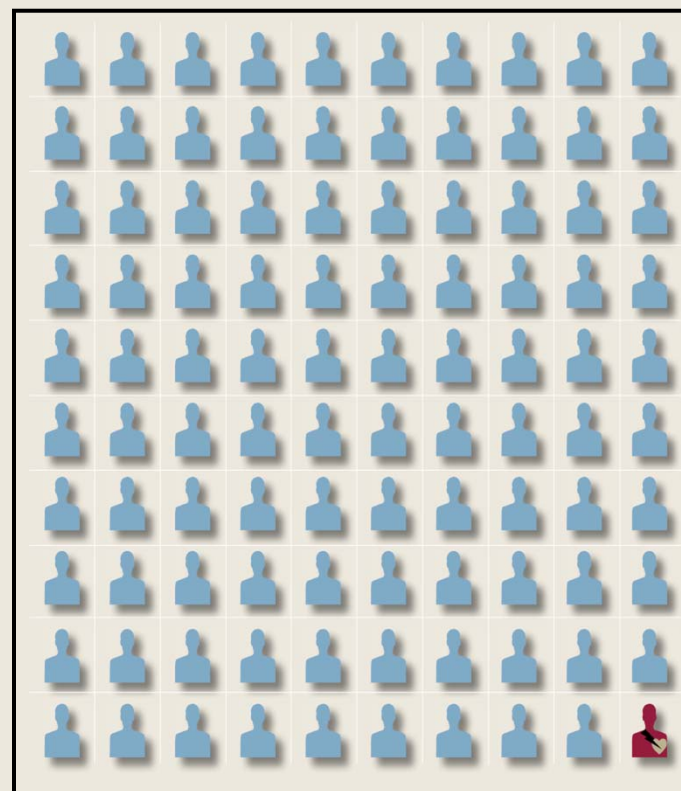
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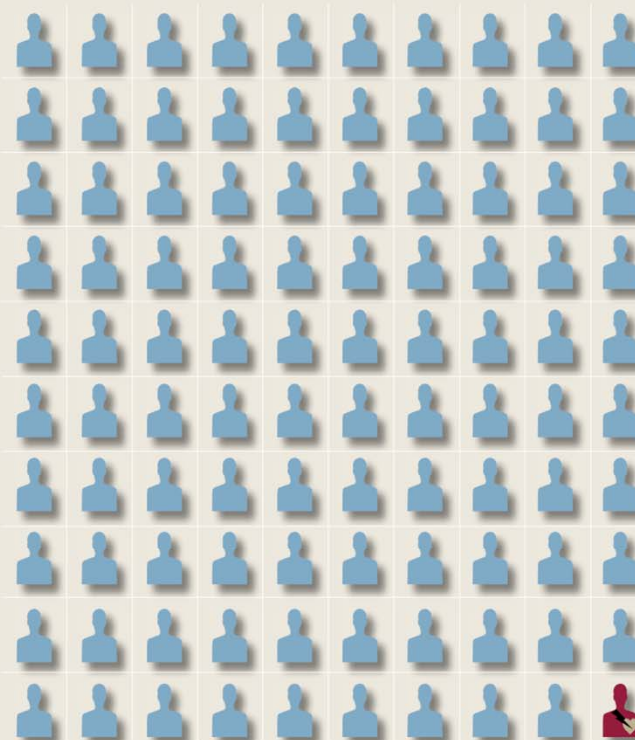
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Chest Pain Choice Pilot Trial

(n=201)

Outcome	Change
Patient knowledge	↑
Patient engagement	↑
Placed in EDOU for stress testing	↓ (19%)
Stress testing within 30 days	↓ (16%)
Provider experience	↑
Outpatient follow-up	↑
Safety	↔



Objectives

Test the effectiveness of Chest Pain Choice in a pragmatic multicenter RCT

Assess the heterogeneity of decision aid effect in potentially vulnerable patient subgroups



Methods



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Design

Patient level RCT

Allocation concealed by password-protected, web-based randomization scheme

Dynamic randomization

1:1 ratio



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Eligibility criteria

- **Inclusion**

- Adults with chest pain considered for EDOU admission for stress testing or coronary CTA

- **Exclusion**

- Ischemic ECG
- Elevated troponin
- Known CAD
- Cocaine use within 72 hours
- Unable to provide informed consent or use DA



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Outcome measures

- Decision quality

Patient knowledge**

Degree of patient participation (OPTION scale)

Acceptability

- CV endpoints

Safety: 30-day MACE

Resource use

- Admitted to EDOU for stress testing or coronary CT
- 30-day rate of stress testing/coronary CT



Heterogeneity of Decision Aid Effect

- Dichotomized patient characteristics
- Tested for interactions between each dichotomized patient characteristic and trial outcomes
 - Regression models included indicators for arm assignment and study site
- Replicated main trial analysis within each subgroup and tested whether the interaction differed significantly from zero

Results



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Baseline characteristics

Variable	Control (n=447)	Intervention (n=451)	P-value
Mean age	50.6	50.0	0.57
Female	58%	56.7	0.41
HTN	55%	1.0	0.70
Dislipidemia	69%	56.9	0.07
Family history of premature CAD	59%	25.4	0.62
Mean PTP of ACS	3.8%	3.6	0.46

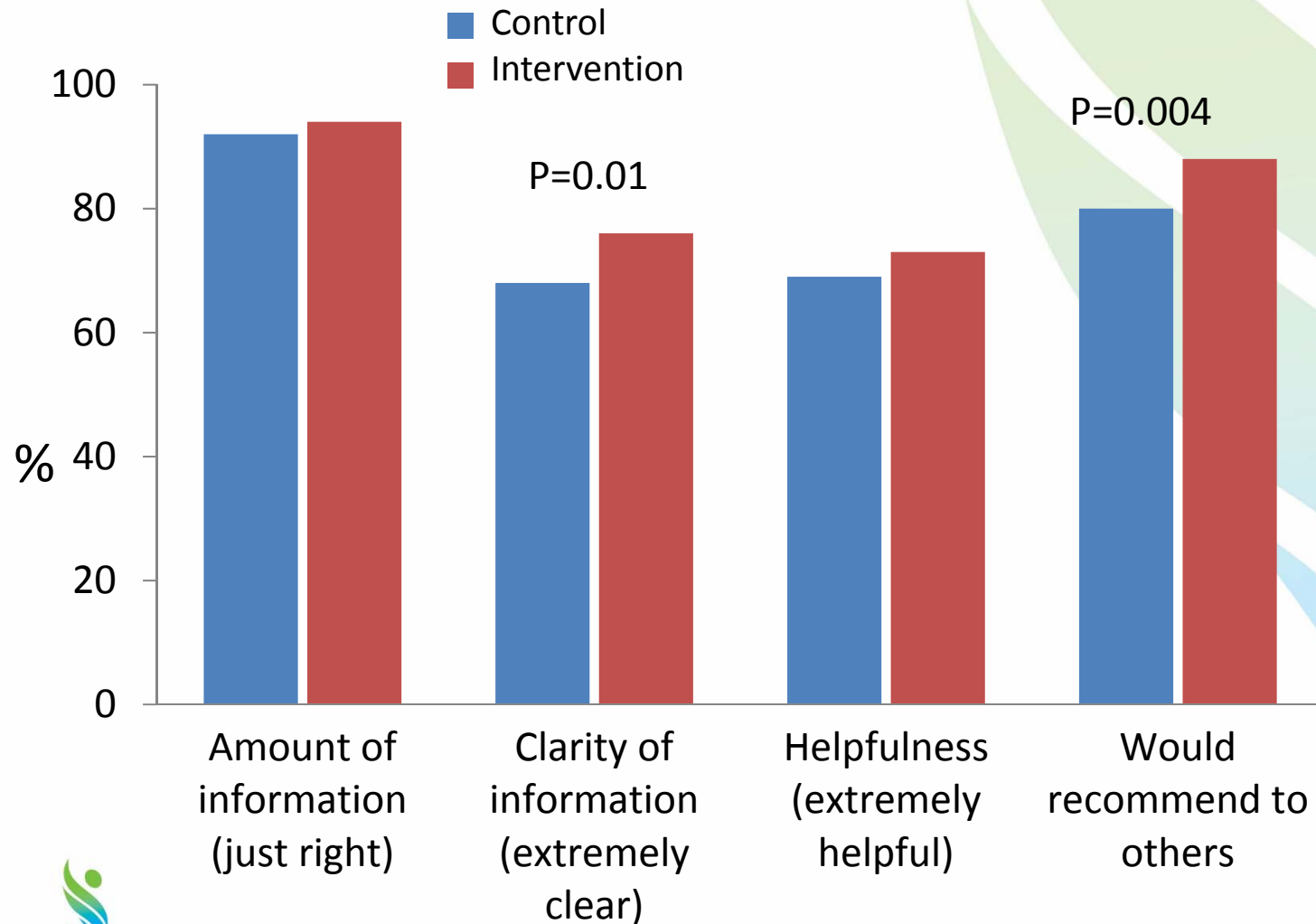
Knowledge and Engagement

Variable	Control (n=447)	Intervention (n=451)	P-value
Knowledge [Mean (SD)]	3.56 (1.50)	4.23 (1.54)	<0.001
Engagement (Option scale)	8	18	<0.001

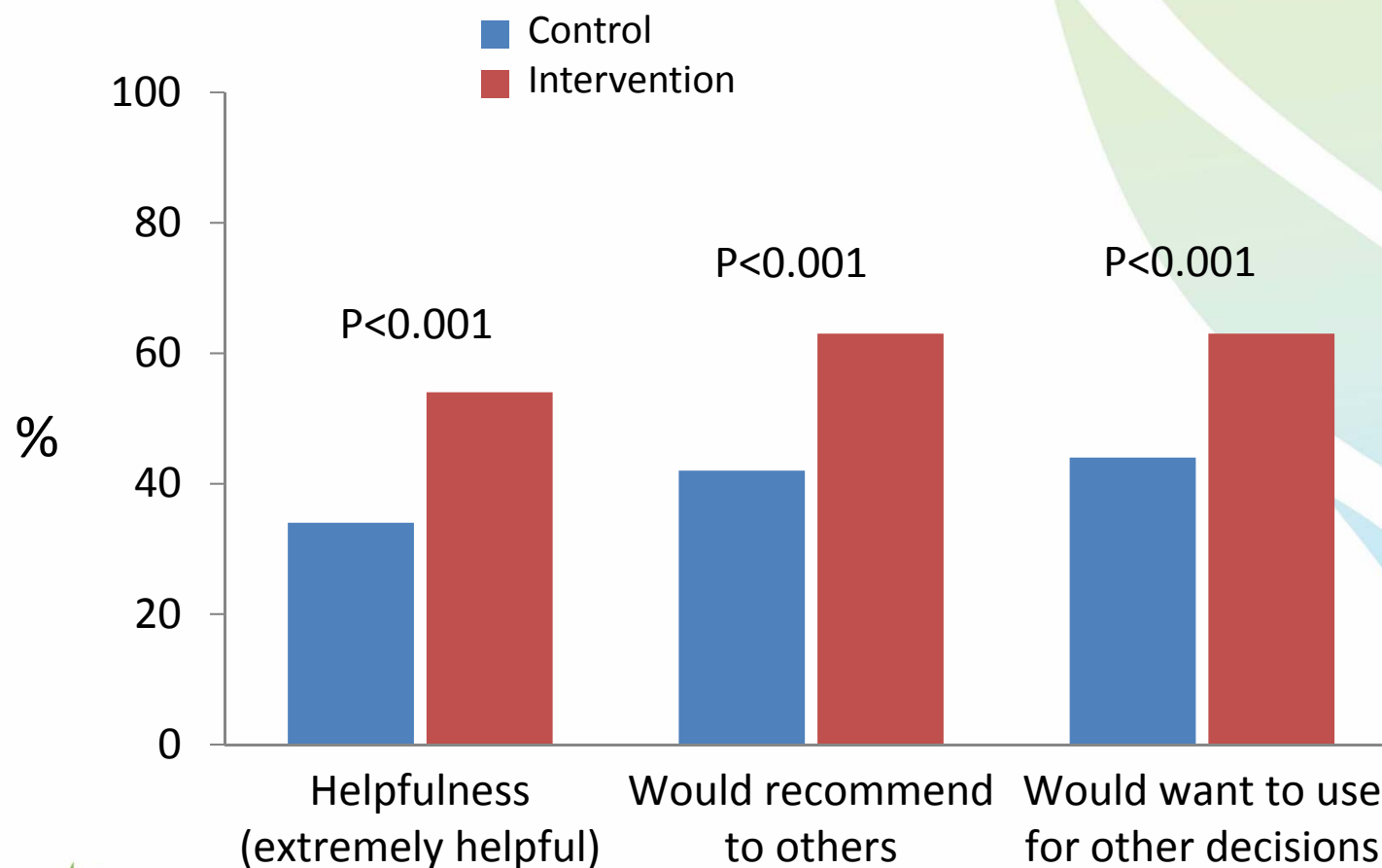


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Decision aid acceptability (patient)



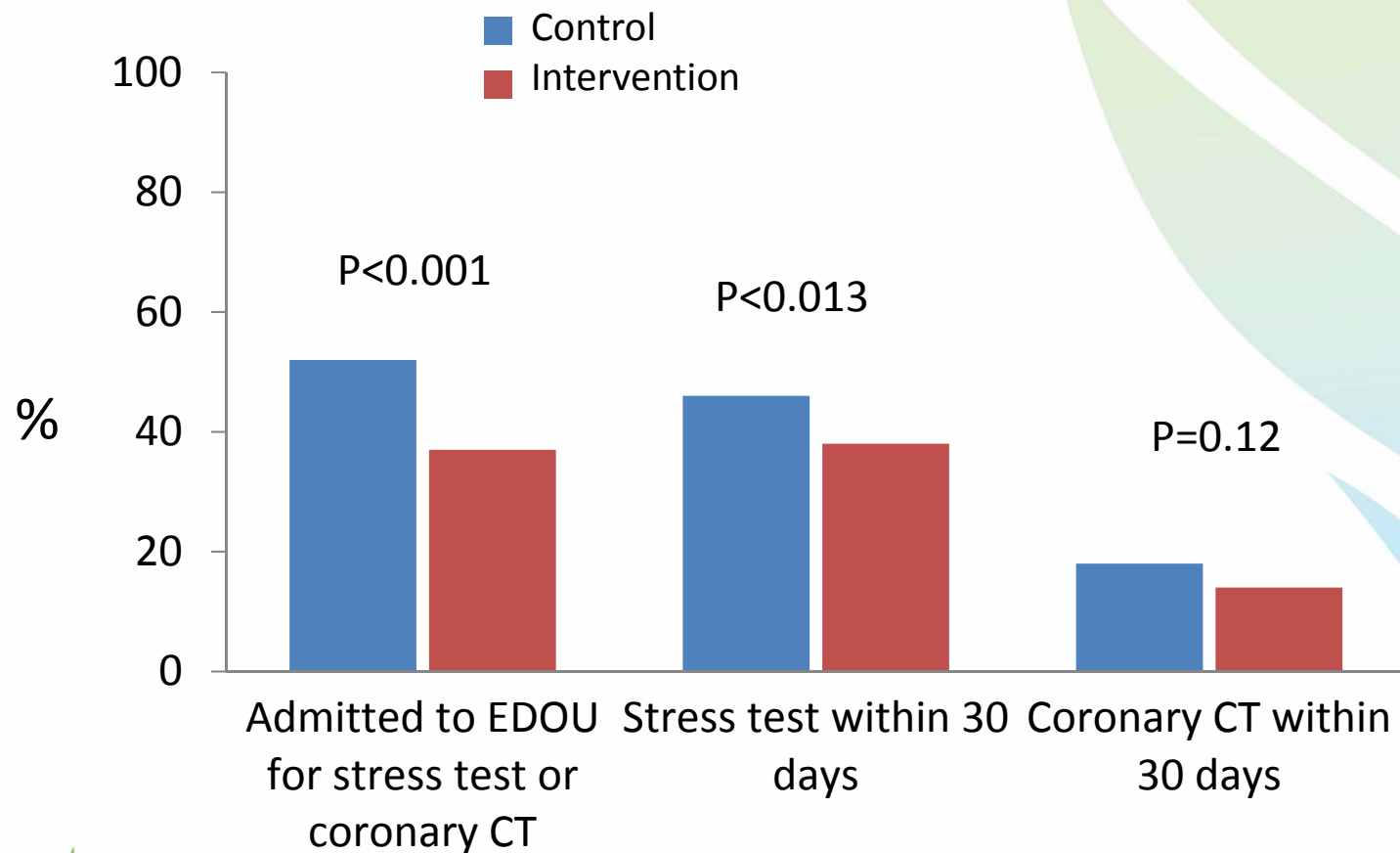
Decision aid acceptability (clinician)



Safety

Variable	Control (n=447)	Intervention (n=451)	P-value
Revascularization	4 (1%)	7 (2%)	0.37
MI	1 (0%)	4 (1%)	1.0
Death	0 (0%)	0 (0%)	1.0
MACE within 30 days of discharge	0 (0%)	1 (0%)	1.0

Resource Use



Results of Interaction testing

- Decision quality
 - Patient knowledge
 - Patient participation (OPTION scale)
 - Physician trust
 - Acceptability
- CV endpoints
 - Safety: 30-day MACE
 - Resource use
 - Admitted to EDOU for stress testing or coronary CT
 - 30-day rate of stress testing/coronary CT

Figure 1 - Knowledge (%) Subgroup Effects

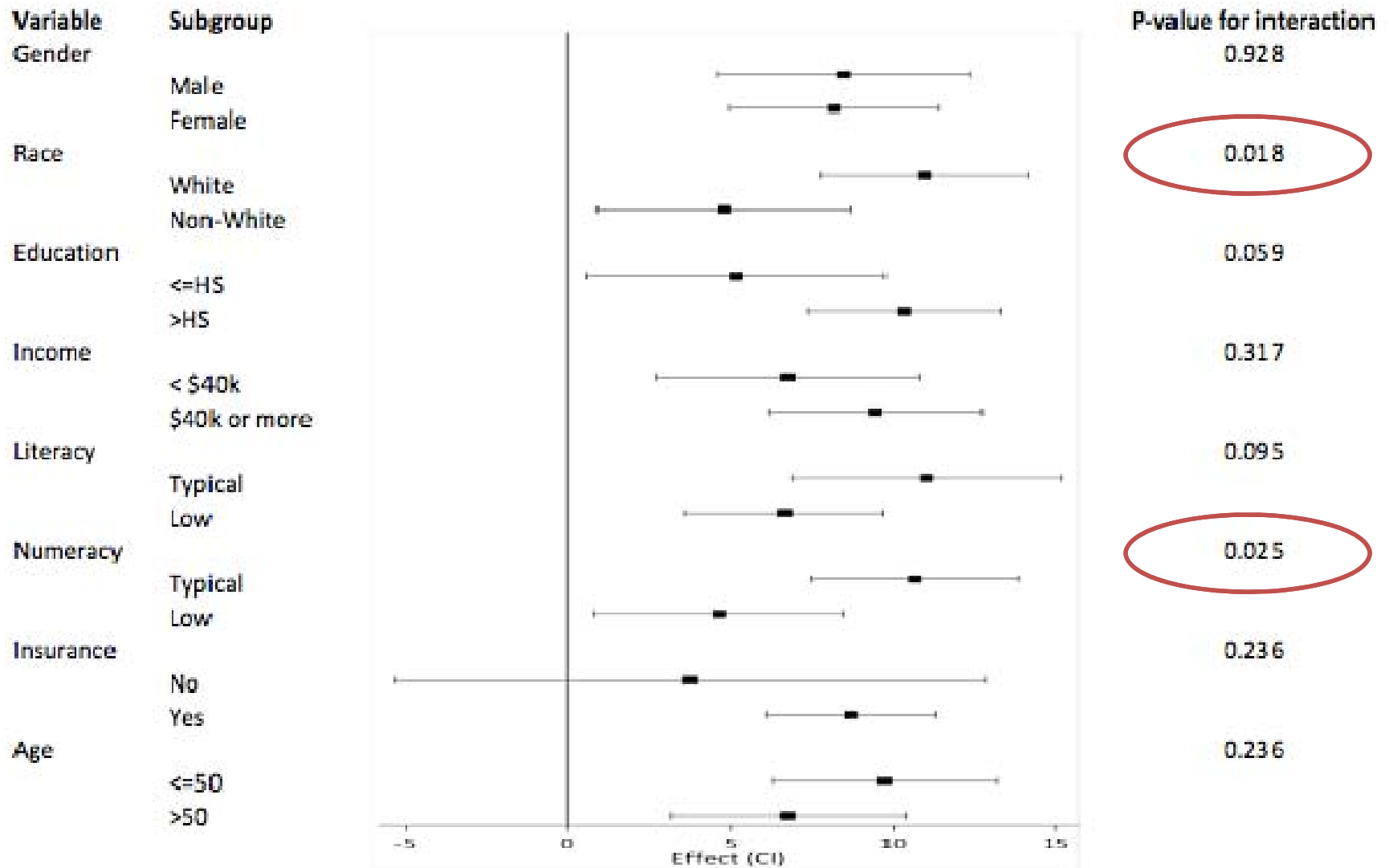


Figure 2 - Trust in Physician Scale Subgroup Effects

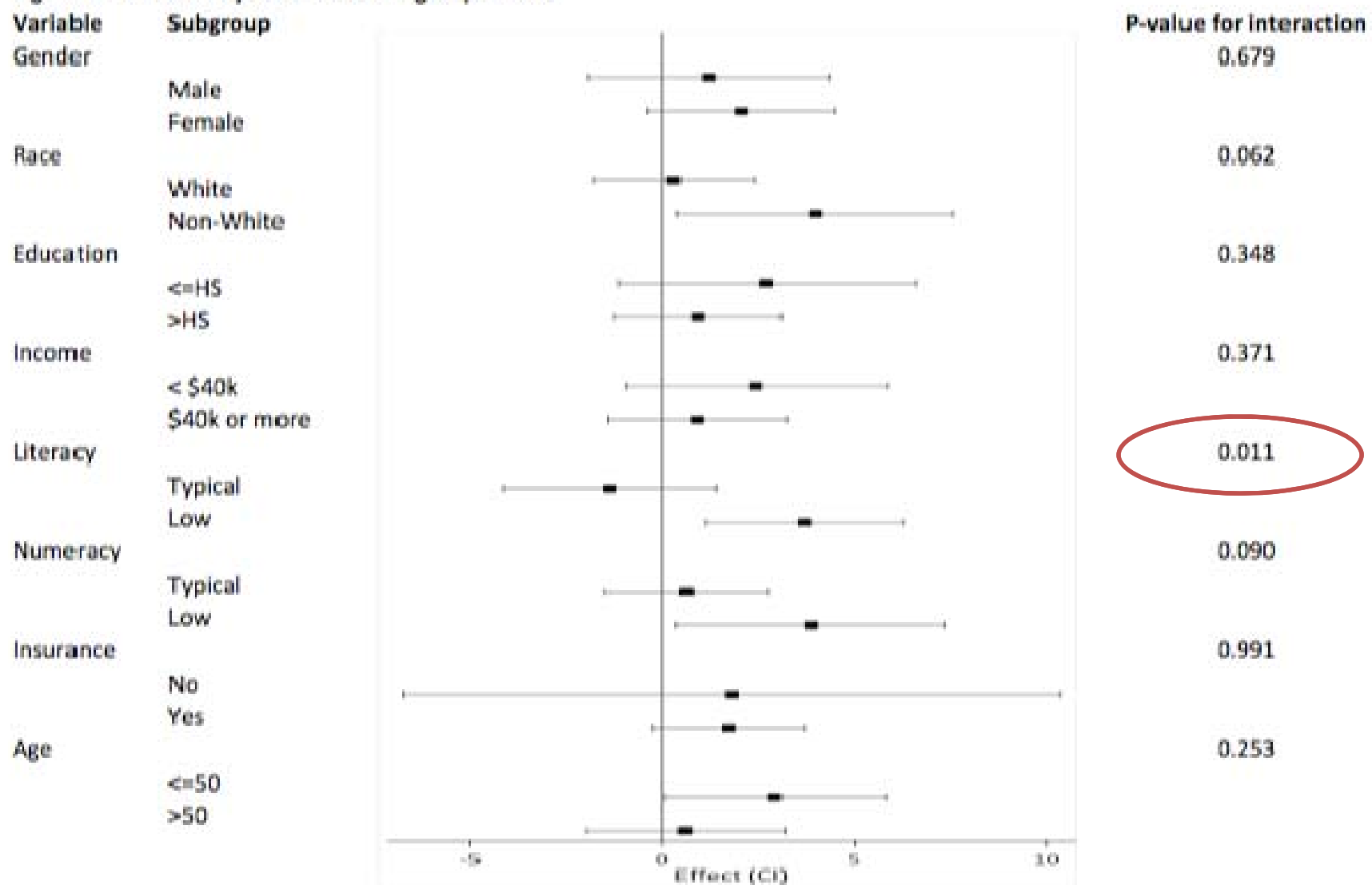
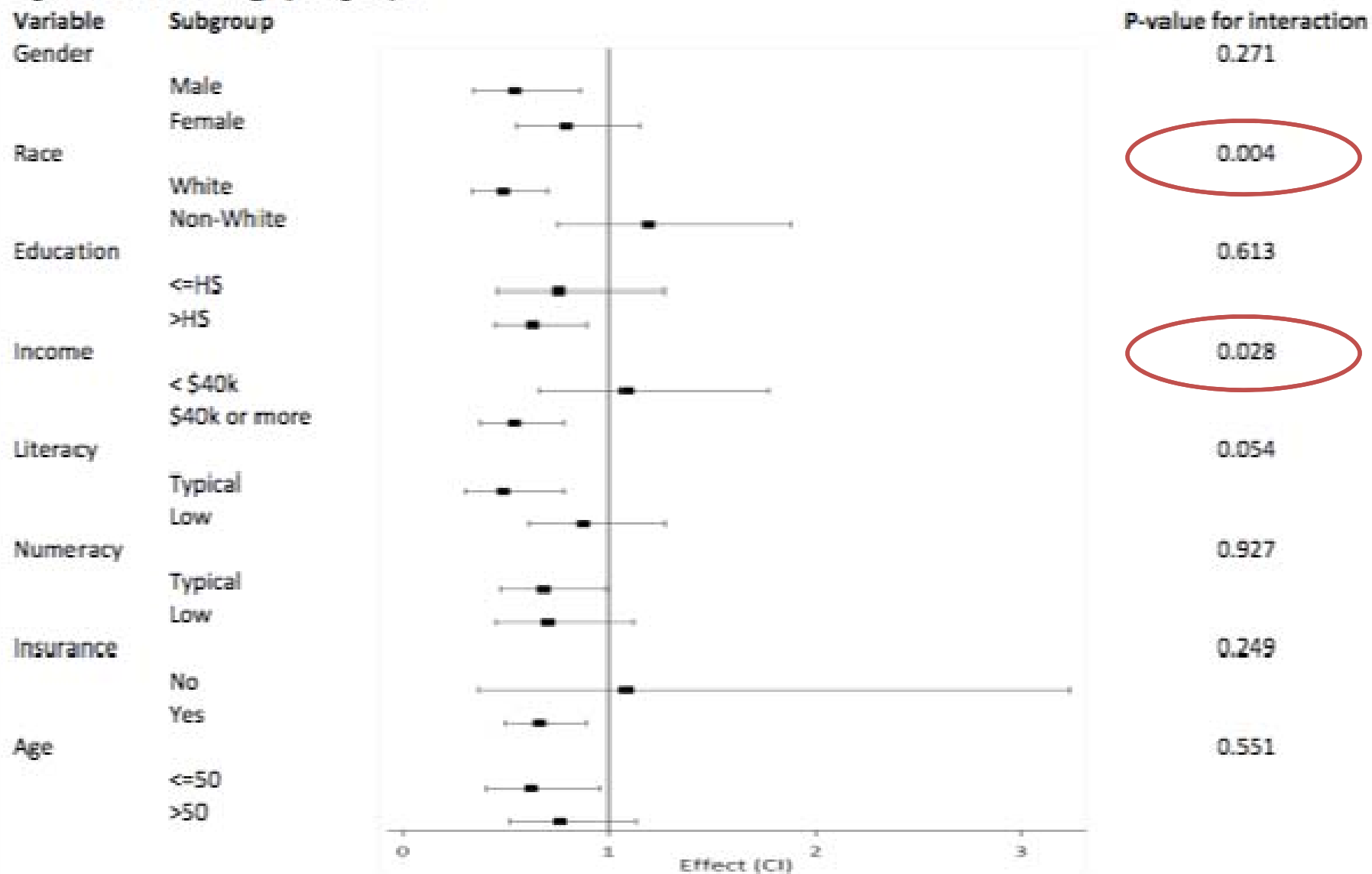


Figure 3 - Stress testing by subgroups



Limitations

- Limited power to demonstrate safety
- Multiple testing: $80 \text{ comparisons} \times 0.05 = 4$
Pre-specified hypotheses
Cautious interpretation of results
- Imprecision around subgroup effects
Still the largest shared decision making trial to date
(N in meta-analysis of 7 RCTs = 771)



Conclusions

Chest Pain Choice

- Increased patient knowledge and engagement
- Acceptable to patients and clinicians
- Safely decreased resource use
- Benefited all sociodemographic groups to a similar extent
 - ↑ knowledge transfer with higher numeracy
 - ↑ physician trust with low health literacy

Next Step: Dissemination and Implementation



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Acknowledgements

- Patient Centered Outcomes Research Institute
- Participating clinicians
- Data safety monitoring board



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Dissemination & Implementation Program Updates

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

May 2017

Joanna Siegel ScD
Director, Dissemination & Implementation



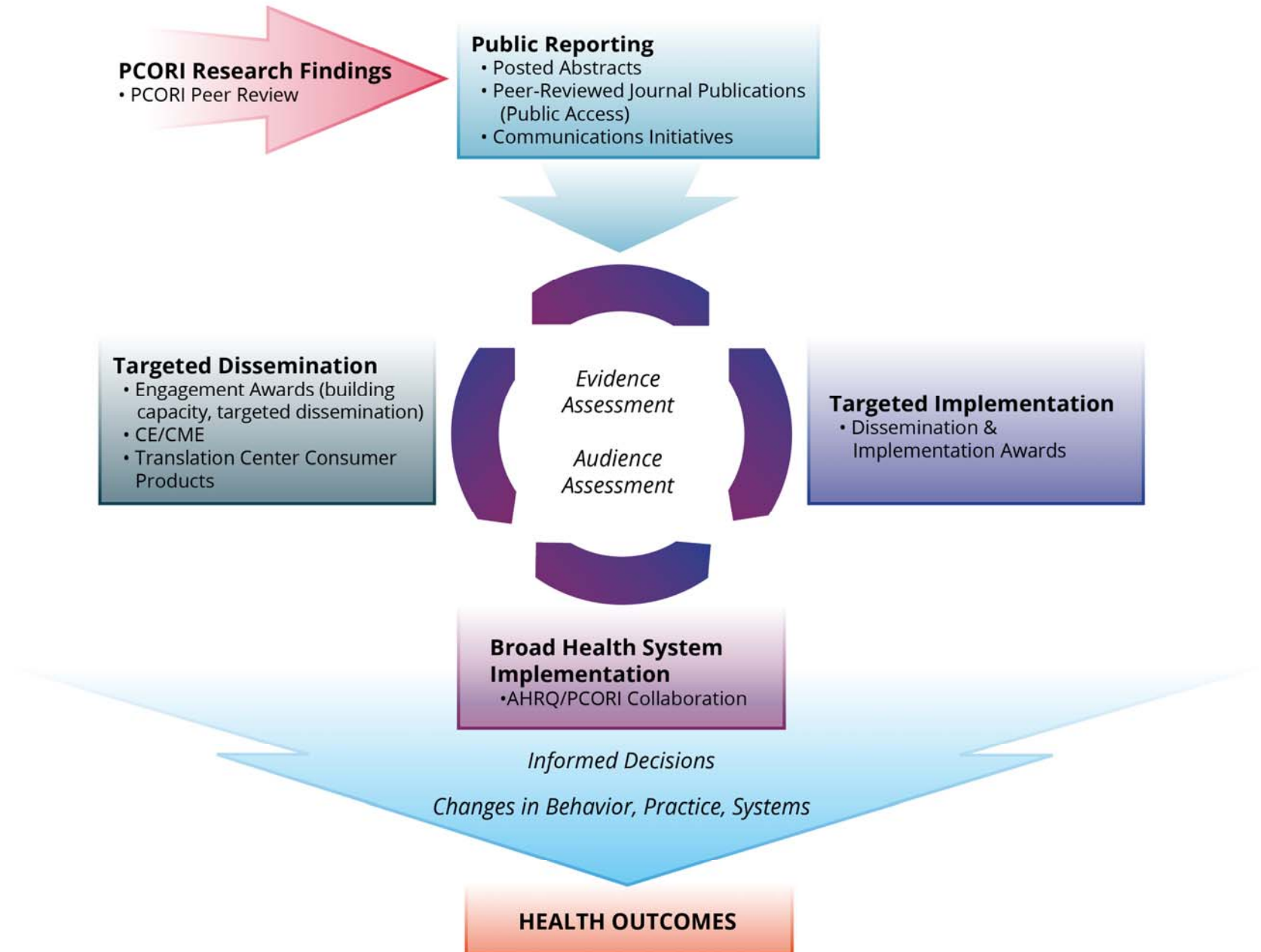
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PCORI Dissemination & Implementation Program

- The D&I Program is charged with heightening awareness of the results of PCORI-funded research, and with advancing efforts to put these findings into practice to improve healthcare delivery and health outcomes.



PCORI DISSEMINATION & IMPLEMENTATION OF RESEARCH RESULTS



Today

- Public Reporting Activities Updates
- Updates on Targeted Implementation -- D&I Award Program

Meeting PCORI's Public Reporting Mandate



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Mandated Public Reporting of PCORI Research Findings

PCORI's [authorizing language and the processes adopted by the Board](#) outline approach for releasing findings.

- Post to pcori.org within 90 days of PCORI's acceptance of the draft final research report following peer review:
 - 500-word **public abstract**
 - 500-word **professional abstract**

Promotes accessibility and comprehensibility of research findings.

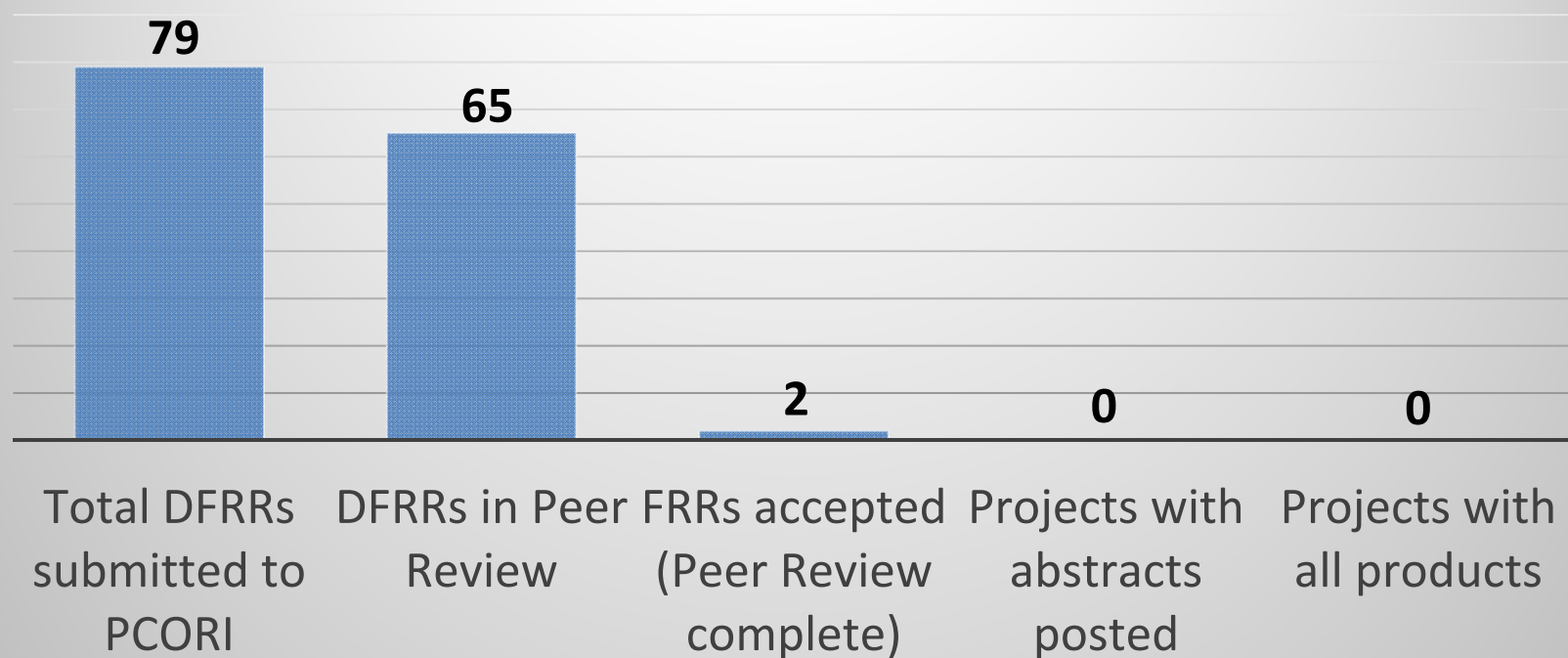
Assures full transparency in reporting results from all PCORI studies

Public and Professional Abstracts for Primary Research Results

- PCORI's Translation Center completed templates for these abstracts December 2016.
 - Cognitive testing included patients/consumers, clinicians, and other PCORI stakeholders.
- Translation Center is preparing drafts of abstracts for the first submitted research findings.
- Abstracts will be finalized when peer review is complete.

Public Release of PCORI Research Findings

Public and Professional Abstracts



Current as of May 12, 2017

Posting Primary Study Results to PCORI.ORG

pcori Patient-Centered Outcomes Research Institute

BLOG NEWSROOM SUBSCRIBE CAREERS CONTACT

ABOUT US FUNDING OPPORTUNITIES RESEARCH & RESULTS GET INVOLVED MEETINGS & EVENTS

See all PCORI completed projects

This project has results available

Comparing Oral to IV Antibiotics for Children With Serious Infections

Public Abstract Audio (English) Resumen en Español Professional Abstract

For a downloadable version of the abstract, click here.

What was the research about?

Researchers studied whether children who have been in the hospital with serious infections do better when they go home with antibiotics by mouth or by IV.

What did the research team learn?

Both ways of delivering antibiotics work about the same at treating infection. Some children who had antibiotics by IV had problems with the IV equipment, not the medicine. These children were more likely to come back to the emergency room or stay in the hospital again because of those problems. Children who took antibiotics by mouth had fewer problems than those who got antibiotics by IV.

How can these results help people make better choices?

When children have serious appendicitis, pneumonia, or bone infections, their families and doctors can use this information to decide which way to give antibiotics after the children leave the hospital.

If your child is in the hospital for serious appendicitis, pneumonia, or bone infections, talk with your doctor about treatment plans for when your child goes home. You may have a choice of antibiotics by mouth or IV.

Who was in the study?

Researchers looked at health records for more than 8,600 children and teens between the ages of 2 months and 18 years.

Read More

Project Information

Principal Investigator	Other Principal Investigator
Ron Keren, MD, MPH	John Smith, MD, MPH
Project Title	Project End Date
Comparative Effectiveness of Intravenous vs. Oral Antibiotic Therapy for Serious Bacterial Infections	Apr 2016

pcori PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

ABSTRACT

PROJECT INFORMATION PUBLISHED DECEMBER 2016

Comparing Antibiotics by Mouth or IV for Children with Serious Infections

Principal Investigator: Ron Keren, MD, MPH Organization: The Children's Hospital of Philadelphia State: Pennsylvania

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WHAT DID THE RESEARCH TEAM DO?

The researchers looked at the health records for children who had been in the hospital for serious

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WHAT WERE THE LIMITS OF THE STUDY?

Patients might not have gone back to the hospital where they were treated the first time. Researchers may have missed health problems if children went back to a different hospital.

There are many types of oral and IV antibiotics. Not all hospitals use the same antibiotics to treat infections. Some antibiotics might work better than others. In the future, researchers could compare different types of antibiotics to see if one works better than others.

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Web Analytics

- Number of times the page was viewed
- Number of unique visitors to the page
- How long visitors stayed on the page
- What promotional mechanism drove the visitors to the page (email, social media, search, direct traffic, etc.)
- Visitors clicking on outbound links, including literature links within the "More on this Project" section
- Use of the “social share” functionality to print, email, or share the page on social media (Twitter, Facebook, LinkedIn, etc)
- Clicks on the public, professional, and Spanish abstract tabs
- Number of PDF downloads and audio plays
- How far users advance through the page either by scrolling down the page manually or clicking within the right-hand menu
- How many users click on “Read more”/“Read less” link
- Clicks on the “see all projects with results posted” button



Other products in process

For Primary Findings:

- Downloadable versions of public abstract
- Spanish and audio versions of public abstract
- High-level summary of peer review comments

For Pilot projects:

- Public and Technical versions

For Ongoing Research:

- Revised summaries of ongoing PCORI research on the website



Revising the Project Summaries

Improving consistency, comprehensibility, and accuracy of **ongoing project** summaries

Project Summary

What is the research about?

Who can this research help?

What is the research doing?

Research methods at a glance

- First revised summaries are posted



PCORI Dissemination and Implementation Awards (Limited Competition)

Key Information

Full Announcement: Dissemination and Implementation of PCORI-funded Patient-Centered Outcomes Research Results

Purpose: Offer PCORI awardee teams an opportunity to propose *investigator-initiated* strategies for disseminating and implementing their research results.

Eligibility: Current Awardee; *draft final research report submitted*

Funding Level: \$350,000 total direct costs. Greater budget levels may be considered with appropriate justification.

Letters of Intent: Competitive

Project Period: 2 years. Longer projects may be considered with appropriate justification.

First Awards Announced: Dec 2016

Funding Cycles Per Year: 3

Purpose and Objectives of the Award Program

D&I Awards are designed to give PCORI awardee teams an opportunity to:

- Propose **investigator-initiated** strategies for disseminating and implementing findings from their PCORI-funded studies
- Undertake the **next step(s)** for making their research results more useful, actionable, and accessible to targeted end users
- Promote and facilitate the **effective and timely uptake** of important research evidence in practice



Supported D&I Project Approaches

- **Develop and demonstrate approaches** for incorporating PCORI research results in specific decision-making settings.
- **Adapt** the content, format, or vehicle for delivering PCORI findings for different populations and/or across different settings.
- Take programs and products found effective **to scale** in diverse settings and populations.

PCORI Dissemination and Implementation Awards (Limited Competition)

To date

- **2 Projects Awarded December 2016**
- **3 Projects Awarded March 2017**

Next awards

- **September 2017**

Preventing Venous Thromboembolism (VTE) in Hospitalized Patients

Original PCORI Study tested a patient education intervention to prevent VTE in hospitalized patients. *Aug 2013 (APDTO CY2; \$1,536,559).*

- Real-time EMR alert notified a health educator immediately when a patient missed a dose of VTE prophylaxis
- Health educator provided one-on-one, face-to-face education about risks of VTE and potential benefit from prophylaxis.

PCORI Study Findings

- The intervention led to a 57% reduction in non-administration (missed doses and refusals) of VTE prophylaxis across intervention floors ($p < .001$).



- AHRQ has called VTE prevention the number one strategy to improve patient safety in hospitals.
- Proper administration of VTE prophylaxis is associated with reduction in VTE risk.
- Omitting even a single dose of VTE prophylaxis is associated with an event.

*Elliott Haut, MD,
Johns Hopkins University
Baltimore, MD*

*Dissemination & Implementation of PCORI Funded
Patient-Centered Outcomes Research Results and
Products, awarded December 2016*

Preventing Venous Thromboembolism in Hospitalized Patients

Dissemination & Implementation Project: Preventing VTE: Engaging Patients to Reduce Preventable Harm from Missed/Refused Doses of VTE Prophylaxis

Aims

- Implement intervention in
 - all floors of a large teaching hospital (Johns Hopkins)
 - a medium-sized, community, non-teaching hospital (Howard County General)
- Examine effect of VTE prophylaxis for inpatients at both hospitals

If successful, this D&I project will result in

- Improved quality of patient-nurse communication about VTE and VTE prophylaxis.
- More informed patient decisions regarding the choice to take VTE prophylaxis.
- Decreased VTE events; decreased mortality and morbidity (heart attack, stroke, organ damage) associated with VTE events

Evaluation Plan

- Measuring rates of missed doses, patient refusal, and VTE events
- Will capture VTE rates in hospital and 30 days post-discharge through diagnosed VTE in 2 hospital EDs, readmissions, 38 outpatient clinics, and other sources.

A Virtual Care Model for Parkinson Disease Specialty Care

Original PCORI Study evaluated the feasibility, effectiveness, and satisfaction associated with telehealth care visits for patients with Parkinson Disease.

PCORI Study Findings

- Telehealth visits successfully delivered: 98% of study patients had 1 or more video house calls.
- Intervention group spent less time on appointments and more time interacting with a doctor ($p < 0.01$).
- 95% of patients were “satisfied” or “very satisfied” with the care, convenience, comfort, and overall quality of the video house calls.
- No significant differences in quality of life, quality of care, or caregiver strain for intervention group versus control.



- Telehealth is growing rapidly; has the potential to improve access to care and reduce health care costs.
- Over 40% of Medicare beneficiaries with Parkinson Disease do not receive care from a neurologist within four years of diagnosis, increasing their risk for morbidity, loss of independence, and death.

*Earl “Ray” Dorsey, MD, MBA
University of Rochester
Rochester, NY*

*Dissemination & Implementation of PCORI Funded
Patient-Centered Outcomes Research Results and
Products, awarded March 2017*

A Virtual Care Model for Parkinson Disease Specialty Care

Dissemination & Implementation Project: Dissemination and implementation of a virtual care model for Parkinson disease and other chronic conditions

Aims

- Refine and expand the telehealth intervention to include multidisciplinary care and address comorbid conditions (anxiety, depression, dementia).
- Implement the revised model into a funded statewide telemedicine program that will provide care to 400-500 individuals with Parkinson Disease.

If successful, this D&I project will

- Increase access to multidisciplinary care for individuals with Parkinson Disease.
- Assess effectiveness of telehealth program as a viable option for providing care for people with restricted access to in-person health care.

Evaluation Plan:

- In addition to patients reached, sites providing the service, and other measures of program implementation, will examine clinical outcomes, quality of life, caregiver burden, and other patient-centered outcomes.

Targeting Interventions to Prevent Diabetes to Patients at Higher Risk

Original PCORI Study assessed heterogeneity of treatment effect in clinical trials. Researchers analyzed individual patient data from 32 studies including the 2002 Diabetes Prevention Program Study.

PCORI Study Findings

- Baseline risk for developing diabetes varies dramatically. Some patients had a 1-2% risk of developing diabetes within 3 years; the risk was 90% for others.
- Low-risk patients showed little benefit from interventions (metformin; lifestyle modification) in the Diabetes Prevention Program Study
- High-risk patients showed significant benefit from these interventions.



- Pre-diabetes affects approximately 86 million people in the US.
- For every patient screened for diabetes who's identified as being diabetic, screening also identifies 3 patients with pre-diabetes.
- The main interventions for pre-diabetes are pharmacotherapy with metformin and an intensive lifestyle program.

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Targeting Interventions to Prevent Diabetes to Patients at Higher Risk

Dissemination & Implementation Project: Improving Diabetes Prevention with Benefit-Based Tailored Treatment: Disseminating Patient-Centered Estimates of Benefit

Aims

- Adapt and incorporate the prediction model based on the Diabetes Prevention Program Study into an EHR-based risk-prediction tool that clinicians can access at the point of care
- Partner with American Medical Group Association (AMGA) to launch the EHR tool in 50 clinic sites within two AMGA-member health care provider organizations.

If successful, this D&I project will:

- Help clinicians triage costly and potentially burdensome preventive interventions to patients with prediabetes based on their risk for developing diabetes, improving the appropriateness of care at all levels.

Evaluation Plan:

- Will assess use of the EHR-based tool, the rate clinicians preferentially refer prediabetic patients at high risk to Diabetes Prevention Program interventions, and patients' acceptance/adherence to their prescribed interventions.



Questions?



Wrap Up

- Next in-person meeting will occur in October 2017
- Questions/Comments?



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Thank you for your participation

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

May 25, 2017



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