



What Should PCORI Study? A Call for Topics from Patients and Stakeholders

December 4, 2012

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

Patient-Centered Outcomes Research Institute



**Joe Selby, MD, MPH,
Executive Director
PCORI**

*What Should PCORI Study? A Call for Topics from Patients
and Stakeholders*

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

Patient-Centered Outcomes Research Institute

Mission

The Patient-Centered Outcomes Research Institute (PCORI) helps people make informed health care decisions, and improves health care delivery and outcomes, by producing and promoting high integrity, evidence-based information that comes from research guided by patients, caregivers and the broader health care community.

PCORI's Board of Governors Represents the Entire Health Care Community



PCORI Board of Governors, March 2012 in Baltimore, MD

Unique role of stakeholders



PCORI's Two Paths to “Getting Specific” in Research Funding

Figure 1: Traditional Process

Investigator-Generated Research Just One Part of the Process

PCORI issues broad funding announcements

Researchers partner with stakeholders to generate questions

Researchers, stakeholders apply review criteria
in their applications

Peer review prioritizes
applications by level
of alignment
with criteria

Diverse Research Portfolio answering key questions
for patients and clinicians

PCORI's Two Paths to “Getting Specific” in Research Funding

Figure 2: Unique Process

Patient/Stakeholder-Led Approach

PCORI and stakeholders generate and prioritize questions based on review criteria

PCORI issues specific, funding announcements for highest priority topics

Researchers and stakeholders develop responsive proposals

Peer review prioritizes applications by level of alignment with criteria

Diverse Research Portfolio answering key questions for patients and clinicians

It Begins with You



www.pcori.org/questions

[About Us](#) [What We Do](#) [Funding Opportunities](#) [Meetings & Events](#) [Get Involved](#) [News Room](#) [Employment](#)

[Opportunities for Public Comment](#) | [PCORI Reviewers: Help Us Evaluate Research Funding Applications](#)

[Suggest a Patient-Centered Research Question](#) |

Is your question about a certain disease, behavior or health condition?

If yes, please name the disease, behavior or health condition. If no, leave blank and continue.

What is your question?



PCORI's First Targeted Research Topics

- Identified several high-priority, stakeholder-vetted topics for targeted PFAs
- Jumpstarts PCORI's long-term topic generation and research prioritization effort
- Leverages stakeholder input from before PCORI's existence
- Allows us to build on our engagement work

Research Topics:

Treatment Options for Uterine Fibroids

Safety and benefits of treatment options for severe asthma

Fall Prevention in the Elderly



**Susan Hildebrandt, MA
Director of Stakeholder Engagement
PCORI**

*What Should PCORI Study? A Call for Topics from
Patients and Stakeholders*

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

Patient-Centered Outcomes Research Institute

Engagement

Patients and Stakeholders

Tell Us What PCORI Should Study

What questions are most important?
(research prioritization)

What outcomes should be studied?
(topic generation)

Review Proposals and Partner in Research

Review research proposals for impact and patient-centeredness

Participate in conducting research

Tell Us How We Are Doing

How can we improve on what we are doing and how we are doing it?

Help Us Share the Findings

How do we best communicate important research findings?

Tell Us What to Study



Transforming
Patient-Centered
Research: Building
Partnerships and
Promising Models

- October 27-28, 2012
- Washington, DC

What Should
PCORI Study? A
Call for Topics from
Patients and
Stakeholders

- December 4, 2012
- Washington, DC

PCORI
Methodology
Workshop for
Prioritizing Specific
Research Topics

- December 5, 2012
- Washington, DC

Review PCORI Funding Applications

- Help PCORI review PCORI Funding Applications
- Apply to be a Stakeholder or Scientific Reviewer
- pcori.org/get-involved/reviewers



Form Research Partnerships

The engagement of patients and stakeholders should include:

- Participation in formulation of research questions
- Defining essential characteristics of study participants, comparators, and outcomes
- Monitoring of study conduct and progress
- Dissemination of research results



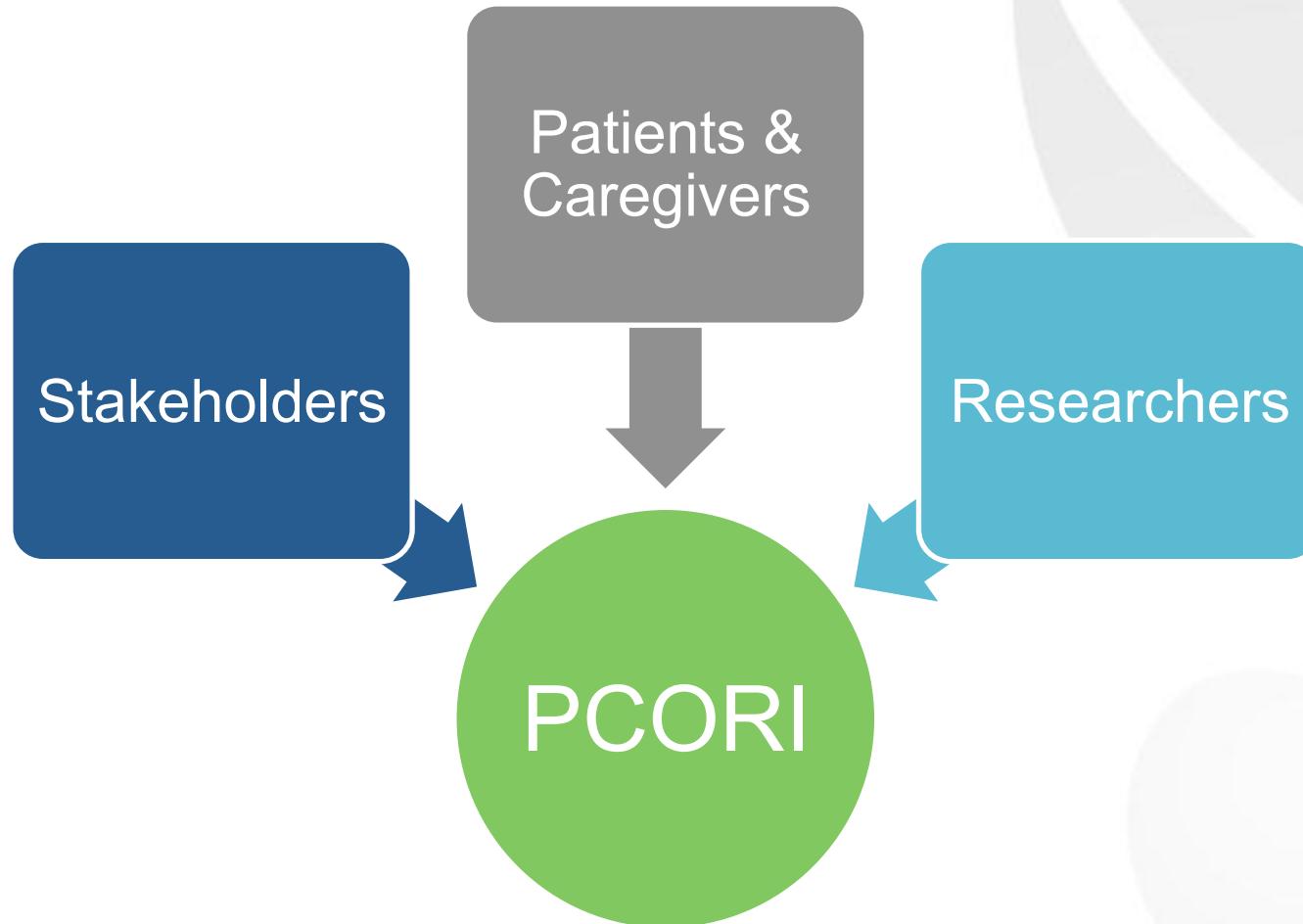
Help Us Spread the Word

Create
Communities

Engage
Meaningfully

Share and
Adopt
Information

Tell Us How We Are Doing



Today's Objectives

The purpose of this workshop is to bring stakeholder representatives together to:

- Solicit research topics for specific funding announcements during breakout sessions on PCORI priority areas
- Report on Patient Engagement Workshop participants' reactions to PCORI's engagement strategies and get additional input
- Report on our draft prioritization process, show how a topic would move through the system, and solicit feedback on the process

Thank You to Our Planning Committee

- Andrew Baskin, Aetna
- Ann Caldwell, The Arc
- Lynne Cuppennull, Alliance of Community Health Plans
- Maureen Dailey, American Nurses Association
- Nancy Foster, American Hospital Association
- Andrea Garcia, Association of State and Territorial Health Officials

Thank You to Our Planning Committee

- Jennifer Graff, National Pharmaceutical Council
- Helen Haskell, Mothers Against Medical Errors
- Dan Leonard, National Pharmaceutical Council
- Jennifer Meeks, American Medical Association
- Jennifer Phillips, Alliance of Community Health Plans
- Sylvia Trujillo, American Medical Association



What Should PCORI Study? A Call for Topics from Patients and Stakeholders

December 4, 2012

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

Patient-Centered Outcomes Research Institute

Workshop Objectives

- Inform Participants on PCORI Mission & Research
- Identify Best Practices in Stakeholder Engagement
- Receive Recommendations on Topics PCORI Should Address

Workshop Agenda

- **8:45 – 10:15:** Engagement & Patient-Centeredness: Sharing Perspectives with PCORI
- **10:15 – 10:30:** Break
- **10:30 – 10:45:** Introduction to Small Group Sessions
- **10:45 – Noon:** Small Group Session #1
- **Noon – 1:15:** Working Lunch: Developing the PCORI Way
- **1:15 – 1:30:** Break

Workshop Agenda

- **1:30 – 2:45:** Small Group Session #2
- **2:45 – 3:00:** Break
- **3:00 – 4:00:** Soliciting Research Topics: What Have We Learned?
- **4:00 – 4:45:** How to Prioritize: A Real World Example
- **4:45 – 5:00:** Closing Remarks

Workshop “Rules”

- Keep Comments Brief So All Can Be Heard
- Allow Facilitator Interruptions To Keep On Time
- Don't Distract With Phones/Email

Small Group Breakout Sessions

- Introductions & Orientation
- Recommendations for Research Topics
- How Organizations Use COR
- Best Practices



Engagement and Patient-Centeredness: Sharing Perspectives with PCORI

8:45 - 10:15 am

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

Patient-Centered Outcomes Research Institute



What Should PCORI Study? A Call for Topics from Patients and Stakeholders

December 4, 2012

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

Patient-Centered Outcomes Research Institute



Introduction to Facilitated Small Group Sessions

10:30 – 10:45 am

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

Patient-Centered Outcomes Research Institute



What Should PCORI Study? A Call for Topics from Patients and Stakeholders

December 4, 2012

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

Patient-Centered Outcomes Research Institute



Research Prioritization: “Developing the PCORI Way”

Rachael Fleurence, PhD
PCORI Scientist

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

Patient-Centered Outcomes Research Institute

Getting to Specificity: Identifying Questions

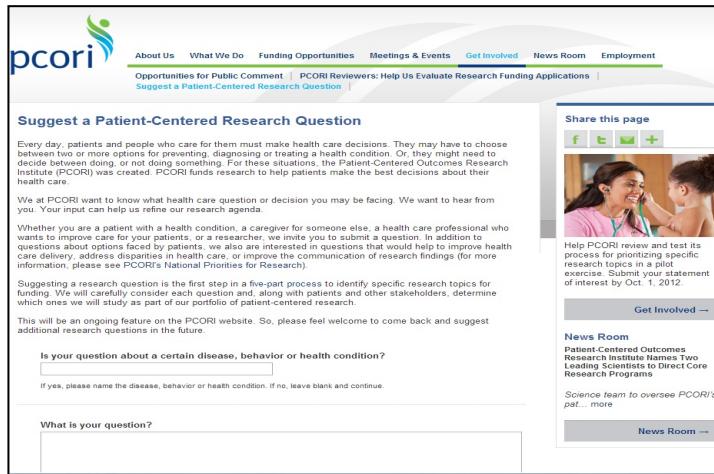
Topic Generation

???????

???????

???????

Getting to Specificity: Identifying Questions



The screenshot shows the PCORI website with a navigation bar at the top. The main content is titled 'Suggest a Patient-Centered Research Question'. It includes a paragraph about the purpose of PCORI, a form for suggesting a research question, and a sidebar with social sharing options and news items.

Suggest a Patient-Centered Research Question

Every day, patients and people who care for them must make health care decisions. They may have to choose between two or more options for preventing, diagnosing or treating a health condition. Or, they might need to decide whether to do, or not to do, something. For these reasons, the Patient-Centered Outcomes Research Institute (PCORI) was created. PCORI funds research to help patients make the best decisions about their health care.

We at PCORI want to know what health care question or decision you may be facing. We want to hear from you. Your input can help us refine our research agenda.

Whether you are a patient with a health condition, a caregiver for someone else, a health care professional who wants to improve care for your patients, or a researcher, we invite you to submit a question. In addition to questions about health care decisions, we are interested in questions that would help improve health care delivery, address disparities in health care, or improve the communication of research findings (for more information, please see PCORI's National Priorities for Research).

Suggesting a research question is the first step in a five-part process to identify specific research topics for funding. We will carefully consider each question and, along with patients and other stakeholders, determine which ones we will study as part of our portfolio of patient-centered research.

This will be an ongoing feature on the PCORI website. So, please feel welcome to come back and suggest additional research questions in the future.

Is your question about a certain disease, behavior or health condition?

If yes, please name the disease, behavior or health condition. If no, leave blank and continue.

What is your question?

Get Involved

Share this page 

Help PCORI review and test its process for prioritizing specific research topics in a pilot exercise. Submit a statement of interest by Oct. 1, 2012.

Get Involved →

News Room

Patient-Centered Outcomes Research Institute Names Two Leading Scientists to Direct Core Research Programs

Science team to oversee PCORI's patient-centered outcomes research

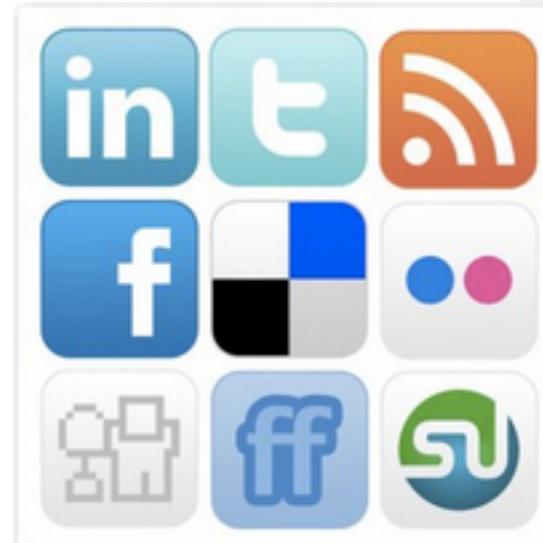
News Room →



Workshops



IOM 100



National Priorities



Guideline Developers



Getting to Specificity: Confirming Research Gaps

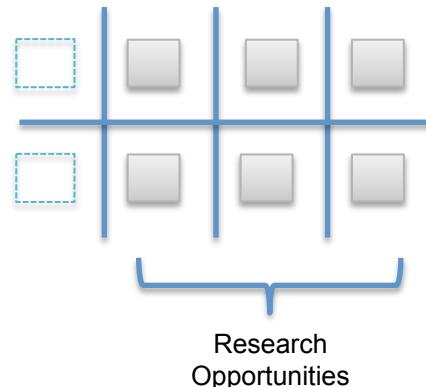
Topic Generation

Gap Confirmation

???????

???????

???????



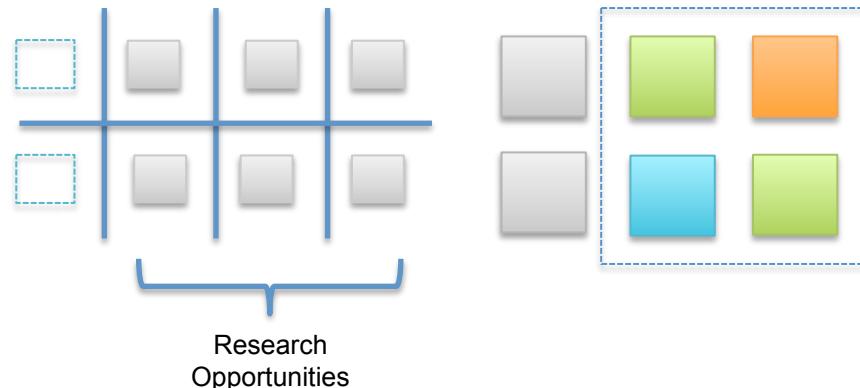
Getting to Specificity: Prioritizing Research Questions

Topic Generation

Gap Confirmation

Research Prioritization

????????
????????
????????



Getting to Specificity: Creating Funding Announcements

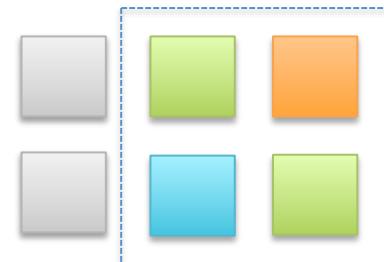
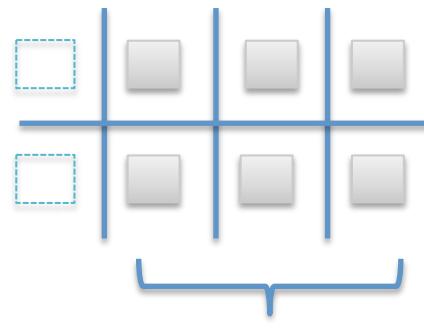
Topic Generation

Gap Confirmation

Research Prioritization

Final Selection for Specific PFAs

????????
????????
????????



Principles to Guide Us: Patients ask for Transparency, Efficiency, Collaboration



Transforming Patient-Centered Research:

Building Partnerships and Promising Models

Washington DC, October 27-28, 2012

Getting to Specificity: PCORI's Progress and Plan for 2013

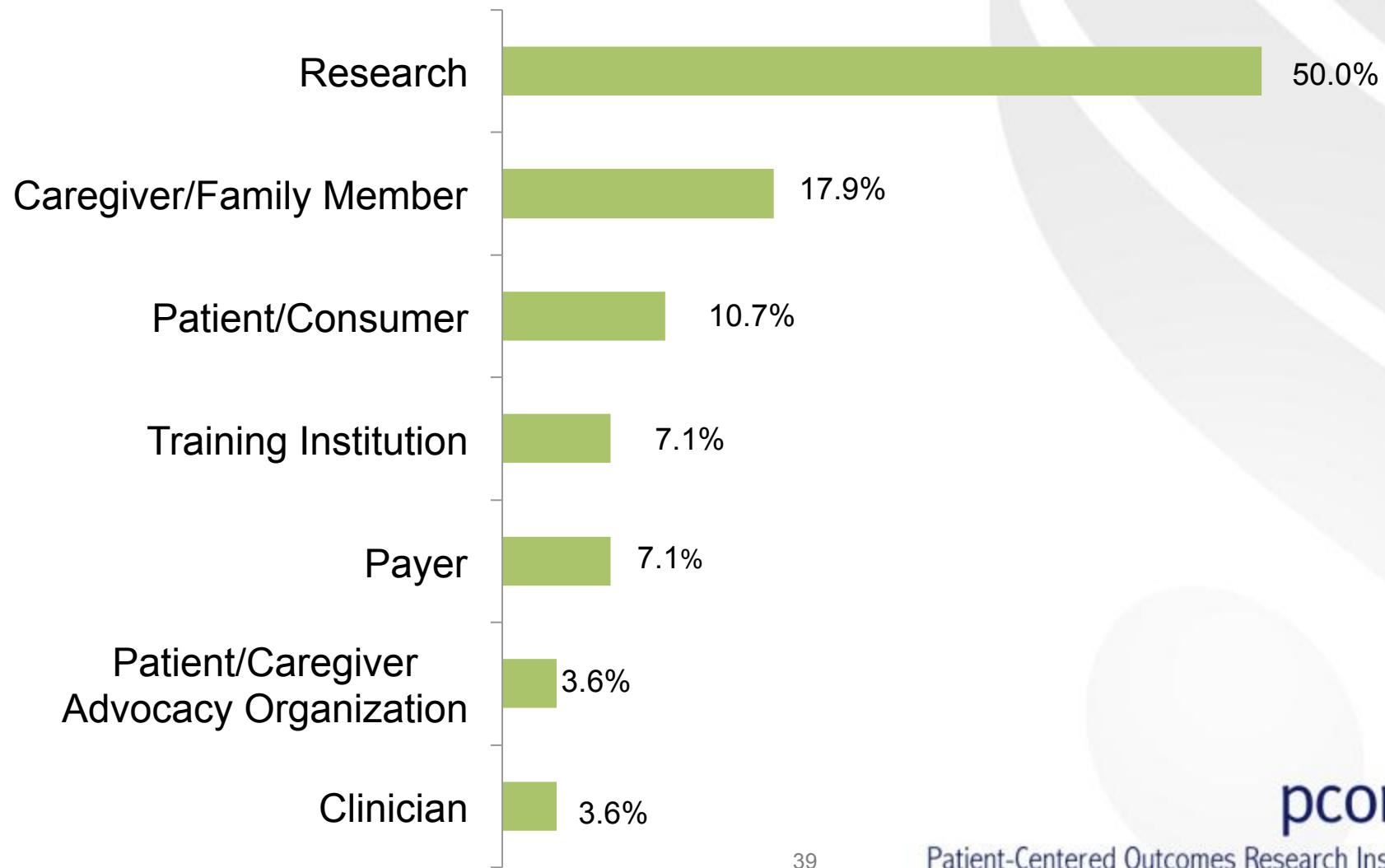
	Aug 2012	Sep	Oct	Nov	Dec	Jan 2013	Feb	Mar 2013
Initial process developed								
Technical Working Group feedback								
Pilot								
Methods Workshop								
Advisory Panel training on Research Prioritization Methods								
Advisory Panels implement and submit results to Board								

Piloting the Process

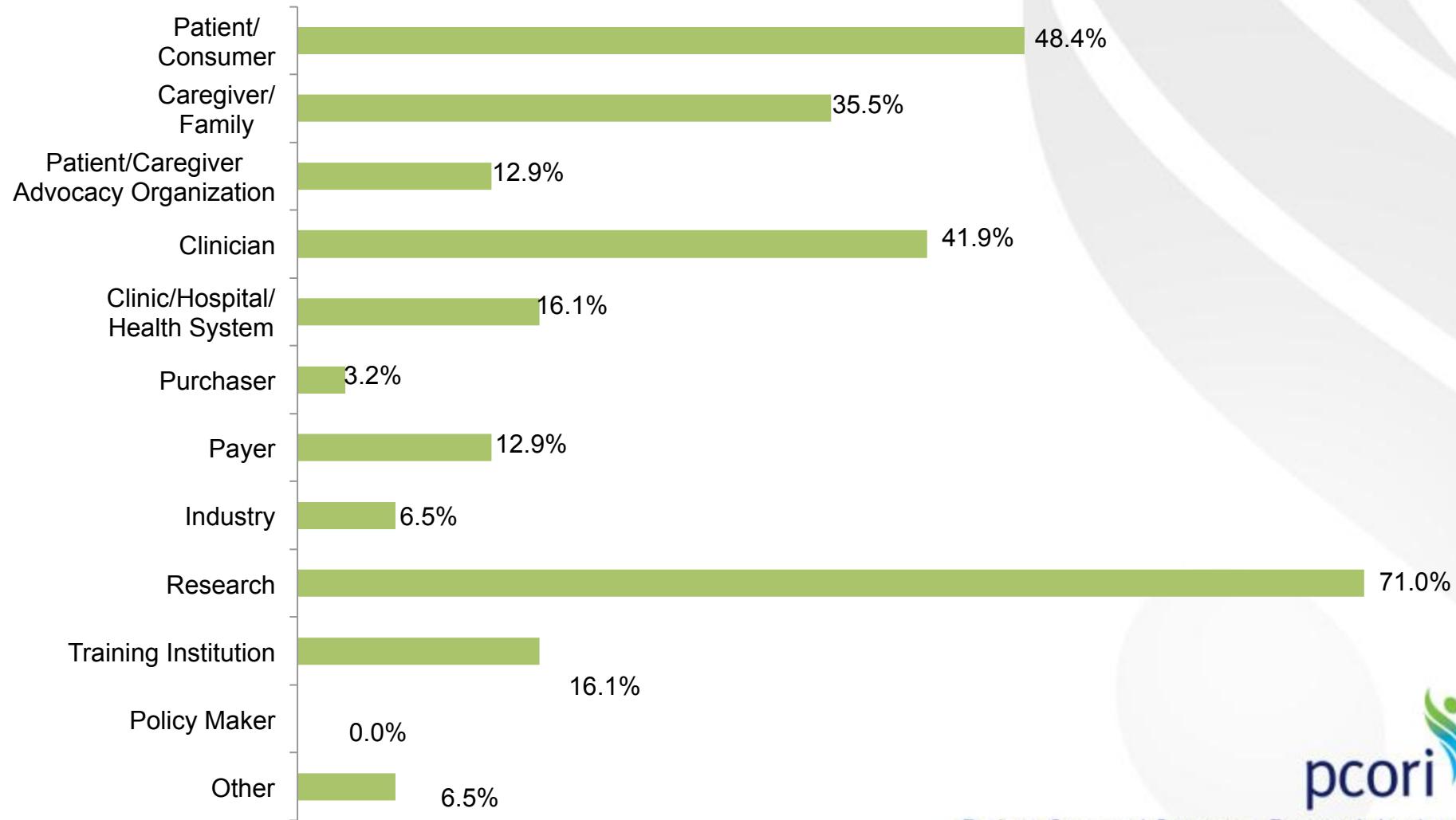


- Piloted from August to November 2012
- 35 Pilot participants
- 8 criteria to prioritize 10 topics
- Results
- Feedback

Composition of the Pilot Group: Primary Identity

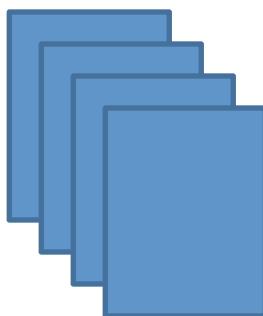


But Pilot Participants Wear Many Different Hats...

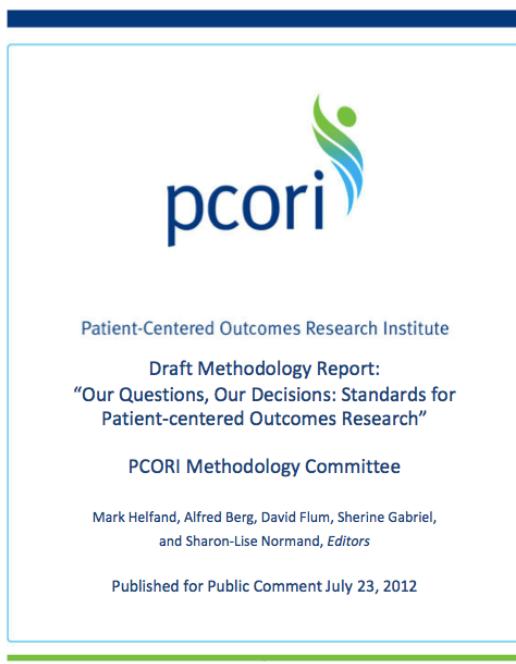


Building on the Existing Evidence Base and Prior Experience

Existing Scientific Work and Literature



Methodology Committee and Methodology Report



Experience of Other Agencies



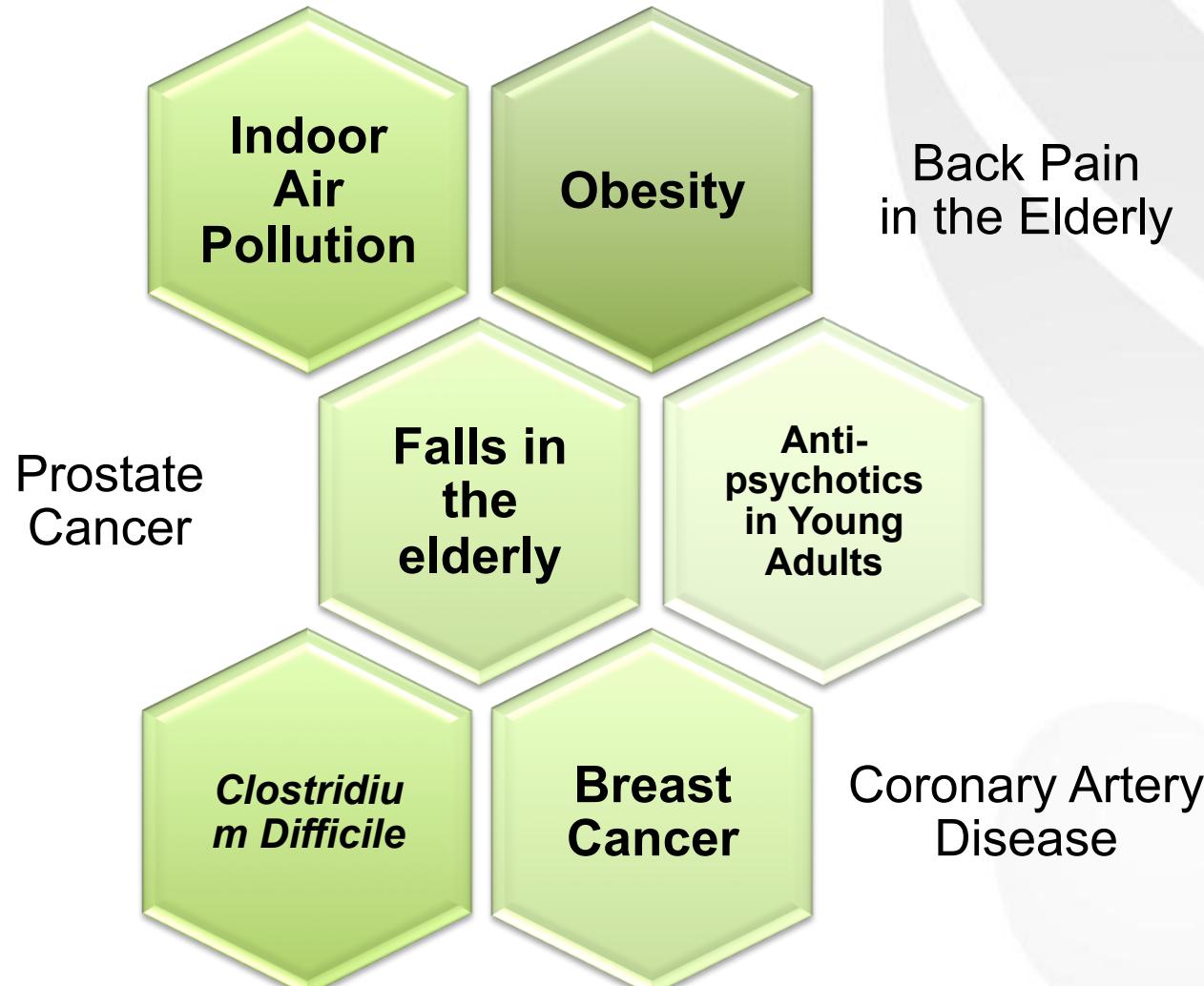
FEDERAL COORDINATING COUNCIL FOR COMPARATIVE EFFECTIVENESS RESEARCH



Original PCORI Criteria for Research Prioritization Process



Questions to Pilot from a Diverse Range of Disease Areas



Pilot Groups used 2 Different Tools to Prioritize



Patient-Centered Outcomes Research Institute

Survey Gizmo

Welcome to PCORI Research Prioritization Tool!

Page One

1. Please rate the following topics. You have 100 points to distribute across the ten research topics. Do not allocate more than 30 points to any one topic. *

- What is the comparative effectiveness of antipsychotics in treating adolescents and young adults, particularly among those with ADHD, bipolar disorder, or schizophrenia?
- What is the comparative effectiveness of management strategies of elderly patients with back pain to evaluate many clinically relevant patient-reported outcomes?
- What is the comparative effectiveness of genetic and biomarker testing to identify optimal candidates for breast cancer risk-reduction medications and interventions?
- What is the comparative effectiveness of percutaneous coronary interventions (PCI) including bare metal stents(BMS), drug eluting stents(DES) and coronary artery bypass graft (CABG) for treatment of coronary artery disease?
- What is the comparative effectiveness of new antibiotic interventions compared with standard therapy (metronidazole, vancomycin) for management of the hospital-acquired diarrheal infection Clostridium difficile?
- What is the comparative effectiveness of management strategies for ductal carcinoma in situ (DCIS) to improve long term patient centered outcomes?
- What is the comparative effectiveness of primary prevention methods, such as exercise and balance training, to prevent falls versus clinical treatments in older adults at varying degrees of risk, including those patients post hip fracture and repair?
- What is the comparative effectiveness of indoor air pollution interventions to improve respiratory and cardiovascular health outcomes among high-risk populations?
- What is the comparative effectiveness of coordinated treatment options for patients with multiple chronic conditions?
- What is the comparative effectiveness of mindfulness-based interventions and usual care for promoting health behaviors to reduce the risk of becoming obese and developing metabolic syndrome?

0 out of 100 Total

Expert Choice – Topic Ranking

 » About » Logout   

Rate Biomarkers Rate Biomarkers for the prevention of breast cancer with respect to the following criteria

Biomarkers for the prevention of breast... 

1. Patient-centeredness

- The question is meaningful to these patients, their caregivers and their clinicians.
- Proposed studies to answer this research question will have the potential for including

Patient-centeredness 

Will this research answer questions that matter to patients and their clinicians in their decision making? Will it make a difference to patients' health outcomes? Is the topic likely to address questions

Impact on individuals and populations 

Differences in benefits 

Reduction in uncertainty 

Probability of implementation 

Durability of information 

Potential for impact on healthcare system performance 

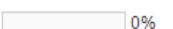
Inclusiveness of different populations 

Biomarkers for... WRT Patient-centered... 

• The question is meaningful to these patients, their caregivers and their clinicians.

• Proposed studies to answer this research question will have the potential for including patient-centered outcomes

Patient-centeredness

Intensity Name	Priority
Not rated	
Exceeds expectations	 100%
Meets expectations exceptionally	 86%
Meets expectations very well	 69%
Meets expectations well	 56%
Meets expectations	 47%
Almost meets expectations	 36%
Moderately meets expectations	 26%
Slightly meets expectations	 4%
Does not meet expectations/cannot assess	 0%

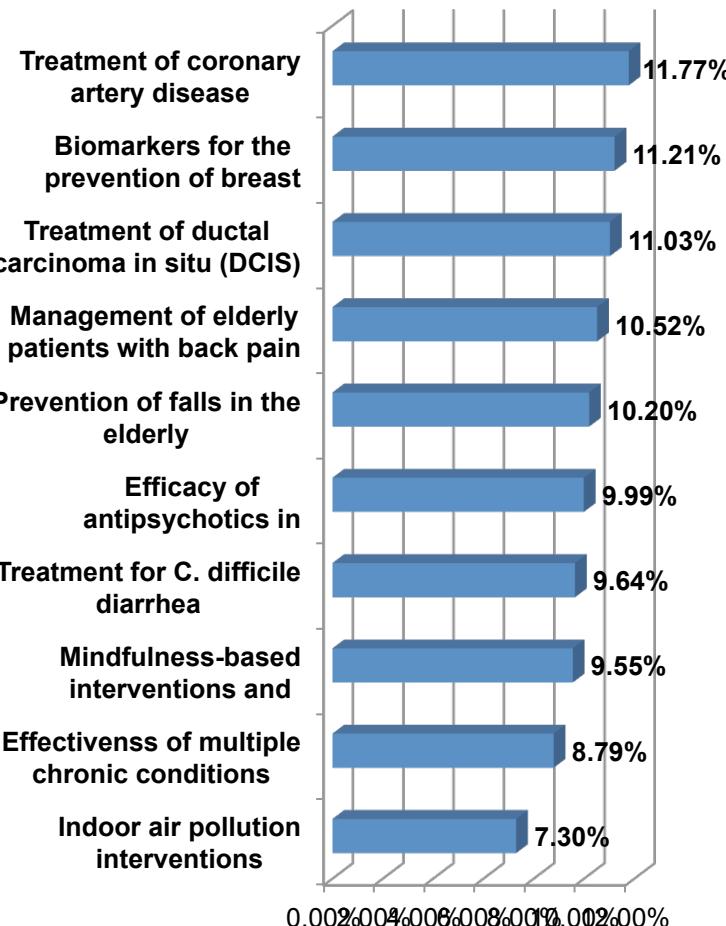
Navigation Box

Steps: 33 ... 30 31 32 33 34 35 36 ... 42 Evaluated: 1/108

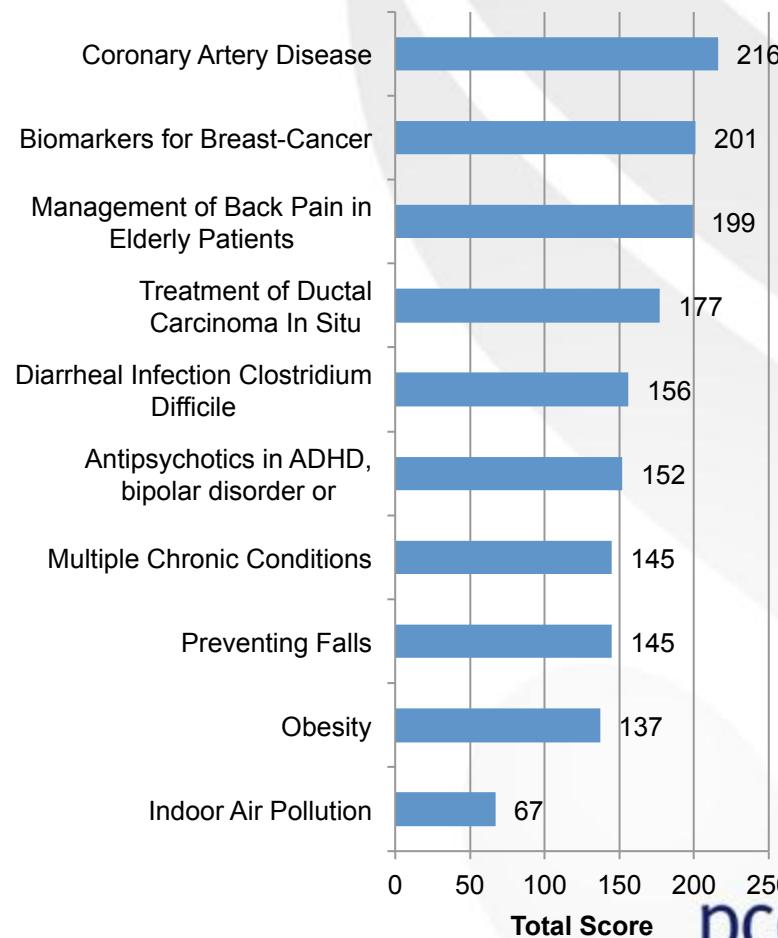
Next Unassessed Previous Next

Group 1 Results Using Two Softwares

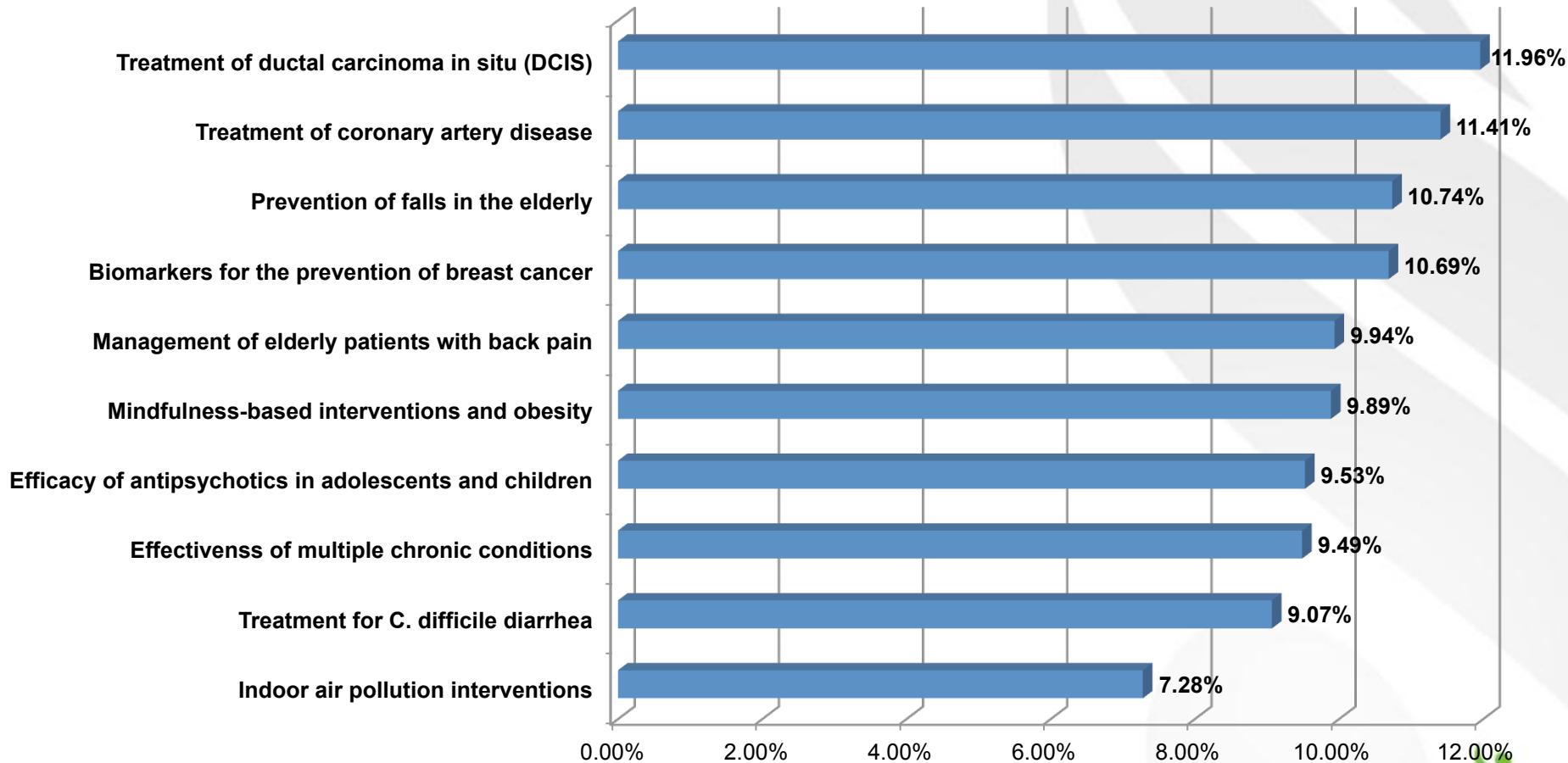
Expert Choice



Survey Gizmo



Group 2 Results



Participants Provided Valuable Insights to Improve the Process

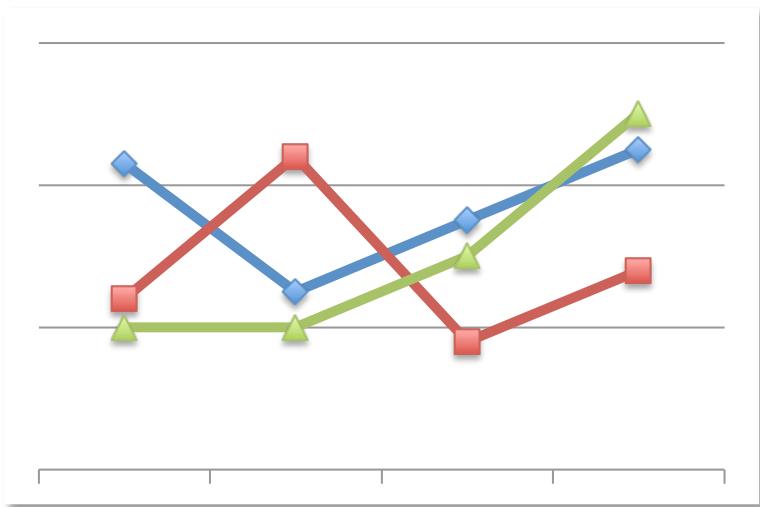


1. Patient-Centeredness



- Are **patients and clinicians** asking for this research ?
- Will research findings make a **difference** to patients and their clinicians when making health care decisions ?

2. Impact on Population and Individual Health



- **Burden of disease** in terms of prevalence, mortality, morbidity, individual suffering, loss of productivity?
- **Rare disease?**

3. Differences in Benefits and Harms, And Reduction in Uncertainty



- Indications of differences in **benefits and harms** sufficient to warrant conducting new research?
- Does current evidence suggest **uncertainty** regarding treatment effectiveness and a need for additional evidence?

4. Implementation in Practice



How likely is it that the research findings will be implemented in practice?

5. Duration of Information



- Will research findings be valid by the time the study has concluded?

Radiation Therapy for Prostate Cancer



- Patient centeredness
- Impact on population and individual health
- Differences in benefits and harms and reduction in uncertainty
- Implementation in practice
- Duration of information

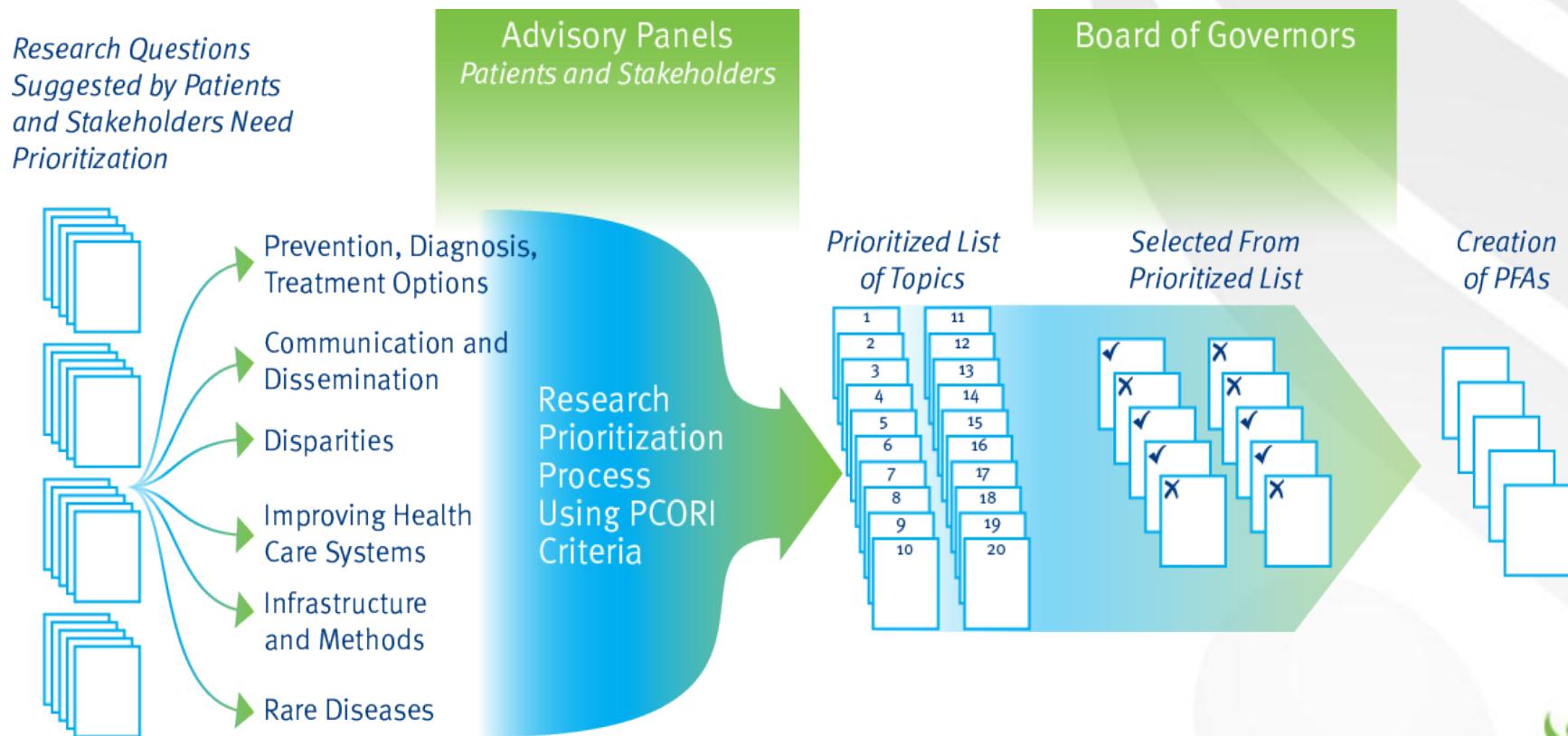
Next Steps



- Revisions
- Implementation
- Learning from ARRA

Launching PCORI's Research Prioritization Process

From Research Questions to Research Studies



Acknowledgements





What Should PCORI Study? A Call for Topics from Patients and Stakeholders

December 4, 2012

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

Patient-Centered Outcomes Research Institute



Soliciting Research Topics: What Have We Learned?

3:00 – 4:00 pm

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

Patient-Centered Outcomes Research Institute



What Should PCORI Study? A Call for Topics from Patients and Stakeholders

December 4, 2012

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

Patient-Centered Outcomes Research Institute



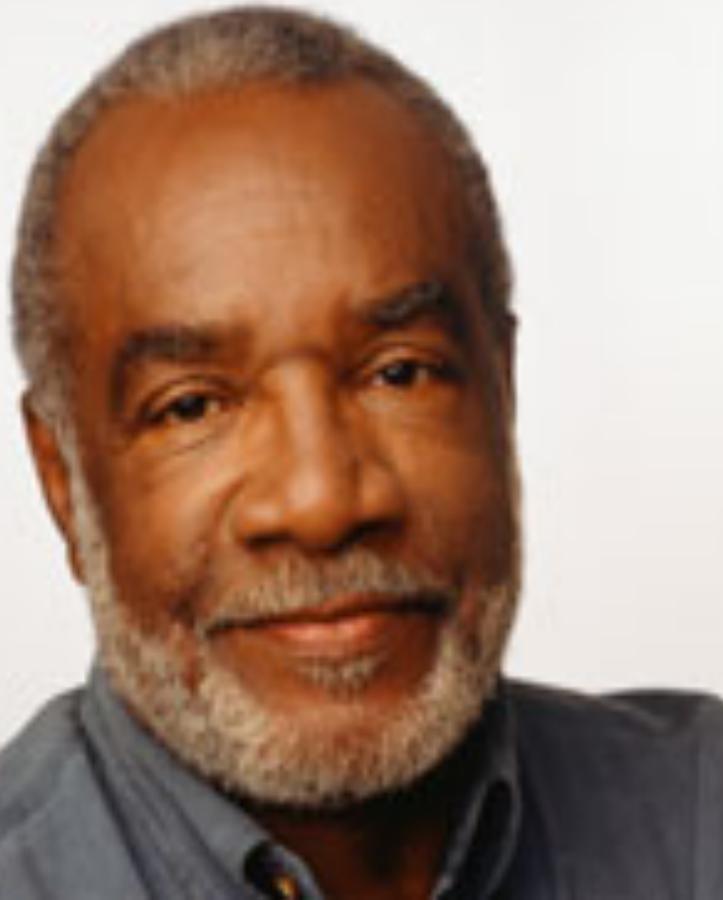
How to Prioritize: A Real World Example

Kara Odom Walker, MD, MPH, MSHS
PCORI Scientist

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

Patient-Centered Outcomes Research Institute

A Real World Question



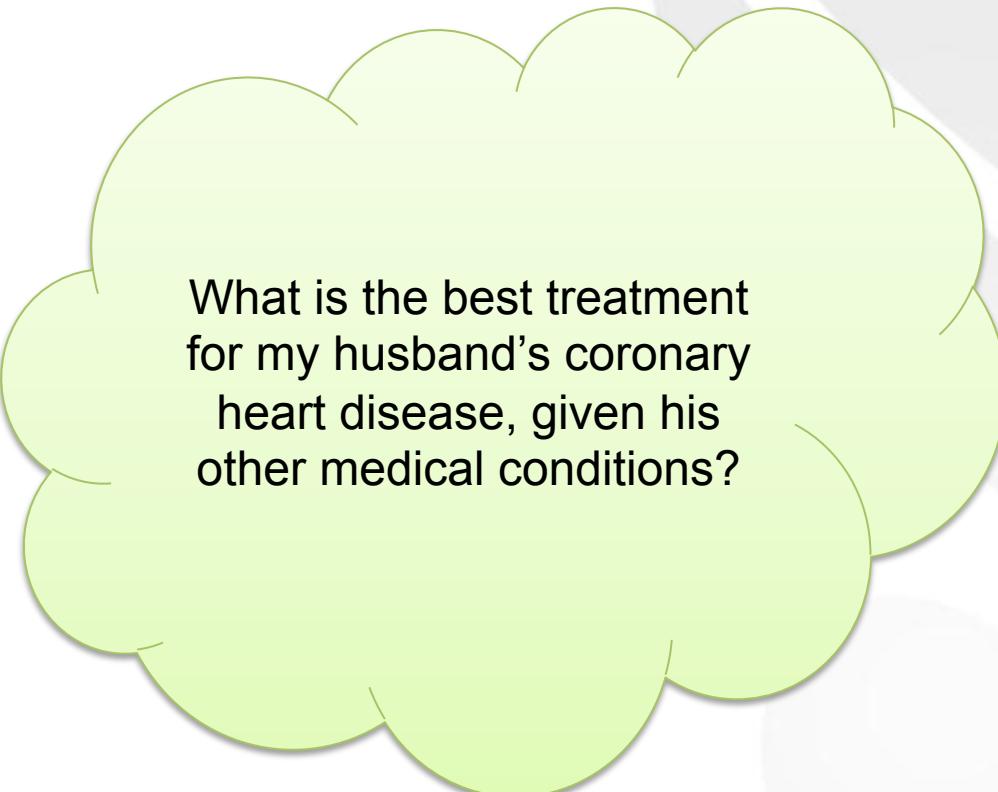
- Mr. Jones is 77 years old and has several medical conditions, including diabetes and congestive heart failure
- Over the Thanksgiving holiday, he experienced chest pain and they rushed to the hospital
- The doctors presented several treatment options to both Mr. and his wife

Mr. Jones listens to the doctors but wonders about making the right choice?

Getting to Specificity: A Multi-Step Process

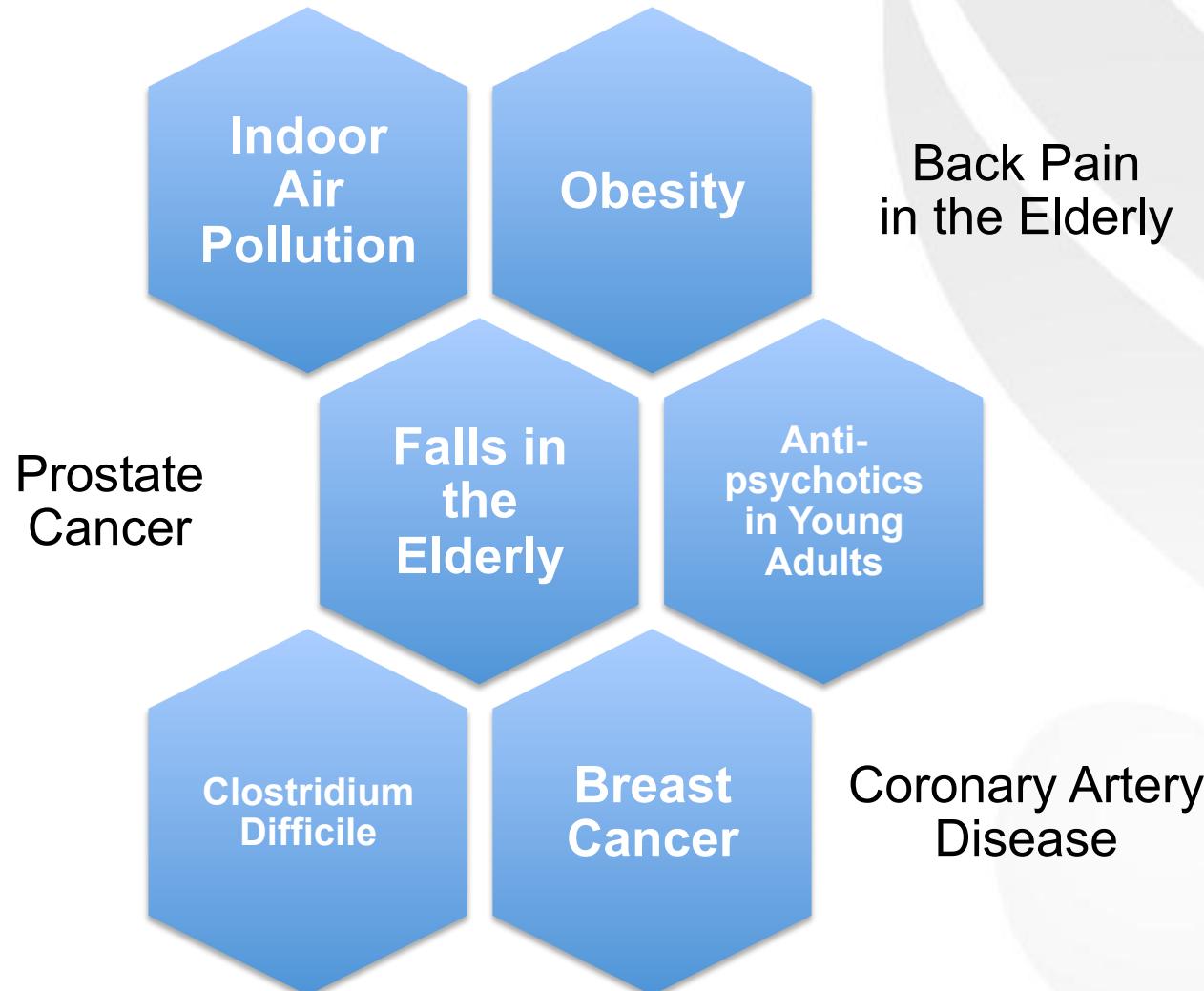
Topic Generation

??????
??????
??????



What is the best treatment
for my husband's coronary
heart disease, given his
other medical conditions?

Other Questions Also Come from a Diverse Range of Disease Areas

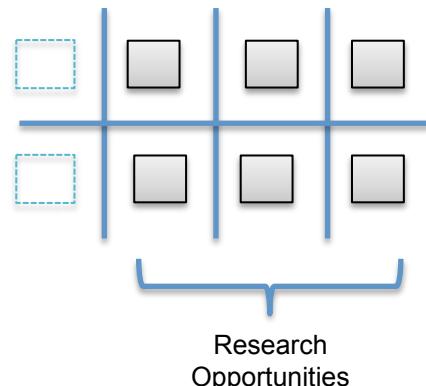


Getting to Specificity: A Multi-Step Process

Topic Generation

Gap
Confirmation

??????
??????
??????



For Example: Treatment of Coronary Heart Disease

Evidence:

- Unknown whether coronary bypass surgery, percutaneous interventions including stents, or medical management are the best option for patients like Mr. Jones, given his preferences, and medical conditions
- More research is needed to help with decision making

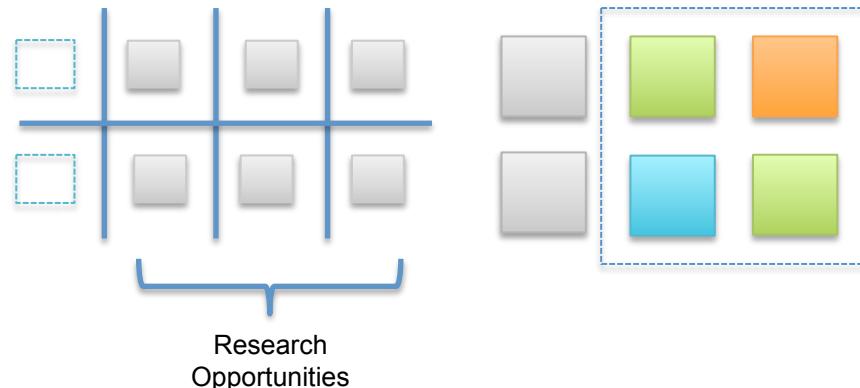
Getting to Specificity: A Multi-Step Process

Topic Generation

Gap Confirmation

Research Prioritization

????????
????????
????????



Original PCORI Criteria for Research Prioritization Process



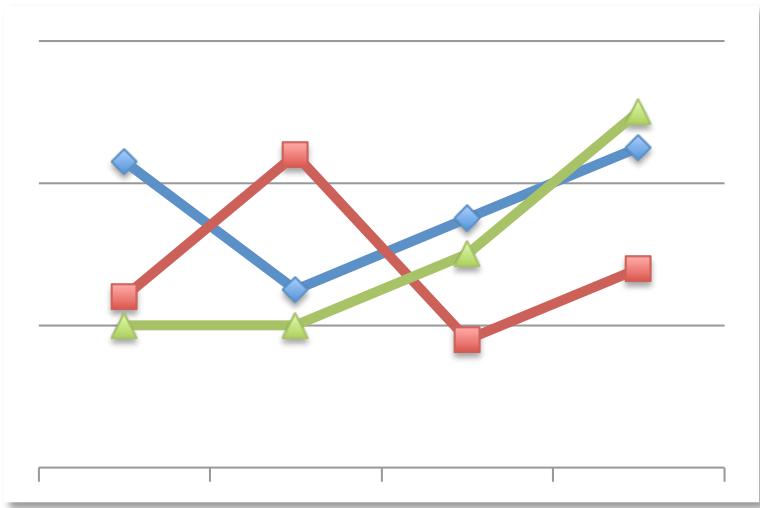
- Patient centeredness
- Impact
- Differences in benefits and harms
- Reduction in uncertainty
- Implementation in practice
- Duration of information
- Healthcare system performance
- Inclusiveness of different populations

1. Patient-Centeredness



- Are **patients and clinicians** asking for this research?
- Will research findings make a **difference** to patients and their clinicians when making health care decisions?

2. Impact on Population and Individual Health



- **Burden of disease** in terms of prevalence, mortality, morbidity, individual suffering, loss of productivity?
- **Rare disease?**

3. Differences in Benefits and Harms, And Reduction in Uncertainty



- Indications of differences in **benefits and harms** sufficient to warrant conducting new research?
- Does current evidence suggest **uncertainty** regarding treatment effectiveness and a need for additional evidence?



4. Implementation in Practice



How likely is it that the research findings will be **implemented** in practice?

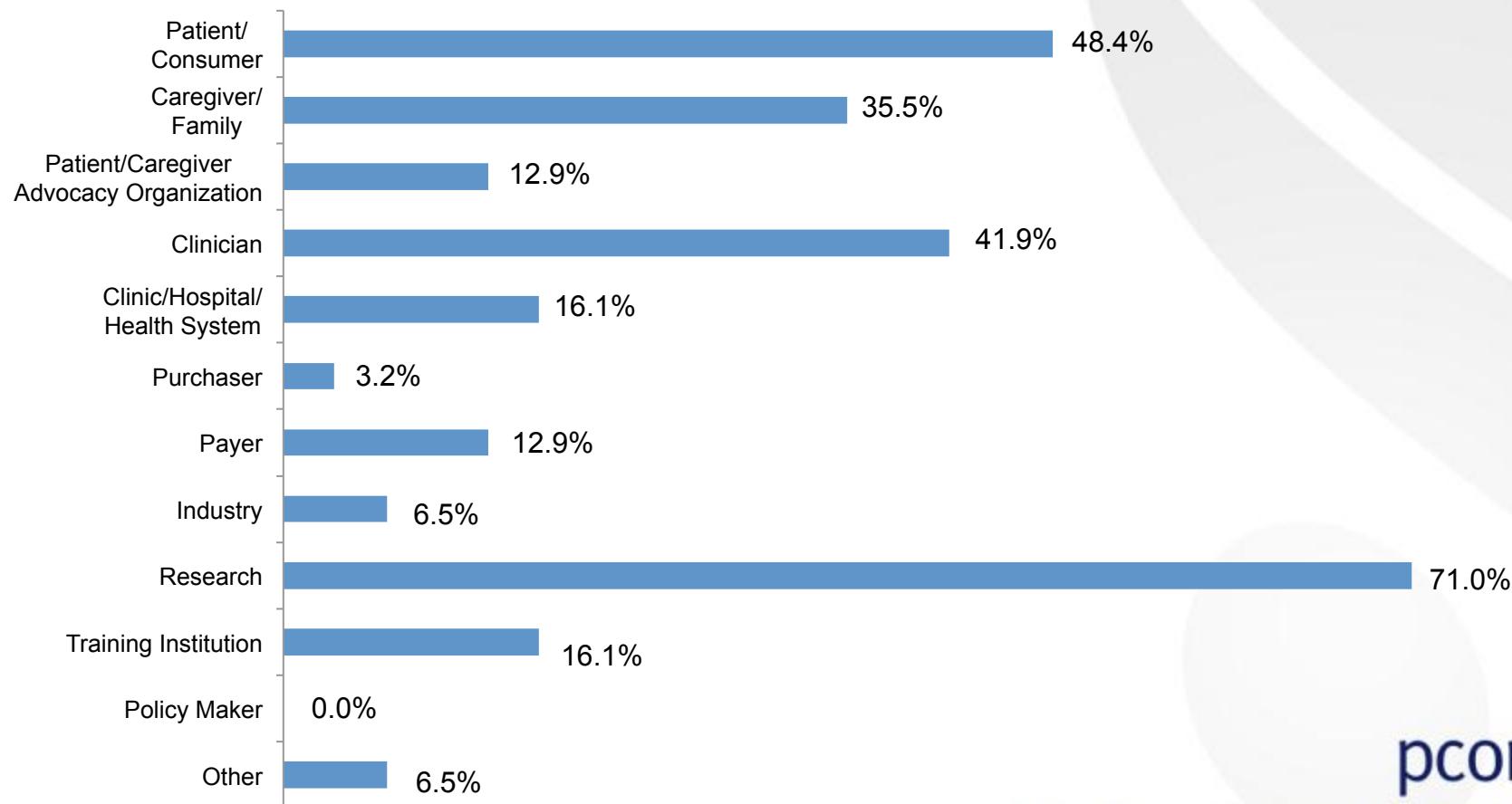
5. Duration of Information



- Will research findings **be valid by the time** the study has concluded?

Who Ranks? Composition of the Pilot Group

“With Which of the Following Communities Do You Identify? (Select All That Apply)”
N = 31



Voting for Topics

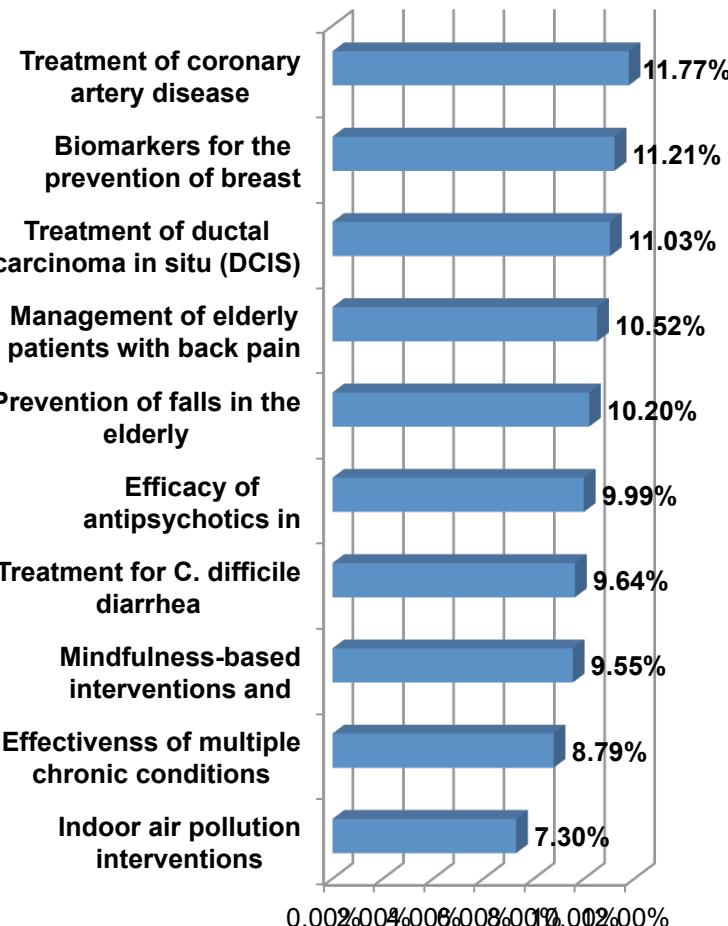


Different Tools

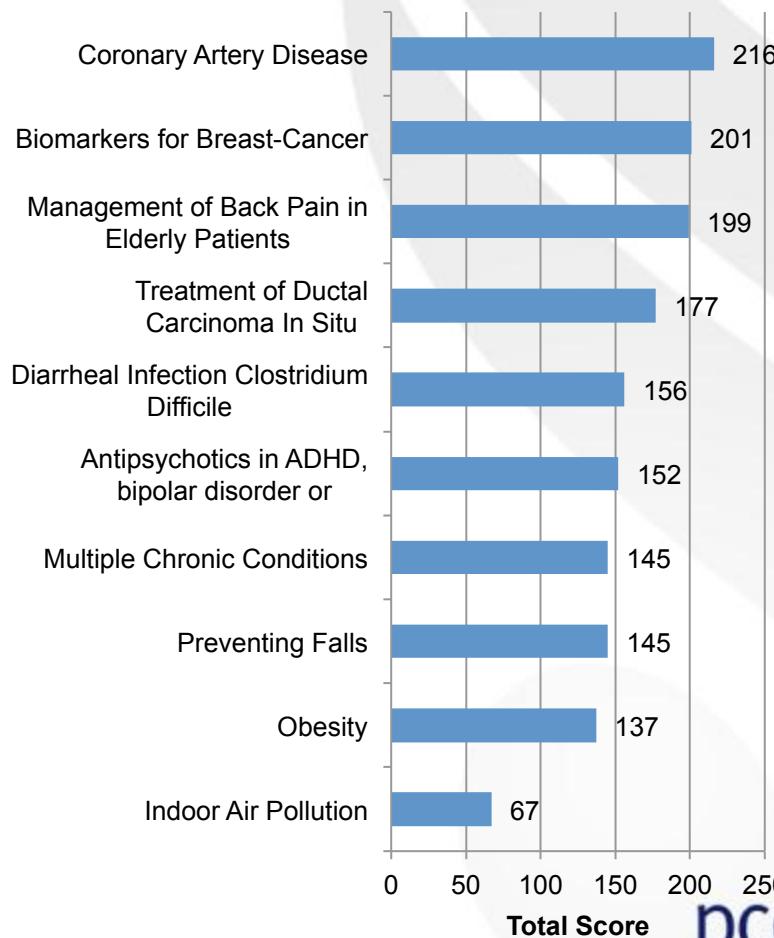


Sample Results: Comparing Lists from Expert Choice and Survey Gizmo

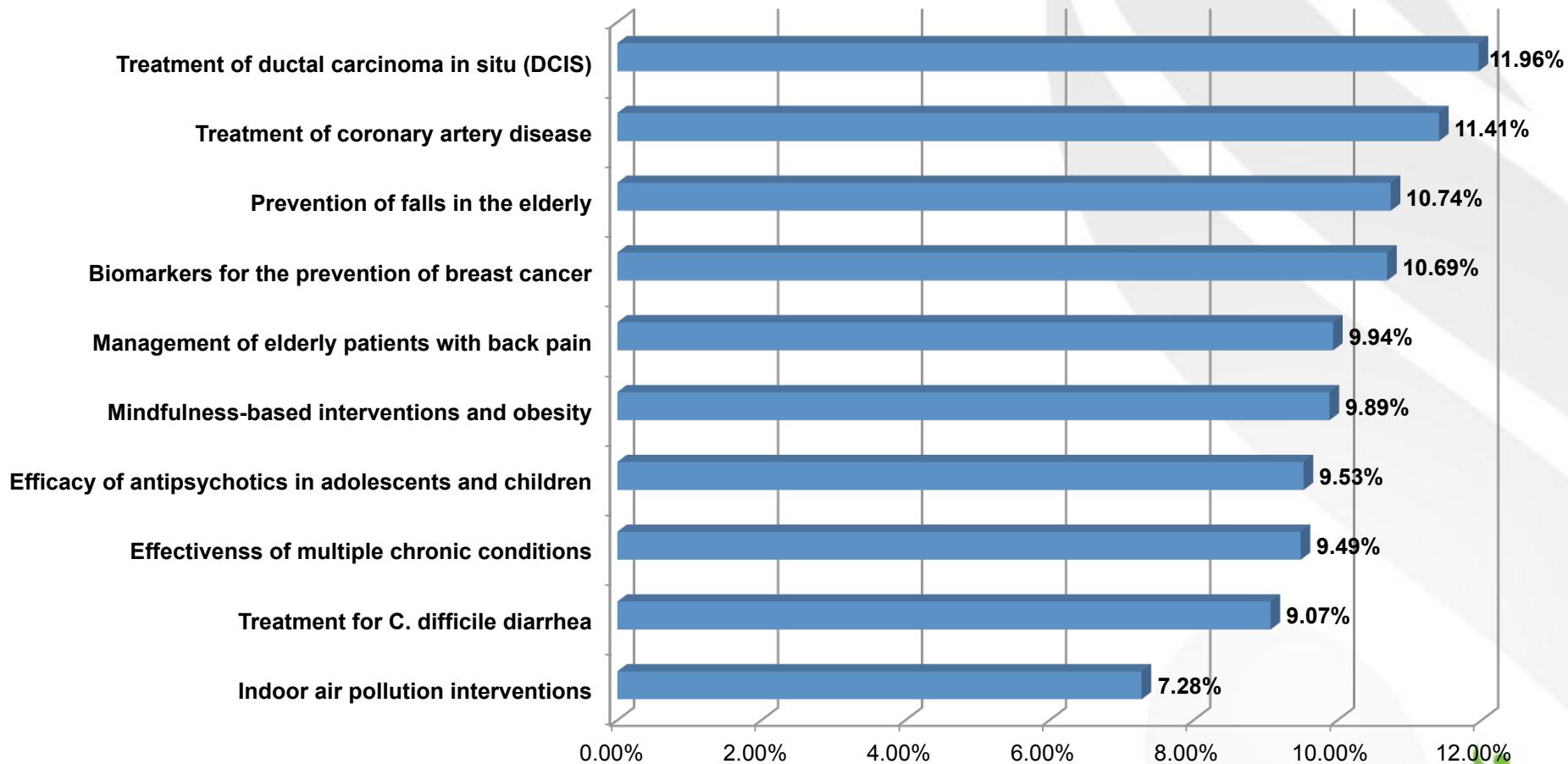
Expert Choice



Survey Gizmo



Sample Results: Ranked Topics with Group Generated Weights



Getting to Specificity: A Multi-Step Process

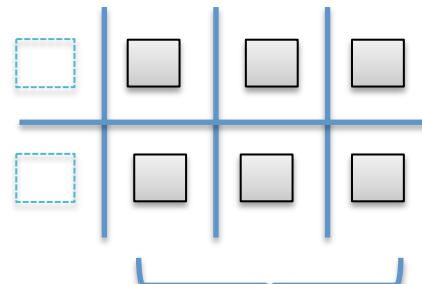
Topic Generation

Gap Confirmation

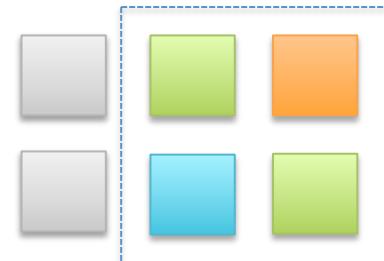
Research Prioritization

Final Selection for Specific PFAs

????????
????????
????????



Research Opportunities



Participants Provided Valuable Insights to Improve the Process

Emphasize Patient's Voice

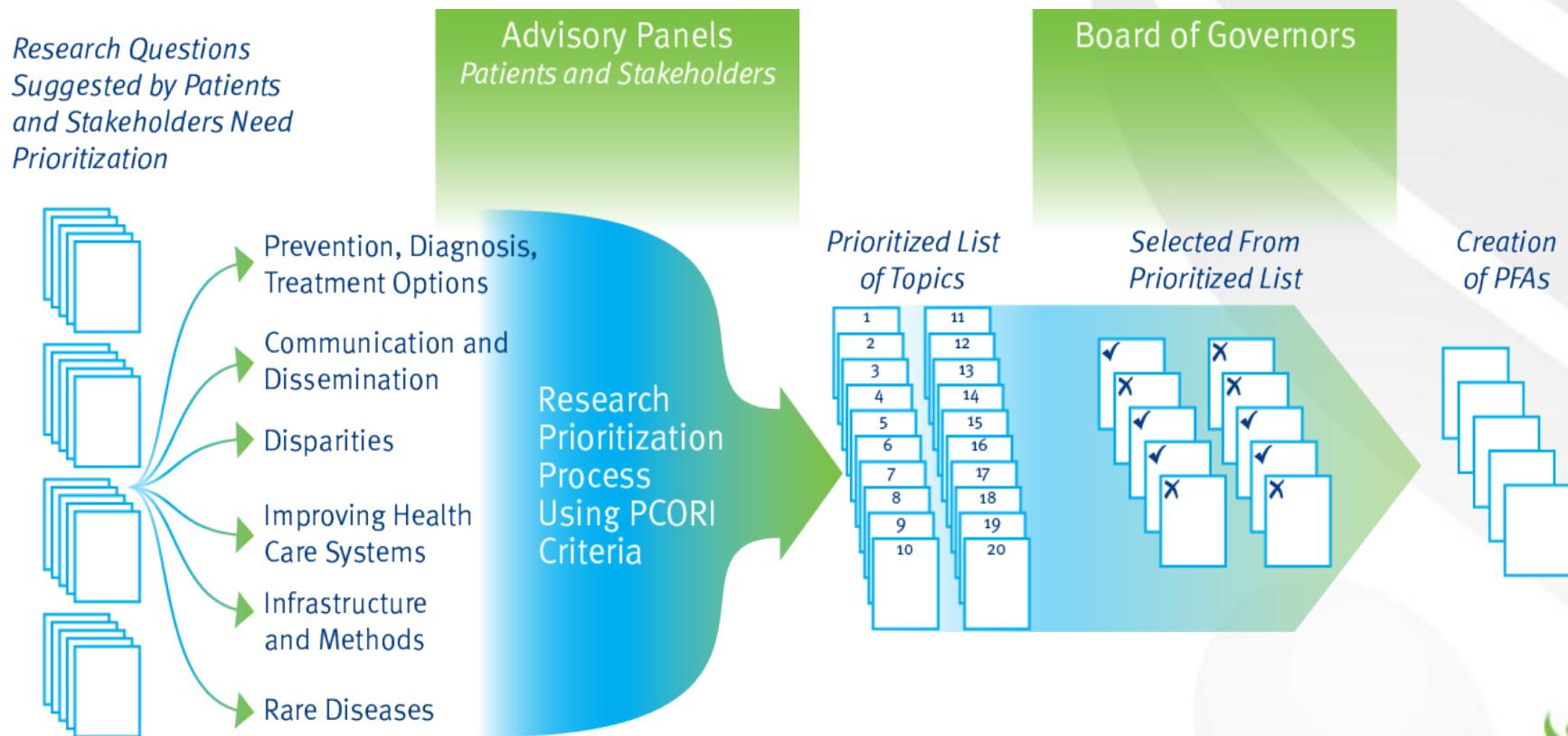
Clarify the Criteria

Improve Supporting Information

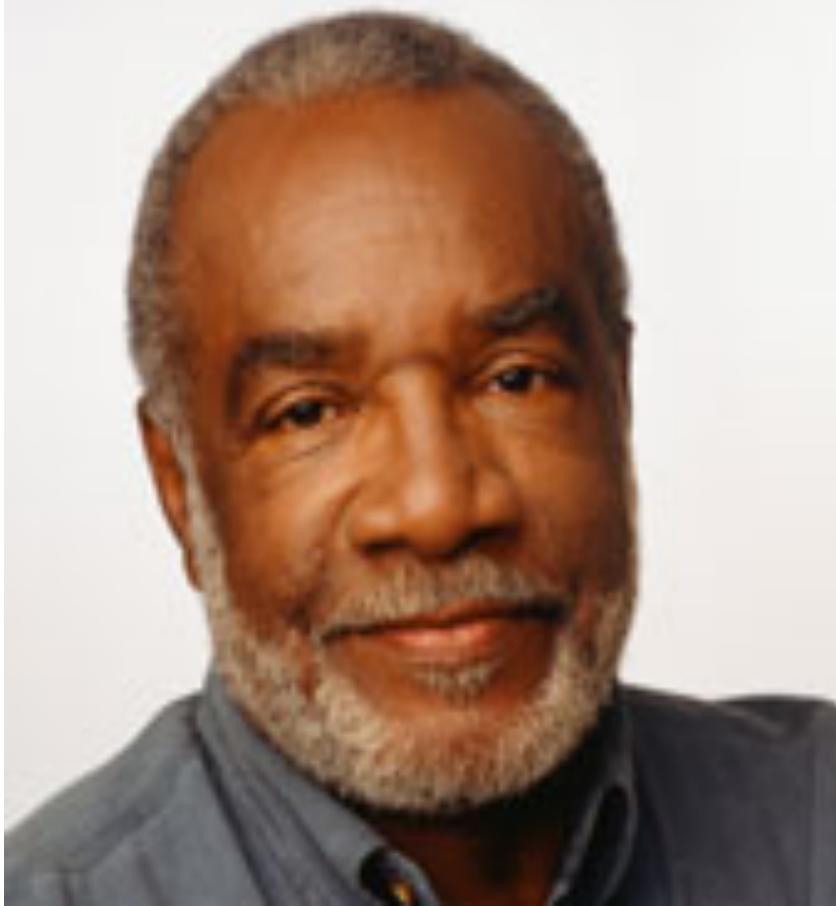
Choose the Tools

Next Steps: PCORI's Research Prioritization Process

From Research Questions to Research Studies



A Potential Answer for Mr. Jones



- ➊ Mr. Jones listens to his options from the doctors and thinks about his choices for his heart disease
- ➋ He chooses the treatment with the fewest risks and the greatest long term benefits for his health

Acknowledgements



- 35 Pilot Group Members
- PCORI's RP Technical Working Group
- MC Working Group on RP
- PCORI staff, Board Members and MC Members
- NORC at University of Chicago



What Should PCORI Study? A Call for Topics from Patients and Stakeholders

December 4, 2012

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

Patient-Centered Outcomes Research Institute



**Anne Beal, MD, MPH, Deputy
Executive Director and COO
PCORI**

*What Should PCORI Study? A Call for Topics from
Patients and Stakeholders*

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

Patient-Centered Outcomes Research Institute



What Should PCORI Study? A Call for Topics from Patients and Stakeholders

December 4, 2012

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

Patient-Centered Outcomes Research Institute