



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*

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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.



Patient-Centered Outcomes Research Institute

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



PCORI Board of Governors, March 2012 in Baltimore, MD

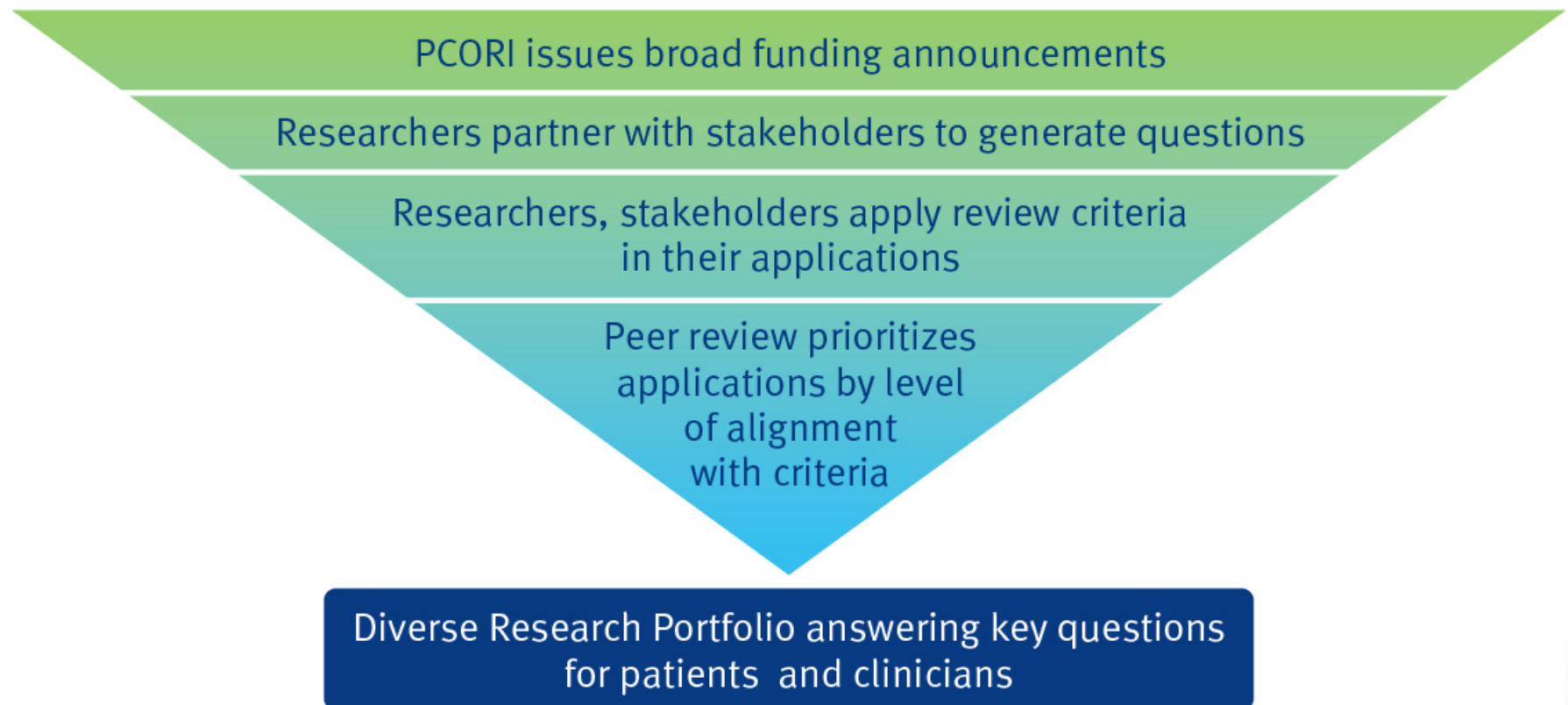
Unique role of stakeholders



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

Figure 1: Traditional Process

Investigator-Generated Research Just One Part of the Process



PCORI'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

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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



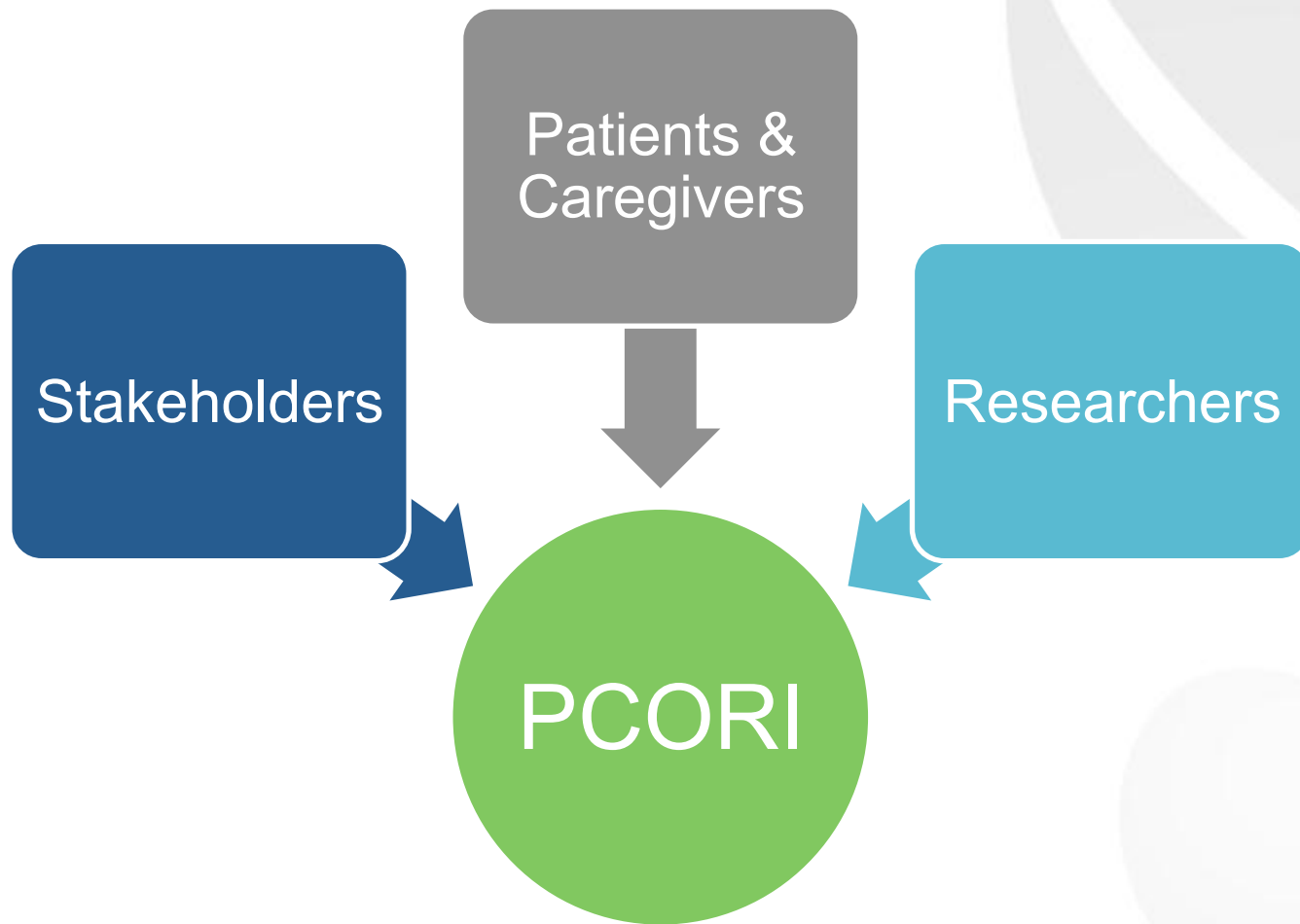
Help Us Spread the Word

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 Cuppernull, 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

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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

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Introduction to Facilitated Small Group Sessions

10:30 – 10:45 am

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Research Prioritization: “Developing the PCORI Way”

Rachael Fleurence, PhD
PCORI Scientist

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Getting to Specificity: Identifying Questions

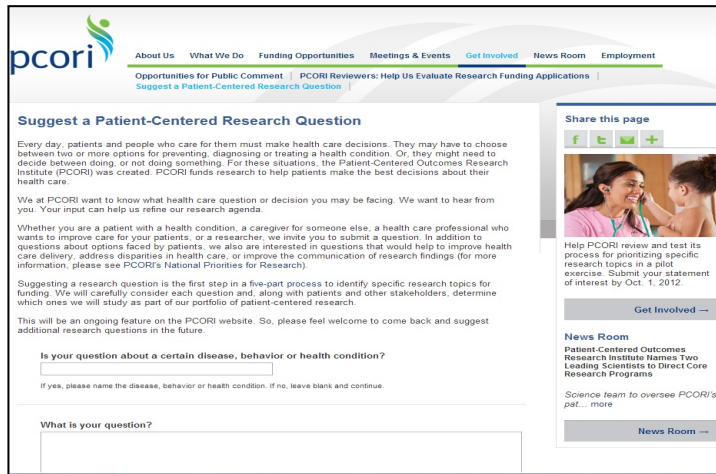
Topic Generation

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Getting to Specificity: Identifying Questions



The screenshot shows the PCORI website's 'Suggest a Patient-Centered Research Question' page. The page includes a navigation bar with links like 'About Us', 'What We Do', 'Funding Opportunities', 'Meetings & Events', 'Get Involved', 'News Room', and 'Employment'. Below the navigation bar, there's a section titled 'Suggest a Patient-Centered Research Question' with a brief introduction to PCORI and its mission. It then asks if the user's question is about a certain disease, behavior, or health condition, and provides a text box for the user to enter the question. There are also links to 'Share this page' and 'Get Involved'.

National Priorities



Workshops



IOM 100



Guideline Developers



Patient-Centered Outcomes Research Institute

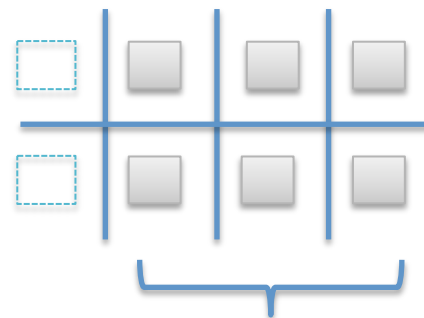
Getting to Specificity: Confirming Research Gaps



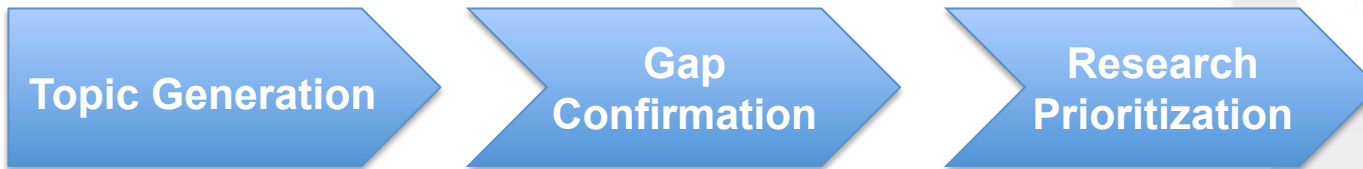
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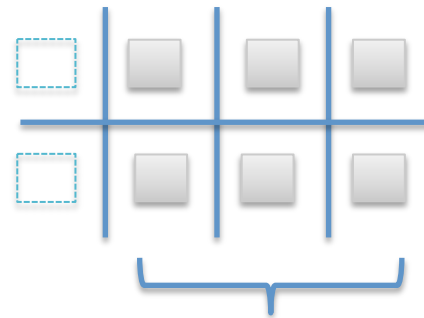
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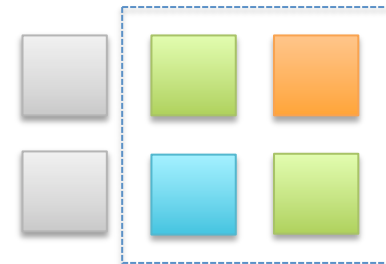
Getting to Specificity: Prioritizing Research Questions



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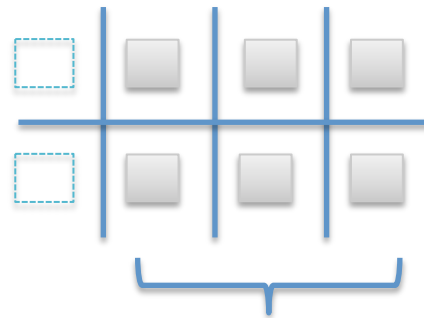
Research Opportunities



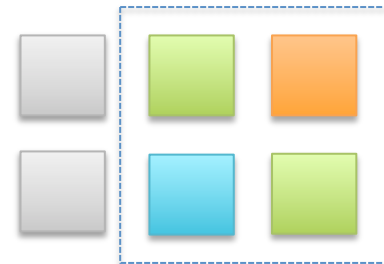
Getting to Specificity: Creating Funding Announcements



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Research Opportunities



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

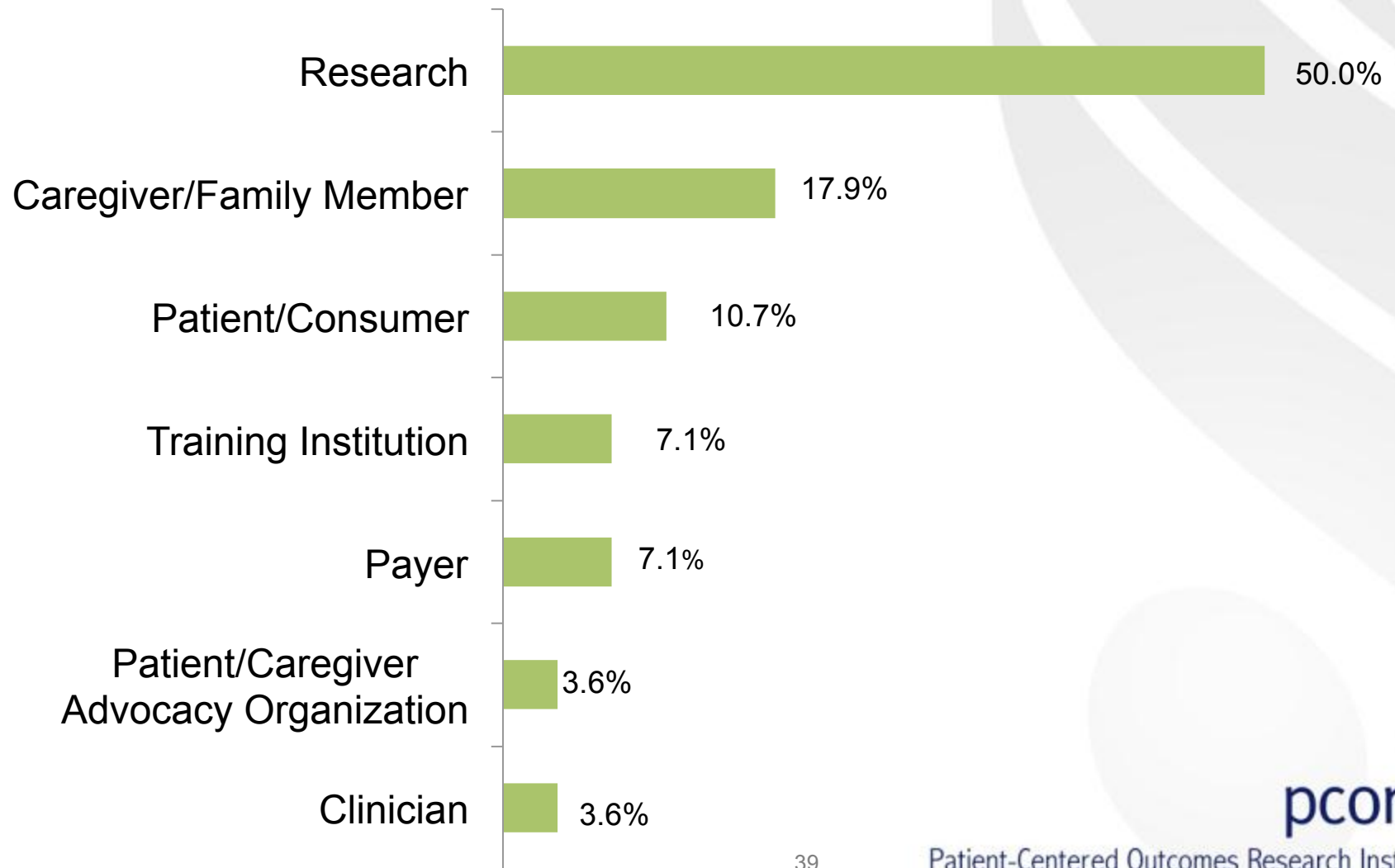
	Aug 2012	Sep	Oct	Nov	Dec	Jan 2013	Feb	Mar 2013
Initial process developed								
Technical Working Group feedback								
Pilot								
Methods Workshop								
Advisory Panel training on Research Prioritization Methods								
Advisory Panels implement and submit results to Board								

Piloting the Process

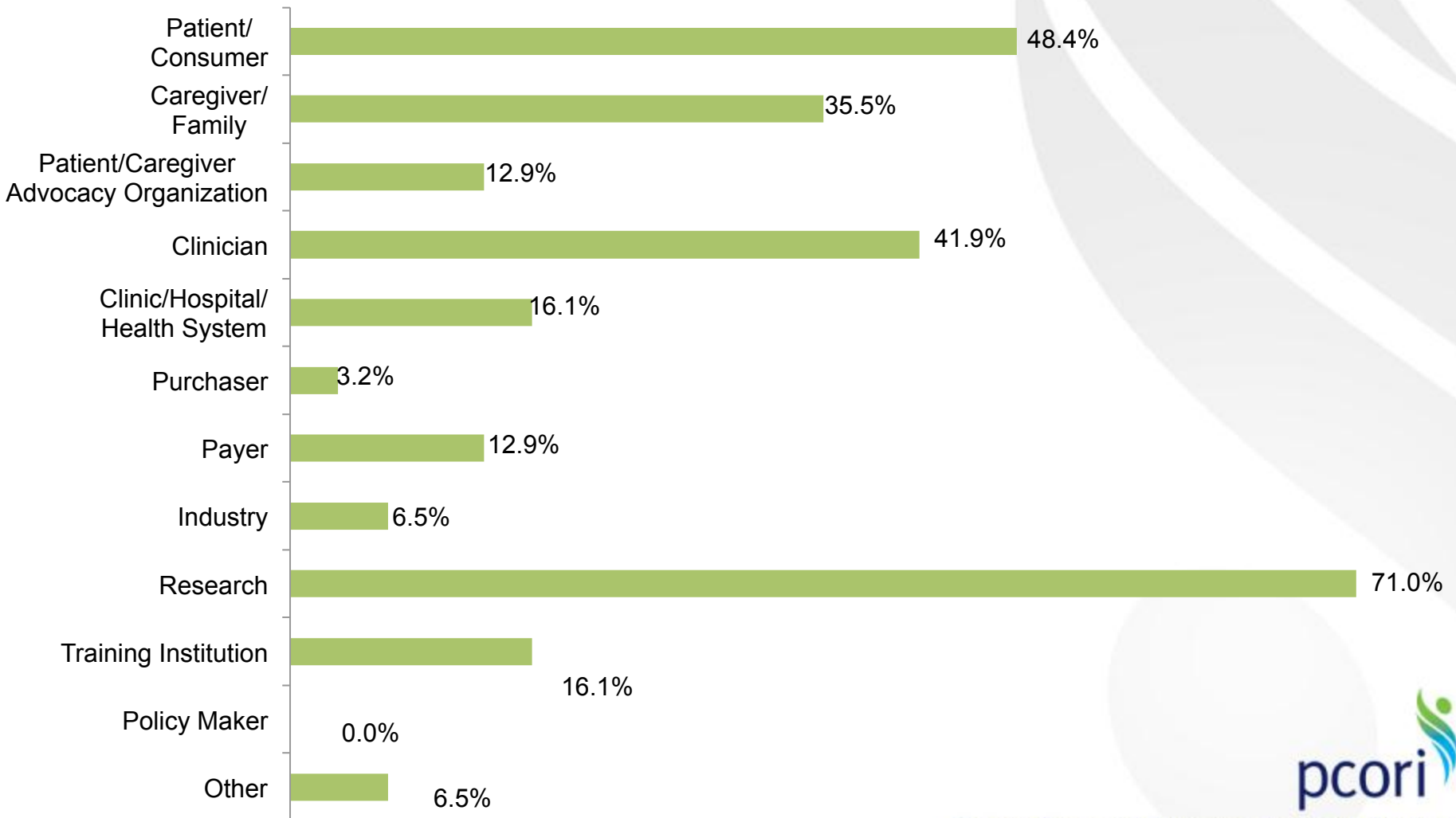


- Piloted from August to November 2012
- 35 Pilot participants
- 8 criteria to prioritize 10 topics
- Results
- Feedback

Composition of the Pilot Group: Primary Identity



But Pilot Participants Wear Many Different Hats...

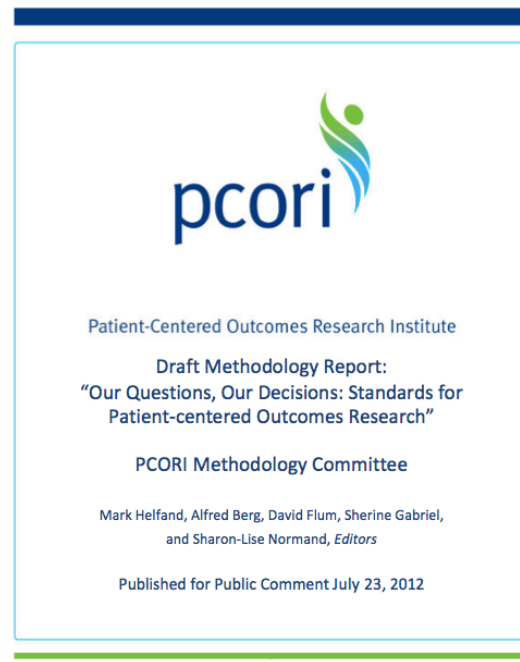


Building on the Existing Evidence Base and Prior Experience

Existing Scientific Work and Literature



Methodology Committee and Methodology Report



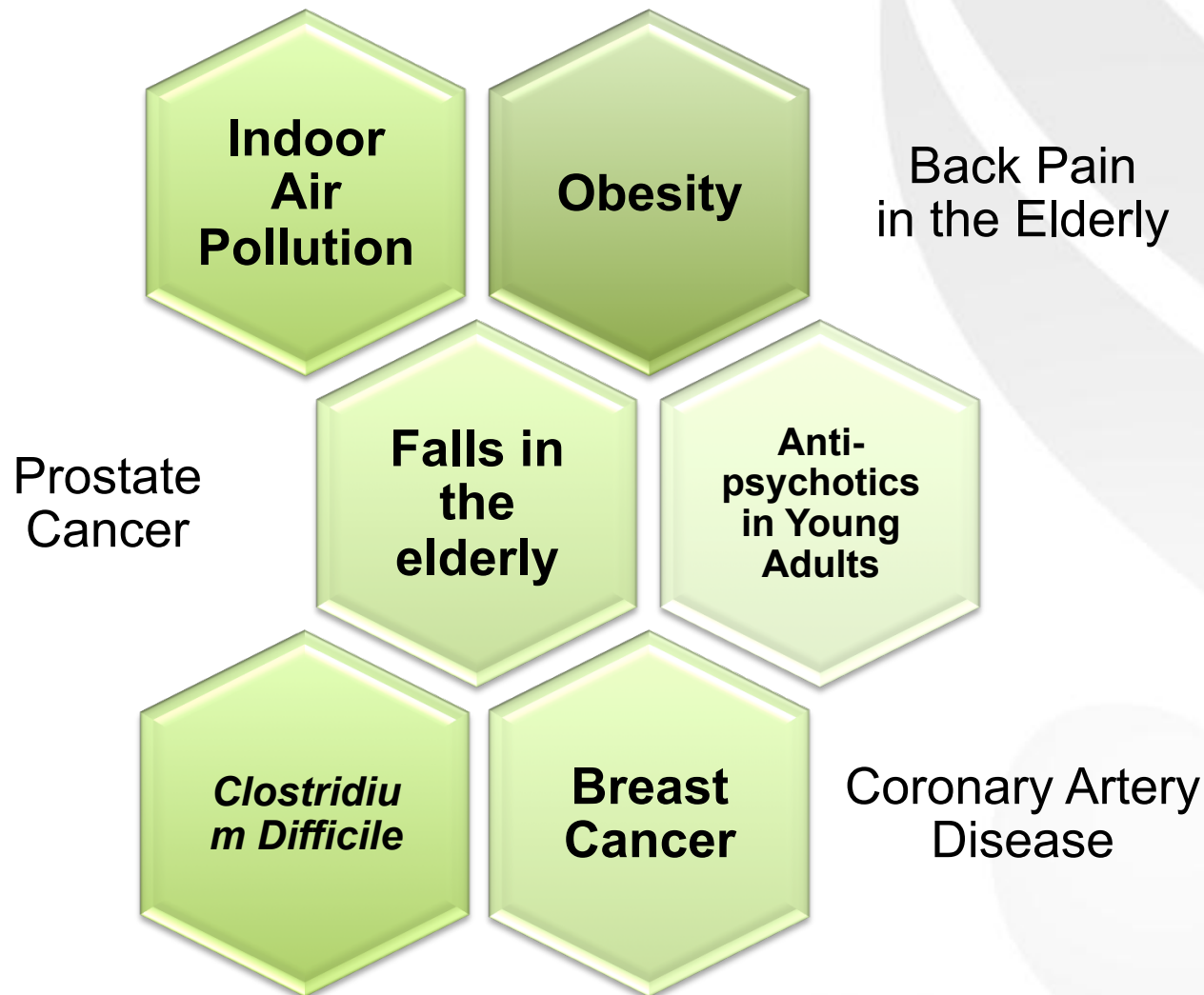
Experience of Other Agencies



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



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

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

☐ 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

<input type="checkbox"/> Patient-centeredness	Not rated
<input type="checkbox"/> Impact on individuals and populations	Not rated
<input type="checkbox"/> Differences in benefits	Not rated
<input type="checkbox"/> Reduction in uncertainty	Not rated
<input type="checkbox"/> Probability of Implementation	Not rated
<input type="checkbox"/> Durability of information	Not rated
<input type="checkbox"/> Potential for impact on healthcare system performance	Not rated
<input type="checkbox"/> Inclusiveness of different populations	Not rated

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: [1](#) ... [30](#) [31](#) [32](#) [33](#) [34](#) [35](#) [36](#) ... [42](#) Evaluated: 1/108

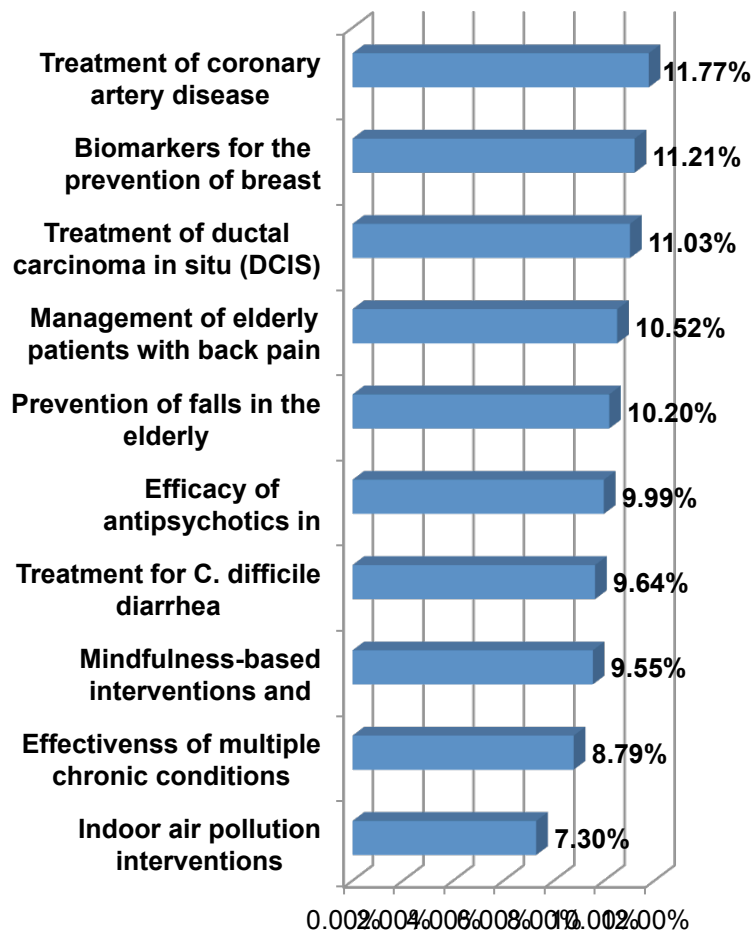
[Next Unassessed](#)

[Previous](#)

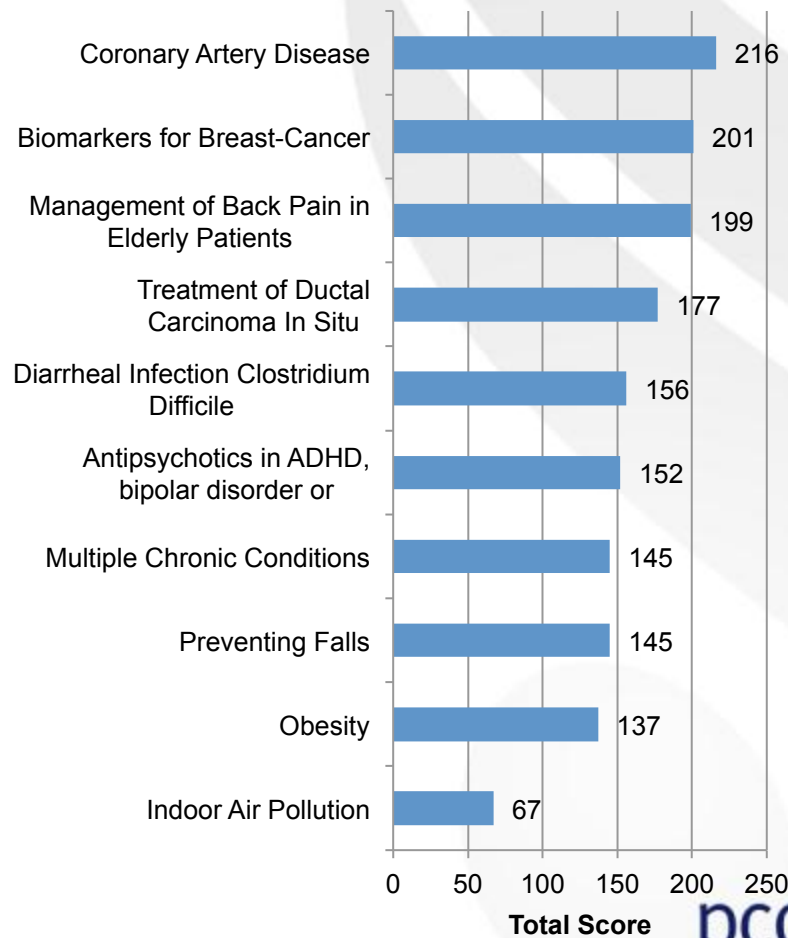
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Group 1 Results Using Two Softwares

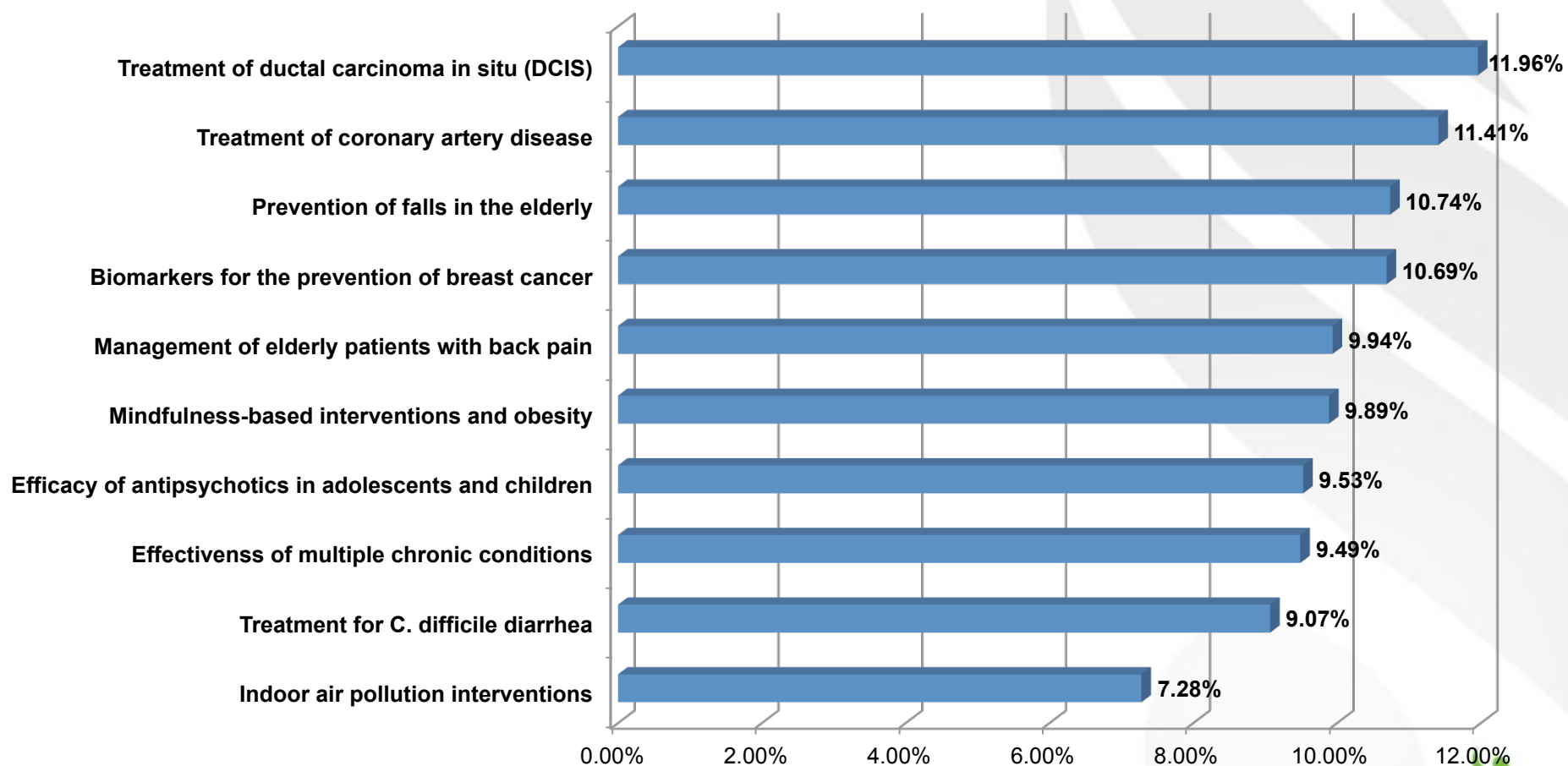
Expert Choice



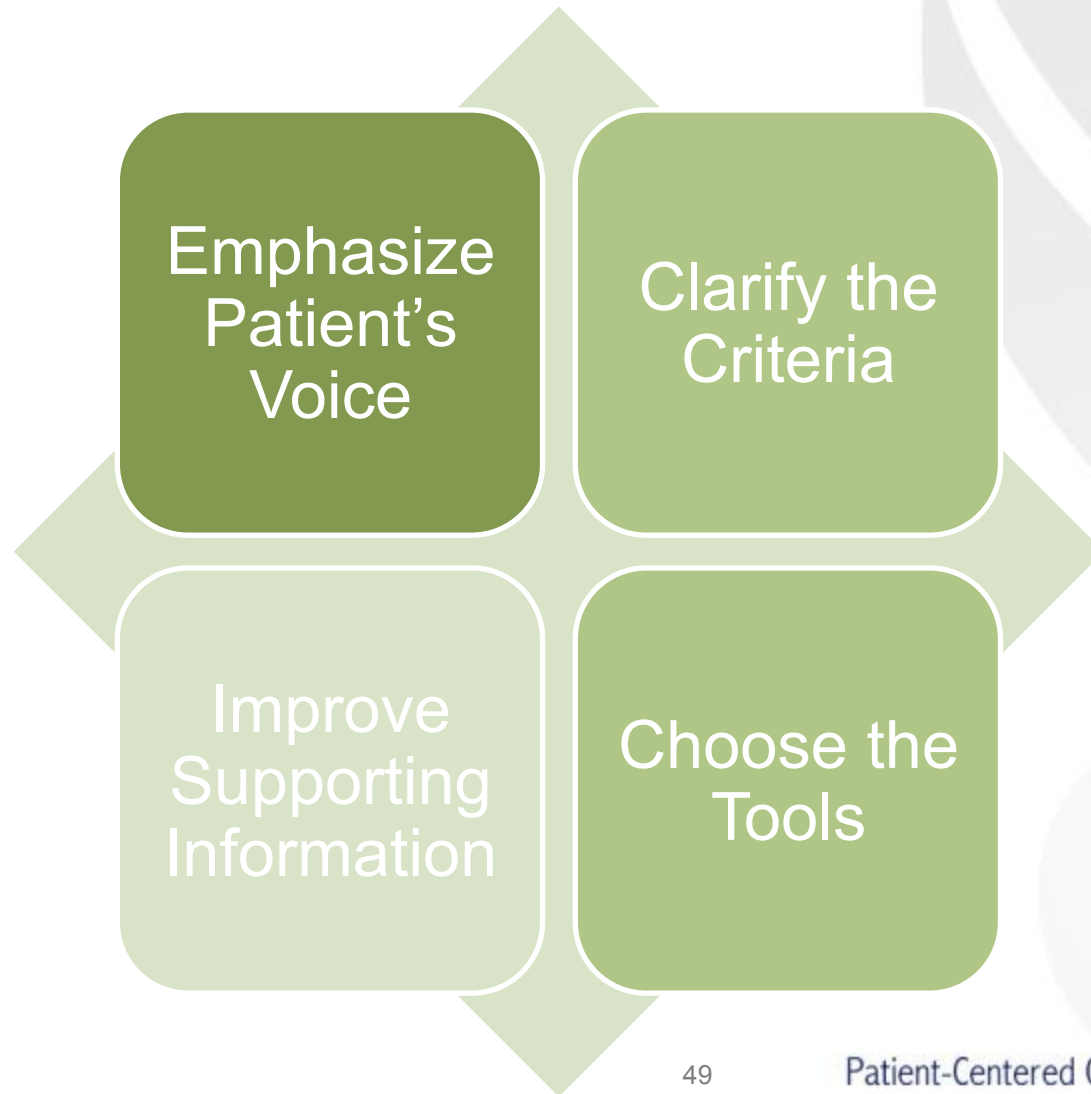
Survey Gizmo



Group 2 Results



Participants Provided Valuable Insights to Improve the Process

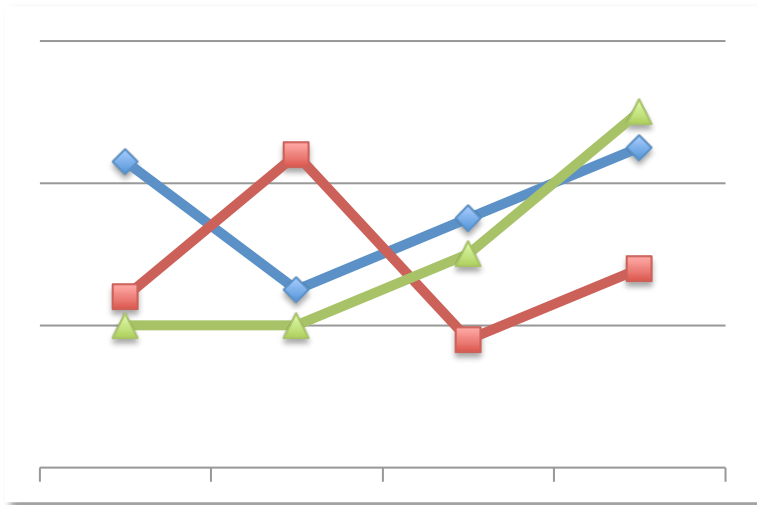


1. Patient-Centeredness



- 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

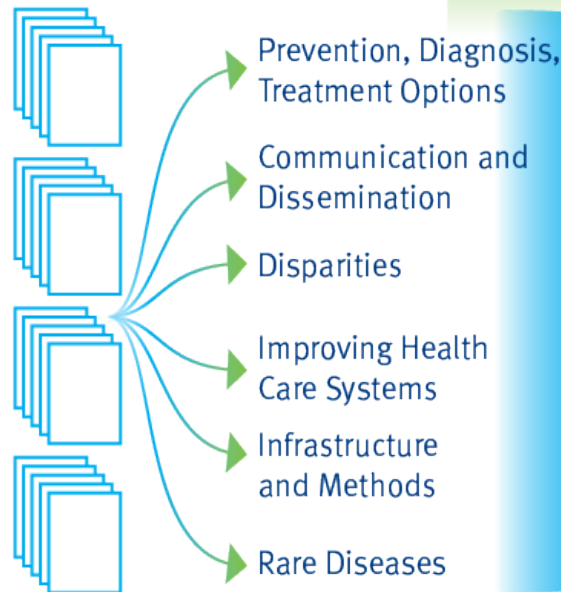


- Revisions
- Implementation
- Learning from ARRA

Launching PCORI's Research Prioritization Process

From Research Questions to Research Studies

Research Questions Suggested by Patients and Stakeholders Need Prioritization



Advisory Panels
Patients and Stakeholders

Research
Prioritization
Process
Using PCORI
Criteria

Board of Governors

Prioritized List of Topics



Selected From Prioritized List



Creation of PFAs



Acknowledgements





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

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Soliciting Research Topics: What Have We Learned?

3:00 – 4:00 pm

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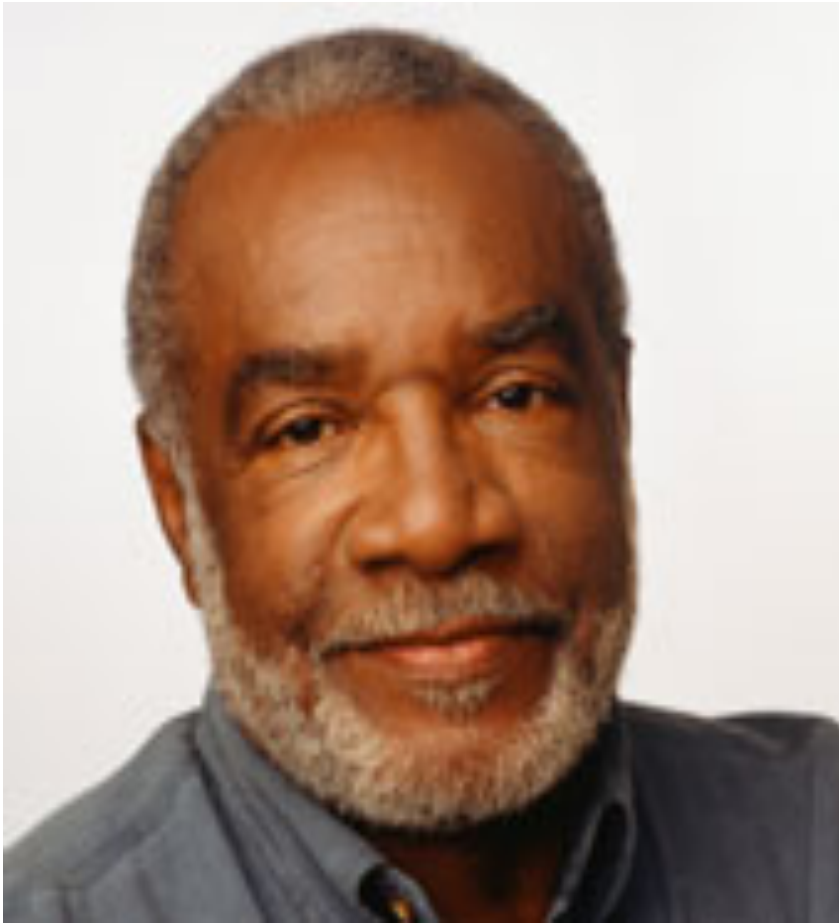
How to Prioritize: A Real World Example

Kara Odom Walker, MD, MPH, MSHS
PCORI Scientist

TWITTER: #PCORI EMAIL: getinvolved@pcori.org

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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

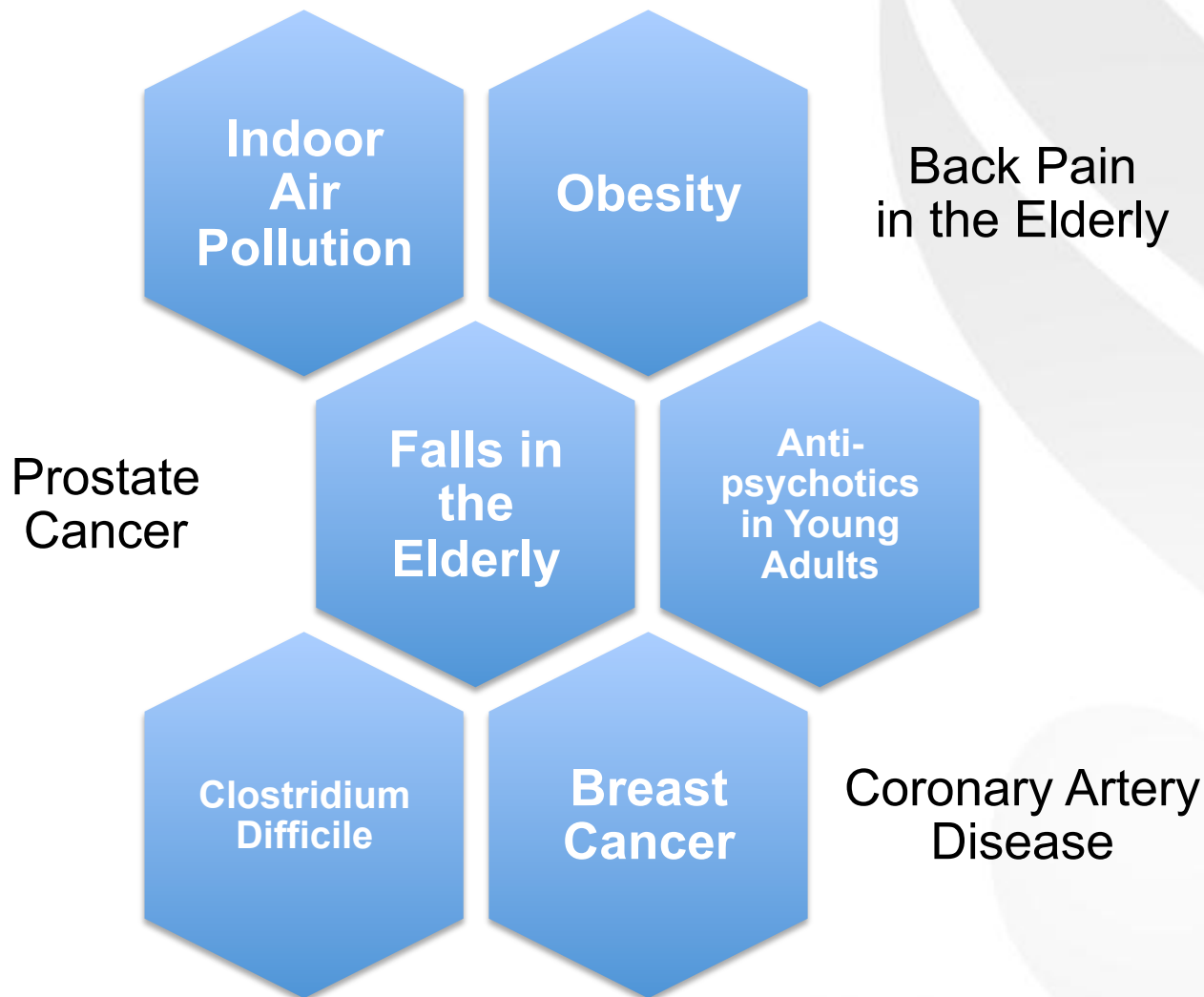
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What is the best treatment
for my husband's coronary
heart disease, given his
other medical conditions?

Other Questions Also Come from a Diverse Range of Disease Areas



Getting to Specificity: A Multi-Step Process

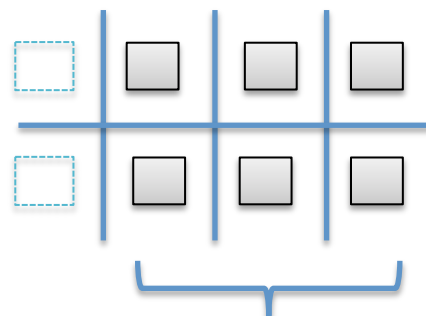
Topic Generation

Gap Confirmation

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For Example: Treatment of Coronary Heart Disease

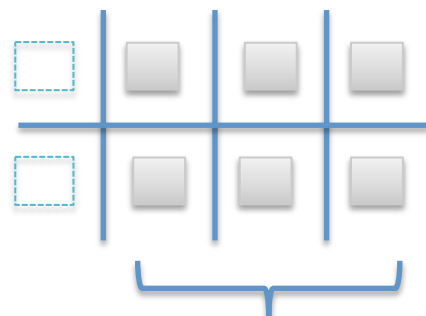
Evidence:

- 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

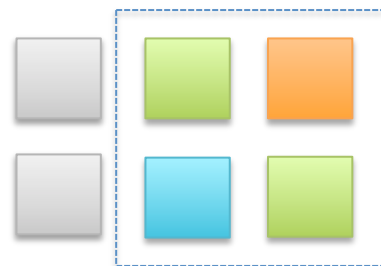
Getting to Specificity: A Multi-Step Process



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Research
Opportunities



Original PCORI Criteria for Research Prioritization Process



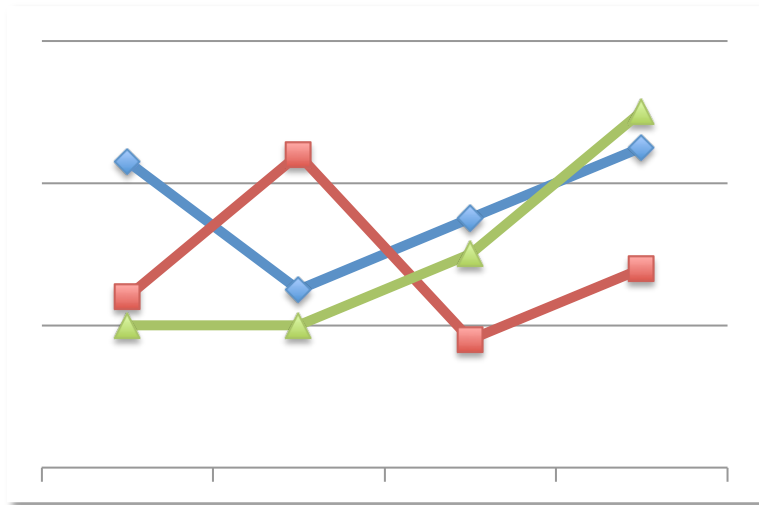
- Patient centeredness
- Impact
- Differences in benefits and harms
- Reduction in uncertainty
- Implementation in practice
- Duration of information
- Healthcare system performance
- Inclusiveness of different populations

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

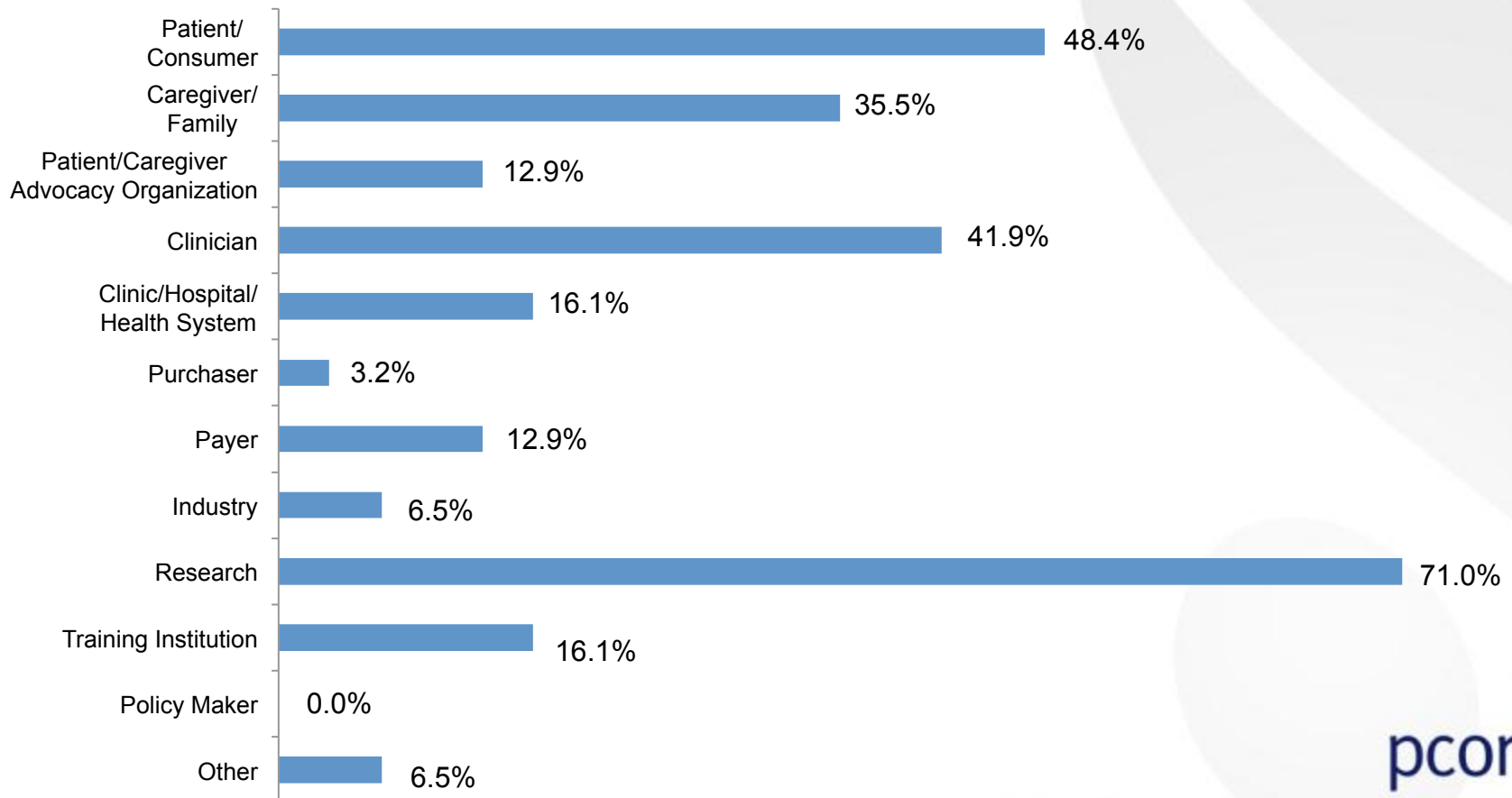


- Will research findings **be valid by the time** the study has concluded?

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

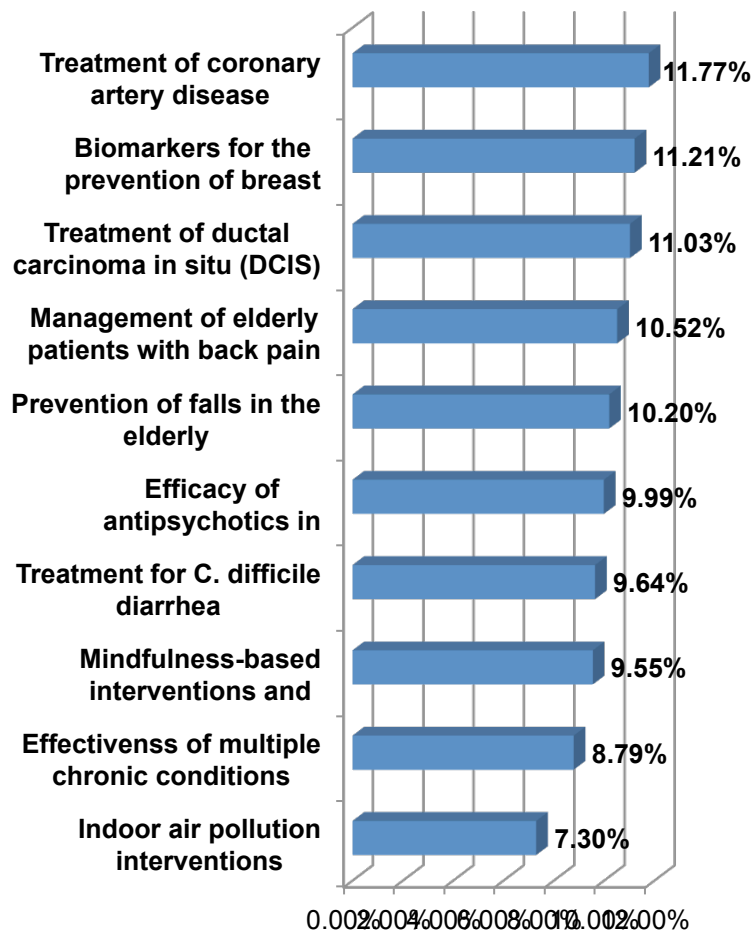


Different Tools

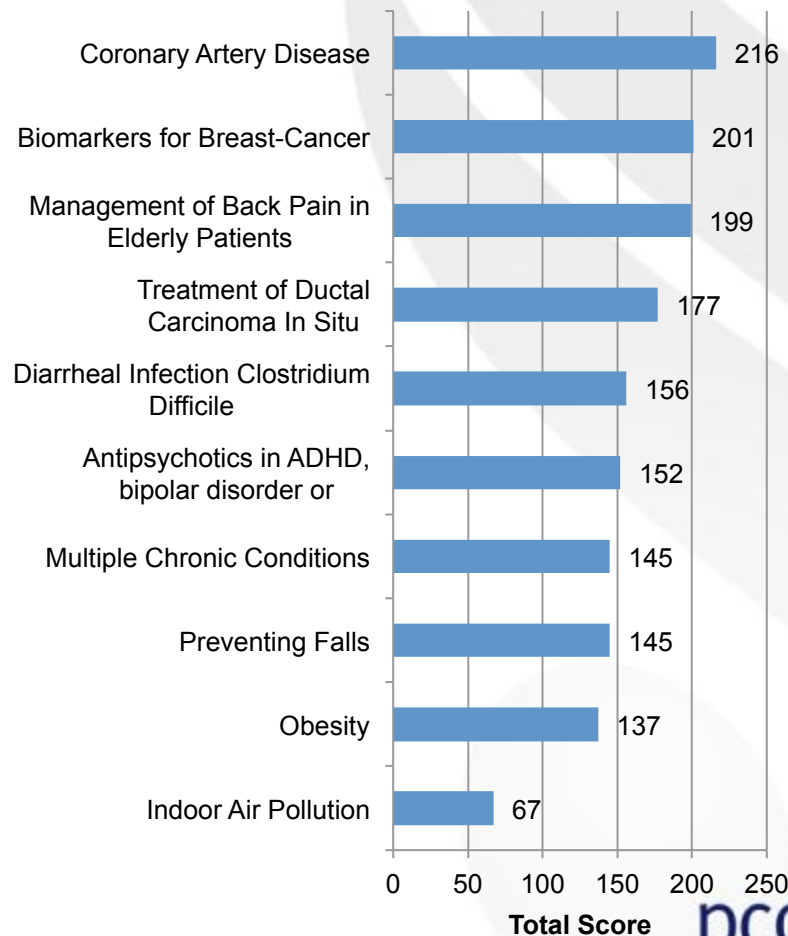


Sample Results: Comparing Lists from Expert Choice and Survey Gizmo

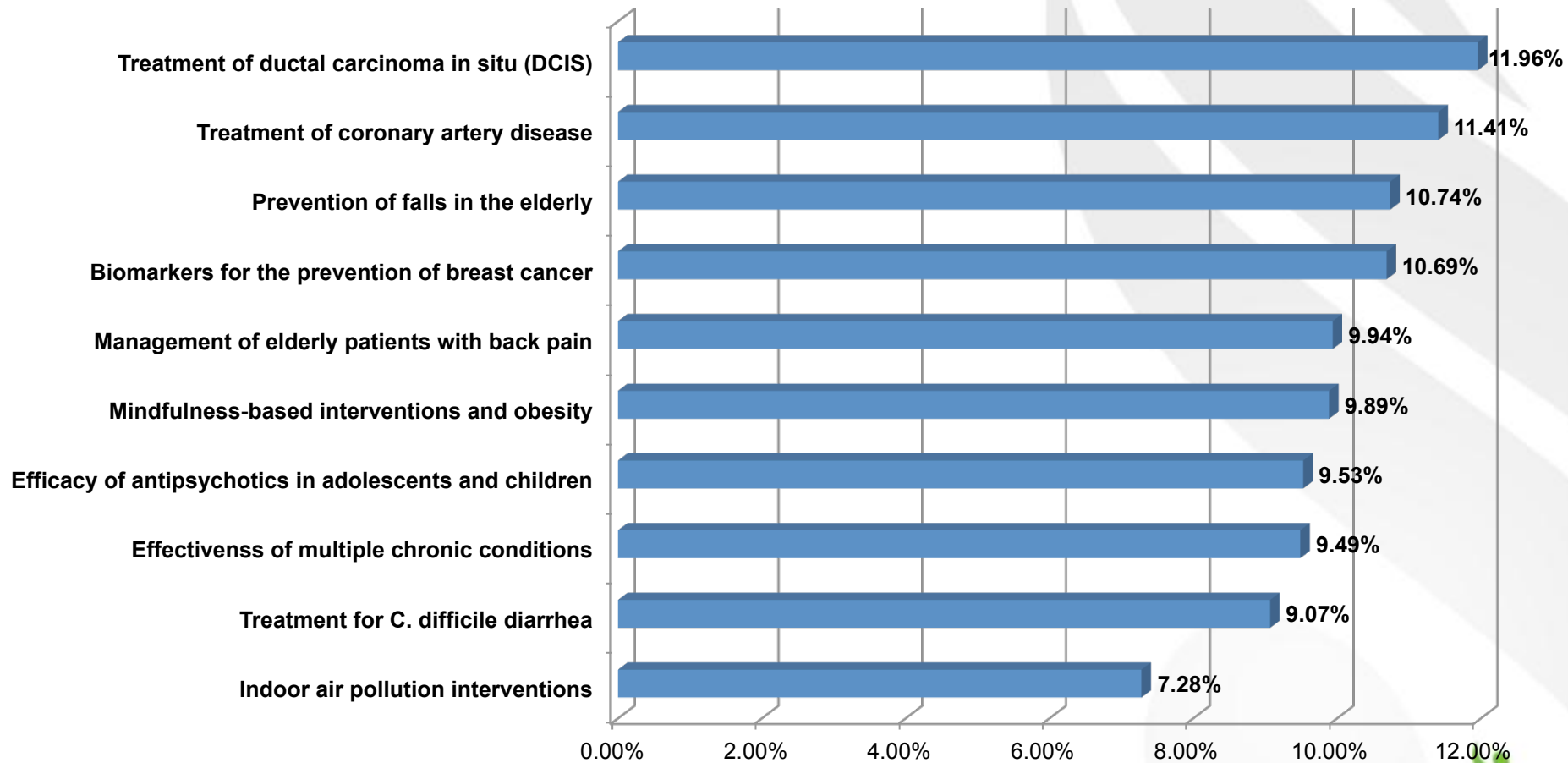
Expert Choice



Survey Gizmo



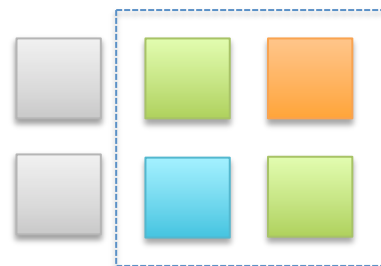
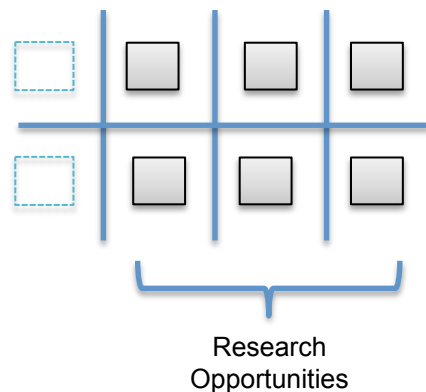
Sample Results: Ranked Topics with Group Generated Weights



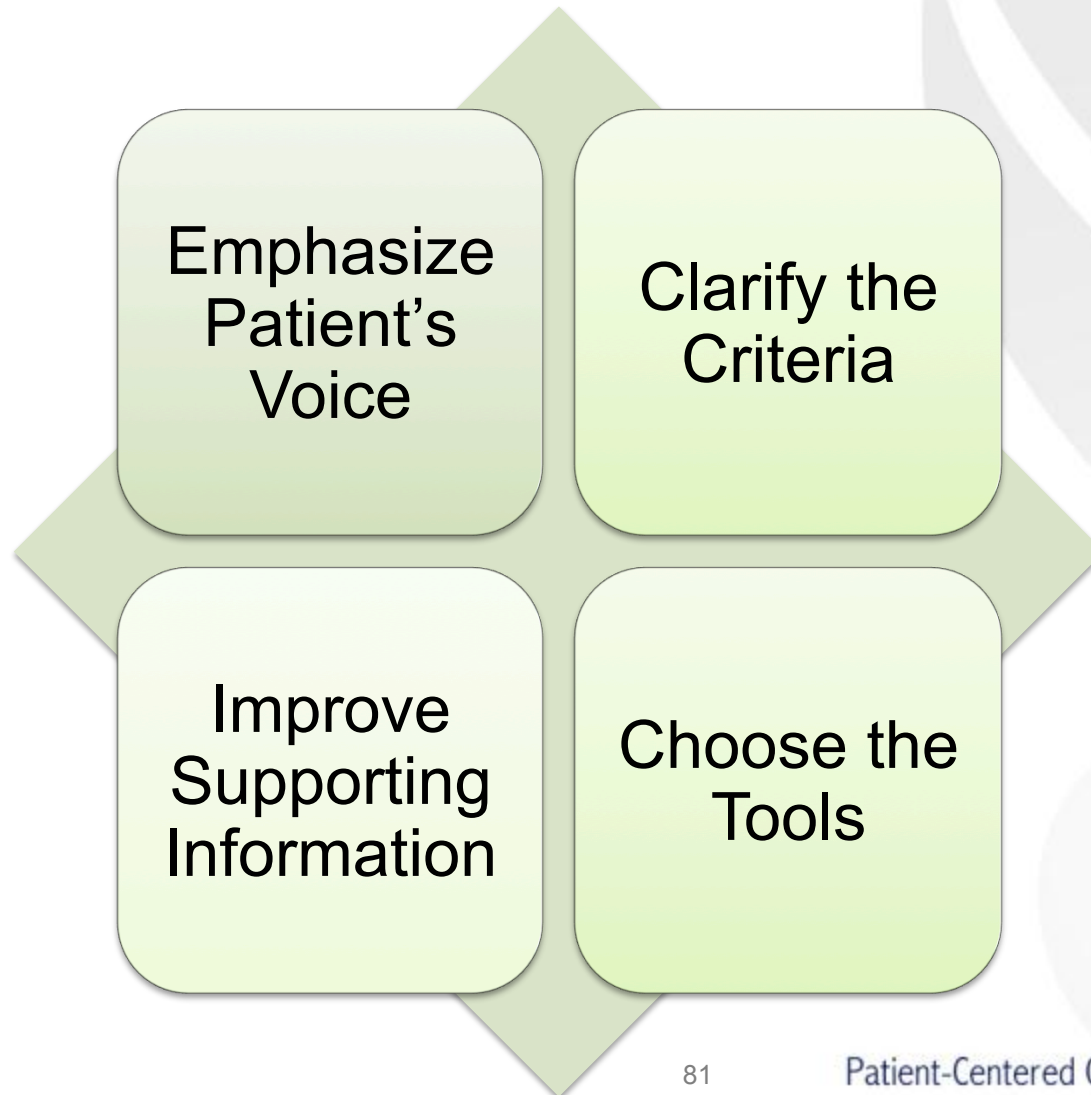
Getting to Specificity: A Multi-Step Process



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Participants Provided Valuable Insights to Improve the Process



Next Steps: PCORI's Research Prioritization Process

From Research Questions to Research Studies

*Research Questions
Suggested by Patients
and Stakeholders Need
Prioritization*



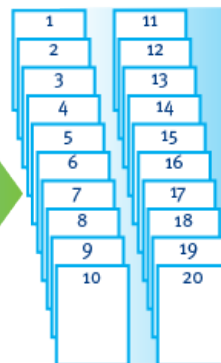
- Prevention, Diagnosis, Treatment Options
- Communication and Dissemination
- Disparities
- Improving Health Care Systems
- Infrastructure and Methods
- Rare Diseases

Advisory Panels
Patients and Stakeholders

Research
Prioritization
Process
Using PCORI
Criteria

Board of Governors

*Prioritized List
of Topics*



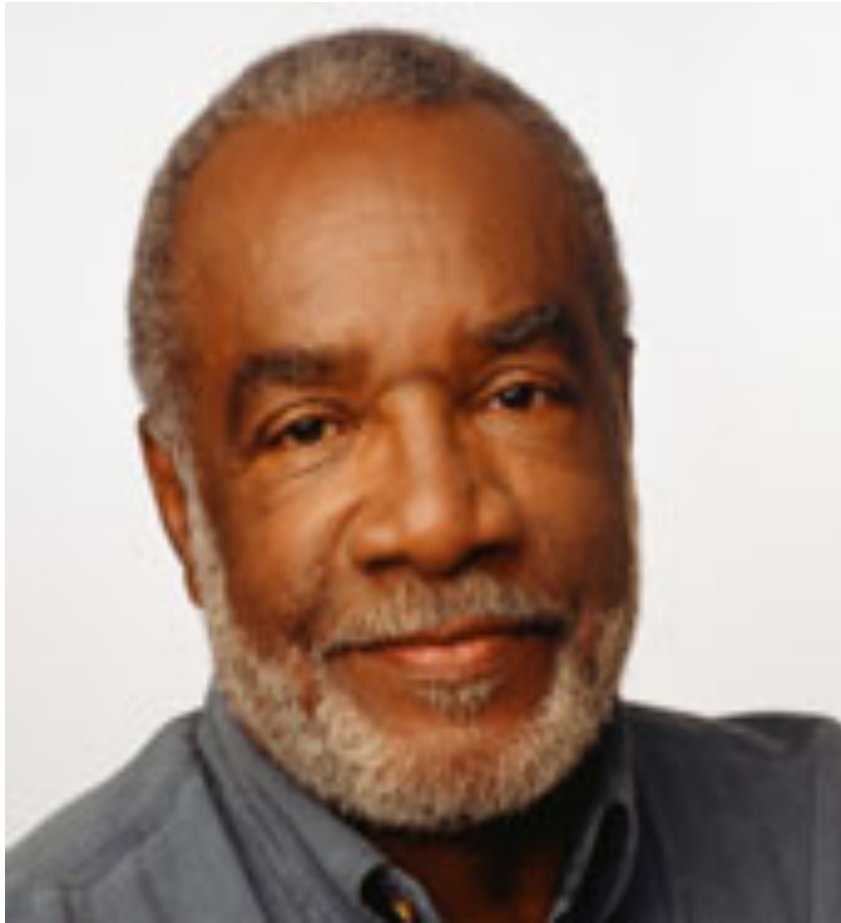
*Selected From
Prioritized List*



*Creation
of PFAs*



A Potential Answer for Mr. Jones



- 🌱 Mr. Jones listens to his options from the doctors and thinks about his choices for his heart disease
- 🌱 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



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

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