Welcome and Approval of Minutes

Eugene Washington, Chair, MD, MSc
PCORI Board of Governors Meeting
Boston, MA
November 2012

Patient-Centered Outcomes Research Institute

Executive Director's Report

Joe Selby, MD, MPH

PCORI Board of Governors Meeting

Boston, MA

November 19, 2012

Patient-Centered Outcomes Research Institute

Engagement


Engagement
Topic Generation
Research Prioritization
Specific PFAs

Transforming Patient-Centered Research: Building Partnerships and Promising Models

October 26-28, 2012

170 in-person attendees
40 states represented
~250 Webinar attendees each day
Video of sessions posted at pcori.org
5 Board members, 1 MC member present

Transforming Patient-Centered Research: Building Partnerships and Promising Models

Transforming Patient-Centered Research: Building Partnerships and Promising Models

Lessons Learned:

- Patient community is prepared and enthusiastic about participating with us in a transformed research enterprise
- PCORI's proposed strategies for engagement endorsed, but refinements offered to many aspects of the process
- Critical points added:
  - Researchers need training to engage with patients
  - Micro-grants could help bring patients and researchers together locally
  - Patients can play a stronger role in the application and in reporting/disseminating results

Upcoming Engagement Events

December 4: Stakeholder Engagement

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

December 5: Research Prioritization

PCORI Methodology Workshop for Prioritizing Specific Research Topics

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Chief Officer for Engagement

- Leads continued development of PCORI's strategic imperative of engagement - with the broad range of our stakeholders
- Builds on PCORI's extensive engagement efforts to date, supports our engagement team in implementing engagement program
- Serves as a principal spokesperson and represents PCORI to the highest levels of key stakeholder organizations and convenes these organizations for planning and conduct of patient-centered outcomes research
- Works closely with PCORI Board of Governors, its Communications, Outreach, and Engagement Committee (COEC) and Methodology Committees, to strengthen our ongoing relationships with stakeholder communities and to evaluate and enhance our efforts

Deputy Executive Director and Chief Operating Officer: Dr. Anne Beal

Deputy Executive Director
- Strategic Planning
- External Relations

Chief Operating Officer
- Contracting
- Finance
- Communication
- HR
- Facilities

Anne Beal, MD MPH

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Laura Forsythe
Research Associate
October 15, 2012

Brittany Jones
Sr. Administrative Assistant
October 15, 2012

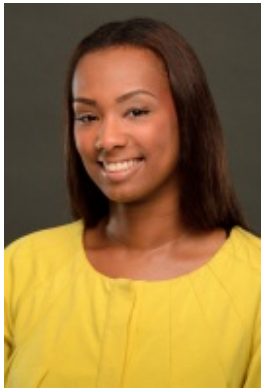
David Hickam
Director, Comparative Assessment of Options Research Program
October 29, 2012

Chad Boult
Director, Improving Healthcare Systems
October 29, 2012

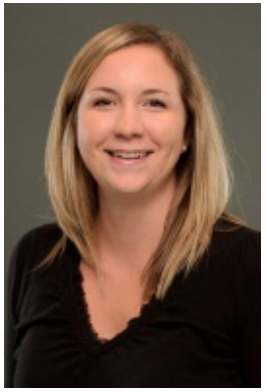
Sean Grande
Project Associate
October 31, 2012

Malik Dean
Sr. Administrative Assistant
November 2, 2012

Natalie Wegener
Project Coordinator
September 24, 2012



Tommesha Allen
Sr. Administrative Assistant
September 24, 2012



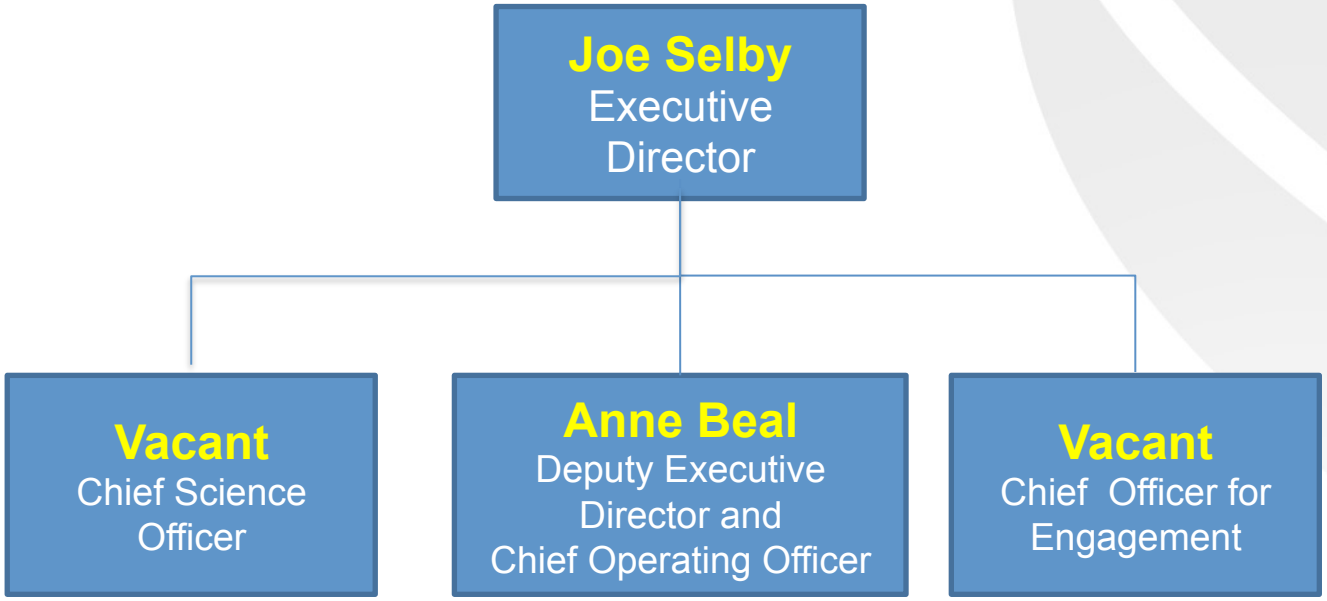
Camille Blackman
Project Coordinator
September 26, 2012

Romana Hasnain-Wynia
Director, Health Disparities Program
October 1, 2012

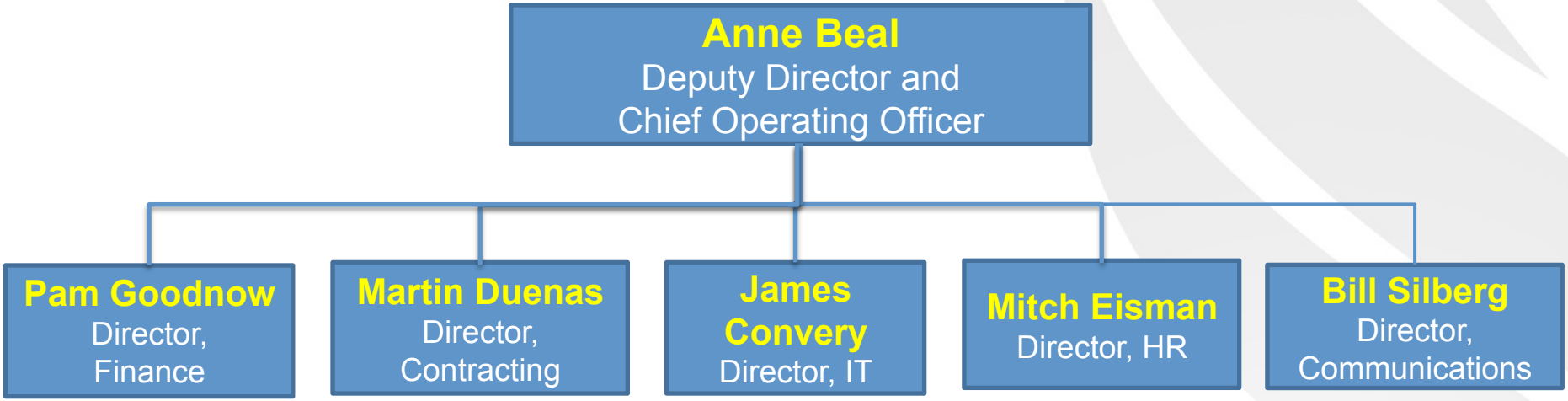
Aingyea Kellom
Project Associate
October 8, 2012

Jim Convery
Director of Information Technology
October 8, 2012

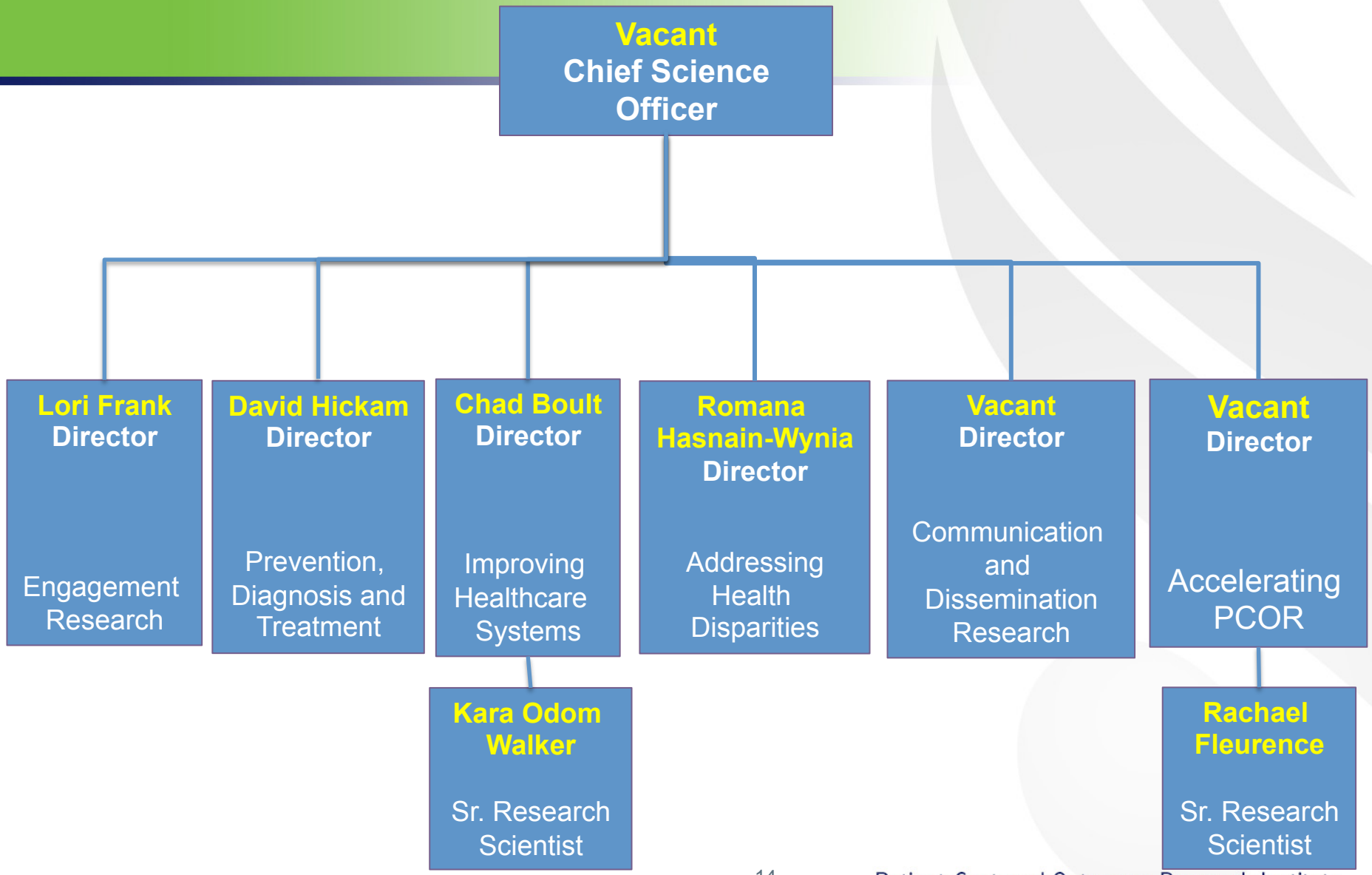
Executive Office



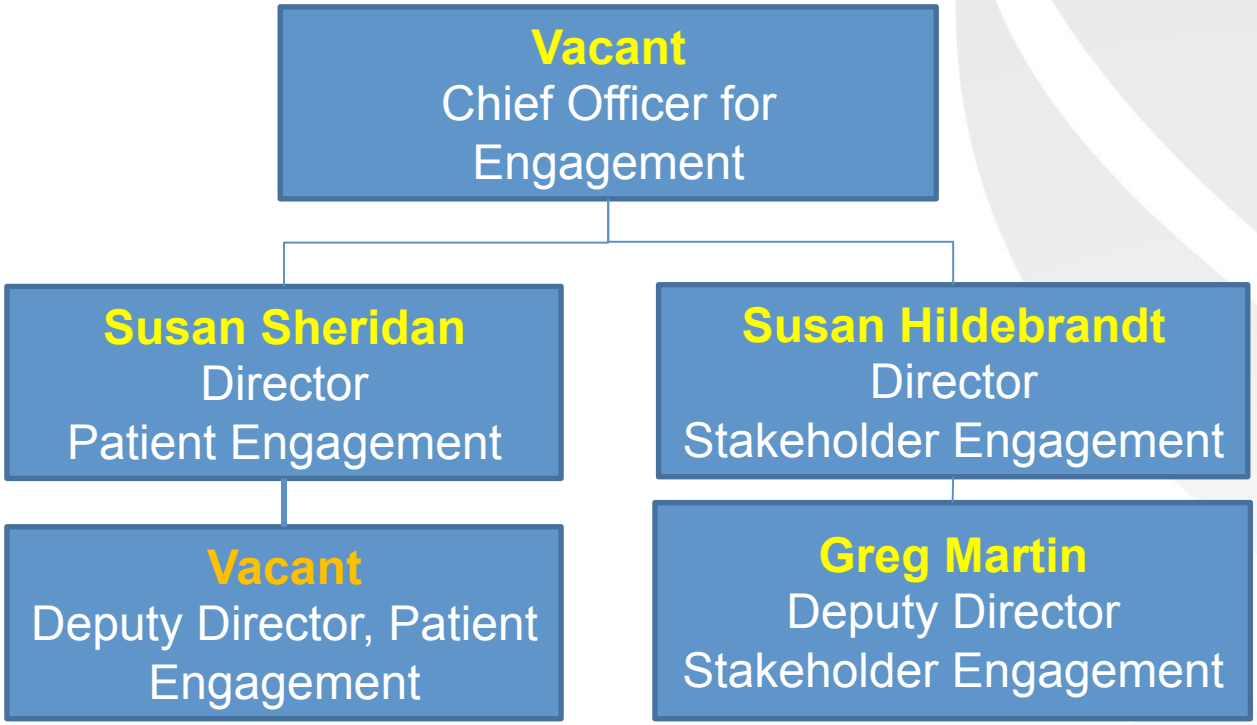
Operations


Anne Beal
Deputy Director and
Chief Operating Officer
Pam Goodnow
Director,
Finance
Martin Duenas
Director,
Contracting
James
Convery
Director, IT
Mitch Eisman
Director, HR
Bill Silberg
Director,
Communications

Science


Vacant
Chief Science Officer
Lori Frank
Director
Engagement Research
David Hickam
Director
Prevention, Diagnosis and Treatment
Chad Boult
Director
Improving Healthcare Systems
Romana Hasnain-Wynia
Director
Addressing Health Disparities
Vacant
Director
Communication and Dissemination Research
Vacant
Director
Accelerating PCOR
Kara Odom Walker
Sr. Research Scientist
Rachael Fleurence
Sr. Research Scientist

Engagement


Vacant
Chief Officer for Engagement
Susan Sheridan
Director
Patient Engagement
Vacant
Deputy Director, Patient Engagement
Susan Hildebrandt
Director
Stakeholder Engagement
Greg Martin
Deputy Director
Stakeholder Engagement

Preview – Today's Meeting

- Methodology Committee Report – Revised Standards
- PCORI Advisory Committee Charters
- Proposed 2013 Budget
- Update on PCORI Pilot Projects
- PFA Cycle 1 – Update on Review Process
- Initial Targeted PCORI Funding Announcements
- Nominating Committee – 2013 Committee Assignments

November Board Meeting
Methodology Committee Briefing

Sherine Gabriel, MD
Sharon-Lise Normand, PhD

PCORI Board of Governors Meeting
Boston, MA
November 2012

Patient-Centered Outcomes Research Institute

Goal for today

- High level update of activities
- Propose adoption of revised standards and recommended actions
  - Endorse dissemination initiative
- Review next steps

Draft Methodology Report – Process

Committee Expertise

1	**Methods Selection**	• Working groups identified and prioritized major research methods questions to be addressed
2	**Information Gathering**	• Researchers contracted to address selected topics • Contractors developed research materials (e.g., reports, summary templates for proposed standard) • MC solicited for external feedback on the translation table (RFI) • Workshops held to discuss contractor findings, with invited experts in attendance
3	**Internal Review**	• MC conducted in-depth internal review of materials developed by contractors, and support staff • MC independently submitted preliminary votes on proposed standards • MC deliberated to reach consensus on recommendations to be endorsed in the report
4	**Report Generation**	• Presented to Board May 2012 • Posted for public comment July 2012

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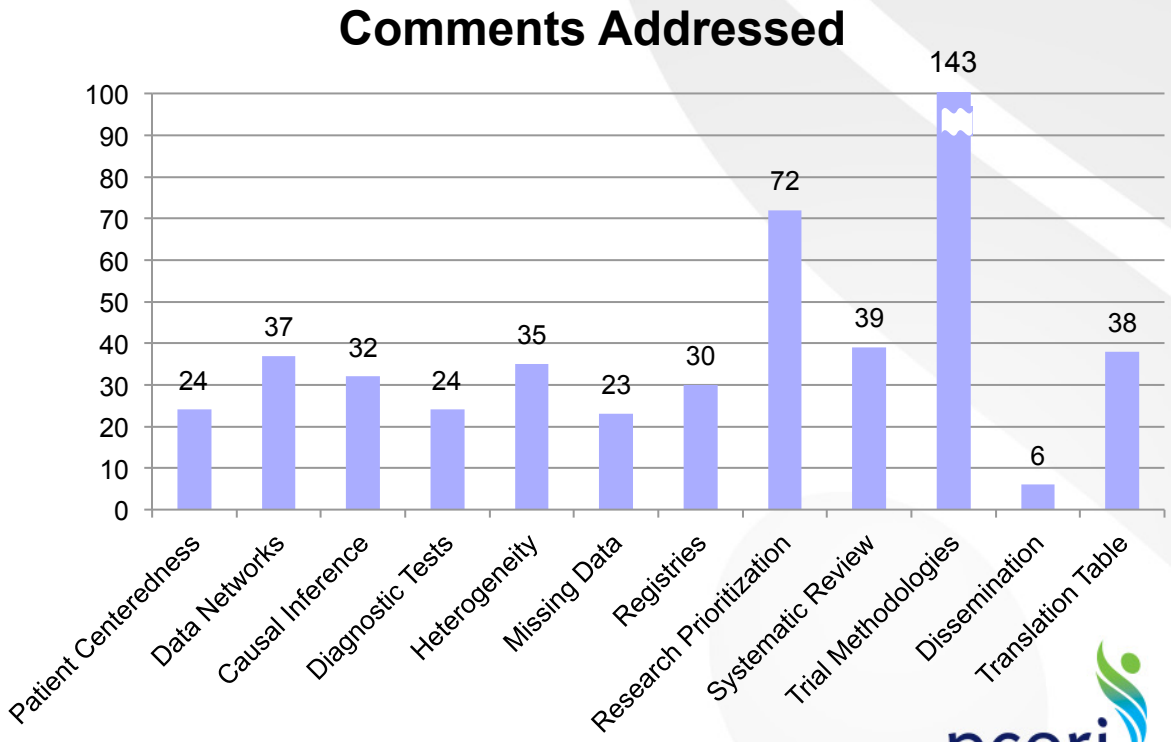
Public Comment Summary

124 groups or individuals submitted comments
Over 1400 comments, 503 applicable to standard topics



Major Themes From Public Comment

Themes

- Guidance on Implementing Standards
- Relation of Standards to Research Funding
- Topic Gaps
- Document Accessibility
- Feasibility of Standards to Produce Patient-Centered Research Findings

Review of Comments, Revision of Standards and Recommended Actions

July-September

- 12 topic areas addressed by Methodology Committee (MC) & Work Groups (WGs)
- WGs met to discuss comments and revisions to Standards and Recommended Actions
- WGs solicited outside expertise for research prioritization, HTE, diagnostic test, and adaptive trials
- WGs drafted proposed revisions to Standards and Recommended Actions

October

- Full MC reviewed comments and proposed revisions *October 12-19*
- Full MC Consensus Meeting held to determine final MC revisions to Standards and Recommended Actions *October 31*

November

- MC unanimously endorsed set of revised standards and recommended actions
- MC delivered revised Standards and Recommended Actions for Board adoption
- MC drafted responses public comment themes

Revisions to Methodological Standards

- 21 were revised
  - 14 Significant changes in content
  - 7 Revisions to wording
- 19 were deleted, expanded, or consolidated
- 21 were not changed

Comment	Summary of Revision	Example
The causal inference standards seem to focus on problems involving point exposures/treatments. They do not seem to address problems involving time-varying treatments/ exposures. However, PCORI's mission includes such longitudinal problems…..	Standards were revised to allow for time varying covariates.	Define Analysis Population Using Covariate Histories ~~Information Available at Study Entry~~ Decisions about whether patients are included in an analysis should be based on information available at each patient's time of study entry ~~and not based on future information such as future changes in exposure~~ in prospective studies or on information from a defined time period prior to the exposure in retrospective studies. For time-varying treatment or exposure regimes, specific time points should be clearly specified and the covariates history up to and not beyond those time points should be used as population descriptors.
..could be expanded to include assessment of common support across comparison groups, and possibly greater clarity in the description of the propensity score model.	The idea of 'common support' or overlap was added to the standard on propensity scores.	~~Assess~~ Report the assumptions underlying the construction of Propensity Scores ~~balance~~ and the comparability of the resulting groups in terms of the balance of covariates and overlap. When conducting analyses that use propensity scores to balance covariate distributions across intervention groups, researchers should assess the overlap and balance achieved across compared groups with respect to potential confounding variables.
….believe 'intervention' should be changed to 'exposure'	Intervention was changed to exposure as it is a more general term	Precisely Define the Timing of the Outcome Assessment Relative to the Initiation and Duration of ~~Intervention~~ Exposure To ensure that an estimate of an exposure or intervention effect corresponds to the question that researchers seek to answer, the researchers must precisely define the timing of the outcome assessment relative to the initiation and duration of the ~~intervention~~ exposure.

Revisions to Recommended Actions

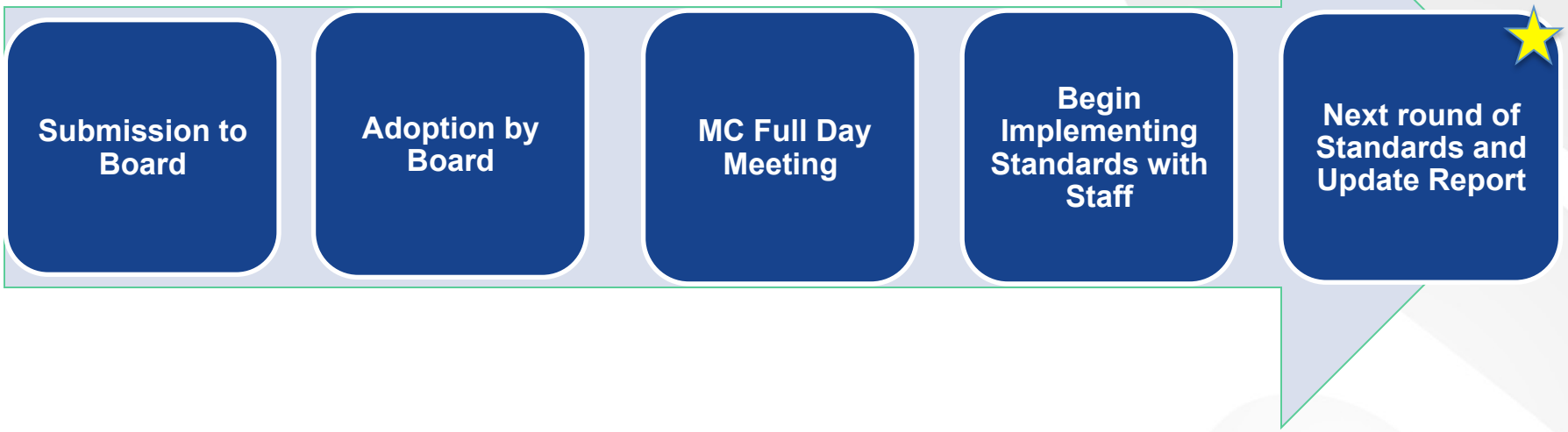
- 13 were revised
- 25 were deleted, expanded, or consolidated
  - Some converted from standards to recommended actions
- 30 were not changed

Next Steps

Deliverable to Board

Determine next round of strategic priorities

Report Revision Complete



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Dissemination and Implementation of the Standards

1. Adherence to the standards will require changes in the ways in which research is **solicited, designed, reviewed and funded, conducted, monitored, reported, and disseminated**.

2. Changing research practice will require multi-component, multi-level, multi-stakeholder coordinated efforts.

3. The Methodology committee with PCORI staff and Board
   (a) coordinate efforts with external groups:
      - including convening advisory committees as needed

   (b) prioritize and stage dissemination activity

Implementation Plan: Questions for Board

- Endorse COEC and MC to develop a new initiative to achieve widespread implementation of the standards

- Endorsement of proposal to convene a new advisory group for this initiative comprising BoG, MC and external stakeholder representatives, with COEC as the key BoG liaison and oversight group

Goal for today

- High level update of activities
- Request approval of revised standards and recommended actions
  - Endorse dissemination initiative
- Review next steps

Sharon-Lise Normand, PhD
Methodology Committee Vice Chair

Thank you for your commitment and service!

Break

Meeting Schedule

DATE	LOCATION
February 3-5, 2013	San Francisco, CA
May 5-7, 2013	Chicago, IL
September 22-24, 2013	Washington, DC
November 17-19, 2013	Atlanta, GA

Advisory Panel Charters

Anne Beal, MD, MPH
Chief Operating Officer, Deputy Executive Director
PCORI Board of Governors Meeting
Boston, MA
November 2012

Patient-Centered Outcomes Research Institute

Reviewed by COEC, October 30, 2012 and PDC, November 13, 2012

Getting Up to Speed: Advisory Panel Recap

Legislative Authorization

What does the law say expert advisory panels should include?

- Expert advisory panels should include clinicians, researchers, patients, and other experts with the appropriate experience and knowledge to assist PCORI in achieving its goals.

Purpose

What is the purpose of advisory panels?

- There is a lot of work to be done!
- With PCORI's staff, Methodology Committee, and Board of Governors, advisory panels will assure meaningful patient engagement in: (1) PCORI's research activities; (2) identifying research priorities and topics; (3) conducting randomized clinical trials; and (4) performing special research studies.
- Leveraging members' expertise will help better inform PCORI's mission and work.

Framework and Composition

How will they be structured?

- Each 12-21 member panel will have a unique charter, term duration, and clearly defined scope of work.
- PCORI staff presents a group of nominees to the Board for approval. The Board appoints a chairperson.
- Members will be selected based on their expertise and ability to contribute to the work of specific panels.
- Members will be compensated and appointed for an initial one-year term with an option to be re-appointment for a second year.

Getting Up to Speed: Advisory Panel Recap

Conflicts of Interest

Will panel members be eligible for future PCORI funding?

- Panel members are not making decisions on funding, programs, or operations.
- Focus on transparency and building information firewalls will prevent conflicts from arising.
- Advisory panel membership generally does not preclude eligibility for funding.
- Members will be advised of unique instances where their role could result in disqualification.

Panel Establishment

When will advisory panels be established?

- Three panels will be established in the first half of 2013.
- More to come in the future.

Questions for Board Consideration

1. Is the scope of work outlined in the three advisory panel charters appropriate?
2. Please comment on the proposed additional advisory panels for Q1/2013.

Advisory Panel Establishment Process

1 Staff Draft and Submit Charter for an Advisory Panel

- Board, Methodology Committee, and/or PCORI staff identify the need to establish an Advisory Panel
- Staff initiates request for an advisory panel by submitting a panel-specific charter

2 Board Reviews the Proposed Advisory Panel Charter

- Board may authorize charter (proceed to step 3)
- Board may request revisions to the charter (return to step 1)

3 Staff Activates Nomination and Selection of Panel Participants

- Staff initiates open call for nominations, via the PCORI Web site and other communications
- Nominees submit an expression of interest, via the PCORI Web site
- Staff evaluates nominees, per evaluation criteria unique to the panel charter

4 Board Approves Panel Participants

- Staff selects and proposes a slate of panel nominees to the Board
- Board authorizes and approves the nominees for panel membership
- Board selects a chairperson from the panel membership

Staff Phase *Board Phase*

Review First Three Advisory Panel Charters

- Patient Engagement
- Comparative Assessment of Options
- Health Disparities

Charters are included in the appendices section

Four Advisory Panels approved by the BOG, September 2012

Proposed Panel: Patient Engagement

- **Purpose:** To assure the highest patient engagement standards and a culture of patient-centeredness in all aspects of PCORI's research and dissemination activities.
- **Term:** 2 years
- **Membership:** Between 12–21 members with 75 percent patients, caregivers, and advocacy organizations and 25 percent researchers and other stakeholders.

Proposed Panel: Comparative Assessment of Options

- **Purpose:** To identify and prioritize critical research questions in PCORI-supported research and to advise PCORI on evaluating potential research topics related to the comparative effectiveness of alternative strategies for prevention, treatment, screening, diagnosis, and management of disease
- **Term:** 2 years
- **Membership:** Between 15–21 members. At least 25 percent of panel members will be patients, caregivers, and advocacy organizations. The remainder members will include clinicians, researchers and other stakeholders

Proposed Panel: Health Disparities

- **Purpose:** To identify and prioritize critical research questions for possible funding under PCORI's research priority addressing health disparities, and provide ongoing feedback and advice on evaluating and disseminating the research conducted under this priority. ***The focus is on studies that will inform the choice of the best strategies to eliminate disparities rather than studies that describe the problem.*** The studies related to addressing disparities must focus on areas of importance to patients and their caregivers, where there are critical disparities that disadvantage members of a particular group and limit their ability to achieve optimal, patient-centered outcomes.
- **Term:** 2 years
- **Membership:** Between 15–21 members to include patients, caregivers, and advocacy organizations and as well as researchers and other stakeholders.

Future Panels: For Board Discussion

Four charter panels will be proposed for February/May 2013

- Randomized Clinical Trials*
- Rare Diseases*
- Health Systems
- TBD

*Required by statute

Questions for Board Consideration

1. Is the scope of work outlined in the three advisory panel charters appropriate?
2. Please comment on the proposed additional advisory panels for Q1/2013.

Board Vote: Recommend Approval

- Patient Engagement
- Comparative Assessment of Options
- Health Disparities

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Appendix A: Advisory Panel: Patient Engagement
Appendix B: Advisory Panel: CER
Appendix C: Advisory Panel: Health Disparities
Appendix D: Selection Criteria: Patient Engagement
Appendix E: Selection Criteria: HD & CER

The 2013 Budget Plan

Kerry Barnett, Chair, FAAC
Anne Beal, Deputy Executive Director and Chief Operating Officer
Pamela Goodnow, Director of Finance
PCORI Board of Governors Meeting
Boston, MA
November 2012

Patient-Centered Outcomes Research Institute

Agenda

- Key Points
- Performance-Based Budgeting
- The 2013 Budget
- Projection for 2014
- Comparative Analysis
- Commitments and Outstanding Obligations
- Cash Flow
- Questions and Answers

Key Points

- Align budget with strategic goals
  - Adjust to lower cash flow expectations
  - Provide flexibility for quick-turnaround, rapid response funding
  - Target for administrative expense set at 10%
  - Focus on infrastructure and operations activities

	Infrastructure and Operations Activities
✓	Refine the staffing model
✓	Reduced reliance on contract staffing
✓	One-time investments in infrastructure

Performance-Based Budgeting

- Definition
  - Performance budgets use the mission and goals to allocate resources to achieve specific objectives based on program goals and measured results.
  - The activities that are required to accomplish the program goals are defined and funded at the department level.

Performance-Based Budgeting

- PCORI Budget Process
  - Define long-term goals
    - Engaging patients and stakeholders so that they can participate in the PCORI research enterprise in a meaningful way
    - Advancing rigorous PCOR methods; methodology standards adopted as best practices across the nation
    - Funding PCOR so that PCORI impacts decision-making, practice, and patient outcomes
    - Communicating and disseminating PCOR findings
    - Developing a sustainable infrastructure for conducting PCOR

Performance-Based Budgeting

- Refine the staffing model

Office	Provides
Chief Executive	Program support and general management
Chief Science	Pre-award PFA/TFA development Post-award monitoring and compliance Methodology Committee support Project management: intramural research
Research	Project management: extramural research
Chief Operating	General management and administration

Performance-Based Budgeting

- Reduced reliance on contract staffing will save over $700,000 per month, which allows for 25 additional FTEs at little additional cost.

DAILY OPERATIONS	Staff	Contractor	Total
2012 FTE	34	38	72
2012 Monthly Spend	$628,067	$1,090,000	$1,718,067
2013 FTE	88	9	97
2013 Monthly Spend	$1,417,689	$326,250	$1,743,939

Performance-Based Budgeting

- Target for administrative expense set at 10%
  - Program expenses are goods and services distributed to fulfill the mission of the organization
  - Administrative expenses are costs of business management, record keeping, budgeting, and finance and other management and administrative activities
  - The percentage of administrative expenses is a measure of a non-profit's efficiency.
  - The industry standard is 15%.

Performance-Based Budgeting

- One-time investment of $6.5 million in infrastructure for program support and operations

	Investment in Infrastructure
✓	Website for interactive Methodology Report
✓	Researcher datamart
✓	Customer Relationship Management software
✓	Digital communications platform
✓	Post-award contract management and compliance
✓	Cash management and financial reporting
✓	Network hardware and software

The 2013 Budget

- Revenue Assumptions: Appropriation
  - $120 million
  - No adjustment has been made for the potential loss of revenue in the sequestration process

The 2013 Budget

- Revenue Assumptions: Fees
  - Assessed on plan years ending October 1 through December 31, 2012 (partial year), and estimated at 25 percent of original funding
  - Timing: funding based on estimates will be received between August 15 and October 15, 2013, in installments and the balance will be received in CY 2014
  - Transfer dates from the CMS Trust Funds have not been published

The 2013 Budget

- Expense Goals, Objectives, and Activities
  - The Methodology Committee and each of the program departments have developed projected expenses at the activity level to achieve their priorities
  - Board governance
  - Priorities for general management and administrative support include commitments to infrastructure, security, and oversight for cash management

The 2013 Budget

	IN MILLIONS	
OPERATING REVENUE	$147.2	
Program Expenses	136.6	86.68%
Administrative Expenses	21.0	13.32%
OPERATING EXPENSE	157.6	
Non-operating Interest Income	0.3	
NET INCOME	($10.1)*	

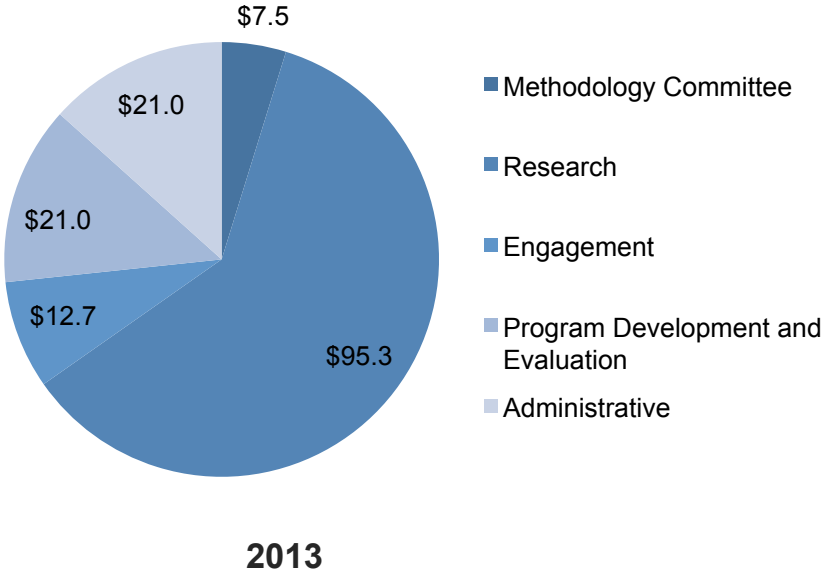
*Does not include monies carried over from 2012.

Projection for 2014

	IN MILLIONS	
OPERATING REVENUE	$543.0	
Program Expenses	272.0	91.58%
Administrative Expenses	25.0	8.42%
OPERATING EXPENSE	297.0	
Non-operating Interest Income	1.1	
NET INCOME	$247.1	

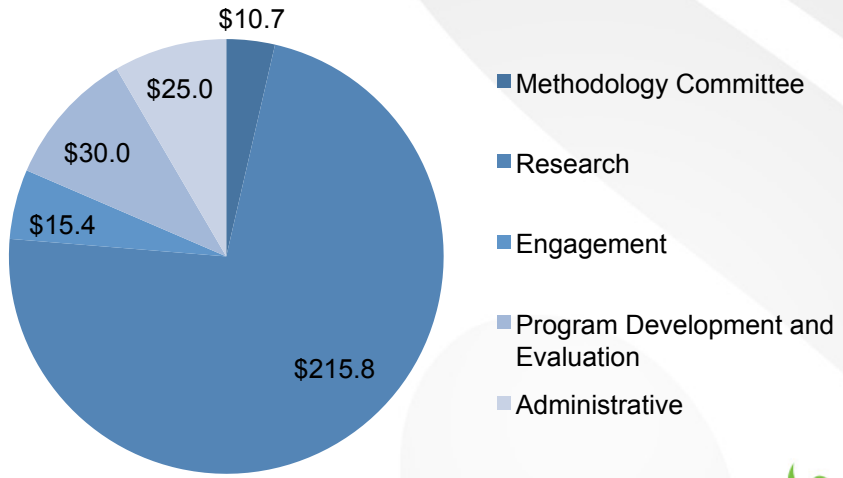
Comparative Analysis

Administrative Expense 13.35%



2013

Administrative Expense 8.42%



2014

Commitments and Outstanding Obligations

- Contracts awarded have two and three year life cycles
  - Contract negotiation for $31 million in PCORI Pilot Project awards will be complete in 2012
  - Contract negotiation for $96 million in research for the 2012 PFA 1 award cycle will be complete in 1Q2013
  - PCORI expects to award $300 million in research contracts during CY2013
  - There will be $304 million in outstanding obligated funding at December 31, 2013

Commitments and Outstanding Obligations

- Commitments and Outstanding Obligations

	IN MILLIONS
COMMITMENTS	
Pilot Projects	$31.0
PFA 2012	96.0
PFA 2013	300.0
	427.0
PCORTF Payments	(123.0)
OUTSTANDING OBLIGATIONS	$304.0

Cash Flow

- Cash Flow
  - Current projections indicate that PCORI will close CY2012 with an available cash balance of $233 million
  - Cash receipts projected at $147 million including interest earnings
  - Cash payments of $112 million will be made on basic research contracts
  - Cash needed for operations: $60 million
  - Projected cash carryover to 2014: $208 million

Cash Flow

- Cash available at the end of 2013

	IN MILLIONS
OPENING CASH BALANCE	$233.0
Cash Receipts	147.3
Cash Requirements	(172.7)
PROJECTED CASH BALANCE	$207.6

  - Reconciliation to financial statements:

	IN MILLIONS
BUDGETED EXPENSES	$157.6
Advance research payments	13.4
Difference in accounts payable	1.7
CASH REQUIREMENTS	$172.7

Questions and Answers

- Open for discussion

Lunch

Meeting Schedule

DATE	LOCATION
February 3-5, 2013	San Francisco, CA
May 5-7, 2013	Chicago, IL
September 22-24, 2013	Washington, DC
November 17-19, 2013	Atlanta, GA

November Board Meeting
Pilot Project Management

Lori Frank
Michele Orza
Joe Selby

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Patient-Centered Outcomes Research Institute

Pilot Projects

The pilot projects will

- Advance the field of patient-centered outcomes research by exploring methods for PCOR
- Help identify gaps to inform PCORI research agenda on methods

Total Awards: $31 million over two years

Pilot Projects—Methods to:

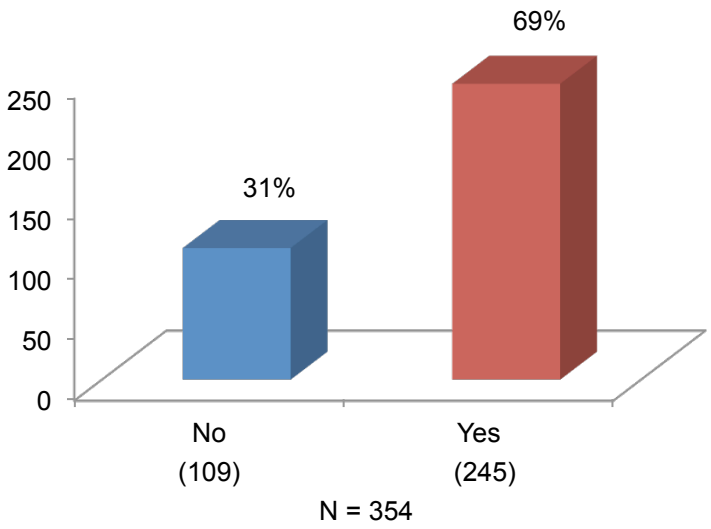
1. Inform the PCORI national priorities
2. Bring together patients, caregivers, and other stakeholders in all stages of a research process
3. Translate evidence-based care into healthcare practice in ways that account for individual patient preferences for various outcomes
4. Identify gaps in comparative effectiveness knowledge
5. Evaluate patient-centered outcomes instruments
6. Assess the patient perspective when researching behaviors, lifestyles, and choices
7. Study the patient care team interaction in situations where multiple options exist
8. Advance analysis of comparative effectiveness research data

Funded PCORI Pilot Projects in 25 States and DC

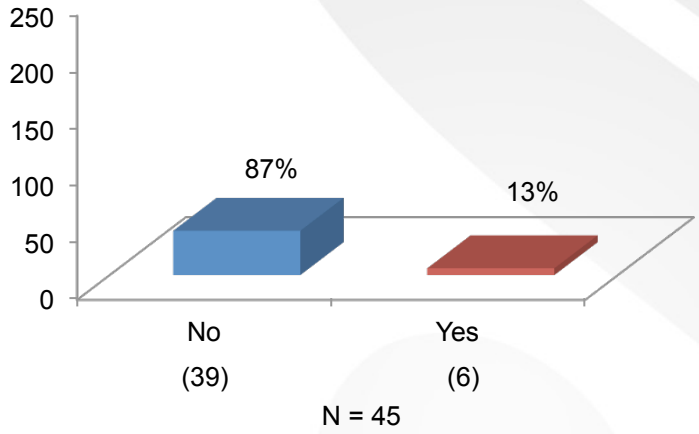
Post-Review Questionnaire: Reviewer Experience

Have you previously participated in a CSR Review?

Scientific Reviewers



Stakeholder Reviewers



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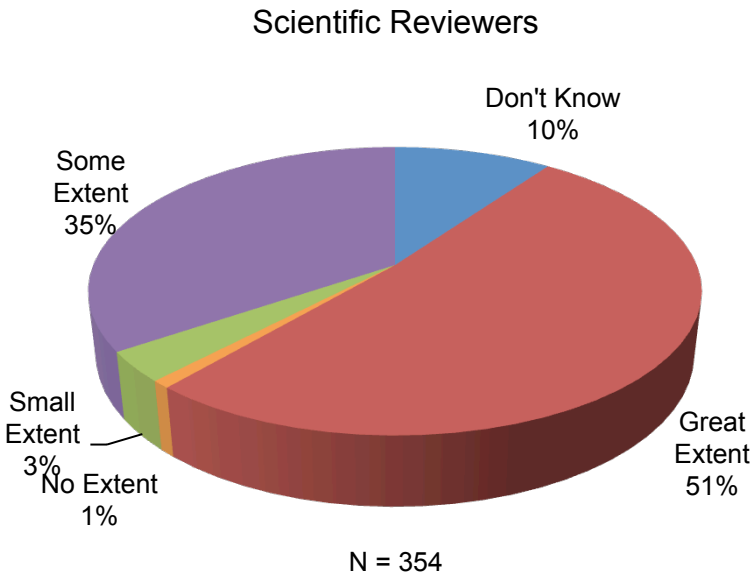
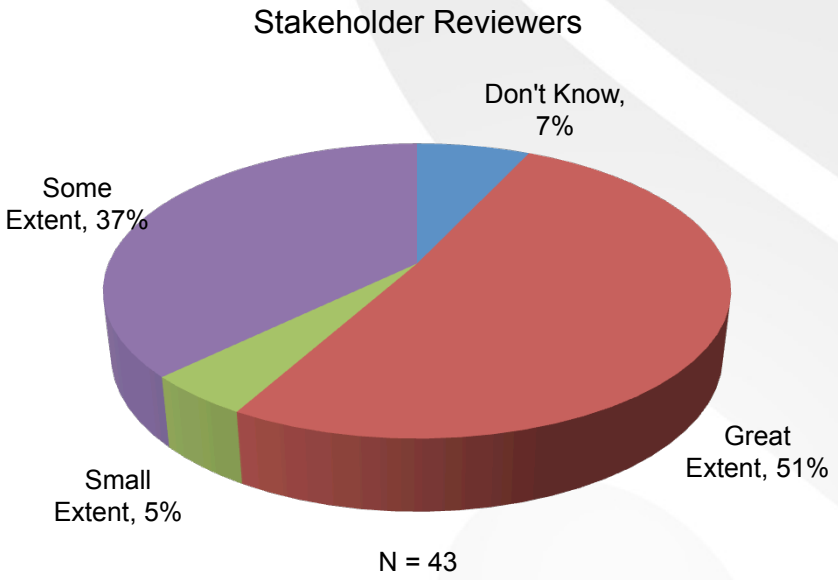
Post-Review Questionnaire: Scientific Reviewer Receptivity

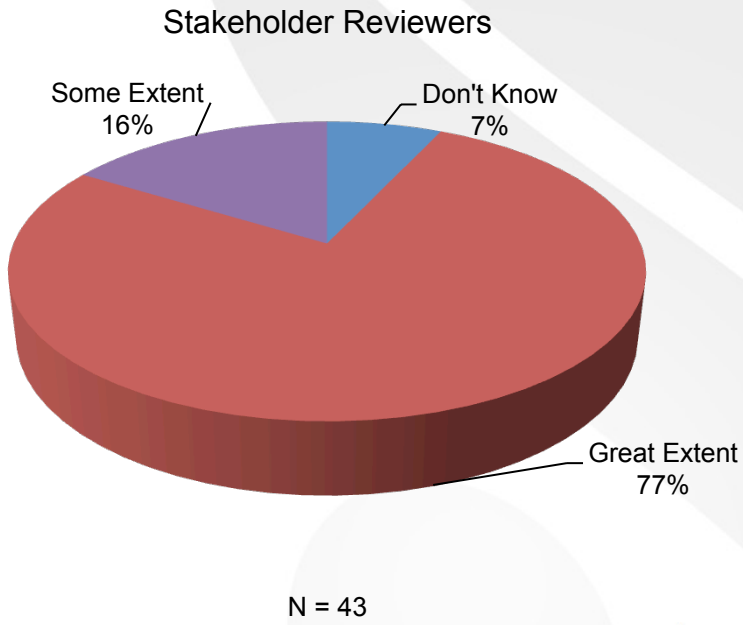
To what extent were SCIENTIFIC reviewers receptive to the comments made by STAKEHOLDER reviewers?





Post-Review Questionnaire: Stakeholder Reviewer Receptivity

To what extent were STAKEHOLDER reviewers receptive to the comments made by SCIENTIFIC reviewers?



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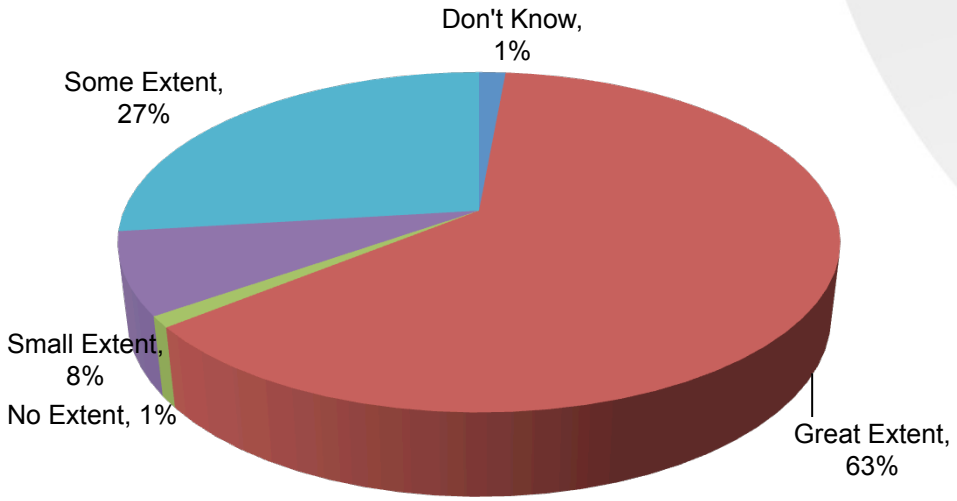
Post-Review Questionnaire: Scientific Reviewer

Compared to other reviews you've participated in, to what extent did having an emphasis on patient engagement impact overall scoring?

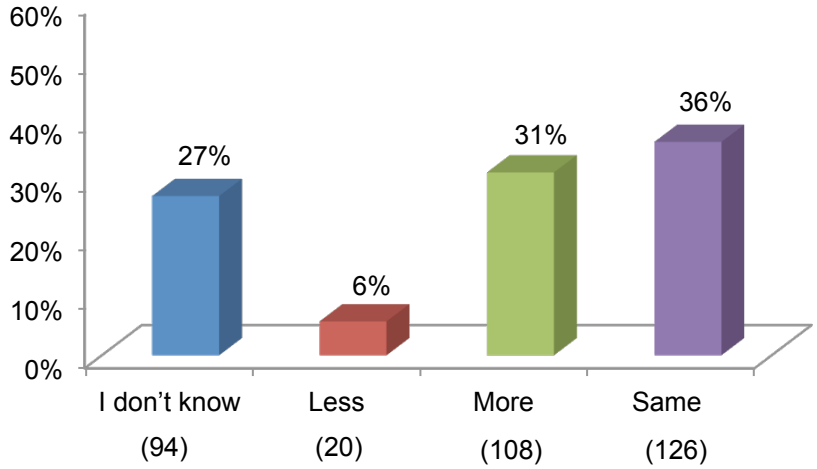


N = 282

Post-Review Questionnaire: Stakeholder Reviewer

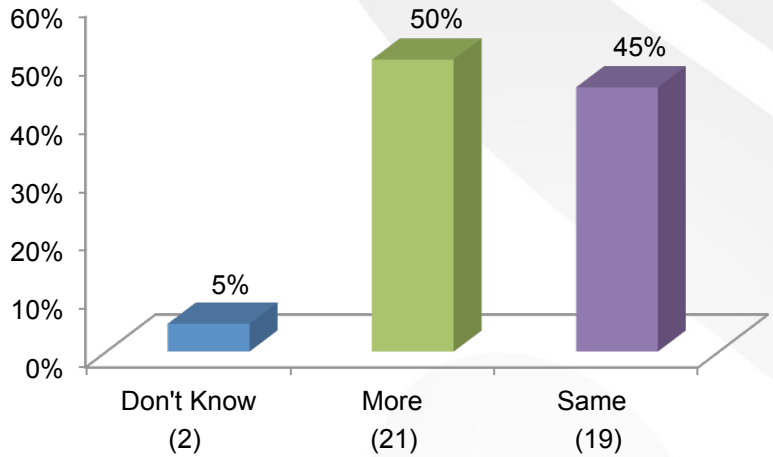
How would you describe the degree of emphasis stakeholder reviewers placed on the patient perspective relative to that placed by scientific reviewers?

Scientific Reviewers



N = 348

Stakeholder Reviewers



N = 42

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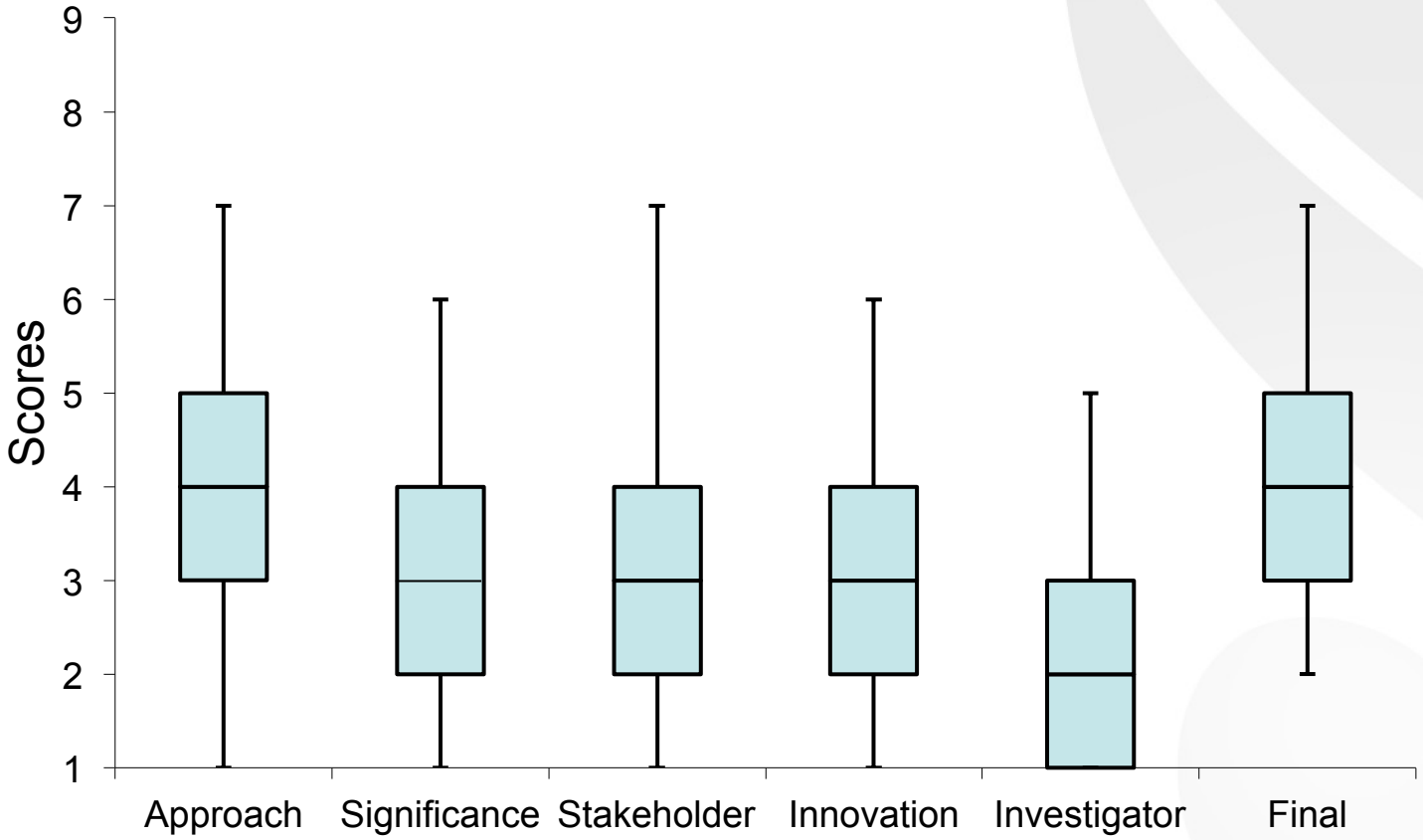
Review Criteria: Distribution of Scores

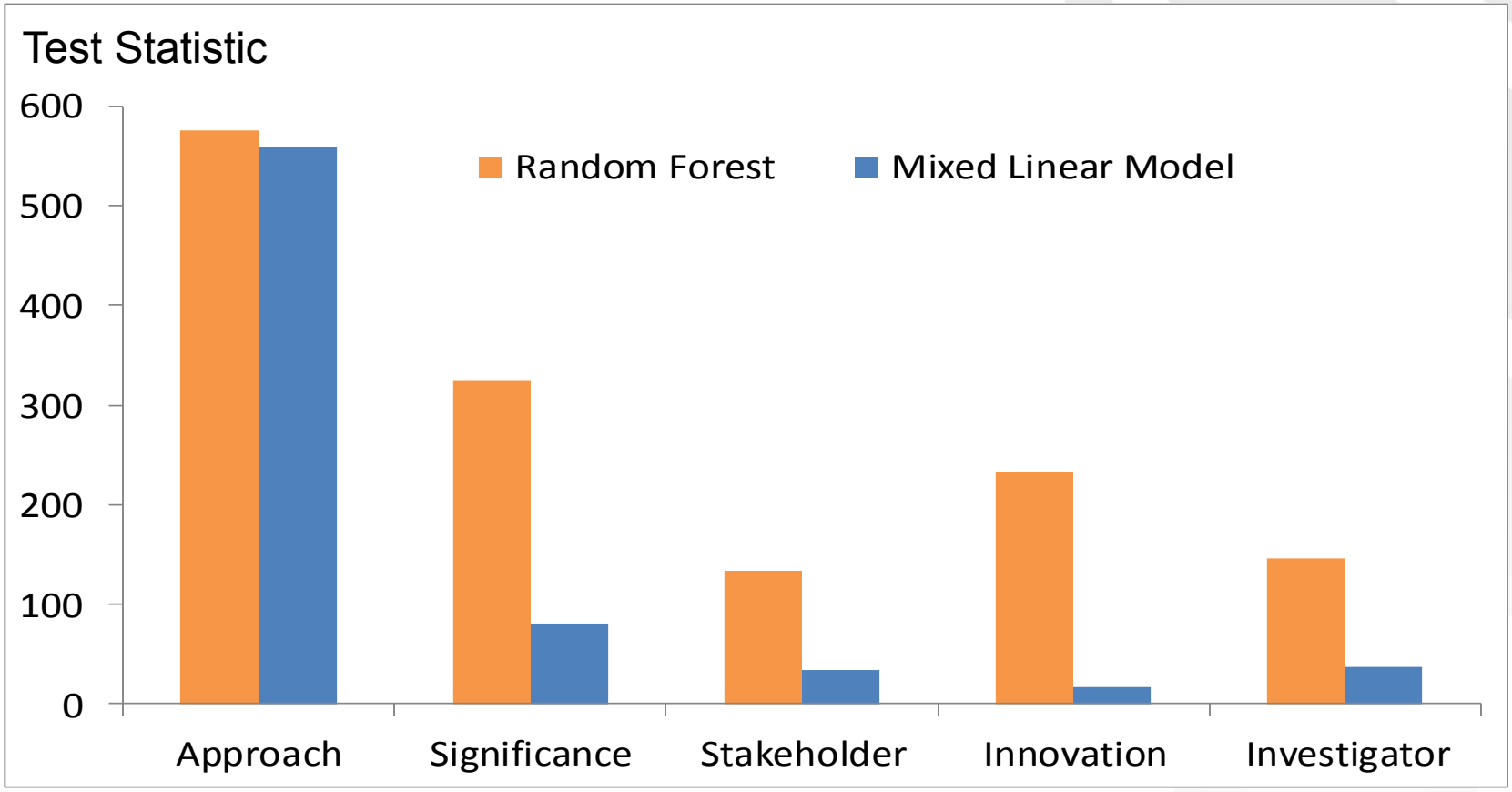
1,259 unique grant reviews across 16 panels



With thanks to Michael Lauer, Richard Fabsitz, and Mona Puggal, 10/12

Review Criteria: Measure of Importance

Which of the sub-component scores are the most important determinants of the final score, given all the others and given groupings within panels?



Data from Michael Lauer, Richard Fabsitz, and Mona Puggal, 10/12

Initiating PCORI's Active Portfolio Management

1. Actively manage and connect awardees

2. Facilitate and accelerate learning across projects

3. Develop and refine engagement framework

4. Elicit the patient view of research engagement

Advancing PCOR Through the Pilot Projects

- Learn about facilitators, barriers, and impact of involving patients in the full cycle of research
- Develop a conceptual framework of PCOR
- Implement a strategy to measure project progress
- Facilitate peer-to-peer learning
- Critically appraise lessons learned
- Identify implications for PCORI research agenda

Pilot Project Management Plan

August – September

- AcademyHealth selected
- Review of pilot project content and related literature

October – November

- Awardee contracts finalized
- Topic/methods subgroups identified to facilitate cross-learning and quick sharing

December

- Coordinate subgroup communication
- Plan for subgroup convenings

Conceptual Framework

Literature review

1. PubMed search and other databases searched, 2005 – present
3. 325 articles filtered through inclusion/exclusion criteria
4. > 50 articles abstracted and reviewed

Framework – initial draft

Constructed based on literature and input from the Patient, Consumer, Researcher Roundtable along with team discussion

Conceptual Framework- Structure Elements

1. Culture of the research entity
2. Governance infrastructure
3. Patient identification and selection infrastructure
4. Engagement infrastructure
5. Training/education infrastructure
6. Support infrastructure
7. Evaluation infrastructure
8. Accountability and transparency infrastructure

Conceptual Framework- Process Elements

1. Nature of engagement
2. Patient identification and selection
3. Patient segmentation and selection
4. Establishing and defining goals and accountability
5. Culture of engagement (research project–specific)
6. Nature and channels for communication and provision of input
7. Continuity and frequency of engagement
8. Stage(s) of the research process
9. Confidentiality and transparency

Conceptual Framework- Outcomes Components

1. Attitudes and perceptions
2. Modifications or refinements
3. Concept appeal
4. Relationships and buy in

Longer Term Outcomes Components:

1. Increased quality of research
2. Increased relevance of research
3. More informed and expansive decision making/uptake of research
4. Improvements in dissemination of and access to research
5. Policy deliberations/changes
6. Improvements in health outcomes and health status

Cycle I Funding Announcement: Merit Review Update

Martin A. Dueñas, Director, Contracts Management
Joe Selby, Chief Executive Officer
Anne Beal, Chief Operating Officer
PCORI Board of Governors Meeting
Boston, MA
November 2012

Patient-Centered Outcomes Research Institute

Questions for Board Consideration

1. Feedback regarding selection criteria?
2. Any additional information PCORI should be collecting?

Overview

- PFA & Timeline
- Merit Review Criteria: Phase I + Phase II
- Applications for Final Review
- Data Collected
- Recommended Selection Approach and Actions

PCORI's Four PFA Areas

PCORI Funding Announcements (PFAs) focus on four areas of research addressing currently unmet needs of patients, their caregivers, clinicians, and other healthcare system stakeholders.

1. Assessment of Prevention, Diagnosis, and Treatment Options
2. Improving Healthcare Systems
3. Communication and Dissemination Research
4. Addressing Disparities

Timeline

Letter of Intent/ Application Deadline
- June 15, 2012 (LOI)
- July 31, 2012 (Application)

Internal Quality Control
- August 1 – 15, 2012

Panel I: Scientific Review
- August 15 – October 26, 2012

Panel II: Impact Review
- Thursday, November 15, 2012

PCORI Review and Board Approval
- November 16– December 15, 2012

Merit Review
Phase II: Focus on Impact

Phase I Review Criteria

Determines Scientific Soundness and Impact

1. Impact of the condition on the health of individuals and populations
2. Innovation and potential for Improvement through research
3. Impact on healthcare performance
4. Patient-centeredness
5. Rigorous research methods
6. Inclusiveness of different populations
7. Research team and environment
8. Efficient use of research resources

Overall Score

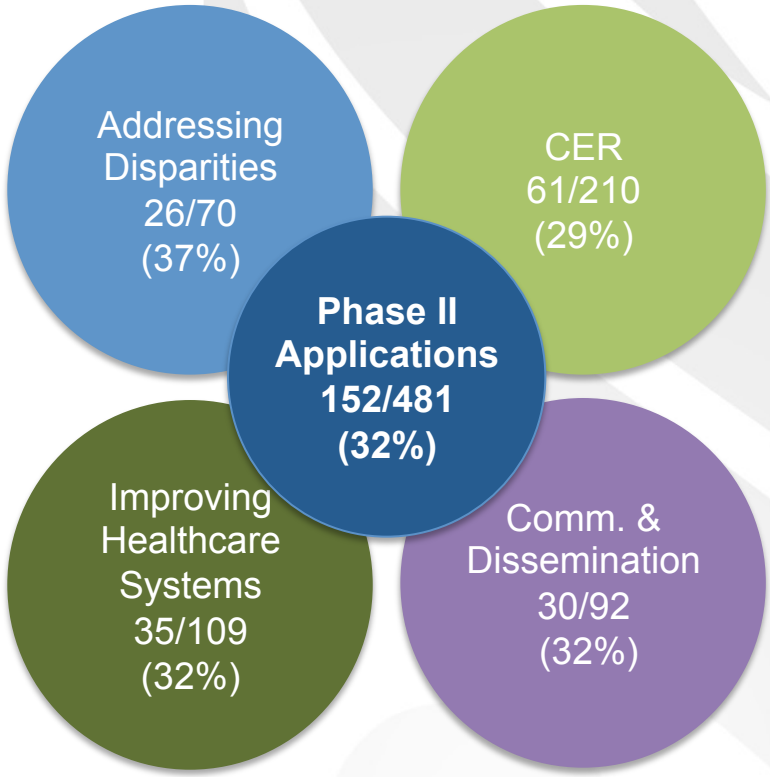
Phase II Review Criteria

Focuses on Impact

2. Innovation and potential for Improvement through research
4. Patient-centeredness
7. Research team and environment

Advanced to Phase II

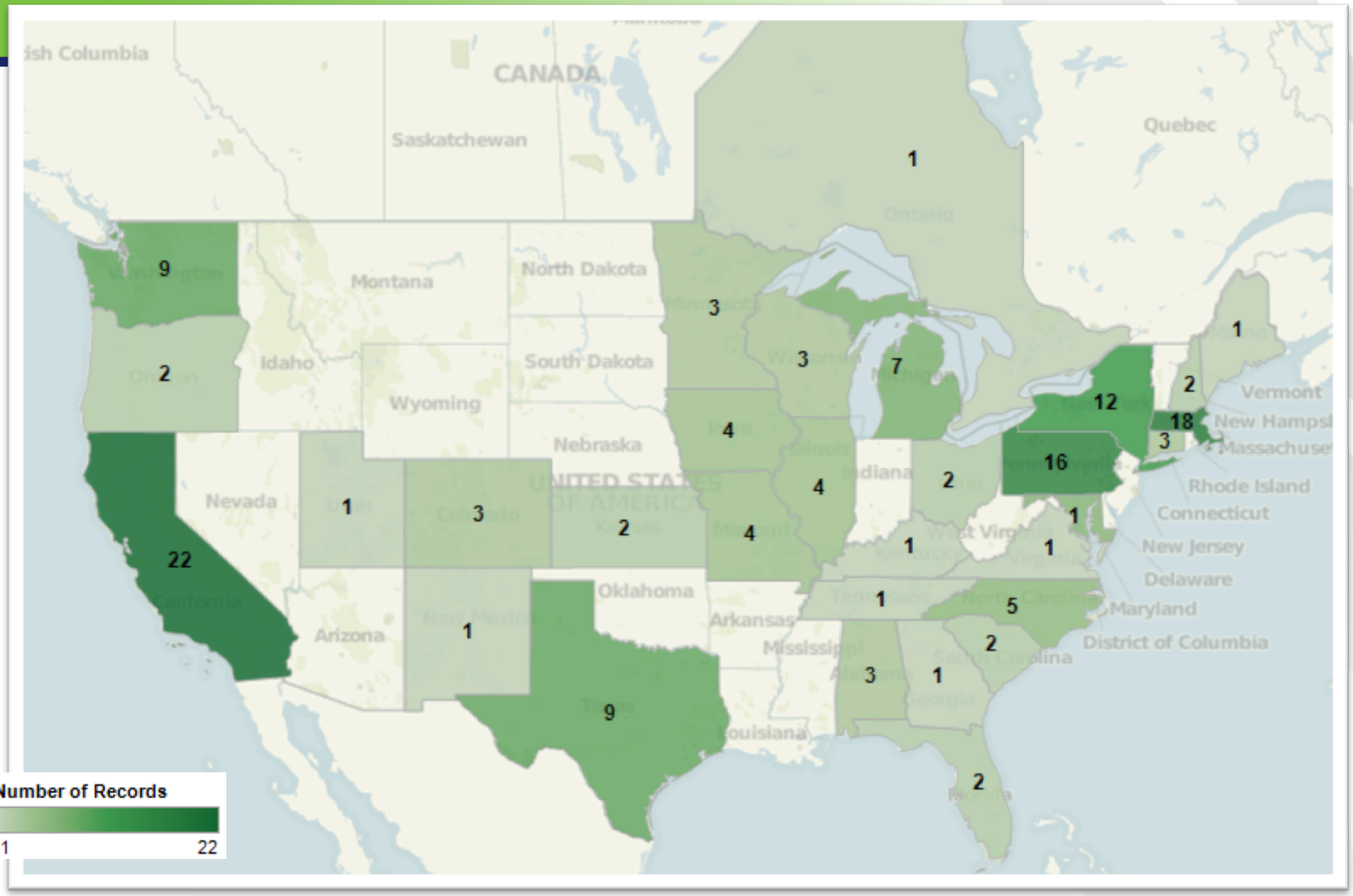
- **Phase I Overall Scores**
- Criteria:
  - Represent the best scoring applications
  - Include about twice the number expected to be funded or about 32% of top scores, depending on score distributions



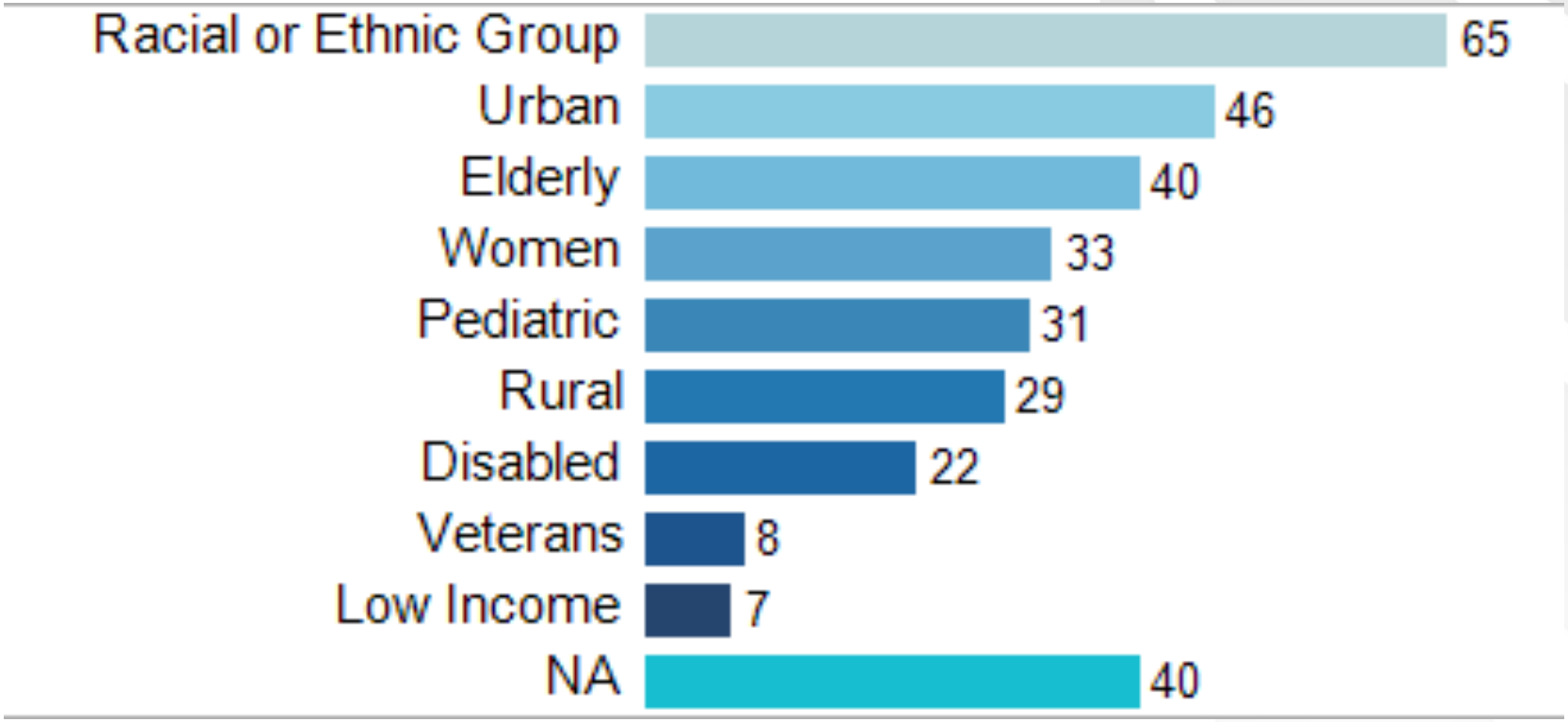
Data Collected for PFAs

BASIC AND ADMINISTRATIVE INFORMATION	PEOPLE	ABOUT THE RESEARCH
Organization (Name, Address, City, State, ZIP, Country)	Principal Investigator (Degrees, Phone, E-mail)	Study design
Project Title	PI years of research experience after terminal degree	Specific analytic methods
Project Start/End Date	PI years of research experience related to the field	Specific aims
Funding Opportunity	PI number of funded grants/contracts	Technical abstract
Funding Cycle	PI total amount for largest grant/contract	Public abstract
Projected Requested Amount	PI agencies from which grants are received	Diseases or conditions addressed
Budget (Personnel Direct Costs)	Key Personnel (Name, E-mail, Organization, Role)	Focus on vulnerable and/or underserved population
		Demographics (Gender, Race/Ethnicity, Income, Locale)
		Specific PCORI priority research area addressed

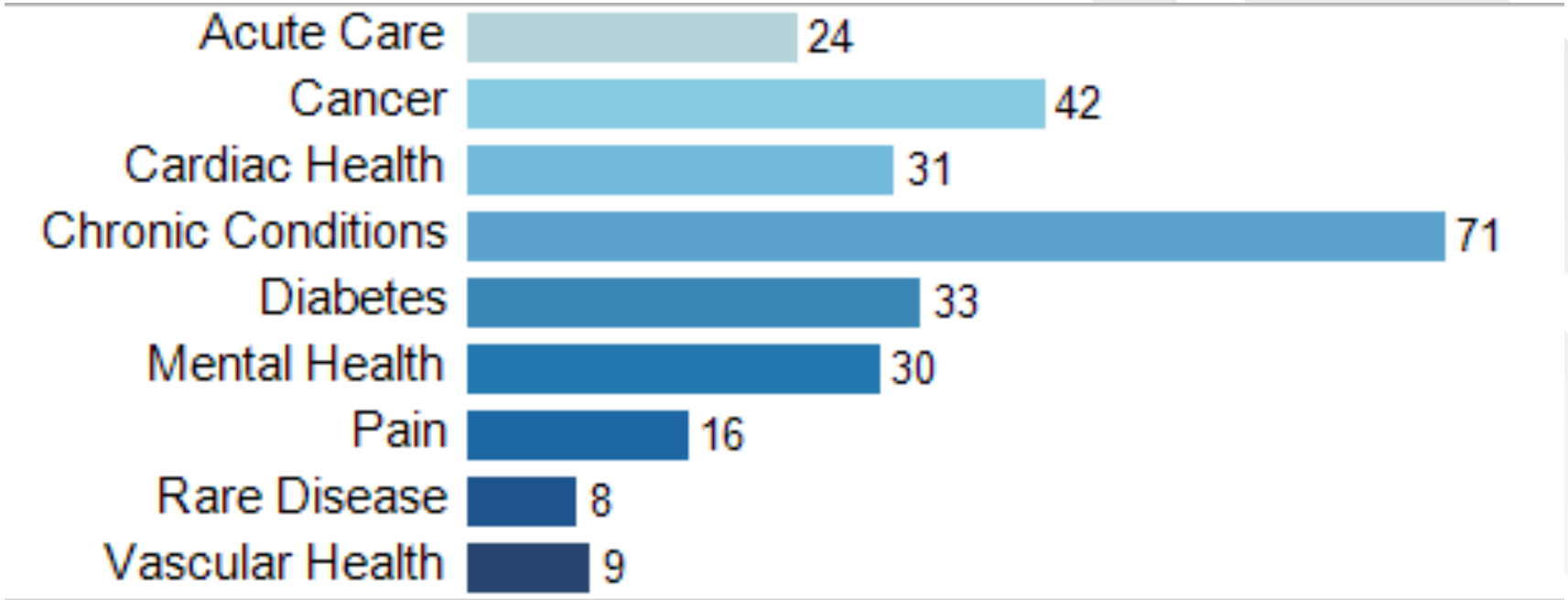
Proposal Advanced to Phase II: Location: 30 States + Canada



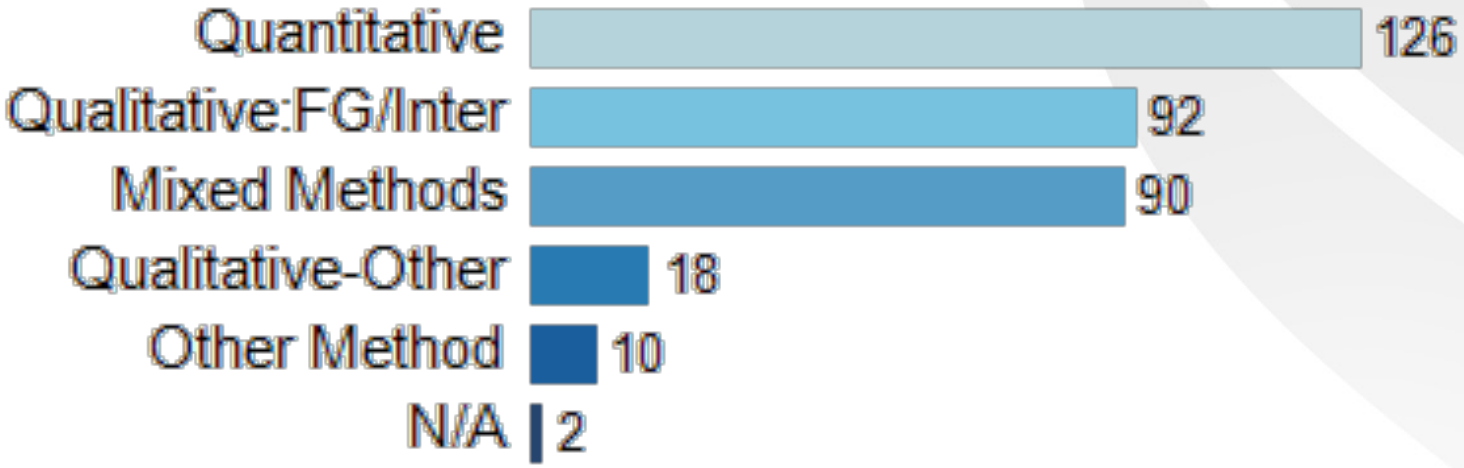
Proposal Advanced to Phase II: Population



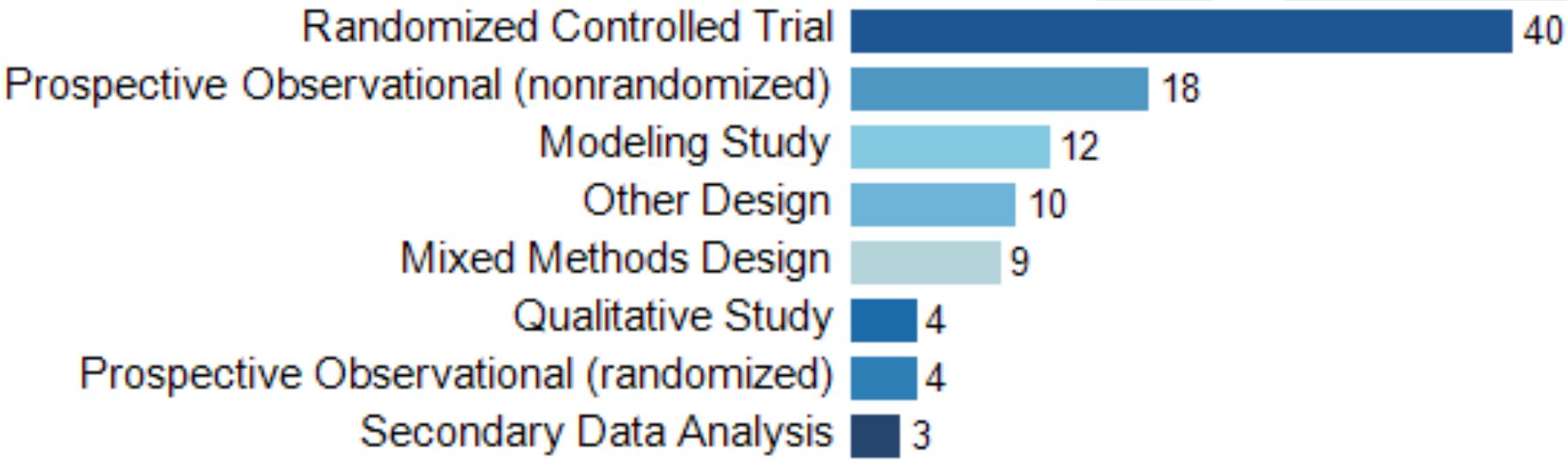
Proposal Advanced to Phase II: Condition



Proposal Advanced to Phase II: Methods



Proposal Advanced to Phase II: Design



Selection Approach and Actions

1. Appoint Board of Governors – Staff Selection Committee
2. Review Data on Characteristic of High Scoring Application
3. Select on basis of 3 Criteria:
   - **Final Score from Phase II**
   - Condition Studied
   - Populations Studied
4. Board Approval in Public Meeting in December

Board Considerations

1. Feedback regarding selection criteria and actions.
2. Is there any additional information PCORI should be collecting?

Break

Meeting Schedule

DATE	LOCATION
February 3-5, 2013	San Francisco, CA
May 5-7, 2013	Chicago, IL
September 22-24, 2013	Washington, DC
November 17-19, 2013	Atlanta, GA

Initial Targeted Funding Announcements

Kara Odom Walker, MD, MPH, MSHS
Joe Selby, MD, MPH, Executive Director
PCORI Board of Governors Meeting
November 2012

Patient-Centered Outcomes Research Institute

Overview

- Rationale for targeted funding announcements now
- Process for identifying high priority topics
- Proposed Topics
- Topic Information
- Next Steps

Rationale

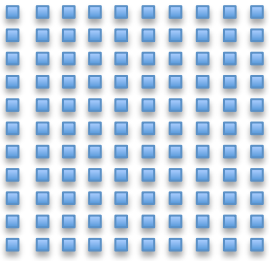
- Responds to widespread concerns that PCORI has NOT gotten specific or identified high-priority research areas
- Responds to board directive to move forward with identifying several high-priority, stakeholder-vetted topics for targeted PFAs
- Jumpstarts PCORI's long-term topic generation and research prioritization effort
- Leverages stakeholder input from before PCORI's existence
- Allows us to build on our engagement work

Process for Identifying Topics for Initial Targeted Funding Announcements

Progress to Date → **Next Steps**

Multiple Stakeholder Efforts

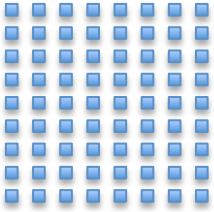
Backlog of critical vetted CER topics



# of topics

Identification of Overlapping Topics

Compile lists of important CER questions



# of topics

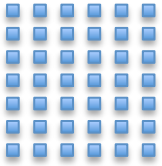
Staff Application of Review Criteria

PCORI Review Criteria

- Patient-centeredness
- Impact of the condition
- Innovation, potential for improvement
- Impact on healthcare performance
- Inclusiveness

Targeted Funding Announcement filter

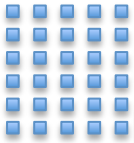
- Salience
- Short-term feasibility
- Stakeholder vetting
- Resource constraints



# of topics

Board Approval

Board-Approved High-priority Topics



# of topics

Expert and Stakeholder Input

Targeted Through Multiple Modes

- Expert Panels
- Webinars
- Public Sessions

First Filter: Seeking Topics Endorsed Through Multiple Processes

Institute of Medicine Top 100 CER National Priorities

- Generated by multiple stakeholders

300+ Topics Considered

- Generated with input from patients, other stakeholders, experts, other funders

Overlapping Topics

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Second Filter: Targeted Funding Specific Factors

- **Salience:** of obvious, recognizable importance— i.e, that the question being addressed is known to represent a fairly common problem
- **Short-term feasibility:** indicates that study results could be available within a two to three year period
- **Unmet research need**: unlikely to be funded without PCORI support
- **Resource constraints:** moderate investments could suffice or could leverage existing co-funding

Third Filter: PCORI Merit Criteria

1. Patient centeredness
2. Impact of the condition on the health of individuals and populations (prevalence, incidence, other measures of burden of disease)
3. Potential for improvement:
   - Preliminary evidence of important differences
   - Opportunity to reduce current uncertainty
   - Likelihood of implementation into practice
   - Durability of information
4. Potential for impact on healthcare performance
5. Potential for inclusiveness of different populations

Ranked on a scale of "does not meet criteria to exceeds criteria"

Ranking Process: Staff Members

Members	Tool	Topics	Top 25
▪ Six reviewers: • Four Science Team staff • Two Non-science Team staff	▪ Independent ranking of criteria ▪ Criteria weighted, with emphasis on PFA-specific filter	▪ Staff ranked 40 potential topics	▪ Top 25 list presented to Program DevelopmentCommittee

Top 25 Topics From Staff Ranking

1. Treatment of uterine fibroids
2. Treatment of localized prostate cancer
3. Diagnosis of suspected renal colic
4. Management of asthma in African Americans
5. Management of maternal fetal and neonatal health outcomes
6. Sleep apnea detection and management
7. Obesity treatment in diverse populations
8. Health system interventions to improve coordination for cancer care
9. Patient navigation and disease management for diverse populations
10. Clinical decision support tools among youth with ADHD
11. Various primary care treatment strategies for ADHD in children
12. Understanding chronic disease self-management programs in patients with multiple chronic conditions
13. Management of elderly patients with back pain
14. School based vs. medical setting health services for diverse populations
15. Clinical decision support systems for imaging in emergency departments
16. Effective and efficient methods to disseminate interventions for chronic condition
17. Breast cancer screening with film, digital/3D mammography, and mammography plus MRI
18. Treatment strategies for neck and back pain
19. Advanced imaging modalities and biomarker tests for prostate cancer
20. Polypharmacy and mortality in schizophrenia
21. Fracture prevention strategies
22. Prevention of falls in the elderly
23. Self-management strategies to manage multiple chronic conditions
24. Effectiveness of comprehensive care coordination programs
25. Management of complex, co-morbid conditions

Initial Targeted Funding Announcement Balancing Criteria

Focus on Balance

Study Population

Condition(s) Addressed

+

Potential for Impact

Recommended Topics

1. Treatment of uterine fibroids
2. Treatment of localized prostate cancer
3. Management of asthma in African Americans
4. Management of maternal fetal and neonatal health outcomes
5. Prevention of falls in the elderly

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6. Sleep apnea detection and management
7. Obesity treatment in diverse populations
8. Health system interventions to improve coordination for cancer care
9. Various primary care treatment strategies for ADHD in children
10. Treatment-related mortality in schizophrenia
11. Treatment strategies for neck and back pain

Recommended Topics

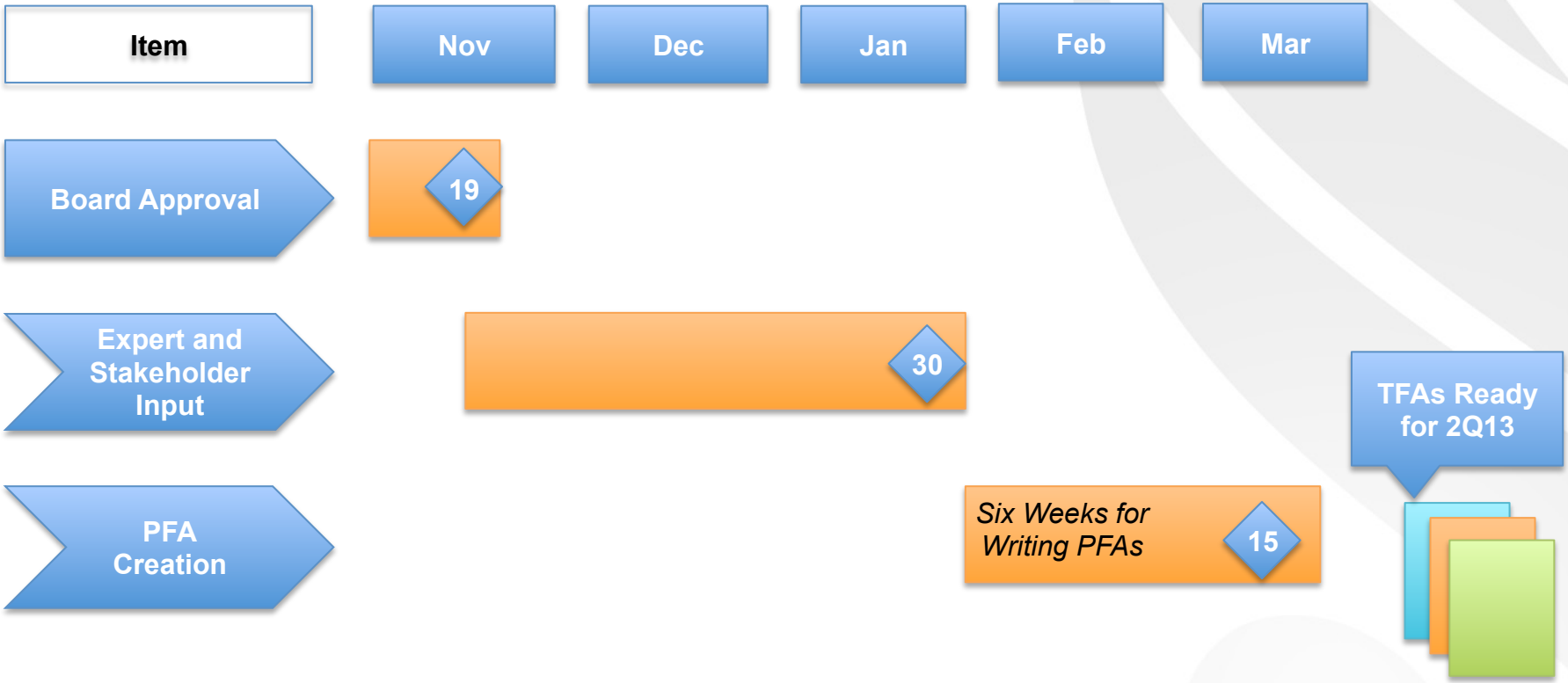
- Treatment Options for Uterine Fibroids
- Safety and benefits of treatment options for severe asthma in African Americans
- Fall Prevention in the Elderly

Topics in Current Headlines

Treatment Options for Uterine Fibroids	The Boston Globe **"Learning from city councilor's fibroid condition"** April 16, 2012
Management of Severe Asthma in African Americans	The Washington Post **"Minority children affected by disparities in asthma health care"** May 31, 2012
Fall Prevention in the Elderly	The New York Times **"Scientists weigh in on fall prevention"** July 12, 2012

Source: Hyperlinks to respective online articles from each newspaper. Logos from respective Web sites.

Proposed Next Steps: Timeline for Initial Targeted Funding Announcements



Potential Funding Strategies

Convene Stakeholder/Expert Panels →

Call for Evidence Synthesis	A Single Study	Portfolio of Studies
• Summarize what is known → disseminate • Identify gaps in evidence, and determine key research questions for future funding	• Identify specific study design • Issue RFP for Contract • Prioritize short turnaround results	• Decide to issue open call for multiple study options • Solicit multiple study approaches and specific research questions • Portfolio of projects, including sequencing projects over time

Next Steps

- Get Board approval to focus on three topics
- Refine important research questions in each topical area
- Convene stakeholder/expert panels for each topic
- Prepare and release PFAs

Call for Vote

- ✓ ✘ Treatment Options for Uterine Fibroids
- ✓ ✘ Safety and Benefits of Treatment Options for Severe Asthma in African Americans
- ✓ ✘ Falls Prevention in the Elderly
- ✓ ✘ Other? Additional?

Appendix

- Appendix A: Topic Briefs
- Reference articles and reports (separate booklet)

Treatment Options for Uterine Fibroids

	Description
Question	• What is the relative effectiveness of the available **procedural or nonprocedural treatments for uterine fibroids**, including: ▪ Procedural treatments (hysterectomy, myomectomy, uterine artery embolization(UAE), magnetic resonance image-guided focused ultrasound, endometrial ablation) ▪ Nonprocedural treatments (hormonal therapies, oral contraceptives, and nonsteroidal anti-inflammatory drugs) ▪ Complementary and alternative medicine ▪ Lifestyle changes ▪ Watchful waiting (no treatment) • What is the optimal sequencing of therapies, considering women's clinical characteristics and preferences? • What are the most important subpopulations to predefine (childbearing aim, race/ethnicity, age, and therapeutic goals)?
Population	• Uterine fibroids are the most common gynecological condition among women, with an incidence that is highest among women ages 30 to 40. Cumulative incidence approaches 70 percent among white women by age 50 and is even higher among African American women.
Research Need	• Treatment options of uterine fibroids include surgical, minimally invasive, and hormonal therapies or other medications. Most women who have uterine fibroids will not experience symptoms severe enough to seek treatment, but for those who do, uterine fibroid disease poses a significant cost and quality of life burden. • Due to the complexity of treatment options, further research is needed to help women target specific treatment options that effectively manage their symptoms.
Mechanism	• Expert and stakeholder panel

Safety and Benefits of Treatment Options for Severe Asthma in African Americans

	Description
Question	• Compare management strategies for **severe asthma in African Americans** for a range of clinical, functional, and healthcare utilization outcomes.
Population	• Asthma affects nearly 20 million Americans. African Americans are one of the highest populations at risk from asthma with almost 4.5 million reports in 2010.
Research Need	• Treatment options include fast-acting inhalers and long-term controlling substances, such as long-acting beta-adrenoceptor agonists (LABAs). African Americans may also be especially sensitive to LABAs. Further research studies are needed to examine the impact of various medical treatments and health education programs to reduce the rate of asthma-associated illness and death in the African American population. • In any patient case, a large Cochrane systematic review for the effectiveness and safety of LABAs has provided evidence that LABAs are safe and beneficial in control of asthma; intriguingly, subgroup analyses indicate that this is true when inhaled corticosteroids are used and in their absence. • There remains a question about which subgroup risk factors may predispose African Americans to increased rates of adverse events–whether it is genetic factors, disease severity, or access to ongoing comprehensive treatment strategies.
Mechanism	• Expert and stakeholder panel

Fall Prevention in the Elderly

	Description
Question	• Compare the effectiveness of primary prevention methods to **prevent falls**, such as exercise and balance training, versus clinical treatments in older adults at varying degrees of risk, including: ▪ Assessing the potential of combining therapeutic agents to achieve additive or synergistic treatment benefits. ▪ Improving adherence to clinical protocols by developing and testing less burdensome dosing regimens or routes of administration and exploring approaches that reduce drug side effects. ▪ Using improved predictors of fracture risk that incorporate aspects of an individual's environment, lifestyle, and medical history to target multi-component prevention programs to high-risk individuals. ▪ Investigating the effect of genetic variation on response to treatments.
Population	• Between 30 and 40 percent of community-dwelling persons 65 years or older fall at least once per year. • Falls are the leading cause of fatal and nonfatal injuries among persons 65 years or older.
Research Need	• Despite the depth of research into interventions, additional research is needed to confirm the context in which multifactorial assessment and intervention, home safety interventions, vitamin D supplementation, and other interventions are effective. • Evidence underpinning the U.S. Preventive Services Task Force recommendations regarding fall prevention in older adults comes from time-limited, randomized, controlled trials involving heterogeneous populations that participated in different combinations of balance, strength, endurance, or general exercise programs in various settings under the supervision of diverse groups of experts (eg, physical therapists, nurses, and exercise physiologists). The trials provide general guidance but no details as to how to construct or conduct a clinical exercise program.
Mechanism	• Expert and stakeholder panel

References: Treatment Options for Uterine Fibroids

- Deng L, Wu T, Chen XY, Xie L, Yang J. Selective estrogen receptor modulators (SERMs) for uterine leiomyomas. Cochrane Database of Systematic Reviews 2012, Issue 10.
- Gliklich RE, Leavy MB, Velentgas P, Campion DM, Mohr P, Sabharwal R, et al. Identification of Future Research Needs in the Comparative Management of Uterine Fibroid Disease. A Report on the Priority-Setting Process, Preliminary Data Analysis, and Research Plan. Effective Healthcare Research Report No. 31. (Prepared by the Outcome DEcIDE Center, under Contract No. HHSA 290-2005-0035-I, TO5). AHRQ Publication No. 11-EHC023-EF. Rockville, MD: Agency for Healthcare Research and Quality. 2011; Available at: http://effectivehealthcare.ahrq.gov/reports/final.cfm.
- Gupta JK, Sinha A, Lumsden M, Hickey M.Uterine artery embolization for symptomatic uterine fibroids. Cochrane Database of Systematic Reviews. 2012; Issue 5.
- Stovall, DW. Alternatives to hysterectomy: focus on global endometrial ablation, uterine fibroid embolization, and magnetic resonance-guided focused ultrasound. Menopause: The Journal of the North American Menopause Society. 2011; 18(4):437.
- Toor SS, Jaberi A, Macdonald DB, McInnes MDF, Schweitzer ME, Rasuli P. Complication Rates and Effectiveness of Uterine Artery Embolization in the Treatment of Symptomatic Leiomyomas: A Systematic Review and Meta-Analysis. American Journal of Roentgenology. 2012; 199(5):1153.
- Tristan M, Orozco LJ, Steed A, Ramírez-Morera A, Stone P. Mifepristone for uterine fibroids. Cochrane Database of Systematic Reviews. 2012; Issue 8.

References: Safety and Benefits of Treatment Options for Severe Asthma in African Americans

- Cazzola M, Matera MG. Safety of long-acting β2-agonists in the treatment of asthma. Therapeutic Advances in Respiratory Disease. 2007; 1(1):35.
- Press VG, Pappalardo AA, Conwell WD, Pincavage AT, Prochaska MH, and Arora VM. Interventions to Improve Outcomes for Minority Adults with Asthma: A Systematic Review. J Gen Intern Med. 2012; 27(8): 1001.
- Torgerson DG, Ampleford EJ, Chiu GY, Gauderman WJ, Gignoux CR, Graves PE, et al. Meta-analysis of Genome-wide Association Studies of Asthma In Ethnically Diverse North American Populations. Nat Genet. 2011; 43(9):887.

References: Falls Prevention in the Elderly

- Moyer, VA, on behalf of the U.S. Preventive Services Task Force. Prevention of Falls in Community-Dwelling Older Adults: U.S. Preventive Services Task Force Recommendation Statement. Ann Intern Med. 2012; 157(3):197.

- Tinetti ME, Brach JS. Translating the Fall Prevention Recommendations Into a Covered Service: Can It Be Done, and Who Should Do It? Ann Intern Med. 2012; 157:213.

Public Comment Period

Patient-Centered Outcomes Research Institute

Nominations

Patient-Centered Outcomes Research Institute

Wrap-up and Adjourn

Break

Meeting Schedule

DATE	LOCATION
February 3-5, 2013	San Francisco, CA
May 5-7, 2013	Chicago, IL
September 22-24, 2013	Washington, DC
November 17-19, 2013	Atlanta, GA