Topic Generation and Research Prioritization

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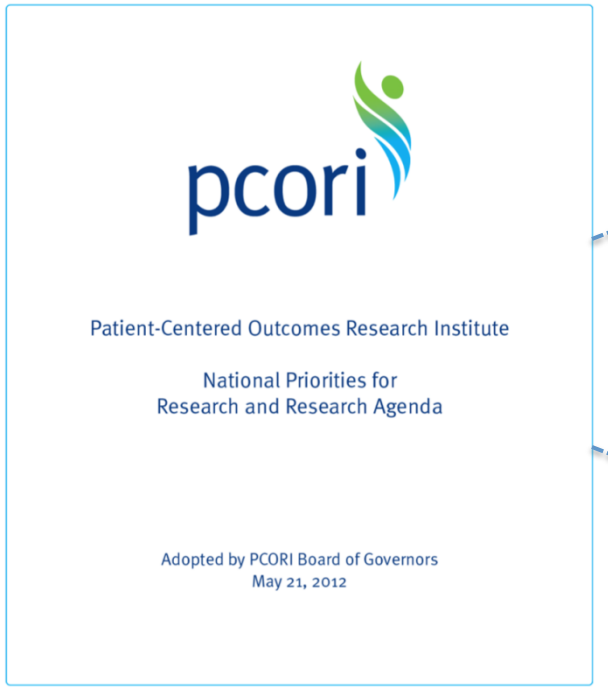
Strategic Questions to Consider

- Does the research prioritization process engage patients and stakeholders at the appropriate level? Is the process transparent and rigorous?
- Will the process enable PCORI to develop a balanced portfolio in line with its mission?
- Does the process enable the optimal level of engagement between the Board of Governors and Advisory Panels?

PCORI's Research Agenda

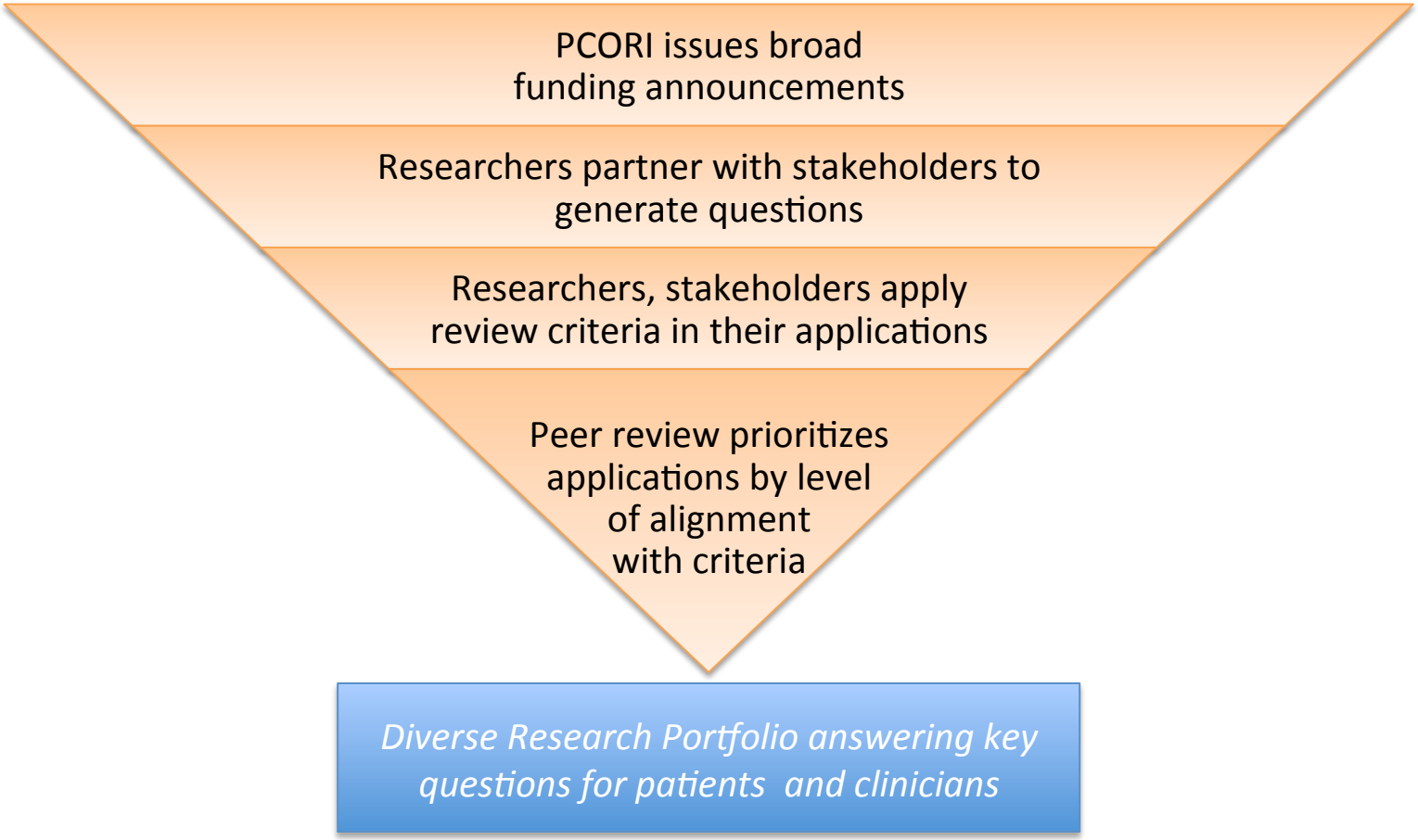
Five Board-Approved Priority Areas



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

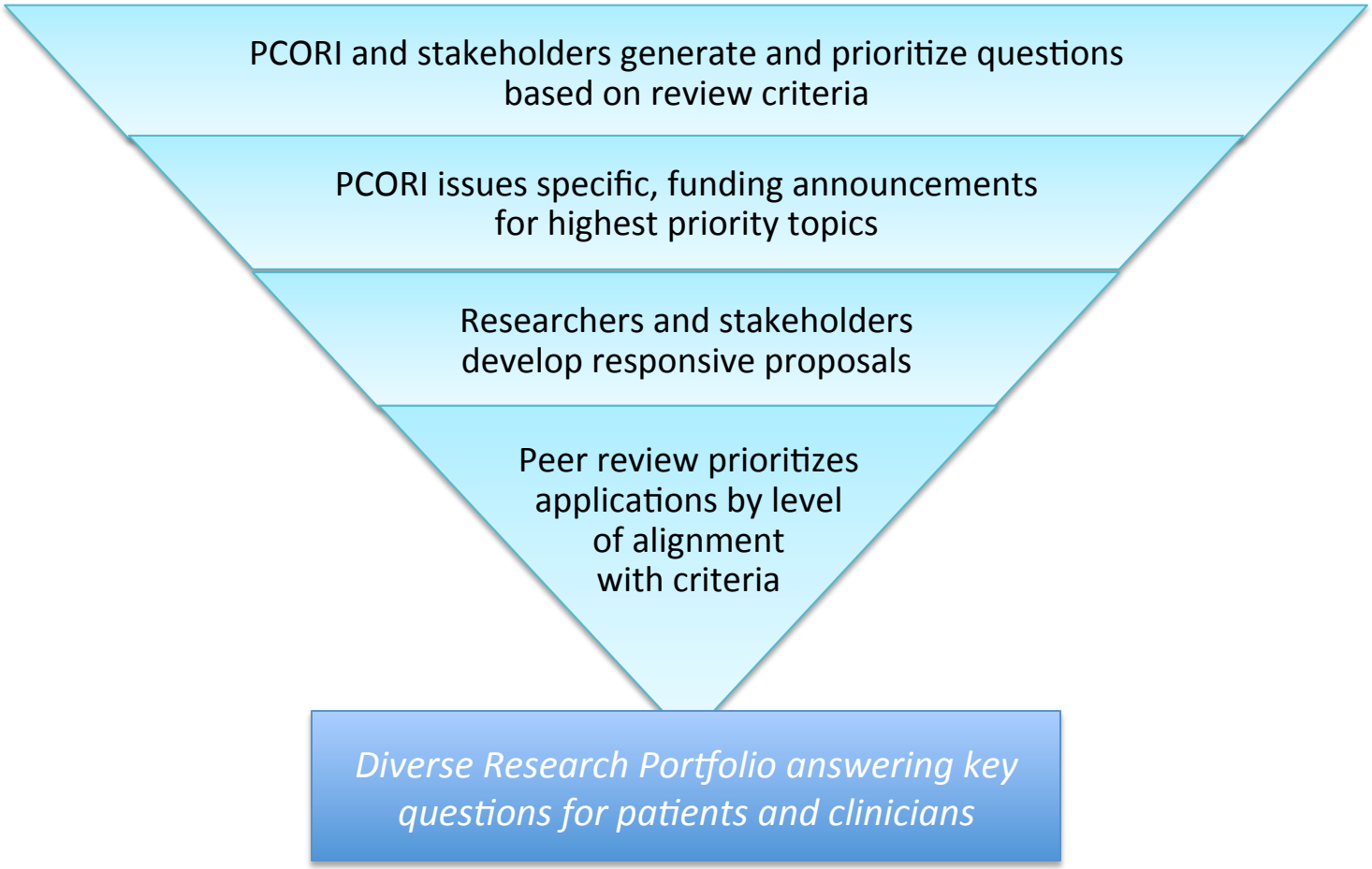
2 Complementary Approaches for Developing PCORI's National Research Agenda

Investigator-Generated Research Just One Part of the Process



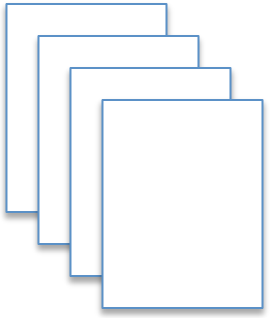
Patient/Stakeholder-Led Approach Unique to PCORI

Patient/Stakeholder-Led Approach

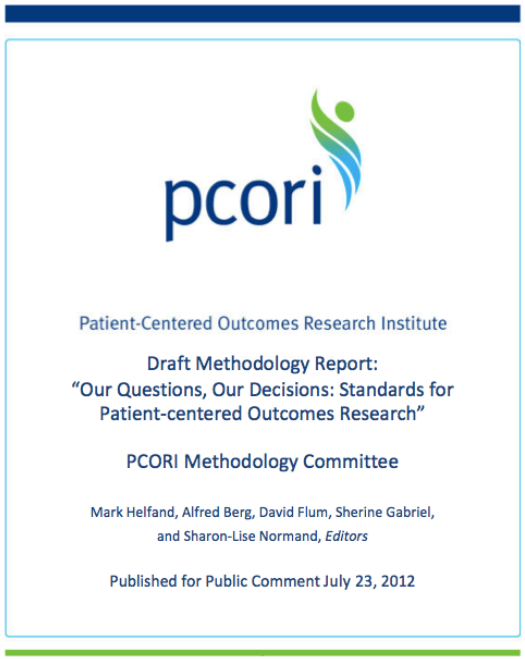


Building on the Existing Evidence Base and Prior Experience

Existing Scientific Work and Literature



Methodology Committee and Methodology Report



Experience of Other Agencies

National Institutes of Health

AHRQ

World Health Organization

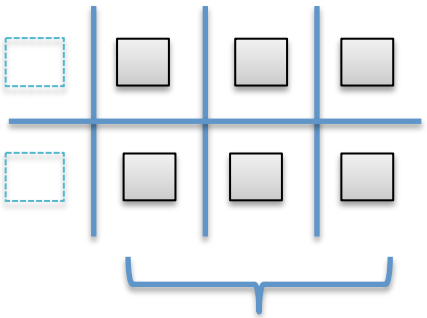
PCORI's Process Transparent, Rigorous

Topic Generation *(Through Multiple Modes)* → Gap Confirmation → Research Prioritization → Final Selection for Specific PFAs (PCORI Board)

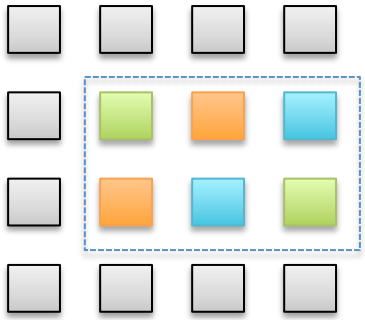
Patients & Stakeholders:
- Web Page
- Social Media
- Workshops

PCORI:
- Continuous Portfolio Review

Other agencies:
- AHRQ gaps
- NIH gaps



Research Opportunities



Phase 1: Topic Generation

Topic Nomination

Process Begins with Patients and Stakeholders

- Web page
- Social media/marketing
- In-person workshops, focus groups
- Topic generation discussed at Patient and Stakeholder Workshops in the Fall

- AHRQ: Future Research Needs (FRNs) Reports, Systematic Gap Review
- Other guidelines development processes (such as NQF)
- Gaps identified by NIH, other funding agencies
- Building on existing work or other organizations, prioritization exercises (eg, IOM 100)

PCORI "Filter"—All Nominated Topics Must:

- ✓ Answer a clinical question around a health care decision
- ✓ Be comparative
- ✓ Not be related to cost/cost effectiveness
- ✓ Align with at least one of PCORI's National Priorities for Research

Gap Confirmation

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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 between doing, or not doing something. For these situations, 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 develop 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 options faced by patients, we also are interested in questions that would help to 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).

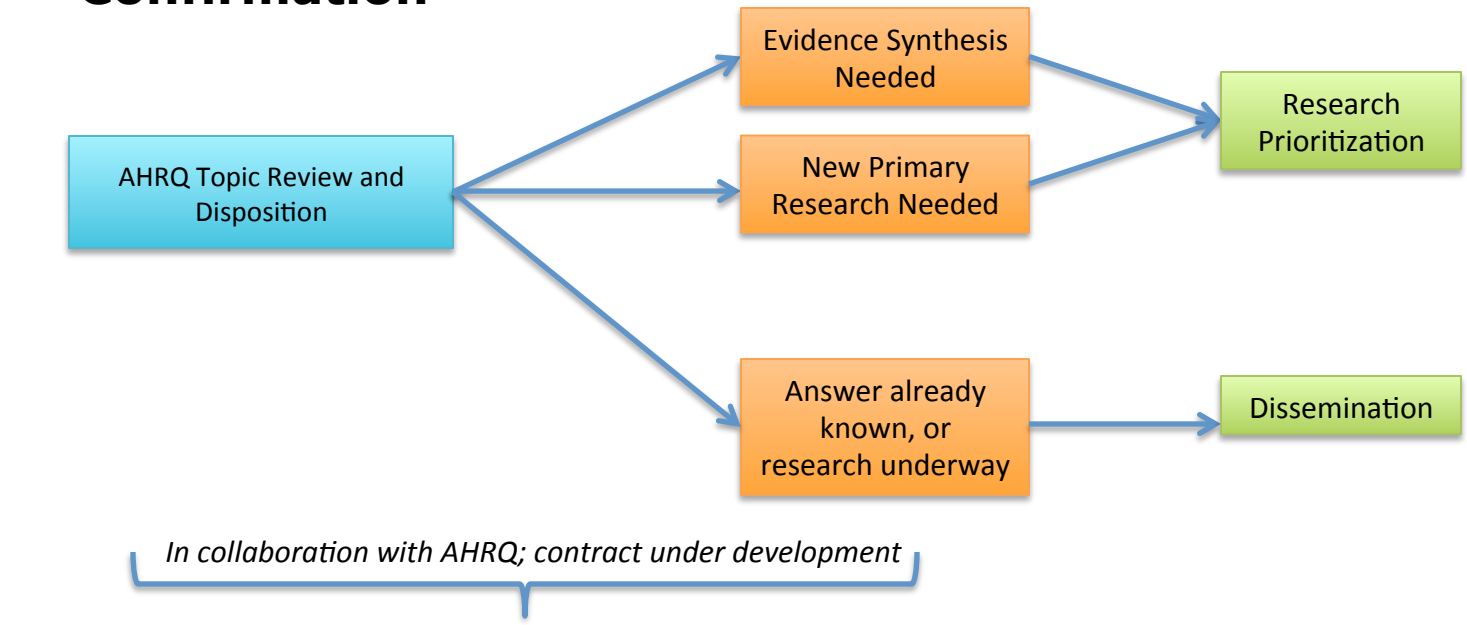
We will carefully consider your question as we plan which questions to study.

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?

Phase 2: Gap Confirmation



Phase 3: Research Prioritization

Advisory Panels
Patients and Stakeholders

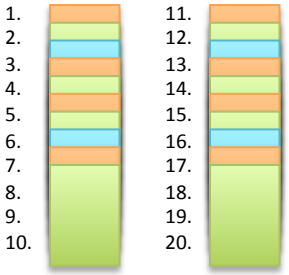
Board of Governors

Research Questions Needing Prioritization

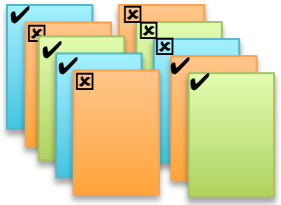
- Prevention, Diagnosis, Treatment Options
- Communication and Dissemination
- Disparities
- Improving Health Care Systems
- Infrastructure and Methods
- Rare Diseases

Research Prioritization Process Using PCORI Criteria

Prioritized List of Topics



Selected From Prioritized List



Creation of PFAs

Five Prioritization Criteria Adapted From PCORI Funding Criteria

1. Patient centeredness
2. Impact of the condition on the health of individuals and populations
(prevalence, incidence, and other measures of burden of disease)
3. Potential for improvement:
    - Are the differences in benefits between the interventions sufficient to warrant conducting a research study?
    - Will the study reduce the uncertainty around the effect of the interventions?
    - How likely are the findings to change practice?
    - How long will the information be valid?
4. Potential for impact on health care performance
5. Potential for inclusiveness of different populations

Developing Topic Briefs for Prioritization of Questions

Sample PCORI Topic Brief

Topic Brief Based on PCORI Review Criteria:

Disease Area	Suggested Research Topic
ADHD	For children less than 6 years of age with disruptive behavior disorder or ADHD, what is the comparative efficacy and effectiveness of specific psychosocial treatments alone compared with pharmacological treatments alone or in combination with psychosocial treatments for patient outcomes?

PCORI Criteria	Brief Description
RESEARCH STRATEGY: Background and Significance	
1. Impact of the condition on the health of individuals and populations	• In 2007, the estimated prevalence of parent-reported ADHD (ever) among children aged 4--17 years was 9.5%, representing 5.4 million children. Of those with a history of ADHD, 78% (4.1 million, or 7.2% of all children aged 4--17 years) were reported to currently have the condition[1] • Among ages 4-10 ever diagnosed with ADHD drops to 6.6% and current ADHD diagnosis sits at 5.5%[2] • Worldwide prevalence for preschool children estimated 2-6%[3] • Health benefit costs, absence days, and employment turnover found to be higher for guardians/caregivers of children with ADHD[4] • Comorbidities include: mood disorders, anxiety disorders, substance disorders, impulse-control disorders, attention-deficit and disruptive behavior disorders, developmental disorders, autism spectrum disorders[5] • Preschoolers treated for ADHD most often have co-occurring noncompliant behaviors, temper, and aggression that impair their relationships with family and care providers, and interfere with social and emotional develo[illegible]nt[6]
	Refers to the current impact of the condition on the health of individuals and populations. **Is the cond[illegible]ase associated with a significant burden in the US population, in terms of prevalence, mortality, morbidity, individual suffer[illegible]ductivity**? A particular emphasis is on patients with chronic conditions, including those patients with multiple chronic conditions.

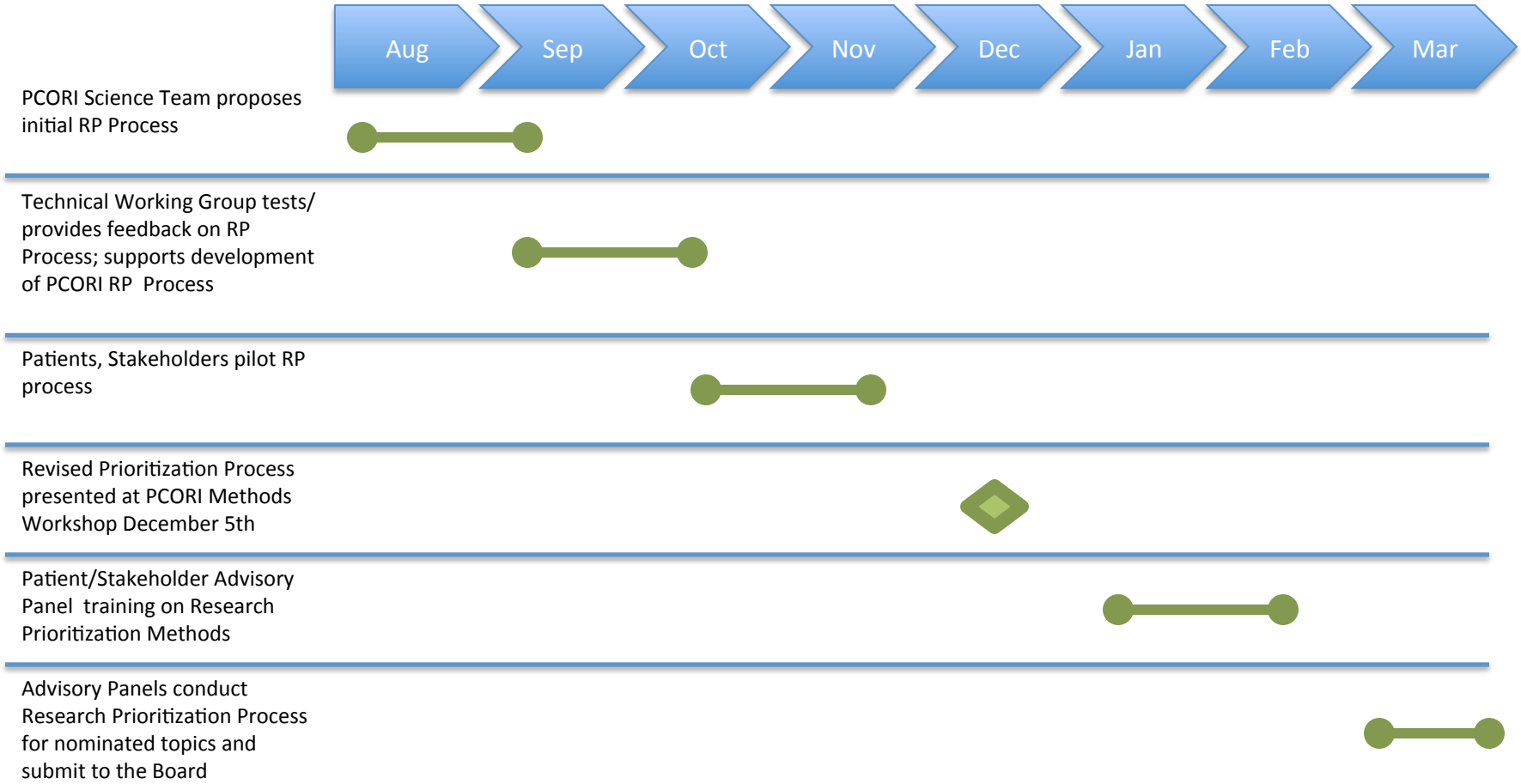
Each Topic Brief explores a research question for which a gap has been identified

Topic Brief addresses PCORI's criteria: patient centeredness, impact of disease, potential for improvement, etc.

Piloting the Process with Patients and keholders

1. Patients and stakeholders will participate in **a pilot prioritization** exercise using topic briefs and online tools. Participation will be by open application.
2. The **pilot group** will convene via several teleconferences to discuss topic briefs and results of initial ranking exercises.
3. The **pilot group** will meet in person to reach consensus on a final prioritized list, and to provide feedback to PCORI. The group will present at the December 5th workshop.
4. The revised process will be used by **Advisory Panels** in Winter 2013 with topics generated from patients and stakeholders across the country.

Research Prioritization Timeline



PCORI's Research Prioritization Team

PCORI Staff and Leadership:

- Joe Selby, Executive Director
- Rachael Fleurence, Scientist
- Katie Wilson, Project Management
- Natalie Wegener, Project Coordination

Technical Working Group:

- Arnie Epstein (BoG)
- Gail Hunt (BoG)
- Neil Kirschner (Stakeholder Representative)
- David Meltzer (Methodology Committee)
- Linda Morgan, RPh, MBA (Patient Representative)
- Jean Slutsky (Methodology Committee)
- Clyde Yancy (Methodology Committee)

Pilot Workshop Participants:

- Approximately 15-20 patients, stakeholders, researchers, and experts trained and prepared in PCORI's research prioritization process

Summarizing PCORI's Unique Approach to Research Prioritization

Patients and stakeholders engaged in each step of a transparent process:

- Patients and stakeholders involved in developing the process, providing feedback, and members of the future Advisory Panels

PCORI criteria for research prioritization:

- Criteria developed to achieve research that will improve patient's health outcomes, is impactful, and has a high probability of changing clinical practice

Transparency/visibility embedded in the process:

- Process shared with public for input and comment
- Pilot group open to applications
- Research prioritization methods workshop on December 5th broadcast via Web cast

Revisiting the Strategic Questions

- Does the research prioritization process engage patients and stakeholders at the appropriate level? Is the process transparent and rigorous?
- Will the process enable PCORI to develop a balanced portfolio in line with its mission?
- Does the process enable the optimal level of engagement between the Board of Governors and Advisory Panels?