Pilot Testing PCORI’s Process for Prioritizing Research Topics

Prepared by NORC at the University of Chicago on behalf of PCORI

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About NORC at the University of Chicago:

NORC at the University of Chicago (www.norc.org) is an independent research organization headquartered in downtown Chicago with additional offices in the University of Chicago campus, the D.C. Metro area, Atlanta, Boston, and San Francisco. NORC also supports a nationwide field staff as well as international research operations. With clients throughout the world, NORC collaborates with government agencies, foundations, education institutions, nonprofit organizations, and businesses to provide data and analysis that support informed decision making in key areas including health, education, crime, justice, energy, security and the environment.
I. Introduction

The Patient-Centered Outcomes Research Institute (PCORI) is an independent organization established to help patients make informed decisions and improve the delivery of healthcare services. PCORI commissions research that is guided by patients, caregivers, and the broader community to produce high integrity, evidence-based information. To this end, PCORI has developed a novel, systematic, transparent research prioritization process that will be used in the future by advisory panels to provide insights as to which research questions are the most important for PCORI to support and answer, given PCORI’s mission. In order to refine this initial process—which PCORI fully expects will continue to evolve with use and experience—PCORI recruited about 33 volunteers who represent patients, caregivers, clinicians, researchers, and other stakeholders to participate in a pilot during October and November 2012. Pilot participants and PCORI members gathered at the PCORI workshop on December 5, 2012, to discuss the pilot experience, hear from experts in the field of prioritization research, and provide feedback for improving the process.

This report, which was commissioned by PCORI and authored by NORC staff—including Jennifer Benz, PhD; Catharine Quirk, BS; Petry Ubri, BS; Tianne Wu, MPH; Jean-Ezra Yeung, MPH; and Wilhelmine Miller, PhD—discusses the rationale behind the proposed prioritization process, provides an overview of the five steps to determine research priorities, describes the pilot process, and presents the feedback and lessons learned for future iterations.

II. Rationale and Planning for Proposed Prioritization Process

PCORI is committed to ensuring that patients, caregivers, clinicians, researchers, health system leaders, and other stakeholders are involved effectively in every aspect of its work. To realize this commitment, PCORI is developing a systematic approach to prioritizing research questions whereby advisory panels comprised of a diverse set of stakeholders including patients and caregivers will utilize a process grounded in the science of research prioritization to recommend research questions that are the most important for PCORI to address and fund. To achieve its objectives, the results of PCORI-funded research will need to matter to patients, their families, and their clinicians and healthcare system leaders. If these results are going to improve healthcare delivery and outcomes, they should also have a high likelihood of being implemented in practice.

In devising its initial approach to research prioritization, outlined in Box 1, PCORI reviewed existing scientific work and literature; best practices from experienced agencies, such as the Agency for Healthcare Research and Quality (AHRQ) and National Institutes of Health (NIH); and predecessors in outcomes research, such as the Federal Coordinating Council for Comparative Effectiveness Research and the James Lind Alliance. The work of PCORI's Methodology Committee was also incorporated into the initial approach to research prioritization, including the work completed on the PCORI Methodology Standards, which outline comprehensive standards for conducting patient-centered outcomes research.
research.\(^1\) The Methodology Committee had commissioned several white papers to inform its report, including some that explored the application of value-of-information (VOI) analysis for setting priorities in research. Following publication of the Methodology Report, researchers David Meltzer and Karl Claxton extended the earlier work for the Methodology Committee in papers that they presented at the December workshop.

**Box 1. Initial Proposed Process for Research Prioritization**

1. **Identify research topics.** PCORI reaches out to its stakeholder community to get a better sense of health and healthcare questions that matter most. Stakeholders can submit the specific research questions for PCORI’s consideration by completing a form on the PCORI website. PCORI also solicits research questions at several PCORI workshops and other public engagements with stakeholders.

2. **Confirm the topic covers a gap in knowledge.** PCORI systematically evaluates current research and evidence, either directly or through contracts, to ensure that questions identified have not been previously answered and are not currently being studied by others.

3. **Assess topics based on PCORI criteria (research prioritization).** PCORI will not be able to fund every research question and needs to make the best decision possible on how to spend its research funds. **Research prioritization** is the process by which advisory panels will provide their insights as to which research questions are most important for PCORI to fund and answer, given PCORI’s mission.

4. **Select topics.** After PCORI’s advisory panels have recommended topics for funding, PCORI’s Board of Governors will approve the final list of topics that the members determine should be the subject of funding announcements that will lead to one or several research studies on that topic.

5. **Create funding announcements for selected topics.** In this final step, PCORI will create targeted funding announcements based on the specific topics identified and vetted by patients and other stakeholders and approved by our Board. The result will be patient- and stakeholder-driven research projects that are part of a broader portfolio that addresses questions of specific interest to patients, their caregivers, and their clinicians.

In addition, three principles that emerged from the Transforming Patient-Centered Research: Building Partnerships and Promising Models PCORI workshop held on October 27, 2012\(^2\), also guided the development process: transparency, efficiency, and collaboration. Transparency refers to the openness

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with which PCORI conducts its investigations and deliberations and shares information among the community of patients, caregivers, and other stakeholders in the clinical research and healthcare enterprise. Efficiency demands that PCORI leverage existing knowledge so that additional research yields new and actionable knowledge. Lastly, collaboration between patients and researchers ensures that PCORI addresses meaningful questions of high importance for health and patient well-being.

Throughout this planning process, the Technical Working Group, composed of PCORI Board members, Methodology Committee members, patient representatives, and stakeholder representations, met bi-weekly over two months to provide feedback on the research prioritization process and supporting materials as they were being developed.

PCORI also engaged National Opinion Research Center at the University of Chicago (NORC) to monitor the pilot of the research prioritization process and document key outcomes. As a neutral third party, NORC provided feedback to PCORI on the process in an effort to inform its revisions and improvements to the prioritization process.

Based on results from the pilot and feedback from the Prioritization Workshop, the process will be further refined and used by future advisory panels. PCORI’s progress and timeline for 2013 are outlined in Figure 1.

![Figure 1. PCORI’s Progress and Plan for 2013](image-url)
III. Research Prioritization Pilot

Development of Initial Approach

The goal of the Research Prioritization pilot was to refine the process that will be used by future advisory panels composed of patients, caregivers, clinicians, researchers, and other stakeholders. This section highlights the key components of the pilot process.

PCORI began by identifying and articulating criteria to be used by the pilot participants to assess and rank research topics. PCORI’s authorizing legislation provides some insights into the prioritization criteria. Section 6301 of the statute decrees that the institute “shall identify national priorities for research, taking into account factors of disease incidence, prevalence, and burden in the United States (with emphasis on chronic conditions), gaps in evidence in terms of clinical outcomes, practice variations and health disparities in terms of delivery and outcomes of care, the potential for new evidence to improve patient health, well-being, and the quality of care, the effect on national expenditures associated with a health care treatment, strategy, or health conditions, as well as patient needs, outcomes, and preferences.” To formulate the criteria for this process, PCORI staff, in conjunction with members of the Technical Working Group, built on the legislation, on existing prioritization research, and on principles of VOI analysis, while maintaining a focus on PCORI’s mission. VOI can be a valuable tool in the research prioritization process; however, it is complex and requires quantified information about a number of aspects of the research question and condition being addressed. Thus, for PCORI’s application of this analytic tool, a generalized and less quantified approach, conceptual VOI, was determined to be a more appropriate tool for the pilot participants and future advisory panels to use for research prioritization. Conceptual VOI\(^3\) could allow panel members to conduct a rapid assessment based on the principles of VOI, with the potential for more in-depth analysis if needed.

The initial criteria also reflected the working definition of patient-centered outcomes research approved by PCORI’s Board of Governors. After responding to public input, PCORI defined patient-centered outcomes research (PCOR) as research that helps people and their caregivers communicate and make informed healthcare decisions, allowing their voices to be heard in assessing the value of healthcare options. This research answers patient-centered questions such as:

- “Given my personal characteristics, conditions, and preferences, what should I expect will happen to me?”
- “What are my options, and what are the potential benefits and harms of those options?”

\(^3\) VOI analysis involves “constructing a complex decision analytic model of the disease and treatment to fully characterize the uncertainty in health outcomes and costs of the treatments or other health interventions being studied.” Conceptual VOI uses elements of VOI, including comprehensive outcome measures and the implementation and durability of findings to provide information on the value of the research, but does not formally quantify the value through modeling. [Hoomans T, Seidenfeld J, Basu A, Meltzer D. Systematizing the Use of Value of Information Analysis in Prioritizing Systematic Reviews. (Prepared by the University of Chicago Medical Center through the Blue Cross and Blue Shield Association Technology Evaluation Center Evidence-based Practice Center under Contract No. 290-2007-10058.) AHRQ Publication No. 12-EHC109-EF. Rockville, MD: Agency for Healthcare Research and Quality. August 2012. Available at: http://www.effectivehealthcare.ahrq.gov/reports/final.cfm]
• "What can I do to improve the outcomes that are most important to me?"
• "How can clinicians and the care delivery systems they work in help me make the best decisions about my health and health care?"  

The initial set of “PCORI-specific criteria” used for the pilot process was as follows (please note that these criteria were subsequently revised based on the feedback PCORI received):

1. **Patient-centeredness**, which is central to PCORI’s mission. Research findings need to have meaningful impact on the patients’ lives and need to answer questions that are impactful on patients, caregivers, and providers.
2. **Impact**, as captured traditionally by the burden of disease, including the prevalence, incidence, mortality and morbidity, and quality of life when applicable.
3. **Differences in benefits and harms** that are significant enough between the interventions under consideration, given what is known at the time of the funding announcement.
4. **Reduction in uncertainty**, namely, the best estimate based on current knowledge of the degree to which the research would reduce the uncertainty around treatment effects.
5. **Implementation in practice**, reflecting how likely the research findings are to be implemented in practice and, thus, change patient outcomes.
6. **Duration of information**, which captures if the intervention will still be current by the time the research is published and the length of time the research results are likely to remain relevant.
7. **Healthcare system performance**, which addresses how the research impacts potential improvements in convenience or wasted resources while maintaining or improving patient outcomes.
8. **Inclusiveness of different populations**, which refers to the inclusion of diverse populations and/or populations that are understudied (e.g., children, elderly, and patients with multiple chronic conditions).

PCORI also developed supporting “briefs” for 10 topics that described the research questions, presenting information addressing each of the eight PCORI-specific criteria as they pertained to the topic. These topic briefs were developed in August 2012 to support the pilot exercise. In the future, these briefs will be developed by a team of scientists and experts in the specific field, who will work with the published evidence. Participants were asked to rank the research questions by applying their own knowledge of the area, in addition to the information contained in the topic briefs. One group of participants was charged with assigning weights to the criteria through a series of pairwise comparison choices. Participants were provided with a guidebook developed by PCORI staff, which provided a detailed description of the process. More detail on this process will be provided in a different section of this report.

**Recruitment**

PCORI staff recruited volunteers through an open, web-based call for statements of interest from patients and caregivers, clinicians, and researchers. To ensure that the pilot participants represented these diverse stakeholder perspectives and affiliations, PCORI’s call solicited responses specifically from

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these three groups. PCORI received more than 150 statements of interest, which PCORI staff reviewed systematically. The first round of review was based on logistical considerations—applicants had to reside in the United States and be available to attend all events, calls, and meetings. Next, PCORI narrowed the pool of applicants to ensure the group reflected a fair representation of minority or underrepresented patient populations, including those with rare conditions or diseases. At this point, PCORI also reconsidered applicants who could not attend all meetings but represented minority or underrepresented patient populations or had rare conditions or diseases. Next, PCORI evaluated the statements of interest submitted by the narrowed pool of applicants. This assessment was based on one of two sets of criteria developed by PCORI: one set of criteria for patients, caregivers, and laypersons and another for researchers, clinicians, and experts. The highest scoring applicants were considered in the final step, in which PCORI had to ensure balance among the applicants based on demographics, geography, clinical or academic affiliation, disease or condition representation, and stakeholder categories.

Ultimately, PCORI selected 35 volunteers; 33 committed to participate in the pilot. Participants had varying levels of scientific knowledge and represented a variety of backgrounds and experiences. Half of the participants primarily affiliated as researchers; the other half was distributed among those who identified primarily as caregiver, family member, patient, consumer, training institution, payer, patient/caregiver advocacy organization, and clinician. However, when asked to indicate multiple affiliations, members of the pilot group belonged to many different categories. Some participated as part of their work; others volunteered their time for the pilot. Table 1, on the following page, presents and compares the characteristics of all of those who expressed interest in participating in the pilot and of those who were selected and participated.

Pilot Process

Pilot process overview: Thirty-three volunteers participated in a series of teleconferences and individually applied PCORI’s research topic rating tools to rank 10 hypothetical research topics online. Preparation for the prioritization exercise included review of the guidebook, participation in two prefatory conference calls (October 11 and October 29, 2012), and close study of a 60-page topic guide discussing the 10 proposed topics.

Kick-off call: During a kick-off call held October 11, PCORI staff provided a thorough introduction of the pilot and the participants and reviewed the research topics and criteria to be considered for prioritization, as well as the topic briefs that were developed to support this process. The overall timeline and goals of the pilot process were also discussed. At the end of the call, participants were tasked with reviewing the topic briefs and nominating vice chairs for each of the two groups.

Following the first call, the participants were split into two groups, which had different tasks. In the online exercise occurring after the second call and before the third call on November 5, 2012, members of Group A used two software tools, Survey Gizmo and Expert Choice, to rate the relative priority of each of the 10 topics. Members of Group B performed an evaluative exercise to establish the relative importance or weights of the eight prioritization criteria and used one of the prioritization tools, Expert Choice, to rate the priority of each of the topics on a Likert scale. The two-hour call conducted by each group separately on November 5 was used to present the results for the group and discuss members’ experiences ranking the topics and/or weighting the priority criteria.
Table 1. Comparison of Characteristics between Pilot Applicants and Pilot Participants

<table>
<thead>
<tr>
<th>Characteristics</th>
<th>Pilot Applicants</th>
<th>Pilot Participants</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Gender</strong></td>
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<tr>
<td>Male</td>
<td>29%</td>
<td>33%</td>
</tr>
<tr>
<td>Female</td>
<td>71%</td>
<td>67%</td>
</tr>
<tr>
<td><strong>Age</strong></td>
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<td></td>
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<tr>
<td>0–25 years</td>
<td>1%</td>
<td>0%</td>
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<tr>
<td>26–40 years</td>
<td>24%</td>
<td>36%</td>
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<tr>
<td>41–65 years</td>
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</tr>
<tr>
<td>65+ years</td>
<td>6%</td>
<td>11%</td>
</tr>
<tr>
<td><strong>Race/Ethnicity</strong></td>
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<td></td>
</tr>
<tr>
<td>American Indian or Alaska Native</td>
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<td>6%</td>
</tr>
<tr>
<td>Asian</td>
<td>14%</td>
<td>19%</td>
</tr>
<tr>
<td>Black or African American</td>
<td>6%</td>
<td>6%</td>
</tr>
<tr>
<td>Hispanic or Latino</td>
<td>2%</td>
<td>0%</td>
</tr>
<tr>
<td>White</td>
<td>76%</td>
<td>69%</td>
</tr>
<tr>
<td><strong>Stakeholder Affiliation</strong></td>
<td></td>
<td></td>
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<tr>
<td>Caregiver</td>
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<td>8%</td>
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<td>Clinician</td>
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<td>6%</td>
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<td>0%</td>
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<td>Consumer</td>
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<td>3%</td>
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<tr>
<td>Industry</td>
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<td>3%</td>
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<tr>
<td>Organizational Provider</td>
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<tr>
<td>Patient</td>
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<td>Patient Advocate</td>
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<td>Patient/Caregiver Advocacy</td>
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<td>Advocacy Organization</td>
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<tr>
<td>Payer</td>
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<td>3%</td>
</tr>
<tr>
<td>Policy Maker</td>
<td>1%</td>
<td>3%</td>
</tr>
<tr>
<td>Researcher</td>
<td>53%</td>
<td>50%</td>
</tr>
<tr>
<td>Training Institution</td>
<td>3%</td>
<td>0%</td>
</tr>
</tbody>
</table>
Second call: Since PCORI was testing two methods for conducting the topic evaluation, during a second call held October 29, participants were assigned to one of two groups, and separate calls were convened for each group. On each call, PCORI introduced the vice chair and provided an overview of the prioritization process and an orientation to the prioritization methods. Each participant received further instructions for the prioritization methods applicable to the particular group via e-mail. Participants were instructed to complete the ranking exercise before November 5, so that results could be presented and discussed on the third call scheduled for that day.

Participants were given three days, during which they had the opportunity to re-rank the research topics based on the November 5 discussions. No one chose to revise his or her initial rankings, however. Following that third call, all participants were asked to complete an online survey with a variety of questions regarding their experiences during the prioritization task and recommendations. Additionally, eight participants (four from each group) were asked to participate in individual one-hour interviews to debrief from and reflect on the prioritization process and tools. Also following the final teleconference, the pilot group participants were paired with another member of their group to get to know each other and share their experiences with the prioritization exercise, and they were encouraged to contact PCORI with any insights arising from these discussions.

Ranking exercise: To simulate the actual prioritization process, PCORI selected 10 research questions, shown in Box 2, from a set of about 500 questions collected and vetted by agencies such as the NIH and AHRQ. The research questions ranked in the pilot prioritization exercise will not result in a funding announcement from PCORI, although some of the same questions could be considered in a future PCORI prioritization process.

Box 2. Ten Research Topics Selected for the Pilot

1. What is the comparative effectiveness of antipsychotics in treating adolescents and young adults, particularly among those with ADHD, bipolar disorder, or schizophrenia?
2. What is the comparative effectiveness of management strategies of elderly patients with back pain to evaluate many clinically relevant patient-reported outcomes?
3. What is the comparative effectiveness of genetic and biomarker testing to identify optimal candidates for breast cancer risk-reduction medications and interventions?
4. 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, specifically for understudied populations?
5. 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?
6. What is the comparative effectiveness of management strategies for ductal carcinoma in situ (DCIS) to improve long-term patient-centered outcomes?
7. 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?

8. What is the comparative effectiveness of indoor air pollution interventions to improve respiratory and cardiovascular health outcomes among high-risk populations?

9. What is the comparative effectiveness of coordinated treatment options for patients with multiple chronic conditions?

10. 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?

After the second call, participants were given one week to complete the ranking exercise. Group A used both Expert Choice and Survey Gizmo to rate the topics; Group B used Expert Choice to rank both the criteria and the topics. Survey Gizmo provided the list of topics in no particular order and asked participants to rank the topics by distributing 100 points across the topics. Participants could allocate more or fewer points on any topic in a range from 0 to 30, using whole, positive numbers. Expert Choice, a decision analysis tool, first asked participants to consider the evaluation criteria and how they should rate against each other. Then, participants were asked to do several pairwise comparisons of the evaluation criteria, which were used to generate a composite weight using the software’s analytic capability. Participants were then asked to evaluate the 10 questions using the criteria. Using this process, Expert Choice took into account the relative weight of the criteria and the ratings of the questions.

**Third call:** During a third call, held on November 5, Groups A and B separately discussed the results of their particular group’s rankings. Prior to the call, each group received a report of the group’s ranking results and a copy of their individual assessment. The vice chair of each group led the respective calls. The group prioritization results were presented during the call; however, the main purpose of the call was to learn more about participants’ experiences in applying the criteria to the topics using the ranking software. Box 3 shows questions used to stimulate discussion during these calls.

**Box 3. Discussion Questions for November 5, 2012**

- How efficient/feasible/difficult was it to apply the criteria to the topics?
- Is the variability in scores expected?
- Is the difference in weighted and unweighted results expected?
- How much did you use primarily your own knowledge and judgment to rank the topics, as opposed to using the topic briefs?
- Can you provide any brief comments to help PCORI improve the process?
Post-ranking survey: At the end of the ranking exercise, participants were asked to complete a survey to evaluate each component of the PCORI prioritization process, including the guidebook, topic briefs, PCORI-specific criteria, ranking tool(s), and their overall experience with the process. The survey had two versions to accommodate the ranking tool(s) tested by each pilot group. The survey consisted of multiple-choice questions, Likert-like scale questions, and opportunities for the participant to provide detailed comments and recommendations.

Paired discussions by group members: In an effort to encourage additional dialogue among pilot participants, PCORI assigned group members into pairs to discuss their experiences with the ranking exercise. Seven pairs, or 14 members, met for 30 to 60 minutes via telephone or Skype and shared their views on various topics, including views on their contribution to the process, personal interests in research prioritization, benefits and challenges of PCORI’s work, and lessons learned from the ranking exercise. Box 4 lists the discussion questions provided to participants for their conversations. Several of the participants sent PCORI summaries of their conversations, noting additional insights and reflections about the prioritization process.

Box 4: Discussion Questions for Paired Discussions

- What personally excites you about being more involved in research prioritization and more involved in patient-centered research?
- What can I bring to the table for the Research Prioritization pilot? What can I contribute to PCORI’s research agenda?
- What do you hope to take with you as you complete this exercise and as you leave the workshop?
- What are the benefits, as well as challenges, of patients and stakeholders being involved in research prioritization?
- What is the most important step you see PCORI can take as a lesson from the pilot that you hope will ignite a positive change in the research prioritization process?

One-on-one interviews: In addition to the conversations by pairs of participants, NORC at the University of Chicago conducted one-on-one interviews to collect in-depth feedback about the ranking exercise. Several participants expressed their willingness to participate in an hour-long interview, and four pilot participants from each group, representing a variety of stakeholder groups, were selected. The interviews, guided by a standard protocol, aimed to understand the participants’ interpretation and application of the criteria, to gather recommendations for revising the criteria and background materials provided to support the process, and to reflect on the overall format of the exercise. The eight interviews were conducted by phone between November 19 and November 30, 2012.

Research Prioritization workshop: PCORI hosted the PCORI Methodology Workshop for Prioritizing Specific Research Topics on December 5, 2012, at the Hilton Alexandria Mark Center in Alexandria, VA (see Appendix 1 for full agenda). Workshop participants included the pilot participants and researchers who are experts in prioritization processes and tools. The workshop began with an overview of the pilot process and initial outcomes, followed by a keynote address from Gail Wilensky of Project HOPE. Several sessions featured experts discussing the science of research prioritization, as well as the clinical, political, and business context of the process. Six pilot participants summarized the experiences and
feedback of the two groups. In addition, there was opportunity for public comments and questions about the process and PCORI’s plans. The workshop provided an opportunity for PCORI staff, stakeholders, and pilot participants to share their experiences and findings. The meeting was highly interactive, with extended question-and-answer periods to engage workshop participants and attendees, both in person and via webcast.

Post-workshop survey: After the workshop, PCORI requested each participant complete an online survey. Participants were asked to evaluate their experiences at the workshop, share their lessons learned, and comment on the challenges and benefits of engaging patients and stakeholders in the prioritization process. Moreover, the survey requested information related to PCORI more generally, such as the participant’s current involvement with the organization, anticipated interaction with PCORI in the future, and feedback for improving future workshops.

IV. Outcomes

PCORI-specific Criteria Revised

By the conclusion of the pilot activities, PCORI had accumulated a wealth of constructive feedback on each component of the ranking exercise and the overall research prioritization process. Participants emphasized the importance of representing diverse perspectives in the advisory groups; emphasized a need to clarify the definition of a research topic; recommended ways to improve the supporting information, such as the format and content of topic briefs; and discussed the PCORI-specific criteria extensively.

PCORI took advantage of the opportunity to refine the initial set of criteria before the Research Prioritization workshop, so that the attendees could comment on the revised criteria on December 5. Overall, pilot participants felt that some criteria were not clear, and others may have overlapped in their meaning. Specifically, participants had difficulty distinguishing the criterion of assessing the balance of benefits and harms and that of reducing uncertainty regarding treatment effectiveness. The criterion of affecting healthcare system performance was considered ambiguous by many. Participants generally viewed the criterion of inclusiveness of different populations as less appropriate to research topics or questions and more applicable in reviewing specific research study designs. In response, PCORI refined the criteria to include the following five:

(1) Patient-Centeredness
The core of PCORI’s mission is to address patient-centered research questions, the answers to which have significant potential for improving patient health. This criterion considers whether the results of a research study would help patients, clinicians, or healthcare leaders in making important healthcare decisions. Another aspect of patient-centeredness to consider is whether patients, caregivers, and their healthcare providers have expressed a real need for this information to support their decision-making.
(2) Impact of the Condition on the Health of Individuals and Populations
This criterion refers to the burden of disease. The burden of disease may be measured by how many people have the disease (prevalence); how many new cases occur every year (incidence); and other measures such as mortality, morbidity, individual suffering, and loss of productivity. PCORI is also interested in rare diseases so these should be given due consideration.

(3) Options for Addressing the Issue
This criterion addresses the current landscape of management options and what is known about the relative benefits and harms of the available management options, based on recent systematic reviews (e.g., Cochrane) and evidence-based guidelines (e.g., AHRQ Clearinghouse). What could new research contribute to achieving better patient-centered outcomes? Have recent innovations (e.g., a new technology or a new policy) made research on this topic especially compelling? How widely does care now vary? What is the pace of other research on this topic (as indicated by recent publications and ongoing trials)? How likely is it that new comparative effectiveness research on this topic would provide better information to guide clinical decision making? How likely is it that new research on this topic would increase our certainty about how to address the problem?

(4) Likelihood of Implementation in Practice
This criterion addresses how likely the research results will be implemented in practice. Research is valuable when it leads to the use of more beneficial treatments or interventions. Providing new evidence by itself does not ensure that the results of research will be used in clinical practice.

(5) Durability of Information
This criterion addresses whether the results of a research study on a particular treatment or intervention would be durable over time. This durability of information is generally associated with the rate at which new clinical evidence and/or better alternatives for patient management are emerging. Durability might be limited when there are rapid modifications to procedures and techniques.

Research Prioritization Workshop: Summary of Discussion
The Research Prioritization workshop, moderated by Paul Wallace of the Lewin Group, began with a brief introduction of PCORI and the proposed research prioritization process. Gail Wilensky’s keynote address focused on the political and fiscal context of how comparative clinical effectiveness research can be used to improve quality and efficiency of care in the future. Then, the discussion moved swiftly into an expert discussion on improving research prioritization methods and reactions to the proposed process, followed by feedback from pilot participants and the public.
On improving research prioritization methods: Two presenters showcased VOI analysis methods to help improve methods for research study prioritization and convey key considerations in conducting these kinds of analyses: “Pragmatic Approaches to Value of Information Analysis” by David Meltzer and “Expected Health Benefits of Additional Evidence” by Claire McKenna.

Dr. Meltzer’s presentation provided an overview of VOI analysis and practical approaches for how it can be used for research prioritization. VOI provides a method for estimating the population health impact of research questions using complex modeling. Because VOI methods are complex and can be resource-intensive, Dr. Meltzer described other VOI modeling approaches that are less complex, for example, conceptual VOI. Conceptual VOI applies the concepts of VOI analysis but does not require a complex model, which allows for quick analysis and increased accessibility to the layperson. Using conceptual VOI, if a proposed research study is found to provide a good return on investment, then no further kind of modeling approach would be necessary. On the other hand, if the topic also clusters with others in its topic domain, then a maximal modeling approach should be used to distinguish its potential value from other research studies. If there is no clustering and comprehensive outcomes are already available, a minimal modeling can be used instead; and if no comprehensive outcomes are available and data collection is potentially costly, then a full modeling approach should be used. Otherwise, a non-costly data collection potential research topic warrants no further modeling.

Dr. McKenna’s presentation summarized a white paper authored by Karl Claxton, herself, and colleagues, “Expected health benefits of additional evidence: Principles, methods and applications.” Dr. McKenna discussed four case studies that highlighted key considerations in measuring the expected health benefits of additional evidence. One case study illustrated the difference between the value of evidence and value of implementation, where the former refers to the contribution of an additional study to the body of evidence and the latter is the actual impact on clinical practice. Another case study demonstrated how estimates of effectiveness can change when additional outcomes linked to the primary endpoint are taken into account. The third case study demonstrated how different weights can be used to reflect the relevance of evidence (for example, whether previous trials were of less quality) and how to select the appropriate statistical approach. Finally, the fourth case study showed how to synthesize study results through mixed treatment comparisons that use all the available evidence.

Expert reactions to PCORI’s proposed research prioritization process: Expert panelists were invited to comment on the proposed process outlined earlier in the day. The discussion was moderated by Jean Slutsky of the Center for Outcomes and Evidence at AHRQ. The panelists included: Robert Dubois, MD, PhD (Chief Science Officer, National Pharmaceutical Council); Veronica Goff, MS (Vice President, Nation Business Group on Health); and Sally Morton, PhD (Professor and Chair, Department of Biostatistics, University of Pittsburgh Graduate School of Public Health).

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Transparency and reliability were two recurring themes of the panel. While much of the decision making will reside with PCORI staff and will require approval from the Board, transparency will help everyone in the priority-setting enterprise, including stakeholders and patients, to feel that their voices are heard. It is important that everyone understands how topics are gathered, how topics are chosen for prioritization, and how the prioritization process is operationalized. Transparency will be a key aspect for credibility. Regarding reliability, it was suggested that PCORI may consider conducting reliability experiments and have at least two advisory groups rate each the topics, although this may be difficult to set up in practice. Similarly, members of the advisory panels will change over time, and PCORI must account for this variability effect.

**Pilot group feedback on research prioritization process:** The panel of pilot participants represented both groups and included the vice chairs and four pilot group members. The panelists touched on four areas for improvement: diversity in recruitment, emphasis on the patient’s voice, PCORI-specific criteria, and an evaluation of the software tools. Box 5 shows the pilot group members who participated in this session.

**Box 5: Participants in the Pilot Group Feedback Session**

**Group 1**
- Fouza Yusuf, MS, MPH, Medical College of Wisconsin (Group 1 Vice Chair)
- Kirk Allison, PhD, MS, Program in Human Rights and Health, University of Minnesota School of Public Health
- Dan Cherkin, PhD, Group Health Research Institute/Bastyr University Research Institute

**Group 2**
- Liz Jacobs, MD, University of Wisconsin School of Medicine and Public Health (Group 2 Vice Chair)
- Lisa Hopp, PhD, RN, FAAN, Indiana Center for Evidence-Based Nursing Practice
- Ting Pun, Patient and Caregiver, Portola Valley, CA

PCORI applied a systematic approach to ensure diversity in the pilot group. At the same time, however, it was recognized that there might be self-selection for the pilot, among those who were already familiar with PCORI and aware of the call for letters of interest. With more time, PCORI could recruit more intentionally through social media, advocacy groups, and word of mouth. To further the diversity of the advisory panels and increase the focus on research of great interest to patients, panelists suggested including non-physicians such as occupational therapists, midwives, and pharmacists. These professionals have rich experience at the frontlines and can offer valuable insight about outcomes that are less commonly reported in traditional clinical studies.

The panelists also felt that the research prioritization process should place more emphasis on the patient’s voice in order to better support PCORI’s mission and vision. Based on the 10 sample topic briefs used in the prioritization exercise, participants found the statement of the topic not sufficiently or obviously patient-centered: What evidence exists for why a particular topic is of concern to patients or clinicians? How will research on a topic inform the care of individual patients and distinctive characteristics? Moreover, the overall prioritization process remains very structured and scientific. For
example, some participants found the topic briefs to be too technical and sometimes too detailed. Many actionable recommendations regarding these issues surfaced in the discussion, including the use of plain language wherever possible, presentation of supporting information in two tiers (lay and technical) to accommodate different needs, and providing patient vignettes for each topic in order to cue the advisory group to think from the patient’s perspective.

Throughout the prioritization exercise, participants consistently expressed concern in the lack of clarity in the PCORI-specific criteria and felt that it was difficult to evaluate topics across the eight criteria. Therefore, collapsing the number of criteria to the five was well received. This revision improved the clarity of the criteria and reduced or eliminated overlaps between the initial set of criteria. In addition, the future advisory panels will be able to focus on the most important criteria.

Lastly, the panelists commented on Survey Gizmo and Expert Choice, the software tools used in the prioritization exercise. Participants judged Survey Gizmo as easy to use and found it allowed them to complete the ranking process relatively quickly. However, it required subjective assessments. Participants also found it difficult to consider all eight criteria and 10 topics at the same time, when comparing different topics. As for Expert Choice, participants described it as imposing a more systematic structure on the rating process, which made the ranking more manageable. At the same time, the tool was time-consuming. Overall, both tools have advantages and disadvantages. Participants felt that PCORI can use both tools to validate the rankings.

**Public feedback on proposed research prioritization process:** During this final session for comments and feedback, workshop participants remained engaged and asked many thoughtful questions about PCORI’s prioritization process. The audience wrestled with the tension between PCORI’s responsibility to fund research that can produce meaningful results quickly and the labor-intensive process of identifying topics that align with PCORI’s mission and mandate.

Moreover, one workshop participant probed the research prioritization process further and wondered if the PCORI-specific criteria should also emphasize the importance of communicating well with a lay audience, rather than solely focusing on the research audience. To bridge the disconnect between medical community “speak” and lay “speak,” the participant suggested that PCORI set a new standard so researchers can partner with communication experts to develop effective communication with end users about outcomes, process, and intent. It was further suggested that future applicants to PCORI funding would be responsible for writing as simply as possible, even as the research itself uses appropriately rigorous research methods. A key aspect to patient-centered research is that the research and findings must be accessible and understandable to the layperson. Workshop participants suggested that if the research is not understandable to the layperson, then it is not patient-centered, and, therefore, PCORI should not fund the research. It was suggested that PCORI could consider this to be a requirement or component of the patient-centeredness criterion.

Continuing on that theme, discussants pointed out, over the course of the workshop, that the number of stakeholders in research prioritization has expanded significantly. Indeed, different types of information require input from different stakeholders. There is a question of how to integrate the pieces of information and, given that different stakeholders will communicate at varying levels of scientific and methodological detail, ensure that all pieces of information are understood and interpreted correctly.
V. Next Steps

From the beginning, PCORI has been committed to an open, flexible approach for developing its research prioritization process. It has relied on feedback from PCORI’s Methodology Committee, the Technical Working Group, pilot exercise, and discussions at the workshop to refine the process for the first round of research prioritization in 2013. PCORI intends to continue learning from its research prioritization process and the feedback from the advisory panels. Based on the feedback from the Research Prioritization pilot and workshop, PCORI has identified several next steps:

- Revise the process based on the feedback from the pilot exercise
- Review and incorporate insights from the ARRA comparative effectiveness research methodology studies as appropriate
- Produce a revised guidebook for the advisory panel training in March-April 2013
- Improve topic briefs in terms of consistency in presentation and level of sophistication, including the possibility of engaging other expert organizations in the development of the topic briefs
- Integrate the improvements proposed at the workshop by the methodologists
- Finalize logistic items such as the choice of software and the ranking process
Appendix A: Workshop Agenda—Methodology for Prioritizing Specific Research Topics

Patient-Centered Outcomes Research Institute

Methodology Workshop for Prioritizing Specific Research Topics

December 5, 2012 | Hilton Alexandria Mark Center
5000 Seminary Road, Alexandria, VA

Agenda (All Times Eastern)

Note: Registration for the workshop is full. Individuals not able to attend in person can follow the workshop’s plenary sessions via webcast.

Tuesday, December 4, 2012

<table>
<thead>
<tr>
<th>Time</th>
<th>Topic</th>
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<tr>
<td>6:00 – 8:00 p.m.</td>
<td>Pre-event Welcome Reception</td>
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Wednesday, December 5, 2012

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<tr>
<th>Time</th>
<th>Topic</th>
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<tr>
<td>7:45 – 8:30 a.m.</td>
<td>Breakfast</td>
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<td>8:30 – 8:40 a.m.</td>
<td>Welcome and Introductions</td>
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<tr>
<td></td>
<td>Paul Wallace, MD</td>
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<td>Senior Vice President and Director, Center for Comparative Effectiveness Research, The Lewin Group</td>
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<td>8:40 – 9:00 a.m.</td>
<td>Opening Remarks</td>
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<td>Joe Selby, MD, MPH</td>
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<td>Executive Director, Patient-Centered Outcomes Research Institute</td>
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<tr>
<td>9:00 – 9:30 a.m.</td>
<td>Proposing a Research Prioritization Process for PCORI</td>
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<td>Rachael Fleurence, PhD</td>
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<td>Scientist, Patient-Centered Outcomes Research Institute</td>
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<td>9:30 – 10:00 a.m.</td>
<td>Keynote Address</td>
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<td>Gail Wilensky, PhD</td>
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<td>Economist, Senior Fellow, Project HOPE</td>
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<td>10:00 – 10:15 a.m.</td>
<td>Break</td>
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<td>10:15 – 11:30 a.m.</td>
<td><strong>Improving Research Prioritization Methods</strong></td>
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<td></td>
<td>David Meltzer, MD, PhD</td>
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<td></td>
<td>Director, Center for Health and the Social Sciences, and Chief, Section</td>
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<td></td>
<td>of Hospital Medicine and Associate Professor, Department of Economics,</td>
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<td></td>
<td>Graduate School of Public Policy Studies, University of Chicago</td>
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<td></td>
<td>Member, PCORI Methodology Committee</td>
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<td></td>
<td>Claire Mckenna, PhD, MPH, MSc, presenting on behalf of Yoni Claxton, Ph.D</td>
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<td>Professor, Department of Economics and Related Studies, University of</td>
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<td>11:30 a.m. – 12:15 p.m.</td>
<td><strong>Lunch</strong></td>
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<td>12:15 – 1:30 p.m.</td>
<td><strong>Panel: Experts' Reactions to PCORI's Proposed Research Prioritization Process</strong></td>
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<td>Jean Slutsky, PA, MSPH (Moderator)</td>
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<td>Center for Outcomes and Evidence, Agency for Healthcare Research and</td>
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<td>Robert Dubois, MD, PhD</td>
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<td>Chief Science Officer, National Pharmaceutical Council</td>
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<td>Veronica Goff, MS</td>
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<td>Vice President, Motion Business Group on Health</td>
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<td>Sally Morton, PhD</td>
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<td>Professor and Chair, Department of Biostatistics, University of Pittsburgh</td>
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<td>Graduate School of Public Health</td>
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<td>1:30 – 2:30 p.m.</td>
<td><strong>Pilot Group Feedback on Research Prioritization Process</strong></td>
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<td>Moderator Paul Wallack, MD</td>
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<td>Fouzara Yusuf, MS, MPH</td>
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<td>Lisa Hopp, PhD, RN, FAAN</td>
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<td>Ting Fu, Patient and Caregiver</td>
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<td>2:30 – 3:30 p.m.</td>
<td><strong>Public Feedback on Proposed Research Prioritization Process</strong></td>
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<td>Moderator Paul Wallack, MD</td>
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<td>Joe Selby, MD, MPH</td>
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<td>Rachel Fluorence, PhD</td>
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<td>David Meltzer, MD, PhD</td>
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<td>David Hickam, MD, MPH</td>
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<td>PCORI Scientific Program Leader, Comparative Assessment of Options Research</td>
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<td>3:30 – 3:45 p.m.</td>
<td>Break</td>
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| 3:45 – 4:15 p.m. | PCORI Perspectives on Input into Research Prioritization Process | Joe Selby, MD, MPH  
Rachael Fluorence, PhD |
| 4:15 – 4:30 p.m. | Closing Remarks and Adjourn                            | Joe Selby, MD, MPH                              |
Appendix B: Acknowledgements

Pilot Group Chair
Tim Carey, Sheps Center for Health Services Research, University of North Carolina Chapel Hill

Pilot Group Vice Chairs
Elizabeth Jacobs, University of Wisconsin School of Medicine and Public Health
Fouza Yusuf, Medical College of Wisconsin

Pilot Group Participants
Kirk Allison, Program in Human Rights and Health, University of Minnesota School of Public Health
Erika Augustine, University of Rochester Medical Center
Laura Bernard, Consultant
Marissa Brooks, SEIU Healthcare NW Health Benefits Trust
Jesus Casida, University of Michigan
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Bobby Dubois, National Pharmaceutical Council
Lisa Hopp, Indiana Center for Evidence-Based Nursing Practice
Jeannie Huang, University of California San Diego
Barbara Hunt, CADASIL Association
Deborah Hunt, College of New Rochelle
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Allison Kalloo, Clinical Ambassador LLC
Charlotte Kauffman, State of Illinois Division of Mental Health
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Jane Kogan, UPMC Center for High Value Health Care
Carrie Levin, Informed Medical Decisions Foundation
Michelle Luo, Baxter Healthcare
Michele Maiers, Northwestern Health Sciences University
Rebecca Malouin, Michigan State University
Michael Miller, Ann & Robert H. Lurie Children’s Hospital of Chicago
Dana Mukamel, University of California Irvine
Huseyin Naci, Harvard Medical School
Linda Nguyen, Alliance for Children and Families
Ting Pun, Patient and Caregiver, Portola Valley, CA
Alex Reed, Family Medicine Residency of Idaho
Leif Solberg, Health Partners Institute for Education and Research
Louis Tharp, Global Healthy Living Foundation
Nalini Visvanath, Patient and Patient Advocate, Washington, DC
Renda Wiener, Boston University School of Medicine
Pilot Technical Working Group
Arnie Epstein, PCORI Board of Governors; John H. Foster Professor and Chair of the Department of Health Policy and Management at Harvard University School of Public Health
Gail Hunt, PCORI Board of Governors; President and CEO of the National Alliance for Caregiving
Neil Kirschner, Stakeholder Representative; Senior Associate, Regulatory and Insurer Affairs at American College of Physicians
Linda Morgan, Patient Representative, Parkinson’s Disease Foundation
Jean Slutsky, PCORI Methodology Committee; Director of the Center for Outcomes and Evidence (COE), AHRQ
Clyde Yancy, PCORI Methodology Committee; Chief, Cardiology, Northwestern University Feinberg School of Medicine and Associate Director, The Bluhm Cardiovascular Institute at Northwestern Memorial Hospital

With special thanks to our presenters at the December 5, 2012, workshop
Kirk Allison
Dan Cherkin
Lisa Hopp
Liz Jacobs
Ting Pun
Fouza Yusuf

NORC Staff
Wilhelmine Miller
Jennifer Benz
Catharine Quirk
Petry Ubri
Tianne Wu
Jean-Ezra Yeung

PCORI Staff
Joe V. Selby
Rachael Fleurence
Bill Silberg
Kara Odom Walker
Natalie Wegener
Katie Wilson