

PIPC White Paper on “Road Map for Prioritizing Research”
November, 2013

Background

The issue of how to set priorities for comparative research and health technology assessment has long been recognized as an important one.¹ The statute creating the Patient-Centered Outcomes Research Institute (PCORI) also recognized this, describing specific factors and procedural considerations for defining national priorities for research.² The question of how best to define research priorities, and whether to do so by proactively identifying specific topics or issuing broader solicitations for investigator-initiated prioritization, has been an area of ongoing debate.³

Accordingly, the Partnership to Improve Patient Care (PIPC) has long advocated for PCORI to identify a clear process for defining specific research priorities,⁴ and applauded PCORI’s recent announcements to (a) establish advisory panels to help set research priorities and (b) make more of its research funding decisions based on specific priority topics. This white paper is intended to identify key elements and serve as a high-level “road map” for PCORI’s consideration in setting specific priorities for research.

Rationale for a Targeted Approach to Priority-Setting

As outlined in statute, PCORI’s purpose is to:

“assist patients, clinicians, purchasers, and policy-makers in making informed health decisions by advancing the quality and relevance of evidence concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed through research and

¹ Donaldson MS, Sox HC, eds. Setting Priorities for Health Technology Assessment: A Model Process. Washington, DC: National Academy Press; (1992).

Gibson JL, Martin DK, Singer, PA. Priority setting for new technologies in medicine: A transdisciplinary study. *BMC Health Services Research* 2:14 (2002).

² Patient Protection and Affordable Care Act of 2010, §6301. Available at <http://dpc.senate.gov/dpcissue-sen_health_care_bill.cfm>

³ Sox, Hal, MD. The Patient-Centered Outcomes Research Institute Should Focus On High-Impact Problems That Can Be Solved Quickly, *Health Affairs*, October 2012; Vol. 31, No. 10:2176-2182

⁴ Priority-Setting for Comparative Effectiveness Research: A Review of Key Elements: Partnership to Improve Patient Care. Available at <http://www.pipcpatients.org/pipc-admin/pdf/dbfe1e_White%20Paper.pdf>

evidence synthesis that considers variations in patient subpopulations, and the dissemination of research findings with respect to the relative health outcomes, clinical effectiveness, and appropriateness of the medical treatments, services, and items,”

including many types of healthcare interventions.⁵ To achieve this purpose, and the goal of patient-centeredness, PIPC believes it is essential to define specific research topics, and ensure these topics are informed by input from patients and clinical experts.

The statute creating PCORI recognized that defining national priorities for research would be an important early step for PCORI, and it specified several key elements of priority-setting to help achieve the goal of research that is relevant to patients and providers at the point of decision-making. These elements include a transparent and public process, with guidance from expert advisory panels.⁶

Over the past several months, PCORI has taken positive steps to move beyond research priorities described at the level of broad categories of health care ((1) assessment of prevention, diagnosis and treatment; (2) improving health care systems; (3) communication and dissemination research; (4) addressing disparities; and (5) accelerating patient-centered outcomes research and methodological research) to priorities described at the level of specific research topics. It also has created several expert advisory panels to support this work. PIPC strongly supports this shift to describing priorities at a more detailed level; we believe it is essential to achieving PCORI goals, and will provide broader benefits by establishing consensus priorities for researchers in academia and the private sector.

This level of specificity is essential to fulfill PCORI’s mission of producing evidence that enhances patient and caregiver decision-making. As stated by Dr. Mark McClellan in comments to PCORI:

“Specificity will be necessary to solidify PCORI’s role in ensuring that its funded research addresses the most pressing evidence gaps for patient and caregiver decision making. Given such limited specificity, the broad approach to soliciting research proposals outlined in the current draft of the research agenda may still result in a body of

⁵ Patient Protection and Affordable Care Act of 2010, §6301 amending 2 U.S.C. 1301 et seq. at §1181(c). Available at <http://dpc.senate.gov/dpcissue-sen_health_care_bill.cfm>

⁶ Patient Protection and Affordable Care Act of 2010, §6301 amending 2 U.S.C. 1301 et seq. at §1182(a) and §1181(d)(4). Available at <http://dpc.senate.gov/dpcissue-sen_health_care_bill.cfm>

proposals and studies that resembles currently funded efforts, even with the kind of distinguishing practical criteria for patient-centered research that PCORI has identified.”⁷

The need for a new, efficient approach to setting specific research priorities was echoed by Dr. Hal Sox, who, in 2009, chaired the Institute of Medicine (IOM) Committee on Comparative Effectiveness Research Priorities that published the Initial National Priorities for Comparative Effectiveness.⁸ It is also supported in a recent paper released by the Brookings Institution, which called for a move beyond investigator-initiated research funding:

“A key step in maximizing the impact of CER [comparative effectiveness research] is to develop a process for identifying priority research topics. The predominant process of setting research priorities for federal funding, which involves awarding budgets to granting organizations who in turn give research funding to the most meritorious proposals from investigators, helps assure that research meets technical and relevance standards, but probably does not maximize the value of CER... National Priorities for CER could be more efficiently set and achieved if a more systematic process were developed with criteria that directly reflect the needs of decision makers in both patient care and policies that affect patient care.”⁹

Overview of PIPC’s Road Map to Priority-Setting

Our goal in describing “best practices” in priority-setting is to support PCORI in refining a process that is workable, efficient, and patient-centered, yielding targeted research questions most responsive to the needs of patients and their caregivers. PIPC recognizes that PCORI has already incorporated some of these elements into its decision-making. We hope that this white paper provides further direction for integrating these elements into a cohesive and transparent process for setting specific research priorities.

In developing these recommendations, we drew on prior works describing and implementing best practices in health care priority-setting, and sought to adapt these to PCORI’s particular

⁷ McClellan, Mark, MD, PhD. Comments on PCORI’s Draft National Priorities for Research and Research Agenda, March 15, 2012. Available at <http://www.pcori.org/assets/gravity_forms/2-11659cf47a59e2684bfbe9aba8241776/2012/03/Comments-to-PCORI-20120315.pdf>

⁸ See Sox, *supra* at 2176-2182.

⁹ McClellan, Mark, MD, PhD and Benner, Joshua, PharmD, ScD. Comparative Effectiveness Research: Will it Bend the Health Care Cost Curve and Improve Quality? The Brookings Institute, June, 2009, at 12. Available at <http://www.brookings.edu/~/media/research/files/papers/2009/6/09%20cer%20mclellan/0609_health_care_cer.pdf>

structure and mission.¹⁰ We also drew on recent PCORI-sponsored research that examines best practices for engaging patients throughout the research process. For example, Dr. Daniel Mullins from the University of Maryland conducted a study regarding the integration of patient perspectives in clinical trial design, particularly among hard to reach populations, which provides useful insight for PCORI.¹¹

PIPC's "road map" for priority-setting includes seven key elements:

- A broad and structured solicitation of topics
- Development of rationale and topic briefs
- Ranking of topic list by expert advisory panels
- Development of draft priorities by expert advisory panels
- PCORI staff review and public comment on draft priority list
- Submission to PCORI Board of Governors
- Transparency of the priority-setting process in its entirety

Road Map to Priority Setting

1. Priority-setting should begin with a public solicitation for potential research topics from a broad range of stakeholders, and PCORI should feed this into a structured process for refining draft priorities.

The first step in any priority setting process is to define the range of relevant, potential research priorities. In view of PCORI's particular mandate for patient-centeredness, patients and providers must be engaged at the outset in setting the research agenda. To meet its statutory mandate of setting a targeted, national research agenda, PCORI should start with a broad, structured process for soliciting specific research topics that are of the highest priority to patients and providers. In this process, PCORI should identify the extent to which the solicited topics reflect the priorities of a broad base of affected patients, and are agreed to be priorities by their providers.

One example is the process undertaken in 2009 by the IOM Committee on Comparative Effectiveness Research Priorities, which went through a detailed step-by-step process to identify primary CER topic areas and solicited specific suggestions from a variety of

¹⁰ VanLare JR, Conway PH, Sox HC. Five next steps for a new national program for comparative-effectiveness research. *New Engl J Med.* 2010;362:970-973. Available at <<http://www.nejm.org/doi/full/10.1056/NEJMp1000096>>

¹¹ Interviews to Identify Evidence for Eliciting the Hard to Reach Patient's Perspective in PCOR." Source: PCORI (contract PCORI-SOL-PCWG-002) 2011.

stakeholders via a broad-based email announcement. These stakeholders included individuals from many groups including, but not limited to, consumer and patient advocacy groups, federal government agencies, health care providers and researchers, insurers, integrated health systems, manufacturers, and state government agencies. Additionally, a web-based questionnaire was emailed and posted on the IOM website to solicit topic nominations.¹²

While PCORI has issued broad solicitations for recommended priorities from the public, it has been unclear to many stakeholders how these recommendations ultimately inform PCORI's funding decisions.¹³ This is due, in part, to the fact that initial rounds of PCORI funding awards relied heavily on a traditional investigator-initiated process within five very broad priority areas:

- 1.Assessment of prevention, diagnosis, and treatment options;
- 2.Improving healthcare systems;
- 3.Communication and dissemination;
- 4.Addressing disparities; and
- 5.Accelerating patient-centered and methodological research.

In order to better meet its patient-centered mandate, PCORI should refine its research prioritization process by engaging patients and providers at the outset to inform PCORI on research areas of highest priority, clearly describing how they will serve as starting points for PCORI in defining its priorities for research funding.

2. PCORI staff should perform the initial evaluation and culling of suggested research topics.
The initial, vetted lists compiled by PCORI staff should be prepared with accompanying rationale and topic briefs.

Because there will likely be a large number of responses, an initial evaluation and vetting of suggested research topics will need to be performed. This initial review should focus on eliminating duplicative topics, and also exclude submissions that are either incomplete or clearly outside of PCORI's mandate (e.g., proposals that would study cost-effectiveness). In creating their list of national CER priorities, the 2009 IOM Committee on Comparative Effectiveness Research Priorities did a screening of the initial submissions to remove duplicated entries and incomplete responses. Additionally, this review should ensure proposals are

¹² Initial National Priorities for Comparative Effectiveness Research, Report Brief, Institute of Medicine, June 2009. Available at <<http://www.iom.edu/~media/Files/Report%20Files/2009/ComparativeEffectivenessResearchPriorities/CER%20report%20brief%202008-13-09.pdf>>

¹³ PCORI: Suggest a Patient-Centered Research Question. Available at <<http://www.pcori.org/get-involved/suggest-a-patient-centered-research-question-survey/>>

weighed against the statutorily defined criteria, “taking into account factors of disease incidence, prevalence, and burden, 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, the relevance to patients and clinicians in making informed health care decisions.”¹⁴

After the vetted list is developed, PCORI should release the suggested research topics included on the list, as well as those excluded with an explanation that helps stakeholders to refine future suggestions and ensure they are relevant to PCORI. For those topics included on the initial list, PCORI will need to develop “topic briefs” containing relevant summarizing evidence related to disease prevalence, burden, and care variation, as well as the history and current status of the topic or question. The importance of the question should be clearly described and evidenced with information regarding the patient population, intervention, comparator(s) and outcomes. Issue briefs should offer comprehensive information that enables panelists to make informed decisions as they rank specific topics.

Providing topic briefs on the suggested research topics for prioritization also recognizes that evidence relevant to other statutory criteria (e.g., “relevance to patients and clinicians”)¹⁵ will not be readily available and will require input of patients and clinical experts. As described by the 2009 IOM Committee on Comparative Effectiveness Research Prioritization:

“The process should allow sufficient time to develop robust, consistent topic briefs for use in voting in order to ‘level the playing field.’ The process should provide background information on the condition, address current practice and policy, and document existing research in order to allow explicit consideration of each topic against pre-specified prioritization criteria.”¹⁶

These briefs will play a very important role in informing consideration and prioritization of topics by PCORI, advisory panels, and stakeholders, and thus should be released to the public.

3. Ranking of topic list by expert Advisory Panels.

¹⁴ Patient Protection and Affordable Care Act of 2010, §6301 amending 2 U.S.C. 1301 et seq. at §1181(d)(1)(A). Available at <http://dpc.senate.gov/dpcissue-sen_health_care_bill.cfm>

¹⁵ *id*

¹⁶ Initial National Priorities for Comparative Effectiveness Research. Committee on Comparative Effectiveness Research Prioritization, Board on Health Care Services, Institute of Medicine: National Academies Press, (2009) at 94.

After an initial list and topic briefs are developed by PCORI staff, the topics should be reviewed and ranked by expert advisory panels. To inform the panel's identification of the research framework/questions, PCORI would provide issue briefs containing relevant background research, landscape and evidence gap analyses, and other materials.

As outlined in the Affordable Care Act, expert advisory panels should be appointed on a permanent or ad hoc basis. Generally, these panels will be responsible for identifying and establishing the research project agenda within a specific topic area. Advisory panels should consist of experts identified/appointed by the relevant clinical, patient and researcher methodology communities. This is consistent with the composition of the panels as described in the statute, which states that they should include, "representatives of practicing and research clinicians, patients, and experts in scientific and health services research, health services delivery, and evidence-based medicine who have experience in the relevant topic, and as appropriate, experts in integrative health and primary prevention strategies." Importantly, the statute also notes that technical experts of each manufacturer or medical technology that is part of a research topic be included in the appropriate advisory panel.¹⁷ Having a wide range of input from across the health-care spectrum will enable thoughtful, well-balanced and informed decision-making.

While each advisory panel will have its own charter, the panels on randomized clinical trials and rare diseases are distinct in their purpose and design, as outlined in the PCORI statute. The panel on randomized clinical trials is expected to serve more as a resource when designing trials for specific disease questions (in terms of the research protocol, subgroups, and data analysis), and also may respond to technical questions as they arise.

One of the challenges PCORI will face in the creation of advisory panels is ensuring they fulfill their purpose of eliciting the input of patients, clinical experts and practicing physicians with knowledge and expertise in various diseases and medical specialties. This will be particularly important in the ranking of research priorities for comparative clinical effectiveness research related to disease prevention, diagnosis and treatment, though it will also be valuable for research related care management, organization and delivery.

PCORI may need to consider either creating standing advisory panels representing particular medical specialties (e.g., oncology or cardiology) or creating high-level advisory panels that are structured to draw on the input of leading experts in specific disease areas.

¹⁷ Patient Protection and Affordable Care Act of 2010, §6301 amending 2 U.S.C. 1301 et seq. at §1181(d)(4). Available at <http://dpc.senate.gov/dpcissue-sen_health_bill.cfm>

Each of the expert panels will have a different focus and, as a result, it will be important for PCORI to appoint panelists who offer the necessary expertise to evaluate and consider research topics. While the inclusion of clinicians is unquestionably crucial to this effort, the full range of stakeholders, who each contribute a unique and important perspective, must be included. The IOM's EBM Roundtable in 2007 provided clarification that no sector can, acting alone, bring about the changes necessary to improve outcomes and value in health care.¹⁸ Bringing together the patient voice and clinical experts is an especially important opportunity presented by the expert advisory panel structure.

4. Each Advisory Panel should further develop a recommended priority list of topics.

Each advisory panel should develop a list of research priorities that will, after PCORI staff review and public comment, be submitted to the PCORI Board of Governors for approval. Utilizing their relevant clinical expertise, advisory panel members should prioritize the research topics based on the criteria for prioritization defined in statute. The weighting of topics could potentially be supported by numerical ranking developed via a Delphi process and should represent a broad range of conditions, interventions and populations.

A CER priority-setting effort undertaken in 2011 by the Friends of Cancer Research (FOCR) modeled an approach that was informed and guided by clinical experts and physicians. FOCR held a forum in 2011 to focus on CER and discuss current and future priority areas on oncology that operated in a similar way to that which was envisioned for the advisory panels described in the PCORI statute. Prior to the forum, advocacy societies and professional organizations were asked to gather input and submit summary proposals of priority oncology CER projects. Leaders in the cancer community then discussed and deliberated on potential topics with the Comparative Effectiveness Research Advisory Committee at the forum, resulting in the identification of 13 priority studies within the field of oncology that they recommended be addressed. Because there were representatives from many different stakeholder groups involved in the proceedings, the chosen priorities represent comprehensive and well-vetted selections.¹⁹

5. Review of Advisory Panel priority lists by PCORI staff, to be submitted for public comment.

¹⁸ Olsen, LeighAnne; Aisner, Dara; and McGinnis, J. Michael, editors, *The Learning Healthcare System: Workshop Summary* (IOM Roundtable on Evidence-Based Medicine), Roundtable on Evidence-Based Medicine; ISBN: 0-309-10687-7, 374 pages, 6 x 9, (2007).

¹⁹ Expanding Comparative Effectiveness Research: Priority Areas. Friends of Cancer Research (2011). Available at <<http://www.focr.org/comparative-effectiveness-research-and-pcori>>

After the priority lists are ranked by Advisory Panels, they should undergo further review and refinement by PCORI staff. The purpose of this review would be to develop a final list of priorities that considers rankings received by various advisory panels and assemble a master list of draft priorities that is balanced across the broad areas of inquiry defined in statute for PCORI. This process should result in a unified list of specific draft priorities that is balanced across PCORI's research mandate and includes the issue brief, panel rankings, and PCORI rationale. As stewards of a public investment, these steps would demonstrate that PCORI is open and transparent about its decision-making process. Once finalized, PCORI then would make this initial proposed priority list available for public comment.

As noted earlier, engaging the public in this priority-setting process is essential for ensuring that the selected topic areas reflect the real-world perspectives of patients, caregivers, and other public stakeholders. Furthermore, their input gives important oversight and clarity on the process and the finalized priorities. A 2009 white paper from the Brookings Institution notes that, "public meetings can promote transparency by making available the discussion and collection of information used by the priority-setting body. Public participation can also help ensure the fairness of the proceedings, by offering opportunity for diverse, conflicting points of view to be expressed and incorporated into committee deliberations."²⁰

6. Revision of draft priority list and presentation to PCORI Board for approval.

After receiving public input, PCORI staff should review and revise the list of priority research questions before submitting it to the PCORI Board of Governors for consideration. Board approval should result in a list of specific research topics that comprise national priorities and include the rationale for prioritization of each topic based on statutory criteria and expert clinical and public input. To the extent feasible, PCORI should seek to ensure balance across the broad range of research described in statute and in the Institute's definition of patient-centered outcomes research, including highly prevalent diseases with high burden, health disparities, and rare diseases with a high burden of unmet need and a smaller number of patients.

7. Provide openness and transparency throughout the priority-setting process.

PCORI must also be open and transparent throughout the research prioritization process. After releasing these draft priority areas, PCORI held a 53-day public comment period during which

²⁰ Garber, Alan M., MD, PhD and Meltzer, David O., MD, PhD, Setting Priorities for Comparative Effectiveness Research: The Brookings Institution, June, 2009, at 26. Available at <http://www.brookings.edu/~media/research/files/papers/2009/6/09%20cer%20mclellan/0609_health_care_cer.pdf>

time they received more than 450 comments. These comments were received through multiple channels, including via the PCORI website, e-mail, regular postal mail, and during their first National Patient and Stakeholder Dialogue, which was a full-day event that provided for structured discussion regarding the proposed draft priority areas. PCORI made all public comments available on its website and publicly provided their own analysis and key findings of those comments.²¹ Additionally, PCORI held a Board of Governors meeting during which the Board reviewed the comments (identifying 15 major themes) and decided not to recommend changes to the actual research priorities, stating that, “the public comments did not identify significant gaps in the five proposed priorities.” This meeting was publicly broadcast as a teleconference and webinar.²²

Moving forward, PCORI should strive to ensure the research prioritization process continues to be accessible and open to public input. As PCORI receives recommended research topics via a solicitation for input, the Institute should publicly post them on its website, including PCORI's analysis. This would be consistent with the approach that PCORI took in its initial solicitation for public comment on the proposed National Priorities for Research and Research Agenda.

It is important for PCORI to encourage input through a variety of channels in order to engage a broad range of stakeholders and ensure input is received from all relevant parties. As noted in a 2009 white paper from Biomed Central Health Services Research:

“To ensure adequate engagement, identifying and engaging stakeholders should involve multiple techniques, such as round tables, open forums, departmental meetings. There should be a genuine commitment from the organization to engage stakeholders effectively through partnership and empowerment. Stakeholder engagement is also concerned with stakeholder satisfaction regarding the level of their involvement in the decision-making process.”²³

Therefore, it is important that topic submissions and comments be made publicly available so that the process can proceed with transparency and public oversight, including PCORI's response to the submissions. A 1992 IOM report emphasized the importance of establishing a clear and open process, stating that, “[p]ublic agencies need a comprehensive, proactive

²¹ National Priorities and Research Agenda, PCORI (2012). Available at <<http://www.pcori.org/research-we-support/priorities-agenda/>>

²² Special Board of Governors Teleconference/Webinar, PCORI, April 25, 2012. Available at <<http://www.pcori.org/events/special-board-of-governors-teleconferencewebinar-3/?type=past>>

²³ Sibbald SL, Singer PA, Upshur R, Martin DK. Priority setting: what constitutes success? A conceptual framework for successful priority setting. *BMC Health Services Research* 9:43 (2009). Available at <<http://www.biomedcentral.com/1472-6963/9/43>>

process of public input to ensure that the technology assessment provides the greatest gain to the health of the public. In addition, priority setting must be accountable to the public. It cannot be private, implicit, or internal to the organization, and it must include a process that is open, fair, and credible to discriminate among the array of possible technologies that it might assess or reassess.”²⁴

As described above, key points for ensuring openness and transparency include:

- PCORI should continue to solicit topics from the public
- PCORI should provide opportunities for public input through a variety of channels.
- Topic submissions should be publicly available
- PCORI comment and analysis of submitted comments should be made public
- Open advisory panel meetings and/or proceedings of meetings made publicly available
- Make available a unified list of proposed priorities following advisory panel input

Conclusion

In order to change the culture of research and meet its mandate to conduct patient-centered comparative clinical effectiveness research, PCORI must develop a targeted, national research agenda through an open and transparent process. To help PCORI achieve this goal, PIPC is developing a priority-setting “road map” that:

- Calls on PCORI to establish a targeted research agenda based on a broad and structured solicitation of topics from patients and providers;
- Directs PCORI staff to evaluate and distill the suggested research topics to ensure research topics meet PCORI’s mandate of patient-centeredness and the statutory criteria for research;
- Develops a rationale and topic brief for research topics to provide both the PCORI board and the public with a clear and transparent understanding of PCORI’s research agenda;
- Utilizes the relevant clinical expertise (both within and beyond PCORI’s advisory panels) to help rank the topic list;
- Ensures the opportunity for public comment on the draft priority list and research agenda;
- Provides for input and approval by Board of Governors; and
- Promotes transparency of the priority-setting process in its entirety.

²⁴ Donaldson MS, Sox HC, eds. Setting Priorities for Health Technology Assessment: A Model Process. Washington, DC: National Academy Press (1992).

PCORI was implemented to answer specific research questions that will have the greatest impact on informing patient and provider health care decisions and improving health outcomes. To do so, PCORI “must drive the burgeoning discipline of comparative effectiveness research forward, starting with a research project agenda that conveys a sense of urgency and strategic direction.”²⁵ With a national list of specific research priorities developed from the expertise of patients and providers, PCORI will be able to conduct patient-centered comparative clinical effectiveness research that is of priority to patients and providers.

PIPC intends to continue its support for PCORI in establishing a defined, patient-centered research agenda. As PCORI shifts toward a more targeted research agenda, the best practices outlined herein are intended to be a road map to help PCORI identify more specific research topics. PIPC is committed to supporting a long-term program for patient-centered outcomes research that focuses on the needs and priorities of patients and providers. In addition to presenting PCORI with this “road map,” PIPC will continue to support PCORI’s efforts through our series of roundtables designed to solicit input directly from patients and providers on issues related to CER, including research prioritization and dissemination. PIPC looks forward to continuing its work with PCORI as they implement an effective priority-setting process that best supports the needs of patients and their providers.

²⁵ Sox, Hal, MD. The Patient-Centered Outcomes Research Institute Should Focus On High-Impact Problems That Can Be Solved Quickly, *Health Affairs*, October 2012; Vol. 31, No. 10:2176-2182.