PIPC Partnership to Improve Patient Care

Priority-Setting for

Comparative Effectiveness Research:

A Review of Key Elements

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Introduction

Over the last two years, legislation has provided an influx of new funds to support the generation of more comparative effectiveness research (CER), as well as the infrastructure to sustain this research. Most recently, the Affordable Care Act of 2010 (ACA) established the Patient Centered Outcomes Research Institute (PCORI), a nonprofit corporation charged with identifying research priorities, developing a research project agenda, and executing research to better inform patients and physicians on their treatment choices.

In addition to PCORI, the American Recovery and Re-investment Act (ARRA) of 2009 allotted a one-time $1.1 billion investment to the Agency for Healthcare Research and Quality (AHRQ), the National Institutes of Health (NIH) and the Office of the Secretary of Health and Human Services to promote CER. ARRA funding has led to over 400 grants and contracts focused on evidence development, evidence translation and dissemination, and establishment of CER infrastructure and methodologies. Finally, with the current level of federal attention and funding for CER, many established and new organizations are realigning their research programs to focus on conducting CER in the future.

The process that these federal agencies and private-sector organizations use to set research priorities is central to the benefit that patients, physicians, and others will receive from Congress's investment in CER.

This paper describes key elements, identified by researchers and others, for a priority-setting process that the public understands and accepts. It also explores the presence of these elements in both government and private CER organizations within the United States.

The consideration of a priority-setting process for CER is not a new or unfamiliar concept. Numerous academics have reviewed and discussed concepts related to priority setting, and domestic and international organizations have tried to implement formal processes as part of their health technology assessment and CER programs. In addition, the issue has become increasingly important to stakeholder groups, particularly those representing patients and providers. One factor leading to this was the process led by the Institute of Medicine (IOM) to recommend research priorities for ARRA CER funds, which directly engaged patients and providers in the process. Through a review of relevant literature and stakeholder comments, as well as an examination of existing priority-setting processes, we highlight five key elements for all CER organizations to consider.

1. Transparency and openness
2. Broad stakeholder participation
3. A range of input opportunities, particularly from patients and consumers
4. Clinical input and guidance throughout the process
5. Systematic input by decision-makers of information received

For each of these elements, we provide examples of how existing organizations have approached priority setting or legislative proposals regarding the selection of research topics by CER organizations. Accordingly, this work warrants careful consideration by policymakers, stakeholders, and the leadership of agencies or bodies responsible for the oversight and initiation of increased investments in CER.

1. The process must be transparent and open.

A key element for priority setting is the need for transparency and openness throughout the process. The absence of this element may limit the value of each of the subsequent elements.

If openness and transparency are not present, a priority-setting process viewed by decision-makers as fair and objective may be perceived very differently by patients and providers and by the public at large. This important point was noted in the IOM's 1992 report, "Setting Priorities for Health Technologies Assessment: A Model Process," which noted among its guiding principles that any priority-setting process "must be—and must appear to be—objective, open, and fair." Further, the IOM observed that the process "must present the logic of the process clearly and carefully to others."[1] Likewise, in their proposed "diamond model" for priority setting, Jennifer Gibson and colleagues stated that the process should include "certain procedural safeguards," including transparency, disclosure of conflicts of interest, and fair access to decision-makers. Further, their transdisciplinary model for priority setting offered that institutions "put into place processes to ensure the *transparency* of the decision-making activities and to ensure that the rationales are widely available."[2]

Norman Daniels and colleagues have offered that a fair priority-setting process "will involve transparency about the grounds for decisions; appeals to rationales that all can accept as relevant to meeting health needs fairly;

and procedures for revising decisions in light of challenges to them."[3] When combined, the authors suggest that these components create "accountability for reasonableness," which allows for the education of stakeholders on the material included in deliberations and aligns decision-making "to broader, more fundamental democratic deliberative processes."[4] Finally, Shannon Sibbald and colleagues suggest that transparency may contribute to the overall success of a program through positive media coverage; peer emulation; or changes in policy, legislation, or practice.[5]

Similar sentiments regarding transparency were manifest in the 2009 testimony before the IOM Committee on Comparative Effectiveness Research Priorities. Specifically, the testimony of the American Nurses Association recommended that any "CER program must be developed with objectivity, transparency and accountability."[6] America's Health Insurance Plans offered, "if patients and clinicians are to support the findings of comparative effectiveness research, it will be essential that the priority setting work, research study designs and subsequent

Future Transparency in the PCORI Priority-Setting Process

ACA stipulates that the PCORI Board of Governors set research priorities and make them available to the public for a 45 to 60 day comment window. The legislation also requires that PCORI create additional opportunities for feedback on priorities and other activities, specifically through "forums to increase public awareness and obtain and incorporate public input and feedback through media (such as an Internet website)."[7] Finally, the Board must submit a report to Congress and the President each year, made available to the public, that details the Institute's research priorities and activities.

recommendations based on research results are open, transparent and take into consideration patient preferences that will impact adherence and compliance."[8] These observations, along with the literature discussed above, suggest that transparency and openness are necessary to foster public and stakeholder trust, and may therefore be a key determinant of a CER program's ultimate impact.

2. The process must include broad stakeholder participation.

Another element of an acceptable priority-setting process is the inclusion of all interested and affected stakeholders. In its 1992 report, the IOM offered among its guiding principles that the priority-setting process must be sensitive to political context, which included a requirement for the process to "invite input from a variety of interested parties."[9] The need for stakeholder participation was further highlighted in the report's specific recommendation that the Agency for Health Care Policy and Research (AHCPR—now known as AHRQ) "should involve a broad spectrum of interested parties."[10] Similarly, in a recent paper describing a conceptual framework for priority setting in health technology assessments, Sibbald and colleagues offer stakeholder engagement as a central characteristic. Further, they note the need for "a genuine commitment" by organizations in engaging stakeholders through partnership and empowerment, as well as the connection between engagement and stakeholder satisfaction with their level of involvement in the priority-setting process.[11] Finally, in their review of the current approaches to priority setting across the globe, Hussein Noorani and colleagues observed that a majority (7 out of 12) of priority-setting frameworks used a panel or committee as part of their prioritization process.[12]

AHRQ has taken steps to implement the recommendation for broad stakeholder participation. For example, it has established a stakeholder advisory group that includes representatives from patients, providers,

manufacturers and private payers. Although this group does not hold public meetings, it does provide a forum for stakeholder views to be expressed. In addition, using funds provided under ARRA, AHRQ is setting up a community forum as a mechanism to engage members of the public in discussions related to CER.[13]

The notion that broad stakeholder participation is an essential component of any priority-setting process was reinforced by public comments during ARRA implementation. In testimony provided to the IOM Committee on Comparative Effectiveness Research Priorities, the National Health Council noted that ARRA "offer[ed] few safeguards to ensure a patient-focus or the inclusion of patients as stakeholders in the governance and decision-making process."[14] As a reflection of the lack of safeguards, the National Alliance on Mental Illness specifically called for a process "where all stakeholders have equal voice in governance and input into research priorities."[15]

PCORI's main mechanism for stakeholder participation is through its oversight and/or advisory bodies, only one of which, the Board of Governors, deals explicitly with priority setting. The Government Accountability Office announced the appointments to the 21-member board in September 2010. The Board of Governors includes consumers and patients, physicians and providers, private payers, industry, researchers and government employees. ACA also provides other mechanisms for stakeholder representation, specifying that PCORI "may appoint permanent or ad hoc expert advisory panels as determined appropriate to assist in identifying research priorities."[16] Literature emphasizing the importance of stakeholder engagement as well as the concerns that arose during ARRA implementation suggests that a more robust engagement mechanism than the Board of Governors may be necessary.

3. The process should allow for a range of input opportunities, particularly from patients and consumers.

Under ARRA, the use of public forums and listening sessions offered by the IOM and the temporary Federal Coordinating Council for CER provided an initial means to incorporate the perspectives of stakeholders. As discussed above, PCORI has a 45 to 60 day comment period for the public to respond to research priorities, and also requires the Institute to provide additional input opportunities (e.g., through a Website). However, this approach still might not ensure meaningful and comprehensive input. Coulter and Ham suggest that the need for an unbiased and systematic approach for encouraging patient and public participation in the priority-setting process is "a crucial step toward reducing the democratic deficit in health-care decision making."[17] In addition, they note that true public involvement in the priority-setting process may lead to an emphasis on different topics. For instance, the public may champion the evaluation of existing technologies over new technologies, as the appropriate use of existing technologies may have a more direct impact on patient care.

Sibbald and colleagues recommend that decision-makers adopt multiple techniques to solicit stakeholder feedback, including roundtables, open forums, and administrative meetings.[18] Patient and consumer groups have also noted the importance of multiple opportunities for stakeholder input, chiefly among patients and clinicians. In testimony submitted to the IOM Committee on Comparative Effectiveness Research Priorities, the Consumers Union called for the inclusion of consumers at decision-making levels and for the IOM to make "the process accessible to the public at critical points" by providing opportunities for public input into the process.[19] The National Working Group on Evidence-Based Health Care issued a policy paper in August 2008 that called for patient and consumer engagement in defining a CER

research agenda through the creation of a separate patient/consumer advisory body whose purpose would be to advise the organization throughout the research process. Further, the group provided examples of avenues for public comment, including Web-based solicitation forms, surveys, and public meetings, while noting that proper engagement was central to "generating information that patients/consumers care about."[20] Given these perspectives, PCORI and other CER organizations should strive to implement and ensure meaningful opportunities for public guidance in priority setting.

Example of Input Opportunities for Patients and Other Stakeholders in the Priority-Setting Process: CANCERGEN

Created with ARRA funding, the Center for Comparative Effectiveness Research in Cancer Genomics' (CANCERGEN's) goal is to facilitate CER that evaluates the use of existing and emerging genomic tests against the standard of care. The group has specifically noted that priority setting in cancer as well as personalized medicine has been overly focused on the views of a limited group of experts in the past. CANCERGEN claims a "more balanced approach— one that better meets the needs of those who manage, are served by, and have a stake in the technologies that are moving from discovery to practice."[21] Their plan is to create an External Stakeholders Advisory Group that includes insurers, test developers, clinicians with a range of expertise, researchers, industry, and patient and consumer groups. This diverse advisory group will meet four times a year to determine areas in cancer and genomics where we are most in need of new evidence.

4. Clinical input and guidance must be obtained throughout the process.

Clinical expertise is vital to the priority-setting process, as it enhances the relevance of studies for practicing physicians who must make critical clinical decisions. In addition, the public often views clinicians as trusted advisors, and their inclusion may lead to greater acceptance of the process and advance the communication of results to the masses. This sentiment was acknowledged in a 1992 Dutch Committee on Choice in Health Care report, which noted, "in setting priorities in health care, authentic social values must be combined with professional and expert opinion as to what is meaningful and meaningless medical treatment."[22,23]

Research by Douw and Vondeling found that organizations responsible for health technology assessments often use clinical experts "for filtering or for making the final decision on which health technologies to assess."[24] In many cases, organizations had created links to medical associations, with whom they contracted experts. Often the review committees included clinicians from relevant clinical specialties in addition to other stakeholders.

In 2009, testimony submitted to the IOM Committee on Comparative Effectiveness Research Priorities highlighted a preference for the inclusion of clinicians in the priority-setting process. Specifically, the American Society of Clinical Oncology recommended the "involvement of oncology experts in setting cancer-related priorities" as the committee worked to shape national priorities for CER.[25] Amy Abernethy, a professor at the Duke University Medical Center, recommended that the committee prioritize a "model of comparative effectiveness research that conserves clinical trials resources to important, vetted clinical questions," and "ensure that true comparative effectiveness research includes implementation of lessons learned at the point of care."[26] Consistent with all of these recommendations, 37 percent of the ap-

Examples of Clinical Input and Guidance in the Priority-Setting Process

The goal of the Centers for Medicare & Medicaid Services (CMS) Evidentiary Priorities Initiative is to provide the research community with research topics on clinical practice for which there are significant knowledge gaps. In October 2007, the agency initiated the process by convening a panel at the Medicare Evidence Development and Coverage Advisory Committee (MEDCAC). The discussion at that meeting, which included experts from NIH, AHRQ, and the Centers for Disease Control and Prevention, led to an initial list of priorities. Additional input from experts in cardiovascular disease, diabetes, mental health, aging, cancer, and other fields allowed CMS to further refine the evidentiary priority list. In April 2008, CMS convened a second meeting of the MEDCAC to review and score the list. The updated list is available on CMS' Website.[27]

pointed positions on the PCORI Board of Governors represent physicians and providers, and well over half of the appointees have some clinical experience.

5. Decision-makers must incorporate the input they receive in a systematic manner.

As decision-makers work to finalize priorities for CER, it is important that they use clear procedures in their determination process, notably the use of explicit and transparent criteria that reflect societal values. Further, a systematic priority-setting process should include a rationale for the priorities selected, based on these criteria.

Sibbald and colleagues believe information management

> **Example of a systematic means for the priority-setting process**
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> The IOM's 1992 Report, "Setting Priorities for Health Technologies Assessment: A Model Process," offered a logical stepwise priority-setting process. The report offered suggested prioritization criteria, including prevalence, cost, variation, and burden of illness. The report also recommended the assignment of a weight to each criterion to reflect their relative importance, acknowledging that different organizations may choose distinct criteria and assign differing weights.[28]
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> *Overview of IOM priority-setting process*
> 1. Select priority-setting criteria and assign a weight to each.
> 2. Solicit nominations of candidates for technology assessment.
> 3. Reduce a large list of nominees to those on which to obtain the data set needed for priority ranking.
> 4. Obtain data for priority ranking.
> 5. For each topic, assign a score for each attribute.
> 6. Calculate priority score, and rank topics in order of priority.
> 7. Perform authoritative review of list.

is an important component in the priority-setting process and that "priority setting decisions should be based on reasons that are grounded in clear value choices." This includes the information, both used and unused, made available to decision-makers. Information management also refers to the way information is gathered and assembled, and how values and context are considered. The authors also believe that the measurement of the quality of a priority-setting process could come via the appropriate use of evidence, consistency of reasoning, and acceptance of the priority-setting process by the organization.[29]

In its 1992 report, the IOM offered that the priority-setting process for any agency "should be explicit, so that people can trace backwards from results to inputs and so satisfy themselves that the process was fair." In addition, the IOM proposed seven steps for the priority-setting process that provide a clear framework for an organization's use of the input received through the prioritization process.

As PCORI and other agency leadership moves forward in the priority-setting process, previous efforts and reviews signify the need for a systematic approach to the selection of research topics that offers a clear rationale for the inclusion or exclusion of topics.

Conclusion

Expanded federal investment in comparative effectiveness research obligates decision-makers to implement a priority-setting process that allows for the appropriate selection of research topics and areas of focus. The five elements highlighted in this paper provide the foundation of a process that accomplishes the following goals while engendering broad public acceptance and support.

1. Transparency and openness
2. Broad stakeholder participation
3. A range of input opportunities, particularly from patients and consumers
4. Clinical input and guidance throughout the process
5. Systematic input by decision-makers of information received

As the new PCORI begins its work, we encourage key decision-makers to apply mechanisms to the priority-setting process that reflect these key elements, as a process that includes such mechanisms is likely to lead to more useful outcomes and a greater benefit to society.

Endnotes

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