



PIPC

Partnership to Improve Patient Care

A Procedural Framework for the Conduct of Comparative Clinical Effectiveness Research

Executive Summary

The *Patient Protection and Affordable Care Act (PPACA)* represents a significant and qualitative shift in the way decisions will be made with respect to comparative effectiveness research. For the first time, stakeholder representatives—including patients, consumers, and care-givers—will be decision-makers, instead of advisors, in a federal program to prioritize, fund, and disseminate the results of Comparative Effectiveness Research (CER). To achieve this goal, PPACA establishes a framework for conducting comparative effectiveness reviews that includes several key elements:

- **Stakeholder Involvement**— The Patient Centered Outcomes Research Institute (PCORI) is in essence a test of a new model for the conduct of federally-supported, patient-centered CER by engaging a broad range of stakeholders, including patients and providers throughout the research decision-making process.
- **Transparency**—a requirement that the Institute’s operations and procedures are made clear to the public, and that policy documents are made available in a timely way;
- **Public Participation**—a requirement that public is given an opportunity to attend meetings, comment on policy documents, procedures, and reports; and
- **Open Decision-Making**—a requirement that the public is notified of meetings of the Institute Board of Governors, that meetings are open, and that decisions on comparative effectiveness research priorities are made in full view.

In some areas, these requirements are set forth explicitly in law. In other areas, these requirements are identified in statute but not fully defined, and will need to be addressed in the course of the new law’s implementation. In both cases, it is expected that putting this framework into operation will be influenced by procedures and approaches that have been developed for federal comparative effectiveness research (CER) efforts currently underway, funded prior to the enactment of the *PPACA*.

This PIPC white paper inventories and discusses the basic elements of the framework for CER that are identified in *PPACA* and compares these requirements to current procedures developed and used by the Agency for Healthcare Research and Quality (AHRQ) to conduct CER through its Effective Health Care Program¹ and through the funding provided by the *American Recovery and Reinvestment Act*. Finally, the paper identifies issues related to these elements to monitor as implementation of PCORI gets underway in the coming months.

The following table highlights the key comparisons between PCORI, the CER funds provided by ARRA, and the AHRQ Effective Health Care Program and identifies some of the issues related to PCORI implementation discussed throughout the white paper.

¹ This program was authorized by Section 1013 of the *Medicare Modernization Act of 2003*.

PCORI	ARRA CER SPENDING	AHRQ EFFECTIVE HEALTH CARE PROGRAM	IMPLEMENTATION ISSUES TO MONITOR
STAKEHOLDER PARTICIPATION			
<p>PCORI Board of Governors is responsible for managing the research process, funding specific projects, and releasing research results. Composed of Composition of 21 appointed members: AHRQ Director and NIH Director s, 3 patient reps / consumers; 7 physician / provider reps; 3 payer reps; 3 industry reps; 1 rep for quality improvement or health service researchers; 2 federal government / state reps.</p> <p>Expert Advisory Panels will assist in identifying research priorities, establishing the research project agenda, and for other purposes.</p> <p>PCORI must establish Advisory Panels on clinical trials and rare disease, and may establish other panels as needed.</p> <p>Methodology Committee will develop standards for research studies.</p>	<p>Federal Coordinating Council for CER established by law, limited membership to 15 federal officials. Serves in an advisory capacity to the Secretary of HHS.</p>	<p>A Stakeholder Group, not required by law, serves as a advisory panel. AHRQ is flexible regarding the number and composition of members and provides general selection criteria in public notices soliciting nominations.</p> <p>AHRQ provides only general selection criteria in public notices soliciting nominations.</p> <p>AHRQ is currently combining the management of the new Stakeholder Group (which will begin in the fall of 2010) with an initiative to increase consumer input into CER operations and processes. AHRQ has solicited proposals to establish and support a Community Forum on Effective Health Care to formally engage stakeholders.</p>	<p>PCORI: The ability of individual stakeholders to work as an independent body to define and achieve common goals consistent with the statutory mandate will be crucial to the Institute's success.</p> <p>The selection of PCORI staff, development of by-laws and the appointment of a CER Methodology Committee by GAO.</p> <p>The mix of expertise and stakeholder perspectives on the Advisory Panels appointed by PCORI.</p> <p>How PCORI implements statutory requirement to provide support for patient & consumer reps on the Board.</p>

TRANSPARENCY

PCORI Board of Governors is required to meet in public with the exception of discussions related to personnel for the Institute. The board is also required to make all of its bylaws, processes and proceedings available on a public website.

Federal Coordinating Council working meetings were not open to the public.

Stakeholder Group meets in private at call of AHRQ.

PCORI will make a number of important decisions on how transparent its activities will be to the public:

- appropriate lead time in providing notice of upcoming meetings of the PCORI Board of Governors and;
- opportunity for public input at meetings.

PCORI will decide the extent of transparency in meetings of the methodology committee and its Advisory Committees. Will the same level of transparency that applies to the Board meetings apply to these other meetings?

Will the transparency, public participation, and conflict of interest requirements set out in PPACA be extended to the public and private entities that conduct CER on its behalf (i.e. its contractors)?

PUBLIC PARTICIPATION

Legislation requires public 45-60 day comment periods before finalizing research methods, priorities, peer review processes, and draft findings and that the comments be made publically available. PCORI is also required to increase public awareness and obtain and incorporate public input and feedback.

Federal coordinating council held 3 listening sessions. Comments received were posted on a website. Public comment on specific projects funded by ARRA was not required.

AHRQ provides four-week public comment periods on drafted research questions and key findings of reports.

AHRQ recently began publishing dispositions of comments related to its systematic reviews.

PCORI requirements will open new doors for public participation. However, the mandates set forth in the health reform legislation are general and must be addressed by the Board of Governors soon after its formation. For example:

- Length of comment periods (between 45 & 60 days);
- Extend public comment to areas not specified in statute (i.e. contractors' processes in undertaking the research); and
- What type of peer review process to chose?

DECISION MAKING

PCORI makes decisions about research by a majority vote on national priorities, research project agenda, methodological standards, and peer review process.

Decision making processes about priorities and projects were not made in public.

Decision making processes about priorities and projects are not made public.

PCORI will set precedents on topics such as how to set research priorities, communicate differences among patient subgroups, and assessing individual patient impact.

It will be important to closely monitor the research standards developed by the Methodology Committee.

The law does not address the decision-making process for standing and *ad hoc* committees of the Board of Governors.

The Board will also have to address how it will handle situations where its members disagree on important matters.