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Patient-Centered Outcomes Research Act and Its Impact on Health Services Research

Introduced by Senator Max Baucus (D-MT) and Senator Kent Conrad (D-ND) June 9, the Patient-Centered Outcomes Research Act would establish a nonprofit corporation to conduct comparative clinical effectiveness research and comparative effectiveness research. This new Patient-Centered Outcomes Research Institute, which is neither an agency nor establishment of the United States government, would “assist patients, clinicians, purchasers, and policymakers 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 evidence synthesis that considers variations in patient subpopulations.”

The new Institute would be governed by a board of public and private stakeholders, including “one member representing nonprofit organizations involved in health services research” and “one member representing independent health services researchers.” It would be financed through American Recovery and Reinvestment Act funding in its first year, through annual appropriations in the next three fiscal years, and in subsequent years, through an assessment on the Medicare Trust Fund and health insurance plans (e.g., \$1 per beneficiary).

The following analysis summarizes provisions that would directly impact the field of health services research and that relate to the Coalition for Health Services Research’s policy priorities to build the research infrastructure, improve research coordination, and enhance access to information.

Scope of Work

Whether or not to include the cost of treatments in the analysis of comparative effectiveness is a debate that continues in Washington. Proponents of cost’s inclusion argue that cost should be one factor that various payers could consider and that therefore this research should facilitate consideration of comparative costs. Opponents—including industry and patient groups—argue that cost should not be a factor in judging whether a treatment (drug, device, medical or surgical procedure) is clinically effective. They also assert that the methodologies used in cost effectiveness

research (especially Quality Adjusted Life Years -- QALYs) will ultimately lead to rationing of care.

The Patient-Centered Outcomes Research Act does not specifically address the cost issue, and instead defines this research in two distinct ways. Comparative clinical effectiveness research is defined as “research evaluating and comparing the clinical effectiveness, risks, and benefits of two or more medical treatments, services and items,” including “health care interventions, protocols for treatment, care management and delivery, procedures, medical devices, diagnostic tools, pharmaceuticals (including drugs and biologicals), and any other strategies or items being used in the treatment, management, and diagnosis of , or prevention of, illness or injury in patients.”

Comparative effectiveness research is more generally defined as “research evaluating and comparing the implications and outcomes of two or more health care strategies to address a particular medical condition for specific patient populations.”

The bill provides for the possibility of conducting comparative cost effectiveness research by examining, through a Methodology Committee, “methods by which efficiency and value (including the full range of harms and benefits, such as quality of life) could be assessed in a scientifically valid and standardized way” (see below for description of Methodology Committee).

Research Infrastructure and Coordination

The Patient-Centered Research Outcomes Act includes several provisions to strengthen the research infrastructure.

Methodology Committee

The bill would direct the Institute to establish a Methodology Committee of 17 “experts in their scientific field, such as health services research, clinical research, comparative effectiveness research, biostatistics, genomics, and research methodologies.” Appointed by the Comptroller General of the United States, the Methodology Committee would “develop and improve the science and methods of comparative effectiveness research.” Specifically, the committee would:

- Establish and maintain methodological standards for comparative clinical effectiveness research. Such standards “shall provide specific criteria for internal validity, generalizability, feasibility, and timeliness of such research and for clinical outcomes measures, risk adjustment, and other relevant aspects of research and assessment with respect to the design of such research.”
- Develop a “translation table” to provide guidance and act as a reference for the Institute’s Board of Governors to “determine research methods that are most likely to address each specific comparative clinical effectiveness question.”

- Examine methods to assess the relative effectiveness, benefits, risks, advantages and disadvantages of “various aspects of the health care delivery system (such as benefit design and performance, and health services organization, management, information communication, and delivery).”
- Examine methods to scientifically assess the “efficiency and value” of health care services.

The Methodology Committee would have the authority to “consult or contract” with entities including the IOM, AHRQ, NIH, and “academic, nonprofit, or other private entities with relevant expertise.” The committee could also consult with stakeholders and other entities knowledgeable in relevant fields, as appropriate.

Capacity Building

The bill directs the Institute to build capacity for comparative clinical effectiveness research and methodologies, including research training and development of data sources (e.g., clinical registries). The Institute may use up to 20 percent of its total budget in a fiscal year to “fund extramural efforts of organizations such as the Cochrane Collaboration (or a successor organization) and other organizations that develop and maintain a data network to collect, link, and analyze data on outcomes and effectiveness from multiple sources, including electronic health records.”

Coordination

The Coalition for Health Services Research has long advocated for better coordination of health services research to facilitate planning and accountability across myriad public and private sponsors of health services research. Consistent with recommendations from AcademyHealth’s study, *A First Look at the Cost and Volume of Comparative Effectiveness Research in the United States*, the Patient-Centered Outcomes Research Act charges the Institute with coordinating the “research conducted, commissioned, or otherwise funded by the Institute with comparative clinical effectiveness research and other relevant research and related efforts conducted by public and private agencies and organizations in order to ensure the most efficient use of the Institute’s resources and that research is not duplicated unnecessarily.”

To further facilitate coordination between existing efforts and those of the new Institute, the bill would also amend the American Recovery and Reinvestment Act to include the Chair of the Institute’s Board of Governors as a member of the Federal Coordinating Council for Comparative Effectiveness Research, and would direct the Council to coordinate its activities with the new Institute. The American Recovery and Reinvestment Act does not explicitly state when the Coordinating Council’s authority would expire, and so it is unclear how the Coordinating Council and the new Institute would collaborate going forward, if at all.

Funding Mechanism

Federal entities use various funding mechanisms to support research, which affects the types of research conducted and the degree of autonomy researchers are afforded in conducting their studies. For example, when a grant is awarded, no substantial federal involvement with recipients is anticipated during the performance of a research activity. Conversely, contracts are awarded when a funder's purpose is to acquire goods or services for the direct benefit or use of the funder. In these circumstances, the funder has substantial and direct involvement with the contractor throughout the life of the project.

The Patient-Centered Outcomes Research Act directs the Institute to conduct a study on the feasibility of conducting intramural research. In the meantime, the bill authorizes the Institute to enter into contracts "for the management and conduct of research." Contractors could include federal agencies including AHRQ and "appropriate private sector research or study-conducting entities that have demonstrated the experience and capacity to achieve the goals of comparative effectiveness research." The bill does not explicitly provide the Institute grant making authority.

AcademyHealth's study, *Historical Analysis of Ownership & Publication Rights in Government Contracts for Health Services Research*, found that the role of government contracts in funding research has increased exponentially; at the same time, the clauses in these contracts have become increasingly more restrictive potentially blocking the dissemination of important research results produced under a federal contract.

The Coalition recommends providing the Institute grant making authority to facilitate innovation and competition in the research marketplace, and urges the Institute to not place prior restraint on the publication of research findings developed under federal grants and contracts.

Transparency

The bill requires that the new Institute establish procedures to ensure "transparency, credibility, and access." Among these provisions is the requirement for a public comment period of not less than 45 days and not more than 60 days on the following:

- The national research priorities identified by the Institute and its Board and advisory panel(s);
- The methodological standards developed and updated by the Methodology Committee;
- The peer-review process;

- Dissemination protocols and strategies developed by the Institute; and
- Study designs and findings.

Public comments received during comment periods on study design and findings will be transmitted to the entities conducting research for consideration and incorporation.

The Institute is also required to hold public forums to increase awareness and obtain and incorporate public input and feedback on research priorities, findings, and other activities of the Institute, as appropriate.

The Institute will make available to the public through its Web site:

- The identity of the entity and the researchers conducting research , including any links the entity or the researchers have to industry;
- Draft and final study designs, including research questions, and any public comments on the study design and responses to comments; and
- Research protocols, including methods of research and analysis, research results.

In evaluating the funding, placement, and organization of health services research in the federal government, AcademyHealth adopted five principles to guide decisions related to the placement of comparative effectiveness research. Among these principles, AcademyHealth recommended that the entity conducting comparative effectiveness research should involve key stakeholders to assure transparency of the methods and process, promote public acceptance of research findings, and support for the entities mission. Specifically, AcademyHealth recommended that stakeholders be involved in developing the research agenda and ensuring the validity of the research produced.

As written, the Patient-Centered Outcomes Research Act provides for unprecedented levels of stakeholder involvement in the formal governance structure of the Institute, and in the opportunities for formal and informal public comments on nearly all aspects of the research continuum. However, it will be important that these multiple opportunities for stakeholder input—and the associated time required to solicit, review, and incorporate feedback—do not adversely impact the timeliness and thereby the relevance of the comparative effectiveness research ultimately produced.