

## **Summary of Section 6301 (provision on Patient Centered Outcomes Research) of the Patient Protection and Affordable Care Act**

### **Summary**

Sec. 6301 of the Patient Protection and Affordable Care Act establishes a program for government-supported comparative clinical effectiveness research to be operated by a new Patient-Centered Outcomes Research Institute. The Institute will be governed by a Board comprised of a broad range of stakeholders, including pharmaceutical manufacturers. Board members must be appointed within six months of enactment by the Government Accountability Office (GAO). In addition to funding research, the Institute's duties include establishing research priorities, releasing research results, establishing a Methodology Committee and other advisory panels, providing for a peer-review process, and making public information on research and operations. Research must account for patient differences, and findings can not include guidelines or policy recommendations. The section defines requirements for openness and transparency in the Institute's operations, and requires annual reports from the Institute and oversight by the GAO.

### **Comparative Clinical Effectiveness Research**

Comparative clinical effectiveness research (CER) is defined as research evaluating and comparing the clinical effectiveness, risks, and benefits of two or more medical treatments or services. Services are defined as 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.

### **Patient-Centered Outcomes Research Institute**

The legislation establishes a nonprofit corporation called the Patient-Centered Outcomes Research Institute. The Institute will be funded through a Patient-Centered Outcomes Research Trust Fund. The purpose of the Institute is, "to assist patients, clinicians, purchasers, and policy makers in making informed health decisions by advancing the quality of evidence concerning how health conditions can best be prevented, diagnosed, treated, monitored, and managed through research and 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 medical items and services."

### **Board of Governors**

The Institute is to be governed by a Board of Governors consisting of the Director of AHRQ, the Director of NIH, and 21 members representing various public and private sector interests, including 3 manufacturer (drug, device, and diagnostic) representatives, to be appointed within six months by the Comptroller General of GAO for six year terms. The Board may hire an Executive Director and staff. Board meetings are open to the public.

### **Administration**

The Board of Governors shall carry out the duties of the Institute. Priority setting and adoption of the research project agenda, methodological standards, and peer review processes cannot be delegated.

## **Identifying Research Priorities and Establishing a Research Project Agenda**

The Institute will identify research priorities based on disease incidence, evidence gaps, practice variations and health disparities, potential for quality improvement, the effect on health expenditures associated with a health condition or the use of a particular treatment, patient needs, outcomes and preferences, and the relevance to assist in decision making, and the priorities established by the National Strategy for Quality Care. The Institute will establish a research project agenda to address the priorities identified by the Institute, and conduct a value of information analysis in determining the types of research that might address each priority.

## **Carrying Out the Research Agenda**

The Institute will use systematic reviews of existing research and evidence, and conduct primary research (clinical trials, observational studies), including “molecularly informed trials,” in carrying out the research agenda.

The Institute will contract with private sector researchers and government agencies to conduct the research. The Institute is directed to give preference to AHRQ and NIH when awarding contracts. Any contractor must abide by the section’s requirements for transparency and conflicts of interest, comply with the Institute’s methodological standards, consult with expert advisory panels established under the law, and comply with other requirements including those for making research information public.

The Institute must review and update evidence on a periodic basis.

Differences in patients and treatment modalities: Research must be designed to account for potential differences in effectiveness of interventions in different subpopulations, including those involving racial and ethnic minorities, women, genetic and molecular sub-types, and quality-of-life preferences. Research also must be designed to account for different characteristics of treatment modalities, such as phase of treatment in the innovation cycle and the skill of the operator of the treatment modality.

Data collection: The Secretary shall make available to the Institute such data collected by CMS under Medicare and Medicaid, as well as data collected through electronic databases created under the law, as the Institute and its contractors may require such data to carry out the research agenda.

## **Expert Advisory Panels and Patient Support**

The Institute may appoint permanent or ad hoc advisory panels to assist in setting research priorities and the research agenda “and for other purposes.” The Institute shall appoint expert advisory panels for carrying out randomized clinical trials to inform study designs, and appoint an expert advisory panel for rare diseases to assist in the design of research.

Advisory panels will include patient and clinician representatives and may also include experts in scientific and health services research, health services delivery, and evidence-based medicine. Advisory panel may include a representative of each manufacturer of each medical technology that is included under the relevant research topic.

In addition, the Institute “shall provide support and resources to help patients and consumer representatives effectively participate on the Board and expert advisory panels.”

### **Ensuring Transparency, Credibility, and Access**

The Institute is required to establish public comment periods of 45-60 days prior to adoption of research priorities, the research project agenda, methodological standards, peer-review process, and after release of draft findings of systematic reviews. Comments received on research must be transmitted to the entity conducting research. The Institute will also periodically host public forums to increase public awareness and better incorporate public feedback.

The Institute is also required to make publicly available the process and methods for conducting research, the identity of the entity conducting the research and any links to industry, study designs, research protocols, and progress reports on its official website. It also must provide public notice of comment periods and make public the comments received, as well as the proceedings of the Institute.

### **Methodology Committee**

The Institute shall establish a methodology committee with 15 members appointed by GAO, plus AHRQ and NIH representatives. Within 18 months of establishment of the Institute, the methodology committee shall establish methods standards and a translation table to help align research methods and study questions. Standards shall include methods for incorporating changes in evidence and medical technology into ongoing research, and accounting for patient subpopulations.

The methodology committee may contract with the Institute of Medicine or other entities to carry out its duties.

### **Peer Review Process**

The Institute shall provide for a peer-review process to ensure scientific integrity and adherence to the methodological standards established by the committee. Names of peer reviewers shall be made public on an annual basis. Existing peer-review processes may be utilized where appropriate.

### **Release of Research Findings**

The Institute must make research findings available to patients, clinicians and the general public. Research findings:

- Must be comprehensible and useful to patients and providers
- Must fully convey findings and discuss considerations for subpopulations, risk factors and co-morbidities
- Must include limitations of the research and further research needs
- May not include mandates for practice guidelines or policy recommendations
- Must not release information that would violate patient privacy or confidentiality agreements.

### **Annual Reports**

The Institute shall submit an annual report to Congress and the President, and release it to the public. The report shall describe Institute activities, research priorities, methods standards, project agenda and budget, and other information.

### **Financial and Governmental Oversight**

The Institute will provide for annual financial audits by a private entity, to be reviewed by the Comptroller General and submitted to Congress.

The Comptroller General of the United States will review and report on the processes established by the Institute, its overall effectiveness in utilizing relevant research findings and effect on innovation and the economy, and the adequacy of funding.

### **Rules**

The Institute, its Board of Governors, and its staff, are not permitted to accept gifts or outside funding.

### **Rules of Construction**

The Institute is not permitted to mandate coverage, reimbursement, or other policies for any public or private payer. None of the research findings disseminated by the Institute may include mandates, guidelines or recommendations for payment, coverage or treatment.

### **Dissemination and Infrastructure Development**

Twenty percent of PCOR Trust Fund moneys shall be available to AHRQ and HHS for the research dissemination and infrastructure development activities specified in the Act. The Office of Communication and Knowledge Transfer at AHRQ, in consult with NIH, shall “broadly disseminate the research findings” of the Institute and other government funded comparative clinical effectiveness research. The office shall create informational tools that disseminate research findings to patients, providers, payers, and policy makers. The office also shall, in consultation with medical and clinical associations, promote timely incorporation of findings into clinical practices (e.g., via clinical decision support tools). In addition, the office will create a public database of research findings, and shall create a feedback process for providers, patients, HIT vendors and health plans.

Dissemination materials shall “include a description of considerations for specific subpopulations, the research methodology, and the limitations of the research,” as well as the entities conducting the research. The materials shall not include: “practice guidelines, coverage recommendations, or policy recommendations.”

Regarding infrastructure development, the Secretary [of HHS] shall coordinate relevant Federal health programs to build data capacity for comparative clinical effectiveness research, including through clinical registries and health outcomes research data networks.

### **Limitations on use of CER by the Secretary**

The Secretary “may only use evidence and findings from research” conducted under this section to make a Medicare coverage determination if the agency uses “an iterative and transparent process which considers public comment and considers effect on

subpopulations. The Secretary is not authorized to deny coverage solely on the basis of comparative clinical effectiveness research.

The Secretary shall not use CER evidence in determining coverage, reimbursement, or incentive programs under Medicare in a manner that treats extending the life of an elderly, disabled, or terminally ill individual as of lower value than other patients or that discourages patients from making individual trade-offs between length of life and risk of disability. However, these limitations do not limit “the application of differential copayments [under Medicare] based on factors such as cost or type of service” or prevent the Secretary from using CER findings in determining coverage, reimbursement, or incentive programs based on the comparative effectiveness of interventions in extending an individual’s life due to that individual’s age, disability, or terminal illness.

The Institute shall not develop or employ a cost/QALY or similar cost-effectiveness threshold, and the Secretary shall not utilize such a threshold to determine coverage, reimbursement, or incentive programs under Medicare.

### **Patient-Centered Outcomes Research Trust Fund (PCORTF)**

The PCORTF will be funded by general revenues (\$210 million in first 3 years), amounts from the Medicare Trust Funds phased-in to \$2 per beneficiary annually, and amounts from a \$2 fee per-covered-life assessed annually on insured and self-insured health plans. (The latter two revenue sources do not take effect until 2013). When fully funded, annual funding is estimated to total approximately \$600 million.

- General appropriation amounts for 2010: \$10 mil.
- General appropriation amount for 2011: \$50 mil.
- General appropriation amount for 2012: \$150 mil.
- Beginning in fiscal year 2013, the revenues from the health plan tax take effect. Tax revenues are in addition to \$150 mil. appropriation made each year.

No amounts will be available for expenditures by the Institute after 2019, and any amount still in PCORTF at that time will be transferred to the general fund of the Treasury.

### **Coordination with the Federal Coordinating Council for CER (ARRA)**

The Federal Coordinating Council established under the American Recovery and Reinvestment is disbanded. The requirement for the council to submit an annual report to Congress and the President is eliminated.