

Summary of Patient-Centered Outcomes Research Provisions Prepared by AAMC Government Relations, March 2010

Section 6301 of the Patient Protection and Affordable Care Act (as modified by Section 10602) amends Title XI of the Social Security Act to authorize a Patient-Centered Outcomes Research Institute (PCORI) as a nonprofit corporation that is not “an agency or establishment of the U.S. Government.”

The purpose of the Institute 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 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 treatments, services, and items.”

The Institute will have a 21-member Board of Governors, including the Directors of the Agency for Healthcare Research and Quality (AHRQ) and the National Institutes of Health (NIH), and 19 members appointed by the Comptroller General.

Duties of the Institute include identifying research priorities and establishing and carrying out a research project agenda. The Institute shall:

- establish a standing Methodology Committee to develop and periodically update scientifically based methodological standards for research conducted through PCORI;
- ensure peer review – designed to avoid bias and conflicts of interest on the part of reviewers – to assess scientific integrity and adherence to methodological standards adopted;
- provide public comment periods prior to adoption of national priorities, the research project agenda, the methodological standards, and the peer review process, and after the release of draft findings with respect to systematic reviews of existing research and evidence; and
- make research findings publicly available within 90 days.

The Institute is authorized to “enter into contracts to manage funding and conduct research” with Federal agencies or academic and other entities, with preference to AHRQ and NIH, but only if the research under the contract is authorized by their governing statutes. The entity must comply with transparency, conflicts of interest, methodological, and other requirements of the Institute. Researchers under contract may publish findings in a peer-reviewed journal, as long as the researcher enters into a data use agreement with the Institute.

AHRQ, in consultation with NIH, will be responsible for disseminating research findings published by PCORI and for establishing grants to train researchers in research methods.

There are limitations on the Institute’s and the Secretary’s ability to use evidence and findings from research conducted through PCORI for coverage and reimbursement policies.

The Institute will be supported by a newly established Patient-Centered Outcomes Research Trust Fund (PCORTF), funded through a combination of: appropriations; transfers from the Medicare Federal Hospital Insurance and Federal Supplementary Medical Insurance Trust Funds; and transfers from

health insurance and self-insured health plans. No amounts shall be available for expenditure from the PCORTF after September 30, 2019.

The following summarizes these provisions of the Patient Protection and Affordable Care Act in more detail.

Definitions:

The Act defines “comparative clinical effectiveness research” and “research” as “research evaluating and comparing health outcomes and the clinical effectiveness, risks, and benefits of 2 or more medical treatments, services, and items.”

The Act defines “medical treatments, services, and items” as “health care interventions, protocols for treatment, care management, and delivery, procedures, medical devices, diagnostic tools, pharmaceuticals (including drugs and biologicals), integrative health practices, and any other strategies or items being used in the treatment, management, and diagnosis of, or prevention of illness or injury in, individuals.”

Duties of the Institute:

The duties of the Institute include identifying research priorities and establishing and carrying out a research project agenda.

The Institute “shall identify national priorities for research, taking into account disease incidence, prevalence, and burden in the U.S. (with emphasis on chronic conditions), gaps in evidence in clinical outcomes, practice variations and health disparities in 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 decisions, and priorities in the National Strategy for quality care established under section 399H of the Public Health Service Act...”

The Institute “shall establish and update a research project agenda to address the priorities identified..., taking into consideration the types of research that might address each priority and the relative value (based on the cost of conducting research compared to the potential usefulness of the information produced by research) associated with the different types of research, and other factors the Institute determines appropriate.”

The Institute “shall carry out the research project agenda established... in accordance with the methodological standards adopted [by the institute], including the following”:

- Systematic reviews and assessments of existing and future research and evidence including original research conducted subsequent to the date of the enactment;
- Primary research, such as randomized clinical trials, molecularly informed trials, and observational studies; and

- Any other methodologies recommended by the Methodology Committee that are adopted by the Board (see page 6).

Board of Directors:

The Institute will have a 21-member Board of Governors, consisting of the Directors of the Agency for Healthcare Research and Quality (AHRQ) and the National Institutes of Health (NIH) (or their designees) and 19 members, appointed by the Comptroller General of the United States no later than six months after enactment, including:

- three representing patients/health care consumers;
- seven representing physicians and providers, including four representing physicians (at least one who is a surgeon), one nurse, one State-licensed integrative health care practitioner, and one representative of a hospital;
- three representing private payers, with at least one representing health insurance issuers and at least one representing employers who self-insure employee benefits;
- three representing pharmaceutical, device, and diagnostic manufacturers or developers;
- one representing quality improvement or independent health service researchers; and
- two representing the Federal Government or the States, including at least one representing a Federal health program or agency.

The Board “shall represent a broad range of perspectives and collectively have scientific expertise in clinical health sciences research, including epidemiology, decisions sciences, health economics, and statistics.”

Research Contracts:

The Institute is authorized to “enter into contracts to manage funding and conduct research with appropriate Federal agencies; or appropriate academic research, private sector research, or study-conducting entities.”

In awarding research contracts, the Institute shall give preference to AHRQ and NIH, but only if the research to be conducted or managed under the contract is authorized by the governing statutes of AHRQ or NIH.

A contract shall require that the agency or other entity:

- abide by the Institute’s transparency and conflicts of interest requirements;
- comply with the methodological standards adopted by the Institute;

- consult with the expert advisory panels for clinical trials and rare disease appointed by the Institute (see page 6);
- permit a researcher who conducts original research under a contract with the Institute to publish such research in a peer-reviewed journal or other publication as long as the researcher enters into a data use agreement with the Institute for use of the data from the original research as appropriate;
- have appropriate processes in place to manage data privacy and meet ethical standards for the research;
- comply with the requirements of the Institute for making the information available to the public; and
- comply with other terms and conditions determined by the Institute.

Contracts may allow for coverage of copayments or coinsurance, or allow for other appropriate measures, as are necessary to preserve the validity of a research project.

The Institute shall not allow the subsequent use of data from original research in work-for-hire contracts with individuals, entities, or instrumentalities that have a financial interest in the results, unless approved under a data use agreement with the Institute.

The Institute shall review and update evidence on a periodic basis as appropriate.

Research shall be designed, as appropriate, to take into account the potential for differences in the effectiveness of health care treatments, services, and items as used with various subpopulations, such as racial and ethnic minorities, women, age, and groups of individuals with different comorbidities, genetic and molecular sub-types, or quality of life preferences and include members of such subpopulations as subjects in the research as feasible and appropriate.

Research shall be designed, as appropriate, to take into account different characteristics of treatment modalities that may affect research outcomes, such as the phase of the treatment modality in the innovation cycle and the impact of the skill of the operator of the treatment modality.

Data Collection:

The Secretary shall, with appropriate safeguards for privacy, make available to the Institute data collected by the Centers for Medicare and Medicaid Services (CMS) under Medicare, Medicaid, or the Children's Health Insurance Program, as well as provide access to data networks developed under section 937(f) of the Public Health Service Act, as the Institute and its contractors may require. The Institute may also request and obtain data from Federal, State, or private entities, including data from clinical databases and registries.

The Institute shall only use data provided to the Institute in accordance with laws and regulations governing the release and use of such data, including applicable confidentiality and privacy standards.

Financial and Governmental Oversight:

The Institute is required to have annual financial audits by a private entity. The Comptroller General shall review:

- the financial audits (at least annually);
- the processes established by the Institute, including the research priorities and the conduct of research projects, to determine whether the information produced is objective and credible, is produced in a manner consistent with the requirements under this section, and is developed through a transparent process (at least every 5 years);
- the dissemination and training activities and data networks established, including the methods and products used to disseminate research, the types of training conducted and supported, and the types and functions of the data networks established, to determine whether the activities and data are produced in a manner consistent with the requirements under this section (at least every 5 years);
- the overall effectiveness of activities conducted and the dissemination, training, and capacity building activities, including an analysis of the extent to which research findings are used by health care decision-makers, the effect of the dissemination of such findings on reducing practice variation and disparities in health care, and the effect of the research conducted and disseminated on innovation and the health care economy of the United States (at least every 5 years); and
- the adequacy and use of funding for the Institute and activities, including whether, based on the utilization of research findings by public and private payers, funding sources for the Patient-Centered Outcomes Research Trust Fund (see page 11) are appropriate and whether such sources of funding should be continued or adjusted (within 8 years after enactment).

Not later than April 1 of each year, the Comptroller General shall report to Congress the results of the review conducted for the preceding year (or years), with recommendations for legislation and administrative action as appropriate.

Annual Report to Congress and the President:

The Institute shall submit to Congress and the President (and make available to the public) an annual report that contains:

- a description of the activities conducted, research priorities identified, and methodological standards developed and updated by the Methodology Committee during the preceding year;
- the research project agenda and budget of the Institute for the following year;
- any administrative activities conducted by the Institute during the preceding year;
- the names of individuals contributing to any peer-review process, without identifying them with a particular research project; and

- any other relevant information (including information on the membership of the Board, expert advisory panels, Methodology Committee, and the executive staff of the Institute, any conflicts of interest with respect to these individuals, and any bylaws adopted by the Board during the preceding year).

Expert Advisory Panels:

The Institute may appoint permanent or ad hoc expert advisory panels to assist in identifying research priorities and establishing the research project agenda. Panels shall 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. The Institute may include a technical expert of each manufacturer or each medical technology that is included under the relevant topic, project, or category for which the panel is established.

The Institute shall appoint expert advisory panels in carrying out randomized clinical trials. Panels shall advise the Institute and the agency, instrumentality, or entity conducting the research on the research question involved and the research design or protocol, including important patient subgroups and other parameters of the research.

For a research study for rare disease, the Institute shall appoint an expert advisory panel to assist in the design of the study and determine the relative value and feasibility of conducting the study.

The Institute shall provide support and resources to help patient and consumer representatives effectively participate on the Board and expert advisory panels.

Methodology Committee:

The Institute shall establish a standing Methodology Committee composed of not more than 15 members appointed by the Comptroller General. Members shall be experts in scientific fields, such as health services research, clinical research, comparative clinical effectiveness research, biostatistics, genomics, and research methodologies. The Directors of NIH and AHRQ (or their designees) shall be members of the Committee. The Committee may consult and contract with IOM and academic, nonprofit, or other private and governmental entities with relevant expertise and relevant stakeholders.

The Committee shall, within 18 months of establishment of the Institute, directly or through subcontract, develop and periodically update the following:

- Methodological standards for research that provide specific criteria for internal validity, generalizability, feasibility, and timeliness of research and for health outcomes measures, risk adjustment, and other relevant aspects of research and assessment with respect to the design of research. Methodological standards developed and updated shall be scientifically based and include methods by which new information, data, or advances in technology are considered and incorporated into ongoing research projects by the Institute, as appropriate. The process for developing and updating such standards shall include input from relevant experts, stakeholders, and decisionmakers, and shall provide opportunities for public comment. Such standards shall

include methods by which patient subpopulations can be accounted for and evaluated in different types of research. As appropriate, such standards shall build on existing work on methodological standards for defined categories of health interventions and for each of the major categories of comparative clinical effectiveness research methods (determined as of the date of enactment).

- A translation table designed to provide guidance and act as a reference for the Board to determine research methods that are most likely to address each specific research question.

The Committee shall report to the Board recommendations for the Institute to adopt methodological standards developed and updated by the Committee as well as other actions deemed necessary to comply with such methodological standards.

Peer Review:

The Institute shall ensure peer review process of primary research. Evidence from primary research shall be reviewed to assess scientific integrity and adherence to methodological standards adopted. The names of individuals contributing to any peer-review process during the preceding year or years shall be made public and included in annual reports. The peer-review process shall be designed to avoid bias and conflicts of interest on the part of the reviewers and shall be composed of experts in the scientific field relevant to the research under review.

For a contract or other agreement with another entity for the conduct or management of research, the Institute may utilize the peer-review process of that entity if such process meets the above requirements.

The Institute may utilize the peer-review process of appropriate medical journals if such process meets the above requirements.

Ensuring Transparency, Credibility, and Access:

The Institute shall provide a public comment period of between 45 and 60 days prior to adoption of national priorities, the research project agenda, the methodological standards developed and updated by the Methodology Committee, and the peer-review process, and after the release of draft findings with respect to systematic reviews of existing research and evidence.

The Institute shall support forums to increase public awareness and obtain and incorporate public input and feedback through media (such as an Internet website) on research priorities, research findings, and other duties, activities, or processes the Institute determines appropriate.

The Institute shall make available to the public and disclose through the Web site of the Institute:

- information contained in research findings;
- the process and methods for the conduct of research, including the identity of the entity and the investigators conducting such research and any conflicts of interests of such parties, any direct or indirect links the entity has to industry, and research protocols, including measures taken, methods of research and analysis, research results, and other information the Institute determines appropriate concurrent with the release of research findings;

- notice of public comment periods, including deadlines for public comments;
- subsequent comments received during each of the public comment periods; and
- in accordance with applicable laws and processes and as the Institute determines appropriate, proceedings of the Institute.

Conflicts of Interest:

In appointing the Board, the Comptroller General shall consider and disclose any conflicts of interest. Board members shall be recused from relevant Institute activities in the case where the member (or an immediate family member) has a real conflict of interest directly related to the research project or the matter that could affect or be affected by such participation.

A conflict of interest shall be disclosed in the following manner:

- By the Institute in appointing members to an expert advisory panel, in selecting individuals to contribute to any peer-review process, and for employment as executive staff of the Institute;
- By the Comptroller General in appointing members of the Methodology Committee; and
- By the Institute in the annual report, except that, in the case of individuals contributing to any such peer review process, such description shall be in a manner such that those individuals cannot be identified with a particular research project.

Conflicts of interest shall be disclosed as soon as practicable on the web site of the Institute and of the Government Accountability Office. The information disclosed shall include the type, nature, and magnitude of the interests of the individual involved, except to the extent that the individual recuses himself or herself from participating in the consideration of or any other activity with respect to the study as to which the potential conflict exists.

Release of Research Findings:

The Institute shall, not later than 90 days after the conduct or receipt of research findings, make the findings available to clinicians, patients, and the general public. The Institute shall ensure that the research findings:

- convey the findings in a manner comprehensible and useful to patients and providers in making health care decisions;
- fully convey findings and discuss considerations specific to certain subpopulations, risk factors, and comorbidities, as appropriate;
- include limitations of the research and what further research may be needed as appropriate;

- do not include practice guidelines, coverage recommendations, payment, or policy recommendations; and
- do not include any data that would violate the privacy of research participants or any confidentiality agreements made with respect to the use of data under this section.

Dissemination:

The Office of Communication and Knowledge Transfer at AHRQ (or other office designated by AHRQ), in consultation with NIH, shall broadly disseminate research findings published by the Institute and other government-funded research relevant to comparative clinical effectiveness research. The Office shall create informational tools that organize and disseminate research findings for physicians, health care providers, patients, payers, and policy makers. The Office shall develop a publicly available resource database that collects and contains government-funded evidence and research from public, private, not-for profit, and academic sources.

The Office shall disseminate the Institute's research findings and government-funded research relevant to comparative clinical effectiveness research to physicians, health care providers, patients, vendors of health information technology focused on clinical decision support, appropriate professional associations, and Federal and private health plans. Materials, forums, and media used to disseminate the findings, informational tools, and resource databases shall include a description of considerations for specific subpopulations, the research methodology, and the limitations of the research, and the names of the entities, agencies, instrumentalities, and individuals who conducted any research which was published by the Institute; and not be construed as mandates, guidelines, or recommendations for payment, coverage, or treatment.

The Office, in consultation with relevant medical and clinical associations, shall assist users of health information technology focused on clinical decision support to promote the timely incorporation of research findings disseminated into clinical practices and to promote the ease of use of such incorporation.

The Office shall establish a process to receive feedback from physicians, health care providers, patients, and vendors of health information technology focused on clinical decision support, appropriate professional associations, and Federal and private health plans about the value of the information disseminated and the assistance provided.

Research Training and Building Research Capacity:

AHRQ, in consultation with NIH, shall establish a training grant program in the methods used to conduct such research, including systematic reviews of existing research and primary research such as clinical trials.

The Secretary shall coordinate relevant Federal health programs to build data capacity for comparative clinical effectiveness research, including the development and use of clinical registries and health outcomes research data networks, to develop and maintain a comprehensive, interoperable data network to collect, link, and analyze data on outcomes and effectiveness from multiple sources, including electronic health records.

Limitations on Coverage and Reimbursement Policies:

Nothing in this Act shall be construed to permit the Institute to mandate coverage, reimbursement, or other policies for any public or private payer; or as preventing the Secretary from covering the routine costs of clinical care received by an individual entitled to, or enrolled for, benefits under Medicare, Medicaid, or the Children's Health Insurance Program in the case where such individual is participating in a clinical trial and such costs would otherwise be covered with respect to the beneficiary.

The Secretary may only use evidence and findings from research conducted under this legislation to make a coverage determination under Medicare through an iterative and transparent process that includes public comment and considers the effect on subpopulations.

Nothing in this Act shall be construed as superceding or modifying the coverage of items or services under Medicare that the Secretary determines are reasonable and necessary or authorizing the Secretary to deny coverage of items or services solely on the basis of comparative clinical effectiveness research.

The Secretary shall not use evidence or findings from comparative clinical effectiveness research conducted under this legislation 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 extending the life of an individual who is younger, nondisabled, or not terminally ill. This Act shall not be construed as preventing the Secretary from using evidence or findings from comparative clinical effectiveness research in determining coverage, reimbursement, or incentive programs under Medicare based upon a comparison of the difference in the effectiveness of alternative treatments in extending an individual's life due to the individual's age, disability, or terminal illness.

The Secretary shall not use evidence or findings from comparative clinical effectiveness research in determining coverage, reimbursement, or incentive programs under Medicare in a manner that precludes, or with the intent to discourage, an individual from choosing a health care treatment based on how the individual values the tradeoff between extending the length of their life and the risk of disability. This shall not be construed to limit the application of differential copayments under Medicare based on factors such as cost or type of service; or prevent the Secretary from using evidence or findings from such comparative clinical effectiveness research in determining coverage, reimbursement, or incentive programs under such title based upon a comparison of the difference in the effectiveness of alternative health care treatments in extending an individual's life due to that individual's age, disability, or terminal illness.

Nothing in this Act shall be construed to limit comparative clinical effectiveness research or any other research, evaluation, or dissemination of information concerning the likelihood that a health care treatment will result in disability.

The Institute shall not develop or employ a dollars-per-quality adjusted life year (or similar measure that discounts the value of a life because of an individual's disability) as a threshold to establish what type of health care is cost effective or recommended. The Secretary shall not utilize such an adjusted life year (or such a similar measure) as a threshold to determine coverage, reimbursement, or incentive programs under Medicare.

Funding:

The Act establishes a Patient-Centered Outcomes Research Trust Fund (PCORTF) in the U.S. Treasury. For FYs 2010-2012, the PCORTF is funded by appropriations from the general funds in the Treasury in the following amounts:

- For FY 2010 - \$10 million
- For FY 2011 - \$50 million
- For FY 2012 - \$150 million

For FYs 2013-2019, the PCORTF is funded at \$150 million in appropriations plus transfers from the Medicare Federal Hospital Insurance and Federal Supplementary Medical Insurance Trust Funds and from health insurance and self-insured health plans equal to: the average number of individuals enrolled in the plans (Medicare, health insurance policies, and self-insured plans) multiplied by:

- \$1 for FY 2013;
- \$2 for FY 2014; and
- \$2 increased by annual medical inflation for FYs 2015 through 2019.

No amounts shall be available for expenditure from the PCORTF after September 30, 2019, and any amounts in the Trust Fund after that date shall be transferred to the general fund of the Treasury.

Federal Coordinating Council:

Section 6302 of the Act terminates the Federal Coordinating Council for CER (established under the American Recovery and Reinvestment Act) on the date of enactment.