National Pharmaceutical Council

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Comparative Effectiveness Research and Evidence-Based Medicine: An Informational Series from the National Pharmaceutical Council

In early 2009, Congress approved and President Obama signed into law the American Recovery and Reinvestment Act (ARRA), an economic stimulus package that includes $1.1 billion for comparative effectiveness research (CER). By approving those funds, lawmakers made it clear that CER will be an integral part of health care reform. Throughout the health care debate, CER was included in draft legislation and in the final Patient Protection and Affordable Care Act signed into law in 2010.

Although the concept of CER is not new, it is important to establish a clear definition and understanding of why it is so prominent today and the many related issues and initiatives under consideration in Washington.

To facilitate this understanding, the National Pharmaceutical Council has developed a series of informational pieces, which taken together provide an overview of CER. Each of the following items outlines a specific aspect of CER in a short, easy-to-read format.

- Defining Evidence-Based Medicine and Comparative Effectiveness Research
- A Brief History of Comparative Effectiveness Research and Evidence-Based Medicine
- NPC's Key Considerations on Comparative Effectiveness Research
- Comparative Effectiveness Research Provisions in the Patient Protection and Affordable Care Act
- Comparison of Comparative Effectiveness Research Legislative Activities in the Context of NPC's CER Recommendations to IOM, FCCCER and AHRQ
- Additional Resources

We encourage you to share these informational pieces with your colleagues and other organizations interested in the issue, as well as link to the pieces on NPC's website, where we will be providing updates to the materials on an ongoing basis.

For additional copies of this information, please contact NPC at info@npcnow.org or 703-620-6390.

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Defining Evidence-Based Medicine and Comparative Effectiveness Research

Introduction

Evidence-based medicine (EBM) is a systematic approach to clinical problem solving which allows the integration of the best available research evidence with clinical expertise and patient values.[1]

Under this definition, EBM requires clinical expertise and use of the best evidence available, but must also consider patient preferences, optimal patient outcomes, and the relative effects among competing alternatives. Broadly considered, EBM includes the comparative effectiveness assessments of drugs, treatments and devices, and the appropriate interpretation of evidence from these assessments to support health benefit design and medical decision-making.

EBM is the foundation for Comparative Effectiveness Research (CER), which compares available treatment options utilizing a range of research methods including randomized controlled trials, observational studies, and systematic reviews, a structured assessment of evidence available from multiple primary studies.

Another term that is frequently mentioned with CER and EBM is health technology assessment (HTA). HTA is a rigorous process of appraisal that examines the effects and impact of a health care technology or treatment. These assessments inform decision-makers as to the direct and indirect consequences of a given technology or treatment.[2]

Federal Coordinating Council Definition of CER

The Federal Coordinating Council for Comparative Effectiveness Research (FCCCER) was established in 2009 under the American Recovery and Reinvestment Act (ARRA) to "coordinate comparative effectiveness research across the Federal government. The Council was charged with making recommendations for the framework and prioritization of spending for the $400 million allocated to the Office of the Secretary [of Health and Human Services] for CER."[3]

Following a series of public listening sessions regarding how to define CER and prioritize this research, the FCCCER has developed the following definition and criteria, which it outlined in a report submitted to Congress on June 30, 2009:

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[1] Sackett DL, Strauss SE, Richardson WS, *et al.* Evidence-based Medicine: How to Practice and Teach EBM. London: Churchill-Livingstone, 2000.

[2] Buckley, T. The Complexities of Comparative Effectiveness, October 25, 2007. http://bio.org/healthcare/compeffective/20071025.pdf, accessed June 14, 2009.

[3] Comparative Effectiveness Research Funding: Federal Coordinating Council for Comparative Effectiveness Research, http://www.hhs.gov/recovery/programs/cer/index.html, accessed May 26, 2009.

"Comparative effectiveness research is the conduct and synthesis of research comparing the benefits and harms of different interventions and strategies to prevent, diagnose, treat and monitor health conditions in 'real world' settings. The purpose of this research is to improve health outcomes by developing and disseminating evidence-based information to patients, clinicians, and other decision-makers, responding to their expressed needs, about which interventions are most effective for which patients under specific circumstances.

- "To provide this information, comparative effectiveness research must assess a comprehensive array of health-related outcomes for diverse patient populations and subgroups.
- "Defined interventions compared may include medications, procedures, medical and assistive devices and technologies, diagnostic testing, behavioral change, and delivery system strategies.
- "This research necessitates the development, expansion, and use of a variety of data sources and methods to assess comparative effectiveness and actively disseminate the results."[4]

For a research project first to be considered by the FCCCER, it must meet these criteria:

- "Included within statutory limits of Recovery Act and FCC definition of CER
- "Potential to inform decision-making by patients, clinicians, or other stakeholders
- "Responsiveness to expressed needs of patients, clinicians, or other stakeholders
- "Feasibility of research topic (including time necessary for research)

"The prioritization criteria for scientifically meritorious research and investments are:

- "Potential impact (based on prevalence of condition, burden of disease, variability in outcomes, costs, potential for increased patient benefit or decreased harm)
- "Potential to evaluate comparative effectiveness in diverse populations and patient subgroups and engage communities in research
- "Uncertainty within the clinical and public health communities regarding management decisions and variability in practice
- "Addresses need or gap unlikely to be addressed through other organizations
- "Potential for multiplicative effect (e.g. lays foundation for future CER such as data infrastructure and methods development and training, or generates additional investment outside government)"[5]

The FCCCER also has developed a strategic framework for CER activity and investments to categorize current activity, identify gaps, and inform decisions on high priority recommendations. According to the FCCCER, "This framework is intended to support immediate decisions for investment in CER priorities and to provide a comprehensive foundation for longer-term strategic decisions on CER priorities and the related infrastructure. At the framework's core is responsiveness to expressed needs for comparative effectiveness research to inform health care decision-making by patients, clinicians, and others in the

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[4] Federal Coordinating Council on Comparative Effectiveness Research Report to Congress, June 30, 2009, p. 17.

[5] Ibid., page 17.

clinical and public health communities."[6] The activities and investments are grouped into four main categories – research, human and scientific capital, CER data infrastructure, and dissemination and translation of CER.

Institute of Medicine Definition of CER

The Institute of Medicine (IOM), which was tasked under the ARRA with developing a list of national CER priorities, defines CER this way:

"Comparative effectiveness research (CER) is the generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat and monitor a clinical condition or to improve the delivery of care. The purpose of CER is to assist consumers, clinicians, purchasers, and policy makers to make informed decisions that will improve health care at both the individual and population levels."[7]

In addition to describing 100 specific and prioritized CER recommendations for the Department of Health and Human Services' (HHS) portion of the ARRA CER funds, the IOM makes the following general recommendations:

1. Prioritization of CER topics should be a sustained and continuous process, recognizing the dynamic state of disease, interventions, and public concern.

2. Public (including consumers, patients, and caregivers) participation in the priority-setting process is imperative to provide transparency in the process and input to delineating research questions.

3. Consideration of CER topics requires the development of robust, consistent topic briefs providing background information, current practice, and research status of the condition and its interventions.

4. Regular reporting of the activities and recommendations of the prioritizing body is necessary to evaluate the portfolio's distribution, its impact for discovery, and its translation into clinical care in order to provide a process for continuous quality improvement.

5. The HHS Secretary should establish a mechanism—such as a coordinating advisory body—with the mandate to strategize, organize, monitor, evaluate and report on the implementation and impact of the CER Program.

6. The CER Program should fully involve consumers, patients, and caregivers in key aspects of CER, including strategic planning, priority setting, research proposal development, peer review, and dissemination.

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[6] Ibid., p. 25.

[7] Institute of Medicine, Initial National Priorities for Comparative Effectiveness Research, June 2009, p. 1. http://iom.edu/Object.File/Master/71/107/CER%20report%20brief%206%2030%2009.pdf, accessed July 2, 2009.

7. The CER Program should devote sufficient resources to research and innovation in the methods of CER, including the development of methodological guidance for CER study design such as the appropriate use of observational data and more informative, practical, and efficient clinical trials.

8. The CER Program should help to develop large-scale, clinical and administrative data networks to facilitate better use of data and more efficient ways to collect new data to inform CER.

9. The CER Program should develop and support the workforce for CER to ensure the nation's capacity to carry out the CER mission.

10. The CER Program should promote rapid adoption of CER findings and conduct research to identify the most effective strategies for disseminating new and existing CER findings to health care professionals, consumers, patients, and caregivers and for helping them to implement these results in daily clinical practice.

NPC's Comments on the IOM and FCCCER Reports

NPC, as well as other health care stakeholder organizations, had submitted comments to FCCCER and IOM on their draft definitions, criteria and frameworks. Following the release of the FCCCER and IOM reports, NPC said it was pleased that some of its recommendations were incorporated into their definitions and criteria, such as focusing on conditions with the greatest impact on morbidity and cost, including all major therapeutic options, taking into account patient subgroups, and expressing clear support for the development of new CER methodologies.

In addition, however, NPC said that it would continue to monitor and seek clarification in areas that were unclear or not included in the FCCCER and IOM reports, because it is vital for CER funding decisions to be made in the best possible manner and result in information that improves clinical decision making for health care providers and patients. In particular, NPC wants to ensure that CER has a positive impact on incentives for future innovation and that the proposed prioritization of research topics and studies, their associated research time frames, final study outcomes, and related information will be made transparent to all stakeholders and disseminated in a timely manner.

NPC also outlined other key factors in the selection of the highest priority research:

- First, it will be important to conduct research to define rigorous, high quality, and validated CER methodologies that are focused on providing timely, accurate and balanced information in order to assist clinical decision making.
    - These questions include, but are not limited to, defining how best to address the full range of health effects of a new technology including quality of life, functionality, and productivity, as well as how best to appropriately characterize the strengths, weaknesses, and limitations of various underlying health technology assessment analytic techniques.
    - In order to minimize the likelihood for inaccurate or inappropriate interpretation of CER, a transparent and readily accessible description of the strengths, weaknesses,

limitations, and potential for generalizability of the findings of CER utilizing varied experimental and non-experimental research designs should be included.

- Second, the strategic framework should implicitly assume that health care innovations will be considered as an important external input to a flexible CER framework. That is, it should be encompassed within and considered integral to the framework.
- Third, the agenda for CER should be driven by the clinical condition and the "key unanswered questions" in the context of that condition. Answering these questions may require comparisons between different types of technologies, processes, or procedures that may be considered to treat the condition; for example, the framework should reflect the need for comparisons of drug vs. surgery, drug and diagnostic vs. procedure, procedure vs. surgery, or other combinations.
- Fourth, comparisons should also include delivery system architecture options, insurance plan designs, methods for primary/secondary prevention, and approaches to provider incentives to effect improvements in health.

For further information, see "Key Considerations on CER" or the NPC website, www.npcnow.org.

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A Brief History of Comparative Effectiveness Research And Evidence-Based Medicine

Introduction

The concepts of evidence-based medicine (EBM) and comparative effectiveness research (CER) are not new. Since the 1970's, health industry leaders and the federal government have turned to Health Technology Assessment (HTA), EBM, and more recently, CER as a means to improve quality and consistency and maximize value in the health care delivery system. However, these concepts have taken on prominence since the 1990's when legislation created the Agency for Health Care Policy and Research (later renamed the Agency for Healthcare Research and Quality, or AHRQ), to support studies on the outcomes of health care services and procedures.

These efforts have taken different names over the decades:

1970s: Health Technology Assessment
1980s: Effectiveness Research
1990s: Outcomes Research
2000s: Evidence-based Medicine and Comparative Effectiveness Research

An Overview of Early Efforts

Efforts to improve quality and maximize the value of health care services have been undertaken by both governmental and private entities.

Past governmental efforts include:

- The U.S. Congress Office of Technology Assessment[1] – An agency created by Congress in 1972 to provide analysis of new technologies, including healthcare. The agency was abolished in 1995 as part of the 104th Congress' "Contract with America."
- The Institute of Medicine's Council on Health Care Technology – Established in 1986 "to promote the development and application of technology assessment in health care and to review health care technologies for their appropriate use."[2] The organization lost public funding in 1989.
- The Agency for Health Care Policy and Research – Early iteration of AHRQ; focused on developing clinical guidelines.[3]

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[1] U.S. Congress, Office of Technology Assessment, The OTA Legacy: 1972-1995 (Washington, DC: April 1996) http://www.princeton.edu/~ota/, accessed June 15, 2009.

[2] Medical Technology Assessment Directory: A Pilot Reference to Organizations, Assessments, and Information Resources (1988), Institute of Medicine, p. 633. http://books.nap.edu/openbook.php?record_id=1090&page=633, accessed June 12, 2009.

[3] Luce B, Cohen RS, Hunter C, Cragin L, Johnson J. The Current Evidence-Based Medicine Landscape, April 2008, p. 6.

- RxIntelligence – An independent nonprofit corporation founded by BlueCross BlueShield in 2000, RxIntelligence conducted cost-benefit, cost-effectiveness analyses of pharmaceutical drugs and provided "evaluation of therapeutic interchangeability of drugs." The entity lasted only two years.[4]
- Medicare Coverage Policy – In July 2006, the Centers for Medicare and Medicaid Services (CMS) issued a guidance document that allowed the agency to integrate evidence-based decision making and research into its coverage determination policies.[5] This policy is still currently in use by CMS and is informed by the Medicare Evidence Development and Coverage Advisory Committee (MEDCAC)[6], which is a working group designed to supplement CMS' internal expertise.

Few of these efforts took hold, mostly because they lost political support due to their perceived threat to innovation, medical autonomy, and market access.[7]

Private efforts include:

- Cochrane Collaboration – Founded in 1993, this global nonprofit network is dedicated to evaluating health care interventions through systematic reviews. The major product of the Collaboration is the Cochrane Database of Systematic Reviews, which is published quarterly as part of The Cochrane Library.[8]
- Blue Cross/Blue Shield Technology Evaluation Center – Established in 1995, this entity reviews interventions and evidence to determine effectiveness and guide clinical decision-making.
- Center for Medical Technology Policy (CMTP) – CMTP was created in 2006 to generate reliable and credible information about the real world risks, benefits and costs of promising new medical technologies. Initial funding was provided by the California Healthcare Foundation and Blue Shield of California Foundation, with ongoing funding from organizations including the National Pharmaceutical Council.[9]
- Institute for Clinical and Economic Review (ICER): This organization was created by a grant from the Blue Shield of California Foundation in 2006, and produces appraisals of clinical effectiveness and cost effectiveness of medical innovations, with the goal of providing new information to decision-makers intent on improving the value of health care services. Ongoing funding is provided by a group of organizations, including the National Pharmaceutical Council.[10]
- ECRI Institute (formerly the Emergency Care Research Institute) - ECRI Institute is a nonprofit agency and is a Collaborating Center of the World Health Organization (WHO) and an Evidence-

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[4] Luce B, Cohen RS, Hunter C, A Critical Analysis of the 2008 National Landscape of Evidence-Based Medicine and Comparative Effectiveness Policies, April 2008, p.4.

[5] Ibid, p. 24.

[6] Centers for Medicare and Medicaid Services, Medicare Evidence Development and Coverage Advisory Committee. http://www.cms.hhs.gov/FACA/02_MEDCAC.asp, accessed June 15, 2009.

[7] Bryan R. Luce, PhD, MBA, United BioSource Corporation, Presentation to the National Pharmaceutical Council, April 2008.

[8] The Cochrane Collaboration – About the Cochrane Collaboration, http://www.cochrane.org/docs/descrip.htm, accessed June 12, 2009.

[9] The Center for Medical Technology Policy – About Us, http://www.cmtpnet.org/about-cmtp, accessed June 5, 2009.

[10] Institute for Clinical and Economic Review, http://www.icer-review.org/index.php/support/index.html, accessed June 5, 2009.

based Practice Center (EPC) for AHRQ. ECRI evaluates safety, quality, and cost-effectiveness in health care. It offers more than 10 databases, publications, information services, and technical assistance services.[11]

- Hayes, Inc. – This is an independent organization that specializes in health technology assessment reports for health care organizations, including health plans, managed care companies, hospitals, and health networks. Hayes' medical research analysts assess such technologies as medical and surgical procedures, drugs, biologics, diagnostic and screening tests, medical devices and equipment, and complementary and alternative therapies.[12]
- Oregon Drug Effectiveness Review Project – Established in 2003, this project "produces systematic, evidence-based reviews of the comparative effectiveness and safety of drugs in many widely used drug classes, and applies the findings to inform public policy and related activities in local settings."[13]
- AMCP Format for Formulary Submissions–Established by the Academy of Managed Care Pharmacy, this is a set of guidelines for submitting new and existing pharmaceuticals for a health system's Pharmacy and Therapeutics Committee. The form requires detailed information, not only on the drug's safety and efficacy, but also on its overall clinical and economic value relative to alternative therapies. [14]

It is believed that these private sector activities have succeeded largely because they have been perceived as useful by the market in clinical decision making, purchasing, coverage and formulary placement, and cost containment. For the most part, these initiatives have been insulated from political influence, thus improving their longer term viability.[15]

Growing Interest in CER in Recent Years

The federal government's interest in CER has been accelerating over the past few years, with the creation of new and expansion of several existing initiatives.

In 2003, the Medicare Modernization Act (MMA) ensured funding for CER through AHRQ. Today AHRQ's authority has expanded to generate new knowledge, which it does through a network of research centers and private-public partnerships. In 2005, AHRQ launched its Effective Health Care Program, which has three core mandates:

- To review and synthesize existing knowledge through Evidence-based Practice Centers (EPCs)
- To promote and generate new knowledge through the DEcIDE (Developing Evidence to Inform Decisions about Effectiveness) Research Network
- To compile the findings from the EPCs and DEcIDE Network and then translate that knowledge for consumers, physicians, payers and policy makers.

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[11] Luce B, Cohen RS, Hunter C, Cragin L, Johnson J. The Current Evidence-Based Medicine Landscape, April 2008, p. 47.

[12] Ibid, p. 48.

[13] Oregon Health & Science University – Center for Evidence-based Policy Drug Effectiveness Review Project, http://www.ohsu.edu/ohsuedu/research/policycenter/DERP/index.cfm, accessed June 11, 2009.

[14] Luce B, Cohen RS, Hunter C, Cragin L, Johnson J. The Current Evidence-Based Medicine Landscape, April 2008, p. 48.

[15] Bryan R. Luce, PhD, MBA, United BioSource Corporation, Presentation to the National Pharmaceutical Council, April 2008.

The program is meant to focus on effectiveness, as in the evidence of the relative benefits and risks of alternative interventions; be a transparent and open process; determine usability and real-world applicability; and drive research forward.[16] Since 2005, AHRQ has published more than 150 reports on various interventions and treatments.[17]

In addition to MMA, the Institute of Medicine's Roundtable on Evidence-Based Medicine has engaged major stakeholders in an effort to "help transform the way evidence on clinical effectiveness is generated and used to improve health and health care." Through workshops and publications, the IOM hopes to engage health care stakeholders and "identify key issues that are not being adequately addressed, the nature of the barriers and possible solutions, and the priorities for action" in order to achieve its stated goal for 90 percent of all healthcare decisions to be patient-specific and based on the best available evidence by 2020.[18]

Other ongoing government-supported programs include:

- U.S. Preventive Services Task Force (USPSTF) – Established in 1984, and sponsored by AHRQ since 1998, USPSTF is an independent panel of private-sector experts in prevention and primary care, and conducts assessment of various health services.[19]
- Department of Veterans Affairs and Department of Defense – Both of these entities use data from their patient populations to assess the effectiveness of various interventions and make coverage decisions based on findings. The Department of Veterans Affairs' program is called the Technology Assessment Program (VATAP)[20] and the Department of Defense manages these efforts through TRICARE Management Activity,[21] the Department of Defense agency responsible for administering the health benefits of military beneficiaries.

Under the Obama administration, momentum for the advancement of CER continues to grow. In January 2009, as part of the economic stimulus law known as the American Recovery and Reinvestment Act (ARRA), Congress set aside $1.1 billion in funding for CER. Under ARRA, the funding was distributed among the U.S. Department of Health and Human Services, AHRQ, and National Institutes of Health and must be obligated by September 30, 2010. Additionally, in its FY 2011 Federal Budget, the Obama

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[16] Agency for Healthcare Research and Quality, Effective Health Care – The Program, http://effectivehealthcare.ahrq.gov/aboutUs.cfm?abouttype=program, accessed June 14, 2009.

[17] U.S. Department of Health and Human Services, Agency for Healthcare Research and Quality, FY 2008 Annual Performance Report,http://www.ahrq.gov/about/cj2009/AHRQ%20FY2008%20Annual%20Performance%20Report.pdf, accessed June 5, 2009.

[18] Institute of Medicine Roundtable on Evidence-Based Medicine – Charter and Vision Statement, http://www.iom.edu/CMS/28312/RT-EBM/55066/55223.aspx, accessed June 11, 2009.

[19] Luce B, Cohen RS, Hunter C, Cragin L, Johnson J. The Current Evidence-Based Medicine Landscape, April 2008, p. 9.

[20] U.S. Department of Veterans Affairs, VA Technology Assessment Program. http://www.va.gov/VATAP/index.htm, accessed June 15, 2009.

[21] Jacobsen G, CRS Report for Congress: Comparative Clinical Effectiveness and Cost-Effectiveness Research: Background, History, and Overview, October 15, 2007, p. 28. http://assets.opencrs.com/rpts/RL34208_20071015.pdf, accessed June 15, 2009; and Agency for Healthcare Research and Quality – ECRI. http://www.ahrq.gov/clinic/epc/ecriepc.htm, accessed June 15, 2009.

administration has proposed $286 million for CER under AHRQ. The budget must now undergo consideration by Congress.

In 2010, President Obama signed into law the "Patient Protection and Affordable Care Act," a major health care reform bill that would establish a new CER entity called the Patient-Centered Outcomes Research Institute (PCORI). PCORI would be a public-private partnership outside of any agency or government structure.

For a summary of more recent developments related to CER and EBM, please see "Comparative Effectiveness Research Provisions in the Patient Protection and Affordable Care Act."

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NPC's Key Considerations On Comparative Effectiveness Research

The goal of comparative effectiveness research (CER) should be to support the dialogue between health care providers and patients, thus enhancing the quality of patient care.

To ensure the successful implementation of CER, policy makers should consider the following issues:

1. Provide evidence that will encourage and facilitate good decision-making by health care professionals and patients, recognizing and supporting the physician and patient as the center of the decision-making process.
2. Encompass all healthcare services, including devices, diagnostics, health care delivery methods, pharmaceuticals and medical and surgical procedures, and establish priorities for research in an explicit and transparent manner.
3. CER should be rigorous and transparent, and conducted in accordance with a clear set of methods guidelines.
4. Improve the quality of patient care with focus on clinical effectiveness over simply reducing treatment costs; research should be conducted in the context of health care quality improvement above all else.
5. Appropriately consider the needs of patient subgroups who may respond differently to medicines and treatments based on age, genetic variation and co-morbidities.
6. Encourage an all-inclusive approach that allows for multiple organizations to provide input and generate and evaluate evidence in a fully transparent manner.
7. Utilize a full range of types and sources of evidence that consider both direct and indirect benefits to society, such as quality of life, patient functionality and economic productivity.
8. Be current and allow for amendment when new data emerges.
9. Ensure balanced, effective and timely communication of results to consumers, patients, physicians and health care professionals, including any limitations to findings.
10. A publicly funded CER entity must be perceived as a credible and trusted organization and in order to help ensure that, it is best organized as a public-private partnership outside of any agency or government structure.
11. A national CER effort should remain focused on clinical comparative effectiveness. Value and cost-effectiveness should be considered only after clinical outcomes are assessed and determinations of comparative value may best be considered on a regional or local level where health care decision makers can more accurately incorporate variations in health technology acquisition costs.

In 2009, NPC presented these considerations before the Institute of Medicine's CER Priority Setting Committee, the Agency for Healthcare Research and Quality and the Federal Coordinating Council for Comparative Effectiveness Research. It is critical for these entities to establish appropriate guiding principles that will be used by decision makers in determining CER priorities.

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Comparative Effectiveness Research Provisions in the Patient Protection and Affordable Care Act

On March 23, 2010, President Obama signed into law the "Patient Protection and Affordable Care Act," a major health care reform bill that would establish a new comparative clinical effectiveness research (CER) entity. The CER entity would be a "nonprofit corporation, to be known as the 'Patient-Centered Outcomes Research Institute' which is neither an agency nor establishment of the United States Government." The Institute would generate scientific evidence and new information on how diseases, disorders and other health conditions can be treated to achieve the best clinical outcome for patients.

It would give preference to contracts with federal agencies, such as the Agency for Healthcare Research and Quality (AHRQ) or the National Institutes of Health (NIH), but may also contract with appropriate private entities to conduct the research, which would include both systematic reviews of existing research and primary research. The Institute and its activities would be funded by contributions from both public and private payers, made available to the Institute through a Patient-Centered Outcomes Research Trust Fund.

The Institute and its activities would be governed by an independent, 19-member Board of Governors that includes the director of AHRQ and director of the National Institutes of Health, with the remaining members appointed by the U.S. Comptroller General. Among these appointed members, there are "three members representing pharmaceutical, device, and diagnostic manufacturers or developers."

Other key provisions in the law include the following:

- It would establish a standing methodology committee, a permanent or ad-hoc expert panel for clinical trials, and an expert advisory panel for rare diseases. Within the expert advisory panels, "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 Office of Communication and Knowledge Transfer in AHRQ, in consultation with the NIH, would be in charge of disseminating CER findings to appropriate audiences including physicians, health care providers, vendors of health information technology focused on clinical decision support, patients, payers (federal and private plans), and policy makers.
- It would require a report at least every five years on 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."

The legislation also specified how the Patient-Centered Outcomes Research Institute findings can—or cannot—be used.

In particular:

- The Institute may not mandate coverage, reimbursement, or policy recommendations;
- The Secretary of the Department of Health and Human Services (HHS) is prohibited from denying coverage based *solely* on research by the Institute;
- The Secretary cannot use the Institute's research in a way that treats extending the life of elderly, disabled, or terminally ill patients as of lower value than a person who is younger, non-disabled or not terminally ill; and
- The Institute is prevented from developing or using "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."

Ongoing CER Activities

In 2009, Congress made a major investment in ensuring high-quality, patient-centered health care by allocating $1.1 billion for CER as part of the ARRA. Of that $1.1 billion for CER, AHRQ received $700 million; of that $700 million, $400 million was transferred to the Office of the Director of the NIH to support CER as well as a variety of other research projects. The remaining $400 million was allocated to HHS to be disbursed at the discretion of the Secretary. In addition, the Federal budget for Fiscal Year 2011 includes $286 million for CER under AHRQ.

Within ARRA, Congress created a committee known as the Federal Coordinating Council for Comparative Effectiveness Research (FCCCER) that was composed of 15 senior federal employees who are in leadership roles in government organizations that impact health care. At least half are physicians or other experts with clinical expertise. The task of the committee was to coordinate CER efforts across government agencies, and to make recommendations on CER spending priorities to Congress. The Council submitted its initial report to Congress on June 30, 2009. Funds for CER within ARRA must be obligated by September 30, 2010, and the law states a preference for "quick start" projects that can be initiated within 120 days. Every six months, HHS, AHRQ and NIH must submit a report to Congress detailing how funds have been spent. A recent report from HHS in February 2010 indicated that $198 million of the $1.1 billion for CER as part of ARRA have been allocated.

Under the health care reform law, the FCCCER would be terminated on the date of enactment.

National Pharmaceutical Council

Comparison of Various Comparative Effectiveness Research Legislative Activities in the Context of NPC's CER Recommendations to IOM, FCCCER, and AHRQ

#	NPC Comparative Effectiveness Research Recommendation	Senate Patient Protection and Affordable Care Act	House Affordable Health Care for America Act	ARRA
1	Focus on conditions with high burden of illness and cost such as chronic diseases and provide evidence that will facilitate good decision-making by health care professionals and patients	✓	✓	Silent
2	Encompass all healthcare services, including devices, diagnostics, healthcare delivery methods, pharmaceuticals and medical and surgical procedures, and establish priorities for research in an explicit and transparent manner	✓	✓	✓
3	CER methods should be rigorous and transparent, and conducted in accordance with a clear set of methods guidelines	✓	✓	Silent
4	Study and monitor how best to employ CER in a manner that preserves incentives for continuous innovation of healthcare technologies in areas of unmet need	✓	Silent	Silent
5	Consider the needs of patient subgroups who may respond differently to medicines and treatments based on age, genetic variation and co-morbidities	✓	✓	✓
6	Encourage fully transparent stakeholder involvement that allows for multiple organizations (including industry)to participate in both governance and evidence generation/assessment	✓	Precludes	Precludes
7	Utilize a full range of types and sources of evidence that consider both direct and indirect benefits to society, such as quality of life, patient functionality and economic productivity	✓	✓	✓
8	Be current and allow for amendment when new data emerges	✓	✓	Silent

Legend: Silent= Not mentioned within the Bill Precludes= Not allowed within the Bill

Updated: 3.23.10

#	NPC Comparative Effectiveness Research Recommendation	Senate Patient Protection and Affordable Care Act	House Affordable Health Care for America Act	ARRA
9	Ensure balanced, effective and timely communication of results to consumers, patients, physicians and health care professionals, including any limitations to findings	✓	✓	✓
10	A publicly funded CER entity must be perceived as a credible and trusted organization and in order to help ensure that, it is best organized as a public-private partnership outside of any agency or government structure	✓	Precludes	Precludes
11	A national CER should remain focused on clinical comparative effectiveness. Value and cost-effectiveness should be considered only after clinical outcomes are assessed and determinations of comparative value may best be considered on a regional or local level where healthcare decision makers can more accurately incorporate variations in health technology acquisition costs	✓	Silent	Silent

Legend: Silent= Not mentioned within the Bill Precludes= Not allowed within the Bill

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Additional Resources

Below are additional resource materials from the National Pharmaceutical Council related to the ongoing conversations among U.S. health care stakeholders about evidence-based medicine and comparative effectiveness research (CER). All NPC documents are available at www.npcnow.org.

This document also includes links to key documents from organizations charged with developing the definitions, framework and criteria for CER, such as the Federal Coordinating Council for CER, the Agency for Healthcare Research and Quality, and the Institute of Medicine, among others.

National Pharmaceutical Council Research
(All available online at http://www.npcnow.org/Research.aspx)

- Demystifying Comparative Effectiveness Research: A Case Study Learning Guide (full report and executive summary), December 2009
- National Institute for Health and Clinical Excellence (NICE): How Does it Work and What Are the Implications for the U.S.?, 2008
- The Current Evidence-Based Medicine Landscape, 2008

NPC Commentary/Testimony

- NPC Testimony submitted to the Federal Coordinating Council on Comparative Effectiveness Research on the Draft Definition and Strategic Framework for Comparative Effectiveness Research, June 10, 2009
  http://npcnow.org/News.aspx?newsid=5cf31423-95d3-49de-89b8-d9aa11a2e216
- NPC Testimony before the Federal Coordinating Council on Comparative Effectiveness Research, April 14, 2009
  http://www.hhs.gov/recovery/programs/cer/h1404meeting.html
- NPC Testimony before the Agency for Healthcare Research and Quality, April 3, 2009
  http://www.ahrq.gov/about/nac/npc.htm
- NPC Testimony before the Institute of Medicine Comparative Effectiveness Research Committee, March 20, 2009
  http://www.npcnow.org/News.aspx?newsid=5d8d0e9f-1100-4b39-866e-fcbf453bdf5c
- NPC Podcast: Briefing on Clinical Comparative Effectiveness, February 10, 2009
  http://www.npcnow.org/Issues.aspx?issueid=f0cf1095-8655-4e06-9810-d8578e2d8c39
- NPC Statement on Economic Stimulus Package, February 17, 2009
  http://www.npcnow.org/News.aspx?newsid=39310b95-9bf6-4936-adcf-5f16f694c62a

Agency for Healthcare Research and Quality

- National Advisory Council for Healthcare Research and Quality
  http://www.ahrq.gov/about/council.htm

Congressional Budget Office

- Research on the Comparative Effectiveness of Medical Treatments, December 2007
  http://www.cbo.gov/ftpdocs/88xx/doc8891/12-18-ComparativeEffectiveness.pdf
- CBO Budget Options, Volume I: Health Care, 2008
  http://www.cbo.gov/ftpdocs/99xx/doc9925/12-18-HealthOptions.pdf

Federal Coordinating Council for Comparative Effectiveness Research

- Report to the President and Congress on Comparative Effectiveness Research, June 30, 2009
  http://www.hhs.gov/recovery/programs/cer/cerannualrpt.pdf
- Federal Coordinating Council for Comparative Effectiveness Research, main page
  http://www.hhs.gov/recovery/programs/cer/index.html

Institute of Medicine

- Initial National Priorities for Comparative Effectiveness Research, June 30, 2009
  http://www.iom.edu/CMS/3809/63608/71025.aspx
- Institute of Medicine Comparative Effectiveness Research Prioritization
  http://www.iom.edu/CMS/3809/63608.aspx
- Institute of Medicine Comparative Effectiveness Research Committee Public Meetings, March 20, 2009
  http://www.iom.edu/CMS/3809/63608/63674.aspx

Public Law

- The Patient Protection and Affordable Care Act
  http://thomas.loc.gov/cgi-bin/bdquery/z?d111:h.r.04872:
- Comparative Effectiveness Research Sections of the American Recovery and Reinvestment Act
  http://www.ahrq.gov/fund/cefarraexc.htm