

# Applying Evidence to Insurance Design, Coverage, and Reimbursement Policies

**Summary:** This line of research and policy development seeks to address the ethical challenges that arise from using evidence-based medicine to guide insurance benefit design, coverage, and reimbursement policies. The process of generating and applying evidence on clinical and sometimes cost-effectiveness within insurance systems has long been a controversial flashpoint where the goals of access, innovation, and cost containment often conflict. Recently, in the United States, the questions regarding the use of scientific evidence in medical policies gained new notoriety during the controversies over changed recommendations for mammographic screening and over provisions of the health care reform legislation that creates a new federal initiative in comparative effectiveness research.

Specific questions addressed by this line of research include: how to balance consideration of possible risks and benefits of new technologies; how to manage uncertainty about the effectiveness of new technologies when deciding whether to provide insurance coverage ; how to align benefit designs and reimbursement policies with assessment of the value of new technologies when perceptions of “value” differ between patients, clinicians, and payers; and how to consider the ethical ramifications of the dominant utility-maximizing perspective of cost-effectiveness analysis. The overall objective of this line of research is to provide empirical and normative analyses of approaches to balancing access, innovation, and cost control in support of an equitable and sustainable health care system.

**Section:** Ethics and Health Policy

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**Background:** At a time of rapid innovation, increasing demands for health care services, and rising health care premiums in the setting of limited national resources, there is a broad

consensus that cost control and improved value in the United States health care system will require more explicit application of evidence-based medicine. Yet the process of assessing new technologies in order to make decisions regarding insurance coverage, reimbursement, and patient responsibility for costs carries with it important ethical dimensions whose delineation and consideration remain very controversial. The controversies arise due to the complex interplay between the goals of quality of care improvement, technological innovation, and cost control.

The major policy issues that arise can be categorized as pertaining to either the technical methods of technology appraisal or to the overall political process in which technology appraisal is embedded. The methods of technology appraisal have developed along a technical path dominated by the principles of evidence-based medicine. Some appraisal efforts also include formal efforts to judge value by using cost-effectiveness models to produce estimates of the marginal benefit of the new technology as measured by the cost per life year gained, or per quality-adjusted life year gained. None of the methods of evidence-based medicine and of cost-effectiveness analysis are free from inherent considerations of ethical values, including relative weights given to risks and benefits, reasonable boundaries of uncertainty in evidence, and considerations of the relative severity and “need” of patients with different illnesses.

**Departmental Research Initiative:** Coverage and other insurance system policies can be seen as a component of the general departmental focus on the ethics of priority setting, but with the arrival of Steve Pearson as a Visiting Scientist in 2005 this specific line of research gained a lead investigator and increasing visibility within the department. He initially focused on two major areas: 1) extrapolating policy lessons from the international context as part of formulating a new U.S. initiative in comparative effectiveness research; and 2) exploring the ethical ramifications of a new Medicare policy linking insurance coverage to requirements for patients to participate in clinical research, called the “coverage with evidence development” policy.

Since 2007, Dr. Pearson has continued to work closely with Drs. Emanuel, Danis, and Miller in the Department, and with multiple colleagues outside the NIH, on projects related to evidence, ethics, and insurance policy that reinforce the department’s overall commitment to exploring priority setting as an important component of health policy. These research and policy development efforts can be categorized thusly:

#### *1) Comparative Effectiveness Research*

Dr. Pearson has written extensively on questions related to the scope, design, and policy ramifications of a new federal initiative in comparative effectiveness research, an initiative which became part of the US health care reform legislation passed in 2010. Among his work in this area, Dr. Pearson wrote an article and gave a presentation as part of a panel of senior scholars assembled by the Brookings Institution in 2009 to inform health care reform legislative efforts. This article contained an analysis of possible methods to include cost-effectiveness information within the scope of comparative effectiveness, and was the first to lay out a complete framework for applying comparative effectiveness research results through innovative forms of benefit design and coverage and reimbursement policies. In part, this work built upon previous work that Dr. Pearson had done with Dr. Emanuel and a pre-doctoral fellow in the Department on the ethics of value-based insurance designs.

Dr. Pearson also published an article in *Health Affairs* with co-author Peter Bach that is the first to lay out a specific framework for applying comparative effectiveness research within coverage and reimbursement at Medicare. The authors propose a new payment model that would use evidence to move Medicare toward the principle of paying equally for services that provide comparable patient outcomes. The model would pay more for superior services while granting higher reimbursement only for a time-limited phase for others. Although the political hurdles to changing Medicare reimbursement are high, the article argues that efforts should be made to use comparative effectiveness research results to reward superior services, improve incentives for cost-effective innovation, and place Medicare on a more sustainable financial footing.

Dr. Pearson has written several other articles further developing ideas on how best to apply comparative effectiveness research results. He wrote three articles, two of which were part of Institute of Medicine workshops, on the general issue of setting transparent and justifiable evidence thresholds for applications of comparative effectiveness evidence in coverage and other medical policy decisions. Working with a pre-doctoral fellow in the Department, he also wrote an article in *Health Affairs* that presented a conceptual model for using comparative effectiveness research to identify possible waste in the health care system. This model used the potential findings of comparative effectiveness research to designate four categories of “marginal medicine.” The first two categories are driven by uncertainty due to inadequate evidence of clinical benefit: 1) inadequate evidence of comparative net benefit for any indication; and 2) use beyond boundaries of established net benefit. The last two categories of marginal medicine are grounded in considerations of cost-effectiveness: 3) higher cost when established benefit is comparable to other options; and 4) relatively high cost for incremental benefit compared to other options. This schema has been noted by many policy experts and is being used by the AMA Physician Consortium For Performance Improvement (PCPI) in its efforts to develop measures of “waste” due to physician practice patterns.

Dr. Pearson’s work on both the underlying practice and policy applications of comparative effectiveness research has led to numerous requests to speak and contribute to policy development during the health care reform process. Some selected contributions include:

- Testimony on comparative effectiveness research before the Senate HELP committee
- Presentations to physician societies on applying evidence to insurance policies: ASTRO leadership summit, Heart Rhythm Society, Society of Thoracic Surgeons, Spine Surgeons Value Committee, American Society of Interventional Pain Physicians
- Presentations on CER to policy groups: IOM Evidence-based Medicine Roundtable, NQF, AcademyHealth National Health Policy conference, AHIP Institute, AMA PCPI annual meeting, HTAi, ISPOR, Harvard University Program in Ethics and Health, National Pharmaceutical symposia, and the World Health Care Congress.

## *2) Evidence and insurance: off-label prescribing*

Another area in which questions have been raised about the role of evidence and insurance policy is off-label prescribing. Physicians commonly prescribe FDA-approved drugs for off-label indications. Although some off-label uses are standard of care, many others have little or

no scientific support. Few guidelines regarding assessment of evidence or consent for off-label use are available to physicians. In a paper published in the *Archives of Internal Medicine*, Drs. Pearson and Miller, working with a pre-doctoral fellow, identified four drug characteristics that signal the need for rigorous scrutiny of evidence before off-label prescribing: new drugs; novel off-label indications; serious side-effects; and high cost. For off-label uses characterized by one or more of these signals for scrutiny, the article presented an evidence-guided ethical framework that distinguishes three potentially appropriate categories of off-label use: 1) supported off-label use, 2) suppositional off-label use, and 3) investigational off-label use. These categories are based on the validity and relevance of available evidence. The categories are then linked to specific requirements for appropriate off-label prescribing, including the nature of disclosure to patients of the risks and benefits involved.

### *3) Evidence and insurance: orphan drugs*

Another area of ethical and policy concern regarding the proper role of evidence in insurance policy is orphan drugs. Orphan drugs are targeted, often expensive therapies for diseases that are exceptionally rare. In America, a rare disease or condition is defined by law as one that affects fewer than 200,000 people. The rule of rescue is frequently cited as a motivation for accepting the high prices needed to treat these relatively rare cases. But what should the role of evidence be in relation to the rule of rescue?

This is the central question tackled by another line of work led by Drs. Pearson and Miller in conjunction with a pre-doctoral fellow. In an article they published in *Archives of Internal Medicine*, the rule of rescue is analytically divided into three constituent elements – identifiability, endangered lives, and opportunity costs – each of which is then evaluated with the goal of elucidating ways to use evidence to constrain inappropriate applications of the rule of rescue while continuing to respect its moral core. The purpose of this article is threefold: 1) to describe the historical and political circumstances that have shaped orphan drug policy to date and to explain why blanket coverage at “market” prices will not prove sustainable; 2) to outline and deconstruct the argument from the rule of rescue that is made in support of coverage of orphan drugs; and, 3) to draw on this analysis in order to suggest factors that should be incorporated into coverage decisions so as to make them more transparent and ethically sound.

This conceptual framework offers an advance over current decision making practices because it allows for more transparent, more consistent decision-making than has occurred to date and protects against ill-advised and unethical extremes of a rescue mentality. It also creates the possibility for coverage of expensive orphan drugs where others have seen none.

### *4) Evidence and insurance: financial incentives to reduce unhealthy behavior*

As health care costs continue to rise, an increasing number of self-insured employers are using financial rewards or penalties to promote healthy behaviors and control costs. These incentive programs have triggered a backlash from those concerned that holding employees responsible for their health, particularly through the use of penalties, violates individual liberties and

discriminates against the unhealthy. Dr. Pearson wrote a paper with a pre-doctoral fellow that was published in *Health Affairs*, helped inform the design of incentive regulations contained in the health care reform law, and which continues to generate significant interest among policy makers.

The paper offers an ethical analysis of employee health incentive programs and presents an argument for a set of conditions under which penalties can be used in an ethical and responsible way to contain health care costs and encourage healthier behaviors among employees. The paper argues that employers and employees are jointly responsible for mitigating the consequences of unhealthy behaviors and that a reasonable ethical balance can be established, but only within narrow boundaries, between holding employees responsible and protecting their liberties. The paper provides guidelines specifying the appropriate conditions under which penalty programs can be designed and administered in an ethical way. In order to have ethical legitimacy, penalty programs must arise from a collaborative engagement between employers and employees, must engage in transparent processes for selecting voluntary actions – not simple biometric outcomes - - to be penalized, and they must offer employees fair and equal opportunities to improve health. Programs must also adhere to several criteria for fair administration of penalty programs, including provision of accessible tools to change behavior, availability of an opt-out process, and a fair process for setting reasonable amounts for penalties.

This paper led to presentations by Dr. Pearson to the national Partnership for Prevention and the Congressional Prevention Coalition. In addition, Dr. Pearson was invited to participate in the AARP Public Policy Institute roundtable on the topic of financial incentives to promote health behaviors.

**Future Research Initiatives:** The previously described areas of emphasis continue to provide opportunities for further research and policy development. In addition, several other specific research initiatives are under way within the general area of applying evidence within the health care system.

#### *1) Application of “negative” evidence to insurance coverage and reimbursement policies*

The new federal investment in comparative effectiveness research reflects the hope that rigorous data regarding the effectiveness and safety of interventions can be used to help guide better clinical choices, payer policies, and research initiatives. Yet to attain the anticipated benefits of these investments it is not only essential that relevant data are produced, but that there are clear channels for this evidence to be incorporated into the healthcare system. In August 2009 two “negative” randomized controlled trials of the interventional procedure called vertebroplasty were published in the *New England Journal of Medicine*. Until that time vertebroplasty had become widespread despite any randomized trials demonstrating that the procedure was superior to conservative care. Following the publication of negative RCT results, physician specialty societies put out press releases casting doubt on the studies and calling for no change in insurance coverage. Medicare and several prominent national private insurers started processes to reconsider coverage, but backed off, leaving coverage and reimbursement for vertebroplasty unchanged.

Using the example of vertebroplasty as an initial case study, this line of research will explore what can be learned about the use of “negative” evidence in our health care system, and what proposals can be made to improve objective consideration of evidence. Ultimately, the goal of this line of research will be to inform public policy on incorporating evidence into practice and policy in a way that will improve the safety and value of health care services.

## *2) The Institute for Clinical and Economic Review (ICER)*

As a Visiting Scientist within the Department of Bioethics at the NIH, Dr. Pearson also retains involvement with The Institute for Clinical and Economic Review (ICER) at the Massachusetts General Hospital in Boston. ICER is a research group performing independent evaluations of the clinical effectiveness and comparative value of health care interventions. There are several features of ICER’s focus and methodology that distinguish it from other comparative effectiveness assessment organizations. First, ICER engages more deeply throughout the assessment process with all stakeholders, including patients, clinicians, manufacturers, purchasers, and payers. ICER also includes as part of all its assessments the results of cost-effectiveness analysis, and continues to work with stakeholders to refine a rating system for comparative clinical effectiveness and comparative value to guide health care decisions. Lastly, ICER works with all stakeholders to develop and evaluate applications of evidence to create innovative patient-clinician decision support tools, insurance benefit designs, and coverage and payment policies. Through these activities ICER seeks to achieve its ultimate mission of informing public policy and spurring innovation in the use of evidence to improve the value of health care for all.

Many of ICER’s projects provide additional prospects for research on ethics and applied health policy. For example, in July, 2010 ICER was awarded an AHRQ grant to serve as the lead organization of a consortium of six New England states and regional private payers to form a “New England Comparative Effectiveness Council.” ICER will prepare adapted versions of AHRQ evidence reviews for deliberation by the Council and will facilitate the dissemination and implementation of CER reviews by public and private payers. Dr. Pearson will work with fellows in the Department of Bioethics at the NIH to explore ethical aspects of this innovative attempt to advance the ability of public and private payers to integrate cost-effectiveness information in considerations of comparative effectiveness and to propel the application of evidence in setting coverage, reimbursement, and other key medical policies.

## **Publications:**

### *Earlier Related Publications (2005-2006)*

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Pearson SD. Standards of Evidence. In Olsen L, Aisner, McGinnis JM. The Learning Healthcare System. Institute of Medicine. The National Academies Press, Washington, DC., 2007.