



HEALTH

THE ARTS
CHILD POLICY
CIVIL JUSTICE
EDUCATION
ENERGY AND ENVIRONMENT
HEALTH AND HEALTH CARE
INTERNATIONAL AFFAIRS
NATIONAL SECURITY
POPULATION AND AGING
PUBLIC SAFETY
SCIENCE AND TECHNOLOGY
SUBSTANCE ABUSE
TERRORISM AND
HOMELAND SECURITY
TRANSPORTATION AND
INFRASTRUCTURE
WORKFORCE AND WORKPLACE

This PDF document was made available from www.rand.org as a public service of the RAND Corporation.

[Jump down to document](#) ▼

The RAND Corporation is a nonprofit research organization providing objective analysis and effective solutions that address the challenges facing the public and private sectors around the world.

Support RAND

[Browse Books & Publications](#)

[Make a charitable contribution](#)

For More Information

Visit RAND at www.rand.org

Explore [RAND Health](#)

View [document details](#)

Limited Electronic Distribution Rights

This document and trademark(s) contained herein are protected by law as indicated in a notice appearing later in this work. This electronic representation of RAND intellectual property is provided for non-commercial use only. Unauthorized posting of RAND PDFs to a non-RAND Web site is prohibited. RAND PDFs are protected under copyright law. Permission is required from RAND to reproduce, or reuse in another form, any of our research documents for commercial use. For information on reprint and linking permissions, please see [RAND Permissions](#).

This product is part of the RAND Corporation technical report series. Reports may include research findings on a specific topic that is limited in scope; present discussions of the methodology employed in research; provide literature reviews, survey instruments, modeling exercises, guidelines for practitioners and research professionals, and supporting documentation; or deliver preliminary findings. All RAND reports undergo rigorous peer review to ensure that they meet high standards for research quality and objectivity.

R E P O R T



Feasibility and Design Options for a Potential Entity to Research the Comparative Effectiveness of Medical Treatments

Peter S. Hussey, Emily Meredith Gillen,
Elizabeth A. McGlynn

Sponsored by the Commonwealth of Massachusetts

This work was sponsored by the Commonwealth of Massachusetts. The research was conducted in RAND Health, a division of the RAND Corporation.

The RAND Corporation is a nonprofit research organization providing objective analysis and effective solutions that address the challenges facing the public and private sectors around the world. RAND's publications do not necessarily reflect the opinions of its research clients and sponsors.

RAND® is a registered trademark.

© Copyright 2010 RAND Corporation

Permission is given to duplicate this document for personal use only, as long as it is unaltered and complete. Copies may not be duplicated for commercial purposes. Unauthorized posting of RAND documents to a non-RAND Web site is prohibited. RAND documents are protected under copyright law. For information on reprint and linking permissions, please visit the RAND permissions page (<http://www.rand.org/publications/permissions.html>).

Published 2010 by the RAND Corporation
1776 Main Street, P.O. Box 2138, Santa Monica, CA 90407-2138
1200 South Hayes Street, Arlington, VA 22202-5050
4570 Fifth Avenue, Suite 600, Pittsburgh, PA 15213-2665
RAND URL: <http://www.rand.org>
To order RAND documents or to obtain additional information, contact
Distribution Services: Telephone: (310) 451-7002;
Fax: (310) 451-6915; Email: order@rand.org

Preface

In 2008, the Massachusetts state legislature enacted Chapter 305, An Act to Promote Cost Containment, Transparency and Efficiency in the Delivery of Quality Health Care. Among the various provisions in this act, Section 53 instructed the state Secretary of Health and Human Services to examine the feasibility of establishing an entity to examine the comparative effectiveness of medical treatments. This report was prepared by the RAND Corporation to assist the secretary in meeting this requirement.

The analysis, performed between April and August 2009, is based on a literature review, interviews with comparative effectiveness research experts, and a meeting of representatives from New England states. The contents of this report will be of primary interest to the Massachusetts Department of Health and Human Services but should also be of interest to national and state policymakers, health care organizations, health researchers, and others interested in comparative effectiveness research.

This work was sponsored by the Massachusetts Executive Office for Health and Human Services, for which Joel Weissman served as project officer. The research was conducted in RAND Health, a division of the RAND Corporation. A profile of RAND Health, abstracts of its publications, and ordering information can be found at www.rand.org/health.

Contents

Preface	iii
Figure and Tables	vii
Summary	ix
Acknowledgments	xiii
Abbreviations	xv
CHAPTER ONE	
Introduction	1
CHAPTER TWO	
Methods	3
CHAPTER THREE	
Overview of Comparative Effectiveness Research	5
What Is Comparative Effectiveness Research?	5
Why Is New Comparative Effectiveness Research Needed?	6
How Have the Results of Comparative Effectiveness Research Been Used?	7
How Can Comparative Effectiveness Research Contribute to Reduced Health Care Spending Growth?	7
CHAPTER FOUR	
Existing Comparative Effectiveness Centers	9
Federal Government	9
State Governments	10
Private Sector	10
International	11
CHAPTER FIVE	
Objectives of a Comparative Effectiveness Center	13
Potential Objective 1: Provide Information to Patients and Physicians to Guide Decisionmaking... 13	
Potential Objective 2: Provide Information to Private and Public Insurers to Enable Changes in Reimbursement or Benefit Design	15
Potential Objective 3: Use Information to Guide Coverage Decisions in Insurance Programs	16
CHAPTER SIX	
Design Options for an Interstate Comparative Effectiveness Center	19

CHAPTER SEVEN

Other Design Considerations for a Comparative Effectiveness Center 23
How Will Comparative Effectiveness Information Be Produced? 23
How Will Research Topics Be Selected? 24
Should the CEC Evaluate Clinical Effectiveness or Cost-Effectiveness? 25

CHAPTER EIGHT

Conclusions 27

APPENDIXES

A. Existing Comparative Effectiveness Centers 29
B. Glossary 43
References 45

Figure and Tables

Figure

6.1.	Potential Functions of a Comparative Effectiveness Center	19
------	---	----

Tables

5.1.	Coverage Decisions by Washington Health Technology Assessment	17
6.1.	Funding for Selected Existing Comparative Effectiveness Centers	21
7.1.	Potential Criteria for Selection of Treatments for Comparative Effectiveness Research.....	25
A.1.	Summary of Existing Comparative Effectiveness Centers.....	30

Summary

Section 53 of Chapter 305 of the Massachusetts state legislature's Acts of 2008 requires the Massachusetts Secretary of Health and Human Services, in consultation with the Health Care Quality and Cost Council, to

- (i) examine the feasibility of the commonwealth entering into an interstate compact with 1 or more states to establish an independent entity to research the comparative effectiveness of medical procedures, drugs, devices, and biologics, so that research results can be used as a basis for health care purchasing and payment decisions, and (ii) make recommendations concerning the entity's design. (Massachusetts State Legislature, 2008)

“Comparative effectiveness” research is “the conduct and synthesis of systematic research comparing different interventions and strategies to prevent, diagnose, treat and monitor health conditions” (Federal Coordinating Council on Comparative Effectiveness Research, undated). This report outlines the design options for such an entity, referred to hereafter as a “comparative effectiveness center” (CEC), but it does not recommend specific design options. The report is based on a targeted literature review, interviews with comparative effectiveness research experts, and a meeting of representatives from New England states.

What Are the Objectives of a Comparative Effectiveness Center?

According to the Massachusetts legislature, the information generated by a CEC should be useful for making purchasing and payment decisions. To this end, the objectives of a CEC could include guiding decisions by public and private health insurers. Insurers use comparative effectiveness information to decide whether particular treatments are covered or excluded from a benefits package. A CEC could provide additional information that insurers could use in making these decisions. Another potential objective is to provide information to insurers to enable changes in reimbursement or benefit design. The changes could include tiered copayments, with higher copayments for less-effective treatments; reference pricing, under which the same price would be paid for equivalent treatments; and the provision of financial incentives to physicians for the use of effective treatments.

Another potential objective could be to provide information to physicians and patients to guide their medical decisions. This approach could potentially improve health care quality and reduce costs by improving medical decisionmaking, independent of insurers' benefit and payment policies. The main limitation of dissemination activities is that they may not be sufficient to significantly change treatment decisions (and, in turn, health spending). There are many

examples of new information on the effectiveness of treatments having little effect on practice patterns. New approaches to disseminating comparative effectiveness information may increase its impact on treatment decisions. One potential approach is “shared decisionmaking,” a process through which patients and their care providers are active participants in the process of communication and decisionmaking about their care.

Design Options for an Interstate Comparative Effectiveness Center

Given the extent of existing activities by federal and state governments and the private sector, a compelling question is, How much value would be gained from the establishment of a new CEC, and how would a new CEC’s role be coordinated with other comparative effectiveness activities? Here, we outline five options for the role of Massachusetts in an interstate CEC.

Option 1: An interstate CEC could be established to provide a framework for the use of existing comparative effectiveness reports by regional decisionmakers. Evidence reports are currently available from a number of organizations, including the federal government, states, and the private sector, but there is currently no framework to translate the evidence into actionable information for New England decisionmakers. Reports from various sources could be studied by an independent panel of local clinicians, who would make recommendations based on their public deliberations.

Option 2: An interstate CEC could be established to support new comparative effectiveness research. This option would create the framework for evidence translation as in option 1 and also commission new comparative effectiveness research. By funding new research, the regional center would ensure that comparative effectiveness information was available for priority topics. The required funding for Massachusetts would depend on how many other states participated and how funding responsibility was allocated among participating states.

Option 3: Massachusetts could join existing interstate CECs. The Drug Effectiveness Review Project (DERP) and the Medicaid Evidence-Based Decisions Project (MED) are existing collaborations between states across the country that produce comparative effectiveness evidence reviews. The cost of membership would be approximately \$90,000 per year for DERP and \$130,000 per year for MED. Participating states can provide input on priorities for evidence reviews and have access to reports, summaries, collaboration, and guidance.

Option 4: Massachusetts could join DERP and MED and also establish a regional CEC. Massachusetts could pursue both options 2 and 3 to produce a greater amount of new comparative effectiveness research. This option would take advantage of the existing infrastructure of DERP and MED while allocating additional resources to regional comparative effectiveness priorities through a new CEC.

Option 5: Status quo. Massachusetts could elect not to establish or join a CEC. Local stakeholders could continue to rely on existing decisionmaking processes and activities sponsored by other entities for comparative effectiveness information.

Other Design Considerations for a Comparative Effectiveness Center

How Will Comparative Effectiveness Information Be Produced?

There are several types of comparative effectiveness research that could be sponsored by a CEC, with very different cost implications. *Systematic reviews* provide a rigorous framework for evaluating evidence from existing studies. Systematic reviews are generally less expensive than options that produce new evidence. Most of the existing state and private programs undertake this approach. *Clinical trials* are the gold standard for producing rigorous evidence, but due to the level of funding required, sponsorship of new clinical trials is likely not a viable option for a regional CEC. New *observational studies* could be performed retrospectively using existing data sets, such as insurance claims. These studies can add to the evidence base at lower expense than prospective clinical trials but typically do not provide the same strength of evidence. A CEC could also facilitate the use of *patient registries* to produce new information on the comparative effectiveness of treatments. A policy of “coverage with evidence development” would require patients using approved treatments to participate in a registry to gather outcome information.

How Will Research Topics Be Selected?

If Massachusetts enters into an interstate compact to create a new regional CEC, a transparent process will be needed to prioritize treatments selected for review. A similar process would likely be used whether the CEC was providing a framework for translating existing reports (option 1) or commissioning new research (option 2 or 4). If Massachusetts joins DERP and/or MED (option 3), it would participate in existing prioritization activities. Commonly used prioritization criteria include cost, utilization, strength of existing evidence, decision complexity, and social/legal/ethical concerns.

Should the CEC Evaluate Clinical Effectiveness or Cost-Effectiveness?

Some existing activities compare clinical effectiveness only—not cost. This should decrease political opposition to a CEC. However, it is likely that decisionmakers, such as insurers using the clinical effectiveness reviews, would consider cost information separately. These comparisons, since they would not be conducted within the established CEC review process, would not necessarily be transparent to the public. However, others advocate that, given the growing inaffordability of health care, it is necessary to consider the cost implications of treatment alternatives. Consideration of cost-effectiveness increases the likelihood that comparative effectiveness research could lead to reduced health care spending (Congressional Budget Office, 2007).

Conclusion

This report outlines several design options that Massachusetts could follow in establishing an interstate CEC. The choice of design option will be determined by the specific objectives of the legislature and by the legislature’s prioritization of comparative effectiveness research over other options under consideration for improving quality and reducing spending growth in health care. With the political will, all of the options presented in this report should be technically feasible to implement. However, the implementation of a government-funded CEC would likely encounter significant political opposition.

Participants in a meeting of New England state representatives expressed strong interest in establishing a CEC. Among the design options presented in this report, meeting participants expressed the strongest interest in beginning with option 1, possibly as a first step. In this approach, Massachusetts would enter into an interstate compact with other New England states to create a framework for translating comparative effectiveness information into actionable recommendations for local decisionmakers. Meeting participants felt that recommendations coming from such an organized framework may be viewed as a “trusted source” by local physicians, patients, and other stakeholders. They also felt that collaboration among New England states made sense, given the region’s merged medical marketplaces. The centerpiece of the regional framework was envisioned by meeting participants as an independent group of local clinicians. This panel would hold public meetings to review comparative effectiveness research reports produced by other organizations and make recommendations for purchasing or clinical decisionmaking.

The American Reinvestment and Recovery Act of 2009 (ARRA; Pub. L. 111-5) included funding for comparative effectiveness research that could potentially provide seed money for a regional effort. ARRA allocated \$1.1 billion between the Agency for Healthcare Research and Quality (AHRQ), the National Institutes of Health, and the U.S. Department of Health and Human Services. In a notice of intent to publish grant and contract solicitations, released on August 7, 2009, AHRQ announced that it would provide \$29.5 million to “support innovative translation and dissemination grants” for comparative effectiveness research, with solicitations published beginning in fall 2009 and funding commencing in spring 2010. A potential barrier is that the ARRA prohibits the Federal Coordinating Council on Comparative Effectiveness Research from taking action that could be construed “to mandate coverage, reimbursement, or other policies for any public or private payer.” At this time, it is unclear whether this stipulation will apply beyond the activities of the council itself and apply to all AHRQ grants and contracts for translation and dissemination activities.

In future steps, Massachusetts and other New England states could potentially build on such a framework by commissioning additional comparative effectiveness research to fill gaps in existing information. This could be accomplished by commissioning studies from established research centers (option 2) and/or joining existing state collaborations (DERP and MED) (option 3).

Massachusetts is at the forefront of the national health reform debate and is considering a variety of innovative approaches to improve the quality and affordability of care in the state. However, other state governments have been more active to date in sponsoring and using comparative effectiveness research. Massachusetts could potentially become a leader among states in this area. New England is world-renowned for its clinical research enterprise, and its academic medical centers could help in the establishment and operation of a CEC. In addition, New England states have a track record of collaboration on health policy issues that could be extended to comparative effectiveness research.

Acknowledgments

We thank Mark Benton, Mark Gibson, Leah Hole-Curry, Peter Neumann, Steven Pearson, and Harold Sox for providing input for this project. We are also grateful to Robert Brook, Peter Neumann, Paul Shekelle, and Justin Timbie for comments on an earlier draft of this report. Participants at an August 2009 meeting of representatives from New England states provided valuable input on design options. We also acknowledge the support of the Milbank Memorial Fund for convening the meeting. Finally, we benefited from the input and support of the staff of the Massachusetts Executive Office of Health and Human Services, particularly our project officer Joel Weissman.

Abbreviations

AHRQ	Agency for Healthcare Research and Quality
ARRA	American Reinvestment and Recovery Act of 2009
CEAR	Cost-Effectiveness Analysis Registry
CEC	comparative effectiveness center
CMS	Centers for Medicare and Medicaid Services
CMTP	Center for Medical Technology Policy
CT	computed tomography
DEcIDE	Developing Evidence to Inform Decisions about Effectiveness
DERP	Drug Effectiveness Review Project
EPC	evidence-based practice center
FDA	U.S. Food and Drug Administration
HTA	health technology assessment
ICER	Institute for Clinical and Economic Review
IQWiG	Institute for Quality and Efficiency in Health Care
MED	Medicaid Evidence-Based Decisions Project
MRI	magnetic resonance imaging
NICE	National Institute for Health and Clinical Excellence
NIH	National Institutes of Health
TEC	Technology Evaluation Center

Introduction

Section 53 of Chapter 305 of the Massachusetts legislature's Acts of 2008 requires the state Secretary of Health and Human Services, in consultation with the Health Care Quality and Cost Council, to

- (i) examine the feasibility of the Commonwealth entering into an interstate compact with 1 or more states to establish an independent entity to research the comparative effectiveness of medical procedures, drugs, devices, and biologics, so that research results can be used as a basis for health care purchasing and payment decisions, and (ii) make recommendations concerning the entity's design. (Massachusetts State Legislature, 2008)

This report was prepared to assist the Secretary in meeting this requirement by describing the design options for such an entity, referred to hereafter as a "comparative effectiveness center" (CEC). It does not recommend specific design options, however. The report is organized as follows. We begin by describing our methodology (Chapter Two), defining what is meant by *comparative effectiveness research* (Chapter Three), and identifying the ways in which this research might reduce health care spending (Chapter Four). We then describe existing entities at the federal level, in other states, in the private sector, and in other countries that perform this type of work (Chapter Five). Finally, we review potential objectives and design options for the CEC (Chapter Six), along with other design considerations (Chapter Seven). Chapter Eight presents our conclusions, and Appendix A provides profiles on existing CECs. Appendix B includes a glossary of terms used in report.

Methods

The overview of comparative effectiveness research and definitions of related terms, presented in Chapter Three, was developed through a targeted review of the medical literature using the MedLine database. The descriptions of existing CECs (Chapter Four) were drawn from published literature and Web sites, supplemented through consultations with CEC staff. The CECs discussed in Chapter Four were selected based on (1) the CECs named in Chapter 305 of the Massachusetts Acts of 2008, (2) recommendations from the project officer, and (3) recommendations from consulted experts. The list of CECs is therefore illustrative of all existing activities but not comprehensive.

We developed the design options described in Chapter Five through Seven based on our review of existing CECs in Chapter Four. We gathered input on these design options through a meeting of representatives from New England states, convened in August 2008. The meeting was attended by representatives of state Medicaid programs, state employee insurance programs, state departments of health, legislators and their staffs, and academic researchers with expertise in comparative effectiveness research.

Overview of Comparative Effectiveness Research

What Is Comparative Effectiveness Research?

Comparative effectiveness research is “the conduct and synthesis of systematic research comparing different interventions and strategies to prevent, diagnose, treat and monitor health conditions” (Federal Coordinating Council on Comparative Effectiveness Research, undated). The research can be structured to evaluate treatments with similar modalities (e.g., different types of medication therapy), treatments with different modalities (e.g., medication versus surgery), and treatment versus no treatment (sometimes referred to as “watchful waiting”). The results of comparative effectiveness research can be used in a variety of ways, including for the provision of information to physicians and patients for choosing appropriate treatments, as input into decisions about whether and/or for whom a treatment will be covered under public or private insurance policies, and for setting the level of reimbursement that will be allowed under private or public insurance policies.

Comparative effectiveness research can be conducted in several different ways, including through clinical trials, observational studies using existing databases, medical registries, and systematic reviews of prior research. Each method has strengths and limitations, which we discuss later when we consider options for the structure and functions of a CEC.

Comparative effectiveness research can focus on different types of “effectiveness”—mainly, clinical effectiveness and cost-effectiveness.¹ Clinical effectiveness is “the extent to which a specific intervention, when used under ordinary circumstances, does what it is intended to do” (Cochrane Collaboration, 2005). Clinical effectiveness research can consider a range of clinical outcomes (e.g., mortality rates, functional status, laboratory results, physical examination findings, the presence of side effects). A challenge in measuring clinical effectiveness is in observing the effect of an intervention under “ordinary circumstances”—real-world use, rather than controlled experimental conditions. Many clinical trials instead measure “efficacy,” or “the extent to which an intervention produces a beneficial result under ideal conditions” (Cochrane Collaboration, 2005).

Throughout this report, we distinguish *clinical effectiveness* from *cost-effectiveness*, which is “the costs for some additional health gain” (Cochrane Collaboration, 2005). Variants of cost-effectiveness analysis include cost-benefit analysis, in which both health benefits and costs are measured in monetary units, and cost-utility analysis, in which health effects are measured only in quality-adjusted life years, a measure that combines morbidity and mortality into a single metric.

¹ These and other relevant terms are further defined in the glossary.

There are several challenges to measuring the incremental costs used in cost-effectiveness analyses. The U.S. Public Health Service Panel on Cost-Effectiveness in Health and Medicine recommended a “societal perspective” in measuring costs (Gold, Siegel, and Russell, 1996). *Societal perspective* has been interpreted in different ways, but, in general, it has two components: (1) productivity gains and losses, such as the cost of time missed from work, are included, and (2) costs are measured using “opportunity costs,” or the value of the alternative use of a resource (Garrison et al., 2008). In practice, because the societal perspective can be difficult to operationalize, not all cost measurements include productivity gains and losses, and market prices are often used to measure the value of resources, not opportunity costs. For example, a comparison of the cost of two drugs may compare estimates of their market prices; another may include the costs of related treatments that are needed, such as physician visits and hospitalizations; and other approaches may add the value of wages lost or gained due to the drugs’ effect on productivity. Measurement approaches also vary in the length of time over which costs are considered. Costs could be measured solely for providing the treatments being compared at a given point in time, over the period required to treat a case, or over a longer period that includes follow-up care and long-term health effects.

Why Is New Comparative Effectiveness Research Needed?

Comparative effectiveness research is not new, but it has received increasing interest as new, high-cost medical technology and treatments have proliferated. Some form of evidence on the effectiveness of new medical treatments is usually available, but that evidence is often insufficient to provide a solid basis for decisions among treatment alternatives, for several reasons:

- *Few head-to-head comparisons:* Most clinical trials evaluate whether an intervention produces some form of benefit compared to a control group that does not receive any intervention. Fewer studies directly evaluate whether one intervention performs better than another or whether patients have better outcomes if treated with different modalities (e.g., medical therapy versus surgery). Making such comparisons after the studies have been done can produce misleading results if the original studies enrolled different types of patients, used different outcome measures, or employed different protocols.
- *Limited effectiveness data for off-label use and surgical procedures:* The U.S. Food and Drug Administration (FDA) evaluates the safety and effectiveness of new drugs and medical devices. These evaluations support the process of obtaining approval to market these treatments. The approval is generally restricted to the treatments’ use under particular clinical circumstances and is based on research in fairly narrowly defined patient populations. Once a drug or device is approved, however, its use may not remain restricted to the original indications (“off-label” use). Further, new surgical procedures can enter into use without review by a regulatory body, such as the FDA. The evidence supporting new surgical procedures is often less rigorous than that supporting new drugs and devices and may change rapidly as use diffuses to new practitioners (Congressional Budget Office, 2007).
- *Limited information on treatment effects for different patient subgroups:* It is rarely the case that a treatment works equally well for all patients, however, the original clinical research

is frequently not designed to detect differences among patients of different ages, race/ethnicities, or other subgroups.

How Have the Results of Comparative Effectiveness Research Been Used?

Comparative effectiveness research is used by different types of decisionmakers for different purposes. Physicians and patients may use this information in making decisions about the management of particular health problems. The results of the research may be disseminated through scientific articles in the peer-reviewed literature or made more readily accessible through their incorporation into clinical guidelines from professional societies or structured decision aids for patients and doctors. Insurers may use comparative effectiveness information to decide which services to cover under their benefit plans, particularly for new technologies. It is less common for insurers to revoke coverage unless a treatment is found to be unsafe or completely ineffective. Insurers are also testing and implementing ways to encourage the use of preferred treatments other than the blunt instrument of coverage decisions, such as payment rules and benefit design. For example, comparative effectiveness information is sometimes used not only to determine which prescription drugs are covered in a health plan's formulary, but also to assign drugs to different "tiers" with different copayment obligations for beneficiaries.

How Can Comparative Effectiveness Research Contribute to Reduced Health Care Spending Growth?

The likelihood that comparative effectiveness research can moderate trends in health spending depends on a number of factors. Consider some examples.² First, comparative effectiveness research could conclude that two treatments have equivalent effectiveness for all types of patients, but one treatment is substantially more expensive than the other. The extent to which health care spending growth will be reduced depends on the degree to which this information changes behavior. In general, publication of these findings in a scientific journal will be unlikely to quickly or decisively result in a change in practice. If the information is structured into practice guidelines and physicians' performance is monitored, greater change might occur. If the information is structured into formal decision aids for patients and doctors, the impact on health spending might be greater. If the information is used for coverage decisions, even greater effects on health spending are likely (particularly if the result is to stop coverage for the more expensive treatment). If patients can still get partial coverage for the more expensive but equivalent treatment, the reduction in health spending will be somewhat attenuated.

Second, comparative effectiveness research could conclude that one treatment is substantially more effective than the other while the costs of the two treatments are comparable. In this case, although a patient's health might improve with greater use of the more effective intervention, the impact on spending is less clear-cut. We could observe reductions in spending growth if the less effective treatment led to additional health spending or increased rates of

² In these examples, we assume that research examines both clinical effectiveness and cost; much comparative effectiveness research focuses on clinical effectiveness only.

complications, but it would depend on the frequency and type of complications. For example, sudden death might not be expensive, but repeated hospitalizations would be.

Third, comparative effectiveness research could conclude that one treatment is superior to another and that one treatment is substantially more expensive than the other. Increasing use of a treatment with superior effectiveness and higher cost would increase health spending.³ The opposite scenario is increasing the use of a treatment with superior effectiveness and lower cost, which would decrease health spending.

Fourth, comparative effectiveness research could find no evidence of differences between two treatments. In this case, use of the more expensive treatment could decrease, since there are no proven health benefits that would justify its use. This would result in decreased health spending.

At this point, we do not have information on the likely distribution of comparative effectiveness research results across these illustrative scenarios, which makes it difficult to predict the likely impact on spending.

³ A variation on this scenario is that the research could conclude that a combination of the two treatments is the superior option.

Existing Comparative Effectiveness Centers

Comparative effectiveness research is currently sponsored and conducted by the federal government, a limited number of states, and the private sector. Several models for CECs also exist in other countries. This chapter provides an overview of existing comparative effectiveness activities, then discusses the role of a potential interstate CEC that includes Massachusetts. Appendix A provides detailed descriptions of these centers.

Federal Government

Comparative effectiveness research is currently sponsored by various government agencies, including the Agency for Healthcare Research and Quality (AHRQ), the Centers for Medicare and Medicaid Services (CMS), and the National Institutes of Health (NIH).

- *AHRQ*: AHRQ has the authority to conduct comparative clinical effectiveness research on health services and prescription drugs. AHRQ contracts with evidence-based practice centers (EPCs), affiliated with academic centers and private-sector organizations, to conduct the research, which most commonly involves systematic evidence reviews. AHRQ's annual budget for comparative effectiveness research was \$30 million in fiscal year 2008 (Alliance for Health Reform, 2008). AHRQ will receive an additional \$300 million for comparative effectiveness research under the American Reinvestment and Recovery Act of 2009 (ARRA; Pub. L. 111-5). The additional funding will build on AHRQ's current activities, with \$148 million devoted to evidence generation and \$30 million devoted to translating evidence into changes in practice (Agency for Healthcare Research and Quality, 2009).
- *CMS*: CMS uses information about the clinical effectiveness of treatments in its coverage decisions, but not in establishing provider payment rates. By statute, CMS is prevented from using cost considerations in making coverage decisions for Medicare (Medicare Payment Advisory Commission, 2008). In making coverage decisions, CMS may contract with EPCs or other entities to synthesize evidence.
- *NIH*: The NIH is the largest federal sponsor of clinical trials; however, a minority of its funding has been devoted to head-to-head comparisons of treatment alternatives (Medicare Payment Advisory Commission, 2008). ARRA allocated \$400 million to the NIH for additional comparative effectiveness trials.
- *FDA*: The FDA regulates the safety and effectiveness of new drugs and medical devices. It conducts evaluations before approving new treatments and performs postmarketing

surveillance to identify potential safety problems that did not surface during the clinical trials.

State Governments

State governments fund comparative effectiveness research through several interstate collaboratives. Washington State belongs to such a collaborative and also funds additional comparative effectiveness research.

- *The Drug Effectiveness Review Project (DERP)*: DERP is a self-governing collaboration among 12 organizations overseen by the Center for Evidence-Based Policy in Oregon.¹ DERP produces full systematic reviews of the literature on the effectiveness of major drug classes, such as beta blockers and statins. DERP reports are updated as new evidence for each drug class becomes available. All reports are made available to the public, and the results are used by public and private insurers to inform coverage decisions and by consumer groups as the basis for consumer guides.
- *Medicaid Evidence-Based Decisions Project (MED)*: The Center for Evidence-Based Policy in Oregon also oversees MED. MED reviews evidence on medical therapies, procedures, and devices to inform coverage decisions by state Medicaid programs. To date, MED has focused on reviewing and translating evidence syntheses from such sources as the Cochrane Database of Systematic Reviews and AHRQ. MED reports are available only to its 11 members.²
- *Washington State Health Technology Assessment (HTA) program*: Washington State's HTA program performs systematic reviews of the evidence on the cost, efficacy, and safety of medical devices, procedures, equipment, and diagnostic tests. The assessments are reviewed by a clinical panel, which makes coverage recommendations for state insurance programs, including Washington Health Care Authority plans, Medicaid, and the state Department of Labor and Industries, Department of Corrections, and Department of Veterans Affairs. The reports and decisions are also available to the public.

Private Sector

Several private-sector organizations and academic institutions conduct comparative effectiveness research on behalf of both private-sector clients, such as health plans, and public-sector clients. In this section, we discuss several of the most prominent groups.

- *Technology Evaluation Center (TEC)*: Blue Cross and Blue Shield Association's TEC evaluates such technologies as drugs, devices, procedures, and biological products for their influence on health outcomes, including length of life, quality of life, and functional abil-

¹ The 12 DERP participants are Arkansas, the Canadian Agency for Drugs and Technologies in Health, Colorado, Idaho, Maryland, Missouri, Montana, New York, Oregon, Washington, Wisconsin, and Wyoming.

² MED members include Medicaid programs from Alabama, Alaska, Arkansas, Minnesota, Missouri, New York, Oklahoma, Oregon, Washington, Wisconsin, and West Virginia.

ity. TEC completes between 20 and 25 assessments each year. It reports to both private- and public-sector clients, including Kaiser Permanente, AHRQ, and CMS.

- *Institute for Clinical and Economic Review (ICER)*: ICER, based at Massachusetts General Hospital's Institute for Technology Assessment, reviews evidence on the clinical effectiveness and comparative value of medical treatments and produces a rating system integrating effectiveness and value. ICER's four completed reviews, along with executive summaries, supporting documents, and information on its current project, are available to the public at no cost on the ICER Web site.
- *Tufts*: The Tufts Center for Evaluation of Value and Risk in Health is a federally funded evidence-based practice center that conducts systematic reviews of the cost-effectiveness of health care interventions and maintains the Tufts Medical Center Cost-Effectiveness Analysis Registry (CEAR). The registry provides publicly available electronic access to a comprehensive database of 4,400 cost-effectiveness results from the published medical literature.
- *Center for Medical Technology Policy (CMTP)*: Unlike most other existing CECs, the non-profit CMTP's primary activities are to develop and facilitate clinical studies, not to conduct evidence reviews. The clinical studies focus on methods for rapidly producing information needed for medical decisionmaking. These methods include "pragmatic trials," or clinical studies that are designed specifically to answer the questions faced by decision-makers, and analysis of clinical registries.
- *ECRI Institute*: The ECRI Institute is a nonprofit organization that conducts systematic evidence reviews for drugs, devices, procedures, and systems of care for clients.
- *Drug and device manufacturers*: Individual manufacturers typically sponsor research on the effectiveness of their products.

International

The United Kingdom and Germany have CECs that produce information that is used as the basis for coverage decisions by public insurance programs.

- *National Institute for Health and Clinical Excellence (NICE)*: Part of the United Kingdom's National Health Service and funded by the national government, NICE conducts systematic reviews of existing literature on the cost-effectiveness of medical interventions and prescription drugs. NICE reviews form the basis of coverage decisions by the National Health Service.
- *Institute for Quality and Efficiency in Health Care (IQWiG)*: Established in 2004, IQWiG is an independent German institution that works on commission from the Federal Joint Committee and the Federal Ministry of Health to evaluate the use, quality, and efficiency of medical services. It evaluates therapeutic and diagnostic services, pharmaceuticals, surgical procedures, diagnostic tests, clinical practice guidelines, and disease management programs. The Federal Joint Committee uses IQWiG reports to make binding coverage decisions.

Objectives of a Comparative Effectiveness Center

The goal of a CEC, as indicated in the legislature's instructions to the secretary, is to generate information that would be useful for making purchasing and payment decisions. To this end, the center's objectives could be to guide insurers' decisions about the coverage of specific treatments, benefit design, or provider payment methods. Another potential objective could be to provide information for physicians and patients. This approach, although not directly indicated in the legislature's instructions, could potentially improve health care quality and reduce costs by improving medical decisionmaking, independent of insurers' benefit and payment policies. This chapter outlines potential objectives for the CEC.

Potential Objective 1: Provide Information to Patients and Physicians to Guide Decisionmaking

A stronger evidence base on the comparative effectiveness of treatment alternatives could improve the information that patients and physicians use to decide between treatment alternatives. Dissemination of new information on the effectiveness of treatments to physicians and patients has had significant effects on practice patterns for many conditions. However, practice patterns can be slow to adjust following the publication of new research findings, so some level of utilization of less effective treatments often persists long after new findings are published.

There are several dissemination approaches that could potentially increase the rate of dissemination of new information and make a faster, larger impact on decisionmaking by patients and their physicians. An example of an effort targeted to physicians is the "drug detailing" initiative mandated in Chapter 305 to educate physicians about effective prescribing practices. An example of an initiative targeted to patients is *Consumer Reports Best Buy Drugs*, a consumer guide based on DERP research. AARP, formerly known as the American Association of Retired Persons, has produced a similar consumer guide based on DERP research. An excerpt from the *Consumer Reports* guide on diabetes drugs presents some general findings about the drugs reviewed and recommended before listing its best buys:

Our evaluation of the diabetes medicines found the following:

- Newer drugs are no better. Two drugs from a class called the sulfonylureas and a drug named metformin have been around for more than a decade and work just as well as newer medicines. Indeed, several newer drugs are less effective than the older ones.

- Newer drugs are no safer. All diabetes pills have the potential to cause adverse effects, both minor and serious. The drugs' side effect "profiles" may be the most important factor in your choice.
- Taking two diabetes drugs can improve blood sugar control. Many people with diabetes do not get enough help from one medicine. Two or more drugs may be necessary. However, taking more than one diabetes drug raises the risk of adverse effects and increases costs.

Taking effectiveness, safety, side effects, dosing, and cost into consideration, we have chosen the following as *Consumer Reports Best Buy Drugs*:

- Metformin—alone or with glipizide or glimepiride
- Glipizide and Glipizide Sustained Release—alone or with metformin
- Glimepiride—alone or with metformin

These medicines are available as low-cost generics. We recommend that you try metformin first unless your health status prevents it. If metformin fails to bring your blood glucose into the normal range, we advise adding glipizide or glimepiride. Should either of those cause problems, Actos (pioglitazone) may be an option you and your doctor will want to consider. Be aware that Actos has been linked to a higher risk of heart failure. (*Consumer Reports Best Buy Drugs*, 2009, p. 2)

Another potential dissemination approach is "shared decisionmaking," a process through which patients and their care providers are active participants in the communication and decisionmaking about their care (Charles, Gafni, and Whelan, 1999; Charles, Whelan, and Gafni, 1999). Comparative effectiveness information has been incorporated into "decision aids"—booklets or interactive software programs that are designed to simplify and clarify the decisionmaking process by giving a patient a clearer and more thorough understanding of his or her treatment options. Shared decisionmaking is most useful for decisions that involve significant trade-offs that patients and their physicians must weigh (Wennberg et al., 2007).

A review of 17 studies on patient decision aids showed that the aids generally improved knowledge of medical options and outcomes among patients (O'Connor et al., 1999). The Congressional Budget Office concluded that decision aids reduce the use of aggressive surgical procedures without affecting health outcomes and that the use of such aids on a broader scale could reduce health care spending (Congressional Budget Office, 2008). However, it was unable to estimate the effects increased shared decisionmaking on Medicare expenditures.

The following is an excerpt from a decision aid for patients considering magnetic resonance imaging (MRI) for low back pain that was created by the online health information clearinghouse WebMD:

Consider the following when making your decision about having magnetic resonance imaging (MRI):

- There is a good chance that your new (acute) low back pain will improve within 6 weeks—most cases do.
- Avoid requesting costly imaging tests for acute low back pain. They usually provide no helpful early information. If you don't have signs of a serious medical condition (such as spinal infection or bone cancer), don't ask for or agree to imaging tests unless

you have low back pain that has persisted through at least 4 weeks of nonsurgical treatment.

- MRI is the best test for showing a herniated disc, soft-tissue damage, tumor, or infection. MRI also has the advantage of not using the ionizing radiation that the CT [computed tomography] scan does.
- Your doctor will probably wait to order an MRI for nerve-related symptoms that don't go away within at least 4 weeks, because these symptoms often go away on their own.
- A technique called rapid MRI is probably no better than a standard X-ray for diagnosing the cause of back pain or deciding what treatment to use. (WebMD, 2008)

Potential Objective 2: Provide Information to Private and Public Insurers to Enable Changes in Reimbursement or Benefit Design

New evidence on the comparative effectiveness of treatments could be used by insurers to fine-tune reimbursement policies or benefit designs in order to encourage the use of effective treatments. Insurance benefits could be designed to increase patient demand for services with higher effectiveness and decrease demand for services with lower effectiveness. Previous studies have shown that the level of cost sharing affects consumers' demand for services (Newhouse, 1993). However, cost sharing is an effective tool only in changing utilization patterns in insurance programs in which out-of-pocket payments can be raised to decrease demand for services. In Medicaid, for example, cost-sharing levels are low or nonexistent in order to foster access to care.

While cost sharing targets consumers, there is also interest in targeting provider behavior by changing reimbursement methods, such as paying less for less-effective treatments while still covering them. This removes the current common incentive for providers to perform new procedures before a strong evidence base is necessarily established.

Several types of reimbursement policy could potentially be used to encourage the use of treatments shown to be more effective than alternatives. The main options are reference pricing, which would pay the same price for equivalent treatments, and financial incentives for the use of preferred treatments.

Reference pricing is used in several countries for payment for pharmaceuticals. Under this system, a reference price is determined for a group of drugs—typically the minimum or median price. Insurance companies reimburse the reference price for all drugs in the group. Individuals are responsible for any cost above the reference price. Evidence on the effect of reference pricing on costs is limited and mixed, but, overall, studies have concluded that it results in lower costs (Aaserud et al., 2006; Kanavos and Reinhardt, 2003). There are many challenges to implementing a reference pricing system, however, including the administrative structure for determining reference prices, the definition of “therapeutic clusters” that would be subject to a single reference price, and the determination of appropriate prices. Critics of reference pricing argue that it could stifle innovation and increase barriers to access for lower-income individuals. These effects have not been demonstrated conclusively, however (Kanavos and Reinhardt, 2003).

Another reimbursement approach is to pay bonuses to providers who provide evidence-based care (i.e., “pay for performance”). Existing pay-for-performance programs could be

expanded using new comparative effectiveness information. These programs are easiest to implement when the evidence is clear and indicators of evidence-based care delivery can be readily determined from existing data.

Potential Objective 3: Use Information to Guide Coverage Decisions in Insurance Programs

New comparative effectiveness information could be used to improve the evidence base that public and private insurers use to make coverage decisions. The Massachusetts state Medicaid program, MassHealth, includes cost-effectiveness in its definition of *medical necessity* for purposes of determining coverage for medical services, and other insurers in the state use similar definitions (Massachusetts Office of Health and Human Services, undated). The CEC would produce information tailored for use by insurers in these determinations.

An example of a state program that conducts comparative effectiveness research to inform coverage decisions in state public insurance programs is the Washington State HTA. Most of the decisions made to date have been to deny coverage for some or all Washington State insurance beneficiaries (see Table 5.1).

**Table 5.1
Coverage Decisions by Washington Health Technology Assessment**

Service	Description and Alternative(s)	Coverage Decision	Rationale
Implantable drug delivery system	Infusion pumps are surgically implanted devices used to deliver drugs to a specific site in the body, rather than relying on systemic levels of medication(s) administered orally or by other routes.	Not covered	Uncertain effectiveness and equivalent cost
Discography	Discography is a diagnostic test for discogenic pain. Controversy in using the test exists because the clinical importance of test results is unknown and there is a high rate of false positives.	Not covered	Uncertain effectiveness and higher cost
Upright/positional MRI	Upright/positional MRI is an imaging test designed to be performed on patients in weight-bearing or other positions. Alternative imaging tests used to diagnose spinal and other joint conditions are a regular MRI (lying down), CT myelogram, regular or flexion and extension radiographs (X-rays), and discography.	Not covered	Uncertain effectiveness and higher cost
CT colonography	CT colonography has been proposed as a less invasive alternative to conventional colonoscopy to screen for colorectal cancer, with the potential to induce more individuals to get screened.	Not covered	Equivalent effectiveness and higher cost
Knee arthroscopy	Surgical procedure to treat pain associated with osteoarthritis. Alternatives include physical and occupational therapy.	Not covered	Not effective and higher cost
Lumbar fusion	Lumbar fusion reduces back pain by surgically immobilizing the spinal column vertebrae surrounding the disc(s). Nonsurgical treatment alternatives for chronic low back pain include cognitive behavioral therapy, medications, and rehabilitation.	Covered under certain criteria	Higher effectiveness than usual care, equivalent effectiveness to intensive therapy and cognitive behavioral therapy, and higher cost
Pediatric bariatric surgery	Bariatric surgery is a surgical intervention intended to induce weight loss and resolve co-morbid conditions linked to obesity. Alternatives to bariatric surgery include dietary modification, increasing physical activity and exercise, behavior modification, and pharmacotherapy.	Not covered for patients under age 18; covered for patients 18–20 years old	More effective for patients 18–20 years old, uncertain effectiveness for patients under age 18, and uncertain cost

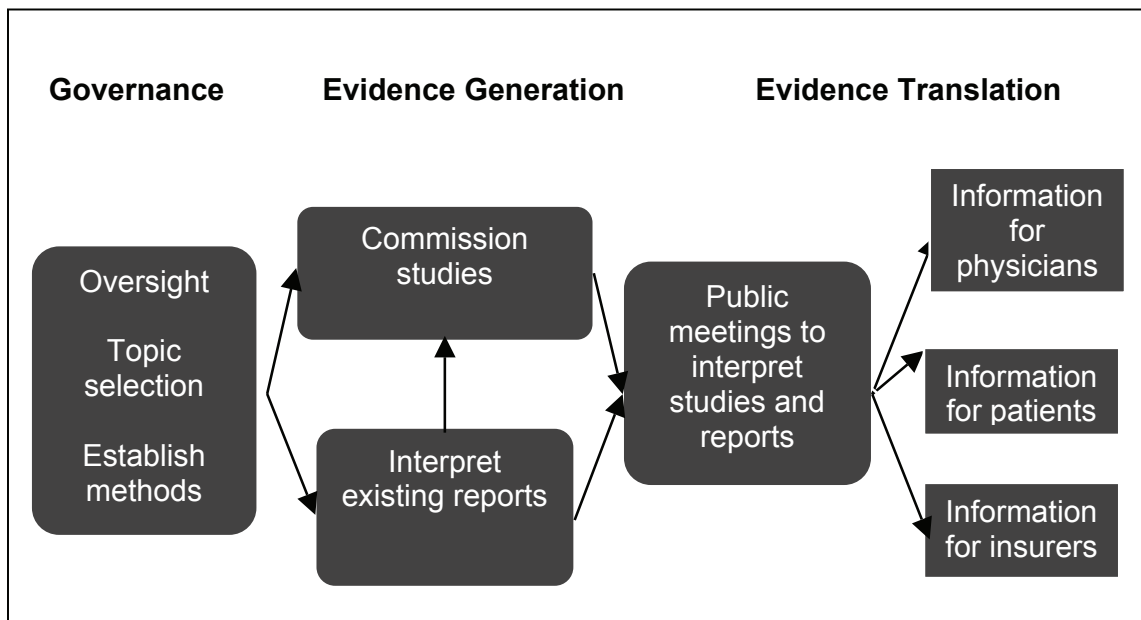
SOURCE: 2009 Washington State Health Technology Assessment Program data.

Design Options for an Interstate Comparative Effectiveness Center

The existing CECs listed in Chapter Four incorporate various models that could guide the establishment of an interstate CEC that includes Massachusetts. However, given the extent of activities by existing centers, a more compelling question is, How much value would be gained by establishing a new CEC, and how would a new CEC's role be coordinated with other comparative effectiveness activities?

Figure 6.1 outlines the main functions that will guide the design of a CEC. First, the CEC will need a process for ensuring oversight of its activities, establishing methods and procedures for those activities, and prioritizing topics for study. Second, the CEC will generate evidence on the comparative effectiveness of treatments. Since there are already many reports from existing centers, it is possible that the CEC will limit its purview to collating and interpreting these reports. Alternatively, the CEC could commission new studies from research institutions. Finally, it will need a process of “evidence translation,” or producing information and recommendations that local decisionmakers can use. This will likely require public meetings of local medical and public health experts who review the evidence generated by the CEC

Figure 6.1
Potential Functions of a Comparative Effectiveness Center



and gather input from stakeholders. Finally, the recommendations made through these public meetings will need to be produced and disseminated to target audiences. The target audiences could include physicians, patients, and insurers, depending on the objectives of the CEC, as discussed earlier.

These functions could all be performed by a new CEC, or some functions could be performed collaboratively by existing organizations. In this chapter, we outline five design options for a CEC.

Option 1: An interstate CEC could be established to provide a framework for use of existing comparative effectiveness reports by regional decisionmakers. This option would establish a new regional organization that would focus on collating, interpreting, and disseminating existing comparative effectiveness information from other sources. This option would address the issue that there are many comparative effectiveness studies that have been published, but there is no organizational framework to translate the evidence into actionable information for New England decisionmakers. This option would establish a new entity either within existing government organizational structures or as an independent, government-funded organization. The CEC would include mechanisms for governance, such as an independent board of directors. The board would approve methods for prioritizing topics for analysis, collecting and interpreting studies, and disseminating results. To perform evidence generation functions, the CEC would collate and summarize evidence reports from external sources. This function could be performed by CEC staff or through contracts with research organizations. To perform evidence translation functions, the CEC would convene a panel of regional physicians and public health experts. The panel would hold public meetings to review comparative effectiveness research reports produced by other organizations and make recommendations for purchasing or clinical decisionmaking, depending on the objectives defined for the CEC.

In establishing a regional CEC, it may be possible to build on infrastructure that is in place in the form of existing regional collaboratives. One example is the New England Collaborative, an initiative by state health departments to coordinate efforts to establish medical home pilot programs. However, comparative effectiveness would have to compete for priority with ongoing work by regional collaboratives.

Option 2: An interstate CEC could be established to support new comparative effectiveness research. This option would create the framework for evidence translation as in option 1 and also commission new comparative effectiveness research studies.¹ The studies would likely be conducted by existing research organizations under contract with the CEC. (Many such highly regarded research institutions are located in New England.) The new research findings would then be used in an evidence translation process as outlined in option 1. In addition, the research findings could be published for use by others outside the region, adding to the scientific evidence base.

A regional center would ensure that comparative effectiveness information was available in priority areas for the region, although many priorities are likely to be shared with other regions of the country. Possible reasons that regional priorities may vary include differences in the population disease profile and differences in the utilization of services. For example, the rate of colonoscopy screening is relatively high in Massachusetts and neighboring states compared

¹ There are several different types of studies that could be commissioned, as discussed in Chapter Seven.

to other regions of the country (Kaiser Family Foundation, 2009); this may affect the interest in, and decisionmaking around, “virtual” colonoscopies, a less invasive test that is covered by many private insurers but not by Medicare. It is possible that priorities for Massachusetts may be more closely aligned with those of states outside the region than with those of other New England states—for example, states that have similar delivery systems (e.g., many academic medical centers), similar urban/rural distribution of the population, or other characteristics.

The establishment of a regional CEC would require resources to establish and administer the program, as well as resources to support the development and dissemination of comparative effectiveness information. The amount of funding required could vary widely, depending on the number of comparative effectiveness studies conducted, the design and scope of the studies, and the extent of dissemination. Based on the budgets of existing CECs, such as Washington State HTA and DERP (see Table 6.1), a rough estimate of the annual funding required would be \$1–1.5 million. Massachusetts’s share would depend on how many other states participated and how funding responsibility was allocated among participating states. Based on the experience of HTA and DERP, a budget of this size could fund approximately seven evidence syntheses per year. However, it is unclear whether seven syntheses per year would be the optimal level of output for a regional CEC that includes Massachusetts.

Option 3: Massachusetts could join existing interstate CECs (i.e., DERP and MED). Joining existing CECs would allow Massachusetts to provide input into the content and priority of comparative effectiveness reviews and would provide Massachusetts with access to comparative effectiveness information that is limited to participants, without incurring the cost of establishing a new CEC. Fourteen states and the Canadian Agency for Drugs and Technologies in Health currently participate in DERP. Eleven state Medicaid programs currently participate in MED. The cost of membership would be approximately \$90,000 per year for DERP and \$130,000 per year for MED.

Unlike a regional CEC (option 1 or 2), participating in DERP and MED may provide information that is less closely aligned with Massachusetts’s priorities, since all participants have equal input. However, participation would offer access to services that can be used in decisionmaking. MED members receive reports that are not available to nonparticipants. Members also have access to policy and evidence consultation experts who provide guidance

Table 6.1
Funding for Selected Existing Comparative Effectiveness Centers

Name	Funding
Washington State HTA	\$1.2 million in 2006
DERP	\$1.4 million annual average since 2002
AHRQ Effective Health Care Program	\$30 million in 2008
Federal funding in the American Recovery and Reinvestment Act of 2009 (ARRA)	\$1.1 billion in 2009, until expended
NICE (UK)	\$35–50 million annually

SOURCES: Agency for Healthcare Research and Quality (2009); Medicare Payment Advisory Commission (2008); Pub. L. 111-5 (2009); University of Washington Office of State Relations data.

for decisionmaking, and members can take advantage of collaboration with other participating state Medicaid programs. DERP evidence reports are available to the public, but DERP participants receive summaries of reports that are not available to nonmembers.

Option 4: Massachusetts could join DERP and MED and also establish a regional CEC.

Massachusetts could combine options 2 and 3 in order to take advantage of the existing infrastructure of DERP and MED while allocating additional resources to regional comparative effectiveness priorities through a new CEC. This would require the highest level of funding of the five options presented here. The option does have a precedent: Washington State participates in both DERP and MED and also supports the HTA program.

Option 5: Status quo. Massachusetts could elect not to establish or join a CEC. Local stakeholders could continue to rely on existing decisionmaking processes and activities sponsored by other entities for comparative effectiveness information.

Other Design Considerations for a Comparative Effectiveness Center

How Will Comparative Effectiveness Information Be Produced?

If a new interstate CEC commissions new research (option 2 or 4), there are four main types of research that could be conducted. Each has very different cost implications. Existing CECs mainly conduct syntheses of existing evidence; under option 1, a New England regional CEC would likely rely on these syntheses.

Conduct systematic reviews of available evidence. Systematic reviews provide a rigorous framework for evaluating evidence from existing studies. A commonly used method for reviews is “meta-analysis,” in which results from multiple studies are pooled using statistical techniques to formulate a single, overall estimate. Many systematic reviews also include products that aim to translate scientific findings for different audiences, such as decisionmakers, consumer advocates, or physicians. This approach is most useful for evaluating a large, mixed evidence base from previous studies. It is least useful when there are few high-quality studies. Systematic reviews are generally less expensive to conduct than new studies. The funding for systematic reviews supported by AHRQ generally averages between \$50,000 and \$300,000 per study (Agency for Healthcare Research and Quality, 2007). Most of the existing comparative effectiveness centers primarily conduct systematic reviews.

Support the generation of new evidence from existing data for treatments with a weak evidence base. New observational studies could be performed retrospectively using existing data sets, such as insurance claims. These studies can add to the evidence base at lower expense than prospective clinical trials but are not as rigorous for two reasons. First, since patients are not randomized to treatments, the studies may be biased by differences between the groups of patients receiving each treatment. Second, readily available data used in observational studies, such as insurance claims, include limited clinical information that may limit the outcomes that can be evaluated or the ability to adjust for differences in the illness severity of patients receiving one treatment versus another. In some instances, these data may be linked to other sources of information to improve their utility. For example, claims data have been linked to the National Death Index to study the survival benefits of different treatments. Other sources of clinical information, such as medical records, are more difficult and costly to collect. In the future, it is possible that expanded use of electronic health records will facilitate the collection of clinical data.

Support the generation of new evidence from patient registries. A patient registry is “a systematic collection of a set of health and demographic data for patients with specific health characteristics held in a defined database for a predefined purpose” (Levine and Julian, 2008). Health plans, medical societies, and product manufacturers maintain such registries to track

the effects of treatments over time. A CEC could facilitate the use of patient registries to produce new information on the comparative effectiveness of treatments. One way registries can be used is through a policy of “coverage with evidence development,” which is used by CMS for a limited number of treatments (Tunis and Pearson, 2006). Under this type of policy, Medicare covers promising new treatments on the condition that data on health outcomes are collected in a registry. The policy is also used to require participation in clinical trials. For example, Medicare is collecting health outcomes data from a mandatory registry for certain patients receiving implantable cardioverter defibrillators (Centers for Medicare and Medicaid Services, 2005). Other registries collect data on patients with a particular disease or condition. Product- or service-specific registries can be used to compare how treatment effects differ across patient subgroups or between providers, while disease-specific registries could be used to compare the effectiveness of different treatments for that disease.

Support the generation of new evidence from clinical trials. A CEC could support prospective randomized clinical trials to generate new information on the relative effectiveness of treatments. While clinical trials have the potential to provide rigorous evidence, they are expensive and time-consuming. Head-to-head clinical trials conducted recently by the NIH averaged \$77.8 million, ranging from \$12 million to \$176 million (National Institutes of Health, 2007). Due to the level of funding required, sponsorship of new clinical trials is likely not a viable option for a regional CEC.

How Will Research Topics Be Selected?

Chapter 305 of the Massachusetts Acts of 2008 states that treatments examined by a potential CEC should include medical procedures, drugs, devices, and biologics. The scope of a CEC could also potentially be expanded to include different methods for care delivery, as has been recommended by the Institute of Medicine. The “treatments” being compared would include defined processes for delivering medical interventions, not just the medical interventions themselves. For example, limited evidence exists on the effectiveness of alternative chronic disease management approaches, such as the use of telephonic reminder systems to encourage medication adherence. IQWiG in Germany is an example of an existing CEC that includes both care delivery methods and medical treatments as research topics.

If Massachusetts enters into an interstate compact to create a new regional CEC, a transparent process must be established to prioritize treatments selected for review. A similar process would be required whether the CEC was providing a framework for translating existing reports (option 1) or commissioning new research (option 2 or 4). If Massachusetts joined existing collaborations, such as DERP and MED (option 3), research topics would be selected using existing prioritization processes.

Candidates for study topics could be identified through nominations from the public and employees of the CEC or state health programs. The Washington HTA and the AHRQ Effective Health Care program are examples of programs that accept nominations by the public via their Web sites. Criteria would then need to be developed and applied to select the subjects for study from among the nominations. Examples of potential criteria for selecting treatments for comparison are summarized in Table 7.1.

Table 7.1
Potential Criteria for Selection of Treatments for Comparative Effectiveness Research

Criterion	Example of How Criterion Could Be Quantified
Impact on health	Mortality rate relevant to condition treated
Impact on health care spending	Total annual spending for treatment
Size of population affected	Number of treatments delivered per year
Geographic variation in utilization rates	Ratio of treatment volume in high- and low-utilization regions
Growth rate in costs and/or volume	Average annual growth in mortality rate or total annual spending for past five years
Strength of existing evidence base	Number of clinical trials addressing treatment
Complexity of treatment decisions	Existence of conflicting evidence on effectiveness
Extent of social, legal, or ethical issues	Number of lawsuits related to treatment

SOURCE: Authors' analysis.

Should the CEC Evaluate Clinical Effectiveness or Cost-Effectiveness?

Many existing CECs evaluate clinical effectiveness only and have excluded cost-effectiveness from their purview. This has been the approach of federal agencies, such as AHRQ and CMS, and other organizations, such as DERP. Political opposition to the use of cost-effectiveness information in decisions affecting treatment alternatives is typically strong. However, others advocate that, given the growing unaffordability of health care, it is necessary to consider the cost implications of treatment alternatives. Consideration of costs increases the likelihood that comparative effectiveness research could lead to reduced health spending (Congressional Budget Office, 2007).

The CEC could review clinical effectiveness only, not cost. Under this approach, the CEC would not be involved in the use of cost considerations to influence treatment availability. This should decrease political opposition to a CEC. However, it is likely that decisionmakers, such as insurers using the clinical effectiveness reviews, would consider cost information separately. For example, insurers often compare the drug effectiveness information published in DERP reports to the prices they pay for drugs to make coverage decisions. These comparisons, since they are not conducted within the established DERP review process, are not necessarily transparent to the public, and the methodological standards of such analyses are unclear. For example, cost comparisons may be conducted using only the price of the treatment or drug. More rigorous evaluations of cost could consider the full range of costs of a particular treatment option over time. The exclusion of explicit consideration of cost could contribute to distrust of a CEC, since stakeholders may assume that cost information is being used in decisionmaking, but in a nontransparent manner (Neumann, 2006).

Alternatively, the CEC could review both clinical effectiveness and cost. There are several ways that this is done by existing CECs, and different approaches could have different effects in diffusing opposition. Several existing CECs report clinical effectiveness and cost separately, rather than together as a single cost-effectiveness ratio. For example, the Washington HTA summarizes its reviews in three categories: clinical effectiveness, safety, and cost-effectiveness.

In its recommendations, HTA indicates whether cost-effectiveness was a strong factor in its decision process or whether the decision was based entirely or predominantly on clinical considerations. ICER reports comparative clinical and cost-effectiveness data using a grid, with clinical effectiveness on one axis and cost on the other. NICE, on the other hand, uses an explicit threshold of cost-effectiveness for its recommendations. However, it has made exceptions in the application of this threshold.

If the CEC reviews cost-effectiveness, an open, transparent process would be critical to diffusing opposition. For example, the Washington HTA has used open, public meetings for decisionmaking and incorporates feedback from patients, drug and device makers, and other stakeholders in decisions. NICE has also convened a citizens' council to allow for greater public input on its recommendations.

Conclusions

This report outlined several design options that Massachusetts could follow in establishing an interstate CEC. The choice of design option will be determined by the specific objectives of the legislature and by the legislature's prioritization of comparative effectiveness research relative to other options under consideration for improving quality and reducing spending growth in health care. All of the options presented in this report should be technically feasible to implement, given the political will. However, as noted earlier, the implementation of a government-funded CEC would likely encounter significant political opposition. Certain design decisions would likely increase political opposition to a CEC. In particular, the consideration of cost-effectiveness and the use of comparative effectiveness information in decisions about the coverage of specific treatments have been very controversial.

Participants of a meeting of New England state representatives expressed strong interest in establishing a CEC. After considering the design options presented in this report, meeting participants expressed the strongest interest in beginning with option 1 and potentially expanding to other options. Under this approach, Massachusetts would enter into an interstate compact with other New England states to create a framework for translating comparative effectiveness information into actionable recommendations for local decisionmakers. Meeting participants felt that recommendations coming from such an organized framework may be viewed as a "trusted source" by local physicians, patients, and other stakeholders. Meeting participants also felt that collaboration among New England states made sense, given its merged medical marketplaces.

ARRA included funding for comparative effectiveness research that could potentially provide seed money for a regional effort. ARRA allocated \$1.1 billion among AHRQ, the NIH, and the U.S. Department of Health and Human Services. In a notice of intent to publish grant and contract solicitations released on August 7, 2009, AHRQ announced that it would provide \$29.5 million to "support innovative translation and dissemination grants" for comparative effectiveness research, with solicitations published beginning in fall 2009 and funding commencing in spring 2010. A potential barrier is that the ARRA prohibits the federal coordinating council overseeing the use of the ARRA comparative effectiveness funding from taking action that could be construed "to mandate coverage, reimbursement, or other policies for any public or private payer." At this time, it is unclear whether this stipulation will apply beyond the activities of the council itself to all AHRQ grants and contracts for translation and dissemination activities.

Representatives from New England states expressed an interest in continuing to meet to discuss design options further. Topics that will need to be addressed include specific objectives for a CEC, funding sources, legislative/regulatory authority, and organizational structure.

In future steps, Massachusetts and other New England states could potentially build on such a framework by commissioning additional comparative effectiveness research to fill gaps in existing information. This could be accomplished by commissioning studies from established research centers (option 2) and/or joining existing state collaborations (DERP and MED) (option 3).

Massachusetts is at the forefront of the national health reform debate and is considering a variety of innovative approaches to improve the quality and affordability of care in the state. However, other state governments have been more active to date in sponsoring and using comparative effectiveness research. Massachusetts could potentially become a leader among states in this area. New England is world-renowned for its clinical research enterprise and its academic medical centers could help in establishment and functioning of a CEC. In addition, New England states have a track record of collaboration on health policy issues that could be extended to comparative effectiveness research.

Given the increase in federal funding and attention to comparative effectiveness research, a central question is whether additional funding by Massachusetts is a priority among the policies being considered. The increasing federal role in this area could be interpreted as a sign that state efforts are becoming less important. On the other hand, the increased federal role could make a regional CEC *more* important. There will be an increasing amount of evidence available that must be weighed by local decisionmakers, and federal funding could also support the creation of a regional CEC.

Existing Comparative Effectiveness Centers

In this appendix, we compare existing CECs according to their role and organization, selection of treatments for comparison, methods used for comparisons, and uses of the comparisons. Table A.1 presents a summary profile of each center.

Table A.1
Summary of Existing Comparative Effectiveness Centers

Characteristic	AHRQ	CMTF	CEAR	DERP	ECRI	ICER	IQWiG	MED	NICE	TEC	HTA
Affiliation	Federal gov't	Private nonprofit	Academic nonprofit	State gov't coalition	Private nonprofit	Academic nonprofit	National gov't (Germany)	State gov't coalition	National gov't (UK)	Private, Blue Cross and Blue Shield Association	State gov't
Funding sources	Federal budget appropriations	Various public- and private-sector sources	Public- and private-sector grants	Participating state gov'ts	Various public- and private-sector sources	Various public- and private-sector sources	National government	Participating state gov'ts	National gov't	Participating Blue Cross and Blue Shield plans	State gov't
Availability of products	Publicly available	Publicly available	Publicly available	Main reports publicly available; other products for members only	Most available to members only	Publicly available	Publicly available (mainly in German)	Available to members only	Publicly available	Publicly available	Publicly available
Type of treatments reviewed	Medical procedures and drugs	Medical procedures	Health care interventions (not specified)	Drug classes	Not specified	Medical procedures, devices, and biologics	Medical procedures, drugs, disease management programs	Medical procedures and devices	Medical procedures, drugs, devices, health promotion activities	Medical procedures, drugs, and biologics	Medical procedures and devices
Basic methods	Systematic reviews	Clinical trials	Systematic reviews	Systematic reviews	Systematic reviews	Systematic reviews and decision modeling	Systematic reviews	Systematic reviews	Systematic reviews	Systematic reviews	Systematic reviews
Intended use of products	Inform consumer and clinician decisionmaking	Inform a variety of applications, including coverage decisions	Not specified	Inform state insurance benefit design and coverage; also used in consumer education	Not specified (may differ by client)	Inform benefit design, reimbursement, and coverage decisions	Inform national insurance coverage decisions	Inform Medicaid coverage decisions	Guidance for providers	Inform benefit design, reimbursement, and coverage decisions	Inform state insurance coverage decisions

Agency for Healthcare Research and Quality

Role and Organization

AHRQ was designed to both sponsor and conduct evidence-based research on health care outcomes, quality, and cost. The Effective Health Care Program, which emerged from Section 1013 of the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (Pub. L. 108-173), is authorized to review and synthesize existing literature and to generate new scientific evidence on clinical effectiveness. The program is a collaboration of EPCs, the Developing Evidence to Inform Decisions about Effectiveness (DEcIDE) Network, and the John M. Eisenberg Clinical Decisions and Communications Science Center.

Selection of Treatments for Comparison

AHRQ's priorities for research are topics that are most relevant for Medicare, Medicaid, and State Children's Health Insurance Program decisionmakers. Effective Health Care Program research focuses on the following conditions: arthritis and nontraumatic joint disorders, cancer, cardiovascular disease (including stroke and hypertension), chronic obstructive pulmonary disease/asthma, dementia (including Alzheimer's), depression (and other mood disorders), diabetes mellitus, functional limitations and disability, infectious diseases (including HIV/AIDS), obesity, peptic ulcer/dyspepsia, pneumonia, and substance abuse. Anyone is allowed to suggest research topics to the Effective Health Care Program.

Methods Used for Comparisons

AHRQ can conduct its own assessment reports or can contract out to its EPCs. The EPCs develop comparative effectiveness reports by performing systematic reviews of all available literature, both published and unpublished, and weighing the risks and benefits of various treatments. The DEcIDE Network is a collection of research centers across the country that runs trials and conducts studies to generate new evidence to fill any existing gaps. The John M. Eisenberg Clinical Decisions and Communications Science Center specializes in translating dense scientific research into audience-specific, digestible information.

Uses of Comparisons

Many AHRQ publications are available for free to the public on the AHRQ Web site; others may need to be purchased. AHRQ shares its guidelines on the National Guidelines Clearinghouse Web site, where they are searchable for the discretionary use of health professionals, providers, and health plans. CMS also uses AHRQ technology assessments to make national Medicare coverage decisions. (See Agency for Healthcare Research and Quality, Effective Health Care Program, undated.)

Center for Medical Technology Policy

Role and Organization

CMTP in Baltimore is a private, nonprofit organization that provides a forum for patients, clinicians, payers, manufacturers, and researchers to collaborate to generate new evidence for health care decisionmakers. CMTP gets 33 percent of its funding from health plans, 20 percent from foundations, 5 percent from the government, and 42 percent from other sources. Funding sources include Aetna, AHRQ, Blue Shield of California Foundation, California Healthcare Foundation, The Commonwealth Fund, Institute of Medicine, Johnson and Johnson, Kaiser, National Pharmaceutical Council, Pfizer, and United Healthcare Foundation.

Selection of Treatments for Comparison

In May of 2006, CMTP selected five new technologies for study: radiation therapy for prostate cancer, delivery of ICU care through telemedicine, minimally invasive bariatric surgery, gene expression profiling tests for early stage breast cancer, and CT angiography for diagnosing coronary artery disease.

Methods Used for Comparisons

Unlike most other existing CECs, CMTP's primary activities are to develop and facilitate clinical studies designed to produce the information needed by decisionmakers, not systematic reviews of available evidence. The clinical studies focus on methods for rapidly producing needed information, such as "pragmatic trials" (clinical studies that are designed specifically to answer the questions faced by decisionmakers) and clinical registries. Multistakeholder working groups identify key questions and develop methodologies and study protocols. Effectiveness Guidance Documents are then drafted and the group reviews the documents and posts them on the CMTP Web site for public comment. These comments are then considered and the completed guidance documents are posted.

Uses of Comparisons

Effectiveness Guidance Documents provide guidance on how to design clinical studies that will provide needed evidence, evidence standards that can be applied in making reimbursement and coverage decisions, and a research framework for researchers and funding agencies. The documents are available to the public. (See Center for Medical Technology Policy, undated.)

Cost-Effectiveness Analysis Registry at Tufts Medical Center

Role and Organization

CEAR was created to be a single source for benchmark information on “cost-utility analysis,” or health-related cost-effectiveness measures that consider quality of life and added life years. CEAR is a part of the nonprofit Institute for Clinical Research and Health Policy Studies at Tufts Medical Center in Massachusetts. CEAR has had a variety of financiers since its inception in 1976 (including the National Science Foundation, AHRQ, National Library of Medicine, and, presently, individual subscribers and other project-related grants such as a recent one for the National Cancer Institute), but it does not accept money attached to research directives. The CEAR catalogue contains more than 5,300 cost-effectiveness ratios and more than 7,000 utility weights from existing published literature.

Selection of Treatments for Comparison

CEAR includes all published articles identified through a systemic review of the literature that include a cost-utility analysis and meet certain methodological criteria.

Methods Used for Comparisons

The CEAR team performs systematic reviews to search for articles that contain an original cost-utility estimate. The team excludes articles that are reviews or editorials or that contain a cost-effectiveness analysis not measured in quality-adjusted life years (though it is considering an expansion to other metrics, such as cost per life or even noneconomic values). Three main types of information are collected on each article: methodology used, cost-effectiveness ratio, and “utility weight,” or measured preferences for particular health outcomes or health states.

Uses of Comparisons

The registry is online and available to the public. CEAR does not track how the information is used. (See Cost Effectiveness Analysis Registry, undated; this section is also based on the authors’ interview with Peter Neumann, CEAR principal investigator.)

Drug Effectiveness Review Project

Role and Organization

DERP is a self-governing collaboration that produces public reports and report updates on the comparative clinical effectiveness of drugs in major drug classes. The Center for Evidence-Based Policy in Oregon oversees DERP, which began as a collaboration among the Oregon Health and Resources Commission, the Washington Health Care Authority, and the Idaho Department of Health and Welfare. DERP has since expanded to 12 states and organizations: Arkansas, the Canadian Agency for Drugs and Technologies in Health, Colorado, Idaho, Maryland, Missouri, Montana, New York, Oregon, Washington, Wisconsin, and Wyoming. Each member contributes equally to DERP funding and has an equal voice in setting the priority list for DERP studies. Each member also has an equal say in the drug classes to be studied, the key questions to be asked, and the timelines and quality of draft reports. The membership cost is contingent on the number of groups involved; currently, membership is \$87,000 annually, with matching funds from the federal government.

Selection of Treatments for Comparison

When choosing a drug class to review, DERP considers disease burden, alternatives, scope, clinical impact, budget impact, evidence, marketing, and benefits/policy considerations. DERP has completed systematic reviews of 32 classes of medications. In drug classes with ongoing research, DERP performs report updates. Members determine which classes will receive updates.

Methods Used for Comparisons

Each study begins with a systematic review of existing literature. Data from existing studies are reviewed for quality and then compiled into evidence tables in a report. Information is solicited from experts and pharmaceutical companies. All reports are peer-reviewed, drafts are circulated among experts, and, when applicable, drafts are posted for public comment. DERP reports contain no cost-effectiveness analysis.

Uses of Comparisons

DERP has both private and public components. The key questions, evidence tables, and all draft and final reports are posted on the DERP Web site for public use. Executive summaries (intended for policymakers), Pharmacy and Therapeutics Committee briefs, and slide presentations are available for the proprietary use of DERP members only. Final DERP reports are not usage guidelines, though states are free to take and use the information at their own discretion. DERP reports are nonbinding and application is subject to normal state and local decisionmaking processes. DERP reports are widely referenced, both nationally and internationally. Consumers Union, publisher of *Consumer Reports* magazine, has created a Web site that uses DERP reports to give consumer advice. The project translates DERP findings and, with additional information from other sources, chooses “best buy” drugs in each drug class. AARP, formerly the American Association of Retired Persons, also uses DERP reports to provide information to patients. (See Center for Evidence-Based Policy, undated[a]; this section is also based on the authors’ interview with Mark Gibson, DERP deputy director.)

ECRI Institute

Role and Organization

The ECRI Institute is a nonprofit research organization whose clients include hospitals, health systems, public and private payers, U.S. federal and state government agencies, ministries of health, voluntary-sector organizations, associations, and accrediting agencies. ECRI has an evidence-based practice center through AHRQ and works with CMS to inform national coverage determinations.

Selection of Treatments for Comparison

ECRI performs customized assessments at the request of its members.

Methods Used for Comparisons

ECRI performs systematic reviews of existing literature in its evidence reports and, when possible, conducts statistical analysis of the existing evidence. Six main products are available ECRI members: evidence reports, systematic reviews with statistical analyses, emerging technology systematic reports, forecasts of health care technologies and services under research and development, guides to the published literature about technologies and services, a health technology trends newsletter, and access to the International Health Technology Assessment Database.

Uses of Comparisons

ECRI reports are used in various ways, including in coverage and reimbursement decisions. (See ECRI Institute, undated.)

Institute for Clinical and Economic Review

Role and Organization

ICER is an academic center that conducts reviews of the clinical and cost-effectiveness of medical treatments. Created in 2006, ICER is physically located at the Massachusetts General Hospital's Institute for Technology Assessment. ICER's executive leadership consists of a president and senior management team, and an advisory board provides strategic guidance. ICER accepts funding from a variety of public and private sources.

Selection of Treatments for Comparison

ICER assesses health care interventions, prioritizing those that have a greater impact on health quality and cost. ICER reviews are also designed to assess interventions in clinical areas where reasonable treatment alternatives are available.

Methods Used for Comparisons

ICER performs systematic reviews of existing literature and confers with experts to determine clinical effectiveness and "comparative value." The evidence review is used to create an assessment, known as the ICER Integrated Evidence Rating™. The rating model, based on the evidence-based medicine matrix, has a three-tiered vertical confidence scale (low, limited, or high) and a four-category horizontal benefit scale (from inferior net benefit to large net benefit). Any technology given a vote of low confidence is automatically labeled "insufficient"; anything rated "limited confidence" is either "insufficient" or "unproven but potential," depending on the net benefit; and anything with high confidence is rated A (for "superior"—high confidence of a moderate-to-large net health benefit), B (for "incremental"—high confidence of a small net health benefit), C (for "comparable"—high confidence of a comparable net health benefit), or D (for "inferior"—high confidence of an inferior net health benefit), depending on the net benefit. The ICER comparative value rating is based on cost savings, cost per additional quality-adjusted life year, cost per key outcome, and cost relative to similar treatments/situations. ICER reviews consider the "downstream effects" that an intervention could have on future health care utilization.

Uses of Comparisons

ICER creates its reports for public dissemination; appraisals are publicized as soon as they are final, along with supporting documents and a final executive summary. Reports contain a summary of existing literature, comparisons to other technologies, recommendations for future reviews, and ratings of comparative value. It is ICER's intention that reports will be used as tools when shaping benefit design, reimbursement strategies, and coverage policies. (See Institute for Clinical and Economic Review, undated; this section is also based on the authors' interview with Steven Pearson, ICER president.)

Institute for Quality and Efficiency in Health Care

Role and Organization

Established in 2004, IQWiG is an independent German institution that works on commission from the German Federal Joint Committee and the Federal Ministry of Health to evaluate the quality and efficiency of medical services. IQWiG has two management boards: the Steering Committee, which comprises department heads and coordinates internal and external affairs, and the Foundation, which is bicameral and includes the Foundation Board, which represents regional health care funds and providers of services, and the Board of Directors, IQWiG's supervising body. Other advisory committees include members of patient groups and national and international scientists. IQWiG is funded through taxes on health care services.

Selection of Treatments for Comparison

IQWiG reports are commissioned by the Federal Joint Committee or Federal Ministry of Health, but working papers and public health Web postings can be created through an autonomous initiative. IQWiG evaluates therapeutic and diagnostic services, pharmaceuticals, surgical procedures, diagnostic tests, clinical practice guidelines, and disease management programs.

Methods Used for Comparisons

IQWiG obtains information from systematic reviews of existing national and international studies; it does not perform its own clinical trials. It looks for studies of services that lengthen life, reduce symptoms or improve quality of life. IQWiG is legally mandated to use evidence-based medicine methods to choose and review the studies. German health care reform in 2007 expanded IQWiG reviews beyond clinical effectiveness to include cost-benefit analysis. The institute solicits input from manufacturers, professional associations, and patients throughout the process.

Uses of Comparisons

The IQWiG produces four different products depending on their intended usage. The Institute publishes health information for consumers and patients on its Web site and produces working papers on methodology for health analysis or general relevant health topics. The IQWiG also publishes both rapid and detailed reports, which are conducted solely on commission. Reports are published on the IQWiG Web site (mainly in German). Commissioned reports help the Federal Joint Committee make binding coverage decisions for Statutory Health Insurance plans although, in addition to the reports, the Committee also considers individual situations and specific populations. (See Institute for Quality and Efficiency in Health Care, 2009.)

Medicaid Evidence-Based Decisions Project

Role and Organization

In addition to DERP, the Center for Evidence-Based Policy in Oregon oversees MED. Like DERP, MED is self-governed by its member organizations, which include Medicaid programs from Alabama, Alaska, Arkansas, Minnesota, Missouri, New York, Oklahoma, Oregon, Washington, Wisconsin, and West Virginia. Membership in MED currently costs \$130,000 annually but is likely to decrease as additional members join. Although both MED and DERP are part of the Center for Evidence-Based Policy, the two projects review different technologies, use different report methodologies, and have different policies about public dissemination of their evidence.

Selection of Treatments for Comparison

MED reviews medical therapies, procedures, and devices. It produces different types of reports depending on usage needs: Rapid appraisal reports are answers to narrow questions, immediate policy/evidence consultations are useful for policymakers, and full systematic reports synthesize and analyze all existing evidence on a topic. Like DERP, MED examines clinical effectiveness only, not cost-effectiveness.

Methods Used for Comparisons

MED uses existing evidence from such sources as the Cochrane Database of Systematic Reviews, AHRQ, NICE, and others and synthesizes and summarizes results for policymakers. The topics covered by MED are more diverse than those covered by DERP, and, therefore, the process for producing each report varies according to available information and the key questions to be addressed.

Uses of Comparisons

MED reports are currently not published online and are the private property of MED's 11 members. The MED members typically use the reviews to inform coverage decisions. (See Center for Evidence-Based Policy, undated[b]; this section is also based on the authors' interview with Mark Gibson, MED deputy director.)

National Institute for Health and Clinical Excellence

Role and Organization

NICE, an independent organization that is part of the United Kingdom's National Health Service and funded by the national government, conducts systematic reviews of existing literature on medical interventions and prescription drugs. NICE is predominantly funded by the UK Department of Health (DOH); in 2007–2008 NICE received £33.4 million from the department and £1 million from other sources. NICE is overseen by a senior management team and a board that sets strategic direction, oversees delivery, and works with collaborative organizations. NICE is divided into centers, which deliver the guidance, and directorates that support the centers.

Selection of Treatments for Comparison

NICE is commissioned to develop three types of health guidance: (1) public health, promotion of good health, and prevention of illness; (2) clinical practice, National Health Service guidance on appropriate treatments, and patient care; and (3) health technologies. Health technologies include medicines, medical devices, diagnostic techniques, surgical procedures, and health-promotion activities. Guideline topics are suggested by a number of sources, including clinical and health professionals, patients, the Department of Health, and the National Horizon Scanning Centre, and from within NICE; Department of Health ministers have the final say in topic selection.

Methods Used for Comparisons

The National Collaborating Centre, with input from NICE and registered stakeholders, determines key questions to frame the scope of NICE reports. Then, a guideline development group of health professionals, representatives of patient groups, and technical experts are assembled to draft the guidelines. Draft guidelines are presented to registered stakeholders and the guideline review panel for consultation and review. Once the final guidelines are approved, they are submitted to the National Health Service. Cost reports estimate the national cost of guidance, while the costing templates guide local NICE implementers on local cost estimates.

Uses of Comparisons

The National Health Service is not the only organization to which NICE guidance applies; all health professionals are expected to consider the guidelines. NICE explicitly states that although its guidance should be regarded when appropriate, it does not replace individual knowledge and care, and, ultimately, it is the provider's decision, in consultation with the patient. NICE guidelines apply, to varying extent, in four countries: England, Wales, Northern Ireland, and Scotland. NICE guidelines can apply across sectors, including local governments, educators, public utilities (such as gas and water companies), and the private sector, in addition to health providers. At the end of each month, NICE circulates all new guidance to a core group of recipients, and all guidance is published online. (See National Institute for Health and Clinical Excellence, undated[a].)

Technology Evaluation Center

Role and Organization

TEC, part of the Blue Cross and Blue Shield Association's, provides evidence-based assessments of clinical effectiveness to both private- and public-sector customers. Assessments are completed by TEC core staff, which includes physicians, nurses, scientists, statisticians, and pharmacology professionals. The TEC Medical Advisory Panel meets three times a year to review TEC assessments. In 2002, Scott Serota, president and chief executive officer of Blue Cross and Blue Shield, estimated that TEC would cost \$500 million to fund in its first year of operation.

Selection of Treatments for Comparison

Treatments are selected by TEC staff with input from Kaiser Permanente technical staff. Additional reviews are requested by such groups as AHRQ and CMS. TEC produces 20–25 assessments per year.

Methods Used for Comparisons

Systematic reviews are used to assess clinical effectiveness according to five criteria: the technology must have final approval from the appropriate governmental regulatory bodies, the scientific evidence must permit conclusions concerning the effect of the technology on health outcomes, the technology must improve the net health outcome, the technology must be as beneficial as any established alternatives, and the improvement must be attainable outside the investigational settings. Occasionally, TEC writes a special report on a specific technology. These reports are less rigid in their end product and could highlight effects of a drug for a specific population or synthesize the cost-effectiveness of a technology.

Uses of Comparisons

The TEC Web site is available to the public for “informational purposes” only and is the private, trademarked property of Blue Cross and Blue Shield and Kaiser. (See Blue Cross and Blue Shield Association, undated.)

Washington Health Technology Assessment Program

Role and Organization

The Washington State HTA program was created by the Washington Health Care Authority to ensure that medical treatments and services covered by its programs are safe and effective. The Health Care Authority oversees three state health care programs: the Public Employees Benefits Board, the Washington Basic Health Plan, and Community Health Services. The Health Care Authority budget allocated \$1.2 million for HTA operations in its first year (2006).

Selection of Treatments for Comparison

The HTA reviews medical and surgical devices and procedures, medical equipment, and diagnostic tests; prescription drugs are not under the purview of the HTA. Technologies to be reviewed are identified by physicians in participating state programs or requested by the public and then sent to the HTA administrator for selection. Prioritization is based on concerns about safety, efficacy, and cost-effectiveness; secondary concerns include the number of individuals potentially affected by the technology, the severity of the condition(s) treated by the technology, any policy-related factors and any special population or ethical concerns. HTA considers the estimated total direct cost per year in its primary criteria for prioritizing technology assessments. The cost criteria are included to estimate the potential budgetary impact from a coverage decision on the technology. The HTA was contractually obligated to review 14 technologies in its first two years of operation.

Methods Used for Comparisons

Once a technology is selected, a systematic review is conducted to assess evidence of safety, effectiveness, and cost compared to alternative technologies; the evidence and methods used to analyze the technology are then reported. Each report summarily ranks the technology (low, medium or high) according to three primary criteria rankings: safety, efficacy, and cost. Completed reports are submitted to 11 physicians and providers who make up the Health Technology Clinical Committee. The committee reviews the reports and uses the evidence to make coverage decisions for Washington State agencies. Reports generally take between two and six months to complete, and the review and decision process can take up to 12 additional months. Technologies are generally re-reviewed every 18 months.

Uses of Comparisons

The Health Technology Clinical Committee reviews the evidence to decide which agencies will and will not pay for a technology and the circumstances that will determine coverage. State agencies participating in HTA decisions are HCA plans, Medicaid, and the Washington Departments of Labor and Industries, Corrections, and Veterans Affairs. Not all agencies have identical coverage allocations. The state agencies cover unique populations, and a coverage decision for one agency may not be appropriate for another. For example, the Department of Labor and Industries covers injured workers, so if the HTA reviews and approves a pediatric procedure, it could permit coverage for Medicaid and not for the Department of Labor and Industries. State agencies also make autonomous decisions about technologies not decided upon by HTA. (See Washington State Health Care Authority, undated.)

Glossary

Clinical effectiveness: “The extent to which a specific intervention, when used under ordinary circumstances, does what it is intended to do. Clinical trials that assess effectiveness are sometimes called pragmatic or management trials” (Cochrane Collaboration, 2005).

Clinical trial: “An experiment to compare the effects of two or more healthcare interventions. Clinical trial is an umbrella term for a variety of designs of healthcare trials, including uncontrolled trials, controlled trials, and randomised controlled trials” (Cochrane Collaboration, 2005).

Comparative effectiveness research: “[T]he conduct and synthesis of systematic research comparing different interventions and strategies to prevent, diagnose, treat and monitor health conditions” (Federal Coordinating Council on Comparative Effectiveness Research, undated.)

Cost-benefit analysis: “An economic analysis that converts effects into the same monetary terms as costs and compares them” (Cochrane Collaboration, 2005).

Cost-effectiveness analysis: “An economic analysis that views effects in terms of overall health specific to the problem, and describes the costs for some additional health gain (e.g. cost per additional stroke prevented)” (Cochrane Collaboration, 2005).

Cost-utility analysis: “A special form of cost effectiveness analysis where health effects are measured in quality adjusted life years. A treatment is assessed in terms of its ability to both extend life and to improve the quality of life” (National Institute for Health and Clinical Excellence, undated[b]).

Efficacy: “The extent to which an intervention produces a beneficial result under ideal conditions. Clinical trials that assess efficacy are sometimes called explanatory trials and are restricted to participants who fully co-operate” (Cochrane Collaboration, 2005).

Meta-analysis: “The use of statistical techniques in a systematic review to integrate the results of included studies. Sometimes misused as a synonym for systematic reviews, where the review includes a meta-analysis” (Cochrane Collaboration, 2005).

Observational study: “A study in which the investigators do not seek to intervene, and simply observe the course of events. Changes or differences in one characteristic (e.g. whether or not people received the intervention of interest) are studied in relation to changes or differences in other characteristic(s) (e.g. whether or not they died), without action by the investigator. There is a greater risk of selection bias than in experimental studies” (Cochrane Collaboration, 2005).

Safety: “Refers to serious adverse effects, such as those that threaten life, require or prolong hospitalization, result in permanent disability, or cause birth defects” (Cochrane Collaboration, 2005).

Systematic review: “A review of a clearly formulated question that uses systematic and explicit methods to identify, select, and critically appraise relevant research, and to collect and analyze data from the studies that are included in the review. Statistical methods (meta-analysis) may or may not be used to analyse and summarise the results of the included studies” (Cochrane Collaboration, 2005).

References

- Aaserud, M., A. T. Dahlgren, J. P. Kusters, A. D. Oxman, C. Ramsay, and H. Sturm, "Pharmaceutical Policies: Effects of Reference Pricing, Other Pricing, and Purchasing Policies," Cochrane Collaboration, April 19, 2006. As of December 1, 2009:
<http://www.cochrane.org/reviews/en/ab005979.html>
- Agency for Healthcare Research and Quality, "Evidence-Based Practice Centers (EPCs) Request for Proposals," 2007. As of December 1, 2009:
<http://www.ahrq.gov/fund/contarchive/rfp0710021.htm>
- , "AHRQ and the Recovery Act," December 2009. As of December 1, 2009:
<http://www.ahrq.gov/fund/cefara.htm>
- Agency for Healthcare Research and Quality, Effective Health Care Program, homepage, undated. As of December 21, 2009:
<http://effectivehealthcare.ahrq.gov/>
- Alliance for Health Reform, "Comparative Effectiveness: Better Value for the Money?" August 2008. As of August 11, 2008:
http://www.allhealth.org/publications/Quality_of_care/Comparative_Effectiveness_Better_Value_for_the_Money_84.pdf
- Blue Cross and Blue Shield Association, Technology Evaluation Center, homepage, undated. As of December 21, 2009:
<http://www.bcbs.com/blueresources/tec/>
- Center for Evidence-Based Policy, Oregon Health and Science University, "DERP Structure," Web page, undated(a). As of December 21, 2009:
<http://www.ohsu.edu/ohsuedu/research/policycenter/DERP/derp-structure.cfm>
- , "Medicaid Evidence-Based Decisions Project (MED)," Web page, undated(b). As of December 21, 2009:
<http://www.ohsu.edu/ohsuedu/research/policycenter/med/index.cfm>
- Center for Medical Technology Policy, homepage, undated. As of December 21, 2009:
<http://www.cmtpnnet.org/>
- Centers for Medicare and Medicaid Services, "Implantable Automatic Defibrillators," Medicare National Coverage Determination, March 4, 2005. As of December 1, 2009:
<http://www.cms.hhs.gov/Transmittals/Downloads/R29NCD.pdf>
- Charles, Cathy, Amiram Gafni, and Tim Whelan, "Decision-Making in the Physician-Patient Encounter: Revisiting the Shared Treatment Decision-Making Model," *Social Science and Medicine*, Vol. 49, No. 5, September 1999, pp. 651–661.
- Charles, Cathy, Tim Whelan, and Amiram Gafni, "What Do We Mean by Partnership in Making Decisions About Treatment?" *British Journal of Medicine*, Vol. 319, No. 7212, September 18, 1999, pp. 780–782.
- Cochrane Collaboration, "Glossary of Cochrane Collaboration and Research Terms," last updated May 2005. As of December 1, 2009:
<http://www.cochrane.org/resources/glossary.htm>

Congressional Budget Office, *Research on the Comparative Effectiveness of Medical Treatments*, Washington, D.C., December 2007. As of December 21, 2009:
<http://www.cbo.gov/doc.cfm?index=8891>

———, *Budget Options, Volume I: Health Care*, Washington, D.C., December 2008. As of February 15, 2009:
<http://www.cbo.gov/doc.cfm?index=9925>

Cost Effectiveness Analysis Registry, Institute for Clinical Research and Health Policy Studies, Tufts Medical Center, homepage, undated. As of December 21, 2009:
<https://research.tufts-nemc.org/cear/default.aspx>

Consumer Reports Best Buy Drugs, Treating Type 2 Diabetes: The Oral Diabetes Drugs, Yonkers, N.Y.: Consumers Union of the U.S., February 2009. As of December 21, 2009:
<http://www.consumerreports.org/health/resources/pdf/best-buy-drugs/DiabetesUpdate-FINAL-Feb09.pdf>

ECRI Institute, homepage, undated. As of December 21, 2009:
<https://www.ecri.org>

Federal Coordinating Council on Comparative Effectiveness Research, “Draft Definition, Prioritization Criteria, and Strategic Framework for Public Comment,” Web page, undated. As of December 21, 2009:
<http://www.hhs.gov/recovery/programs/cear/draftdefinition.html>

Garrison, Louis P., Edward C. Mansley, Thomas A. Abbott, Brian Bresnahan, Joel W. Hay, and James Smeeding, *Good Research Practices for Measuring Drug Costs in Cost Effectiveness Analyses: A Report of the ISPOR Drug Cost Task Force—Part II: A Societal Perspective*, draft, Lawrenceville, N.J.: International Society for Pharmacoeconomics and Outcomes Research, April 25, 2008. As of May 1, 2009:
http://www.ispor.org/councils/documents/ISPOR_Report_Good_Practices_SocietalPerspective.pdf

Gold Marthe R., Joanna E. Siegel, and Louise B. Russell, *Cost-Effectiveness in Health and Medicine*, New York: Oxford University Press, 1996.

Institute for Clinical and Economic Review, homepage, undated. As of December 21, 2009:
<http://www.icer-review.org/>

Institute for Quality and Efficiency in Health Care, homepage, last updated December 22, 2009. As of December 22, 2009:
<http://www.iqwig.de/institute-for-quality-and-efficiency-in-health.2.en.html>

Kaiser Family Foundation, “Percentage of Adults Aged 50 and Over Who Have Ever Had a Sigmoidoscopy or Colonoscopy, 2008,” *StateHealthFacts.org*, 2009. As of December 1, 2009:
<http://www.statehealthfacts.org/comparemaptable.jsp?ind=666&cat=2>

Kanavos, Panos, and Uwe Reinhardt, “Reference Pricing for Drugs: Is It Compatible with U.S. Health Care?” *Health Affairs*, Vol. 22, No. 3, May–June 2003, pp. 16–30.

Levine, Mark N., and Jim A. Julian, “Registries That Show Efficacy: Good, but Not Good Enough,” *Journal of Clinical Oncology*, Vol. 26, No. 33, November 20, 2008, pp. 5316–5319.

Massachusetts Office of Health and Human Services, “Guidelines for Medical Necessity Determination,” Web page, undated. As of December 1, 2009:
<http://www.mass.gov/?pageID=eohhs2subtopic&L=6&L0=Home&L1=Provider&L2=Insurance+%28including+MassHealth%29&L3=MassHealth&L4=Guidelines+for+Clinical+Treatment&L5=Guidelines+for+Medical+Necessity+Determination&sid=Eeohhs2>

Massachusetts State Legislature, Acts of 2008, Chapter 305, An Act to Promote Cost Containment, Transparency and Efficiency in the Delivery of Quality Health Care, 2008.

Medicare Payment Advisory Commission, *Report to the Congress: Reforming the Delivery System*, Washington, D.C., June 2008. As of December 21, 2009:
http://www.medpac.gov/documents/Jun08_EntireReport.pdf

National Institute for Health and Clinical Excellence, homepage, undated[a]. As of December 21, 2009:
<http://www.nice.org.uk/>

———, “Glossary,” undated[b]. As of December 21, 2009:
<http://www.nice.org.uk/website/glossary/>

National Institutes of Health, "Research into What Works Best," fact sheet, August 2007. As of December 1, 2009:

<http://www.nih.gov/about/researchresultsforthepublic/whatworks.pdf>

Neumann, Peter J., "Emerging Lessons from the Drug Effectiveness Review Project," *Health Affairs*, Vol. 25, No. 4, 2006, pp. W262–271.

Newhouse, Joseph P., *Free for All? Lessons from the Rand Health Insurance Experiment*, Cambridge, Mass.: Harvard University Press, 1993.

O'Connor, Annette M., Alaa Rostom, Valeries Fiset, Jacqueline Tetroe, Vikki Entwistle, Hilary Llewellyn-Thomas, Margaret Holmes-Rovner, Michael Barry, and Jean Jones, "Decision Aids for Patients Facing Health Treatment or Screening Decisions: Systematic Review," *British Journal of Medicine*, Vol. 319, No. 7212, September 18, 1999, pp. 731–734.

Public Law 108-173, Medicare Prescription Drug, Improvement, and Modernization Act of 2003, December 8, 2003.

Public Law 111-5, American Recovery and Reinvestment Act of 2009, February 17, 2009.

Tunis, Sean R., and Steven D. Pearson, "Coverage Options for Promising Technologies: Medicare's 'Coverage with Evidence Development,'" *Health Affairs*, Vol. 25, No. 5, 2006, pp. 1218–1230.

Washington State Health Care Authority, Health Technology Assessment Program, homepage, undated. As of December 21, 2009:

<http://www.hta.hca.wa.gov/>

WebMD, "Back Pain Health Center," Web page, last updated February 6, 2008. As of December 1, 2009: <http://www.webmd.com/back-pain/should-i-have-magnetic-resonance-imaging-mri-for-low-back-pain>

Wennberg, John E., Annette M. O'Connor, E. Dale Collins, and James N. Weinstein, "Extending the P4P Agenda, Part 1: How Medicare Can Improve Patient Decision Making and Reduce Unnecessary Care," *Health Affairs*, Vol. 26, No. 6, 2007, pp. 1564–1574.