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.

REPORT

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

RAND HEALTH

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

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

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.