

REPORT BRIEF • JANUARY 2008

KNOWING WHAT WORKS IN HEALTH CARE: A ROADMAP FOR THE NATION

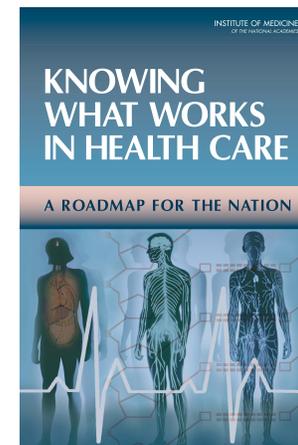
Solutions to some of the nation's most pressing health policy problems hinge on the capacity to identify which diagnostic, treatment, and prevention services really work and under what conditions. Spending on ineffective as well as effective care contributes to soaring health costs and rising insurance premiums. Variations in how health care providers treat specific conditions reflect uncertainty and disagreement about what the clinical practice standards should be, and patients and insurance plans cannot always be assured that providers are delivering the best, most effective care. Health plans are burdened with the need to constantly learn how their covered populations might benefit from—or be harmed by—newly available health services. Meanwhile, proponents of consumer-directed health plans argue that consumers who are equipped with good information will have the power to reduce the cost and improve the quality of care. Yet even the most sophisticated health care consumer struggles to learn which care is appropriate for his or her circumstance.

This report recommends that Congress establish a single national clinical effectiveness assessment program (“the Program”) with sufficient resources, authority, and capacity to facilitate the development of standards and processes that yield credible, unbiased, and understandable syntheses of the available evidence on clinical effectiveness.

Numerous stakeholders, policy makers, and government entities have proposed that new investments be made in comparative effectiveness research to meet the nation's need for evidence on “what works” in health care. These proposals have merit, but more attention is also needed to assure that health care decision makers can discern which evidence is valid, for whom, and under what circumstances. As the evidence base for health care increases, inevitably there will be an even greater need for trustworthy, scientific synthesis and interpretation of the available evidence.

BUILDING A FOUNDATION FOR KNOWING WHAT WORKS IN HEALTH CARE

To address this problem, the Robert Wood Johnson Foundation asked the Institute of Medicine (IOM) to recommend a sustainable, replicable approach to identifying effective clinical services. The IOM committee assessed the status quo and concluded that the United States must strengthen its capacity to assess evidence and provide reliable, unbiased information on the effectiveness of clinical services. The committee recommends a national Program that



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is stable over the long term, produces trusted output, and is independent from external political pressures. In deliberations over the important features of the Program, the committee concluded that eight principles are essential:

- Accountability
- Efficiency
- Objectivity
- Scientific rigor
- Consistency
- Feasibility
- Responsiveness
- Transparency

This report outlines the framework for a national Program designed to optimize the use of evidence to identify effective health care services. Three functions would be central to this mission: (1) setting priorities for evidence assessment; (2) assessing evidence through systematic reviews; and (3) developing (or endorsing) standards for trusted clinical practice guidelines.

Recommendations for a National Clinical Effectiveness Assessment Program

Congress should direct the secretary of the U.S. Department of Health and Human Services to designate a single entity (the Program) with authority, overarching responsibility, sustained resources, and adequate capacity to ensure production of credible, unbiased information about what is known and not known about clinical effectiveness. The Program should

- Set priorities for, fund, and manage systematic reviews of clinical effectiveness and related topics.
- Develop a common language and standards for conducting systematic reviews of the evidence and for generating clinical guidelines and recommendations.
- Provide a forum for addressing conflicting guidelines and recommendations.
- Prepare an annual report to Congress.

The secretary of Health and Human Services should appoint a Clinical Effectiveness Advisory Board to oversee the Program. Its membership should be constituted to minimize bias due to conflict of interest and should include representation of diverse public and private sector expertise and interests.

The Program should develop standards to minimize bias due to conflicts of interest for priority setting, evidence assessment, and recommendations development.

SETTING PRIORITIES FOR EVIDENCE ASSESSMENT

Evidence assessments (systematic reviews) are expensive and resources must be used wisely. Without a central entity setting national priorities, there is no assurance that the most vital topics will be reviewed. The coordination under the Program

would reduce duplication of evidence assessments and ensure that the most important topics are reviewed.

The committee recommends that the Program appoint an independent, free-standing Priority Setting Advisory Committee to develop and implement a priority-setting process that will identify high priority topics that merit systematic evidence assessment. A broad range of topics should be considered, including new, emerging, and well-established health services and technologies for prevention, diagnosis, and treatment. The highest priorities should be the clinical questions of patients and clinicians that have the potential for substantial impact on health outcomes across all ages, burden of disease, health disparities, and undesirable variation in the delivery of health services.

Recommendations for Setting Priorities

The Program should appoint a standing Priority Setting Advisory Committee (PSAC) to identify high priority topics for systematic reviews of clinical effectiveness.

- The priority setting process should be open, transparent, efficient, and timely.
- Priorities should reflect the potential for evidence-based practice to improve health outcomes across the life span, reduce the burden of disease and health disparities, and eliminate undesirable variation.
- Priorities should also consider economic factors, such as the costs of treatment and the economic burden of disease.
- The membership of the PSAC should include a broad mix of expertise and interests and be chosen to minimize committee bias due to conflicts of interest.

If conducted properly, the systematic review should make obvious the gap between what is known about the effectiveness of a particular service and what clinicians and patients want to know.

ASSESSING EVIDENCE THROUGH SYSTEMATIC REVIEWS

A systematic review examines a specific research question by identifying, selecting, assessing, and summarizing all the similar, but separate, research studies focused on a given clinical service or technology. Systematic reviews of available evidence provide a central link between research evidence and clinical decision making. Individual research studies rarely provide definitive answers to clinical effectiveness questions. If conducted properly, the systematic review should make obvious the gap between what is known about the effectiveness of a particular service and what clinicians and patients want to know. As such, systematic reviews are also critical to developing the agenda for further primary research because they reveal where evidence is insufficient and new information is needed. Without systematic reviews, researchers may miss promising leads or pursue questions that have been answered already.

The core of a systematic review is a concise and transparent synthesis of the results of the included studies. The language of the review should be simple and clear so that it is usable and accessible to decision makers.

Under the status quo, the quality of systematic reviews is variable and findings

Recommendations for Developing Clinical Practice Guidelines

Groups developing clinical guidelines or recommendations should use the Program's standards, document their adherence to the standards, and make this documentation publicly available.

To minimize bias due to conflicts of interest, panels should include a balance of competing interests and diverse stakeholders, publish conflict of interest disclosures, and prohibit voting by members with material conflicts.

Providers, public and private payers, purchasers, accrediting organizations, performance measurement groups, patients, consumers, and others should preferentially use clinical recommendations developed according to the Program standards.

CONCLUSION

There is an urgent need for action to change how the nation marshals clinical evidence and applies it to identify the most effective clinical interventions. The nation must significantly expand its capacity to use scientific evidence to know “what works” in health care. This report recommends that Congress direct the U.S. Department of Health and Human Services to designate an entity—either a public or a public-private organization—with the authority, expertise, and funding necessary to set priorities for evaluating clinical services, to conduct systematic reviews of the evidence available on these priorities, and to promote the development and use of standards-based clinical practice guidelines.

There is an urgent need for action to change how the nation marshals clinical evidence and applies it to identify the most effective clinical interventions. The nation must significantly expand its capacity to use scientific evidence to know “what works” in health care.

FOR MORE INFORMATION...

Copies of *Knowing What Works in Health Care: A Roadmap for the Nation* are available from the National Academies Press, 500 Fifth Street, N.W., Lockbox 285, Washington, DC 20055; (800) 624-6242 or (202) 334-3313 (in the Washington metropolitan area); Internet, www.nap.edu. The full text of this report is available at www.nap.edu.

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