Evidence-Based Medicine: A Unified Approach

Two approaches to using evidence to solve clinical problems, and how to unify them.

by David M. Eddy

ABSTRACT: Behind the wide acceptance of the idea of "evidence-based medicine" are two curious facts: There are two very different approaches to applying evidence to medicine, and the most commonly cited definition applies to only one of them. This paper describes the problem that we are asking "evidence" to solve and the different methods by which evidence can be used to help solve that problem, and recommends a unified approach.

THE TERM "EVIDENCE-BASED MEDICINE" (EBM) has spread through medicine with amazing speed during the past fifteen years. The pace speaks to the attraction and fundamental soundness of the core idea: that what happens to patients should be based, to the greatest extent possible, on evidence. Yet behind this wide acceptance is the curious fact that there are several different routes by which the principles of EBM are being introduced into medical practice, and the most commonly cited definition—that EBM is "the conscientious, explicit, and judicious use of current best evidence in making decisions about the care of individual patients"—addresses only one of them.[1] This paper describes two main ways evidence is being applied to improve health care, argues that both are necessary, and offers a new definition of EBM that better captures how it is actually being applied.

The Environment

It is important to begin with a review of the environment from which the phrase "evidence based" arose; the environment created the need, determined the principles, and shaped the evolution of EBM. Up until about forty years ago, medical decisions were doing very well on their own, or so people thought. The complacency was based on a fundamental assumption that through the rigors of medical education, followed by continuing education, journals, individual experiences, and exposure to colleagues, each physician always thought the right thoughts and did the right things. The idea was that when a physician faced a patient, by some

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fundamentally human process called the "art of medicine" or "clinical judgment," the physician would synthesize all of the important information about the patient, relevant research, and experiences with previous patients to determine the best course of action. "Medical decision making" as a field worthy of study did not exist. Analytical methods and mathematical models were limited to research projects. Guidelines were merely a way for experts to pass occasional pieces of advice to nonexperts. Coverage and medical necessity were defined tautologically; if the majority of physicians were doing it, it was medically necessary and should be covered. Diseases did not require any management beyond what physicians were already providing, and performance was taken for granted.

Beginning in the early 1970s, however, two major flaws began to appear in this fundamental assumption. One was a growing body of research showing that key aspects of the assumption were simply wrong. In 1973 John Wennberg and his colleagues began to document wide variations in practice patterns.[2] The implications for the fundamental assumption were undeniable: When different physicians are recommending different things for essentially the same patients, it is impossible to claim that they are all doing the right thing. In the 1980s a group at RAND began publishing studies showing that large proportions of procedures being performed by physicians were considered inappropriate even by the standards of their own experts.[3] These empirical observations were given a disconcerting explanation in a third set of papers that described the complexity of medical decisions, errors in medical reasoning, and wide ranges of uncertainty.[4] It is not possible for anyone, even physicians, to accurately process in their heads all of the information needed for a complex medical decision. Decisions could not be based solely on the art of medicine or clinical judgment; some other anchor had to be found.

A second flaw in the fundamental assumption was the gap between clinical research and what was actually happening in clinical practice. One problem was the lack of good evidence for many important practices. An estimate that only 15 percent of medical practices were based on solid clinical trials became famous.[5] Another problem was that many practices taken for granted by physicians were actually found to be ineffective when subjected to clinical trials. Archie Cochrane, among others, argued persuasively for much more attention to randomized controlled trials (RCTs).[6] A third problem was that even when RCTs were done, it could take years for physicians to actually change their practices to incorporate the new information.[7] The effect of all of this was to identify research evidence as the obvious new anchor for medical decisions.

An additional factor was a deeper appreciation of the role played by guidelines and other types of clinical policies in influencing individual physician-patient decisions. This line of reasoning began with the fact that most medical decisions are far too complicated for the human mind, and then observed that textbooks and articles were filled with thousands of statements saying, essentially, "If you see this,

then do that." It became clear that in fact the great majority of medical decisions were not reasoned from scratch every time a physician faced a patient, but rather were based on relatively simple "if...then" statements.[8] At the very least, these statements served as the initial reference points for decisions, with the physician's task being simplified to tailoring an "if...then" statement up or down to fit an atypical patient. These informal guidelines were playing an essential role in medicine by simplifying decisions to a point manageable by busy physicians. However, when they were tracked back to their origins, it became clear that they were simply the beliefs of the authors, or at best a consensus of experts. In none of the cases was there an explicit rationale tied to evidence. Taken together, these findings highlighted the importance of changing the methods by which the "if...then" statements were created, and they identified a natural and powerful mechanism by which evidence could be transmitted to physicians and incorporated in their decisions. If the "if...then" statements could be based on evidence, then any decisions based on those statements would be based on evidence.

During the same period as this work, a large number of new methods for improving medical decisions and policies were being developed. They included decision trees, utility theory, Bayes theorem for analyzing diagnostic tests, mathematical models, cost-effectiveness analysis, technology assessment, clinical epidemiology, outcomes management, and meta-analysis.[9] These and other tools had two effects. The most obvious is that they began to provide the means by which evidence and related factors could be brought to bear on medical decisions. A more subtle but still powerful effect is that they highlighted the gaps between traditional decision making and more formal methods, and they strengthened the argument for bringing more evidence into decisions.

A final factor looming over everything was the cost of health care, which was increasing at a rate at least twice the general rate of inflation. When this was coupled with the growing awareness of the questionable quality of traditional decision making, the need for corrective action became acute.

'Evidence Based': Two Approaches

■ **Evidence-based guidelines.** It was into this supersaturated solution that the term "evidence based" was dropped. The first published use of the term was in the context of guidelines; it appeared in a series of articles published in the *Journal of the American Medical Association* beginning in 1990.[10] The paper distinguished different methods for designing guidelines ("global subjective judgment" or "consensus based," "evidence based," "outcomes based," and "preference based") and argued that guidelines and related types of policies should be based on evidence, not subjective judgment or consensus. Other articles in the series described the limitations of clinical judgment and expert opinion, the two main steps of a medical decision (analysis of evidence and incorporation of preferences), the role of guidelines in medical decisions, different types of guidelines ("standards," "guidelines," and "op-

tions"), the importance of following an explicit process, the use of evidence to resolve conflicts in guidelines, critical steps in guideline design ("guidelines for guidelines"), methods for balancing benefits and harms (the "balance sheet"), the importance of incorporating costs, resolution of the conflict between the individual and society, and criteria for basing coverage policies and medical necessity on evidence.[11] For convenience, I call this approach to EBM "evidence-based guidelines," or EBG.

The principles of EBG were actually stated a decade earlier, when the American Cancer Society (ACS) developed its guidelines for the cancer-related health checkup:

> First, there must be good evidence that each test or procedure recommended is medically effective in reducing morbidity or mortality; second, the medical benefits must outweigh the risks; third, the cost of each test or procedure must be reasonable compared to its expected benefits; and finally, the recommended actions must be practical and feasible.[12]

That document went on to describe the evidence for each of the common screening tests and derived guidelines based on that evidence. The methods included a systematic review of all pertinent evidence (not just the evidence that supported a particular position), a critical analysis of the quality of the evidence, a synthesis of the evidence, a balancing of benefits and harms, an assessment of feasibility and practicality, a clear statement of the recommendation, and a detailed rationale. As might be expected when the basis for a set of guidelines shifts from expert consensus to evidence, most of the resulting evidence-based guidelines were dramatically different from what the ACS had previously recommended.[13]

During the next twenty years, fueled by the forces described above, more and more organizations began to apply evidence-based methods to their work. In 1981 the American College of Physicians (ACP) began the Clinical Efficacy Assessment Project to write literature reviews for specified topics.[14] Over the next few years, the ACP extended the reviews to include explicit recommendations for practices, and in 1985 it began to publish them as guidelines. In 1987 the Council of Medical Specialty Societies (CMSS) convened a national meeting to promote the idea of guidelines, sponsored training programs for specialty societies, and commissioned a manual of evidence-based methods. It was here that concepts such as problem formulation, evidence tables, direct versus indirect evidence, causal chains, and balance sheets were developed, incorporated into training programs, and eventually published.[15] Shortly after the CMSS conference, the AMA convened a large number of professional societies to help coordinate evidence-based guidelines. In 1989 the U.S. Preventive Services Task Force (USPSTF) was convened to evaluate research and issue guidelines for preventive interventions.[16] In 1993 the Agency for Healthcare Research and Quality (AHRQ, then known as the Agency for Health Care Policy and Research, or AHCPR) launched a program to create evidence-based guidelines, and the Cochrane Collaboration created a thirteen-country network to promote evidence-based health care though a mixture of systematic

reviews and guidelines.[17] In 1995 the BMJ Publishing Group launched *Clinical Evidence*, to summarize the current state of knowledge about important clinical questions.[18] In 1997, after political pressure cut its guidelines program short, AHRQ established Evidence-based Practice Centers (EPCs) to produce evidence reports and technology assessments.[19] Around the same time, a partnership of AHRQ, the AMA, and the American Association of Health Plans (now America's Health Insurance Plans) created the National Guideline Clearinghouse, which emphasized (but did not require) evidence-based methods.[20] And throughout the decade many managed care organizations, specialty societies, and disease-based associations and foundations built guidelines programs.

The work of these organizations took EBG and transformed it from a set of principles with a few sentinel applications into a movement. By the end of the 1990s it was widely accepted that guidelines should be based on evidence, and the only acceptable use of consensus-based methods was when there was insufficient evidence to support an evidence-based approach.

During this period the concepts also spread beyond guidelines to create other branches of EBM. The existence of good evidence of effectiveness was made a threshold criterion for coverage policies ("evidence-based coverage").[21] Another branch is "evidence-based performance measures," where the first step in the design of a measure is a search for evidence that the intervention being measured is effective.[22] The principles of EBM also spread to medical necessity and benefit design.[23] Still other branches are the application of evidence to disease management, quality improvement, regulations, and public policies.[24] For convenience, in this paper I include all of these—coverage, disease management, performance measurement, quality improvement, medical necessity, regulations, and public policy—under the term *policies.*

Each of these represents a different way to use evidence to influence what happens to patients, and they can rightfully be considered different branches of the evidence-based movement. But all of them share four important features, which are important to understand for evaluating the current definition of *evidence-based medicine.* First, in all of them the work of analyzing the evidence and developing a guideline or other policy is done by small groups of specially trained people, usually sponsored by an organization. Second, they all use an explicit, rigorous process. Third, for all of them the "product"—whether it be an evidence review, a guideline, or another type of policy—is generic. It is intended to apply to a class or group of patients defined by some clinical criteria, rather than to an individual patient. Fourth, their effects on care are indirect. That is, they are intended to enable, guide, motivate, or sometimes force physicians and other types of providers to deliver certain types of care to people; they do not directly determine the care provided to a particular patient. Because each branch shares these features, in this paper I lump them together under the acronym EBG.

■ **Evidence-based individual decision making.** The second main type of EBM

appeared shortly after evidence-based guidelines, with an editorial that described how, in "the way of the future," an internist would manage the care of a seventy-year-old man with fatigue by conducting a computerized literature search of the sensitivity and specificity of tests for iron deficiency anemia, selecting a test, assigning a pre-test probability, calculating a post-test probability, and developing a management plan.[25] This paper was soon followed by a more comprehensive article titled "Evidence-based Medicine: A New Approach to Teaching the Practice of Medicine."[26] These were the first publications to use the term "evidence-based medicine," as opposed to "evidence-based guidelines."

However, as the example in the first article and the title of the second imply, this proposal for introducing evidence into medicine is quite different from the evidence-based guidelines and other policies described earlier. The focus here is on educating physicians to help them bring more research and evidence into their individual decisions about individual patients. The bedside, individual-patient orientation is emphasized by the liberal use of patient scenarios to introduce topics (for example, "What should you tell a thirty-three-year-old woman who...?"). The fact that the research and analysis are to be done by individual physicians is emphasized by the instructions given to readers (for example, "You proceed to track down the best available evidence..."). [27] To help them do it, the authors of this branch of EBM wrote a series of Users' Guides that covered such topics as "how to get started [reading the medical literature]," "how to use an article about therapy or prevention," "how to use an article about a diagnostic test," "how to use an article about harm," "how to use an article about prognosis", and "how to use an overview."[28] Several books have been written in the same vein.[29] And it is the original promoters of this branch that offered the commonly cited definition of EBM:

> Evidence based medicine is the conscientious, explicit, and judicious use of current best evidence in making decisions about the care of *individual* patients. The practice of evidence based medicine means integrating *individual* clinical expertise with the best available external clinical evidence from systematic research (emphasis added).[30]

In keeping with this personal, educational, bedside orientation of this branch of the evidence-based movement, and to distinguish it from evidence-based guidelines, I call this it "evidence-based individual decision making," or EBID.

Integrating The Two Approaches

To recap, EBG is done by multidisciplinary teams, using explicit rigorous methods, to produce generic guidelines and other policies that address the needs of groups of people and that affect individual patients indirectly by influencing the decisions of physicians and other health care decisionmakers. EBID is done by individual physicians, using implicit and personal methods, to make decisions about individual patients and directly determine their care. EBG begins with the design of guidelines and other policies and seeks to ensure that they are based on evidence so that everyone downstream (not just physicians, but also nurses, case

managers, and others) will be following evidence-based guidelines and other policies. In contrast, EBID begins with one type of provider—physicians—and seeks to teach them about evidence-based methods so that they can filter and interpret everything coming to them from upstream. Right now, the current definition of *evidence-based medicine* includes the latter but not the former.

The question then becomes whether the definition of EBM should be expanded to include evidence-based guidelines and its related branches. There are several reasons to do this. First, the concept of "medicine" is clearly broader than individual physicians' decision making. The roles played by guidelines, coverage, quality improvement, performance measurement, disease management, public policies, and many other factors speak for themselves. There are other types of providers and decisionmakers. And even if we focus only on physicians, the actual decisions they make are clearly a mixture of guidelines, personal thoughts, incentives, and a wide variety of other influences. Second, evidence-based guidelines clearly meet a test of being based on evidence. Third, evidence-based guidelines and other policies are motivated by the same overall goal of increasing the extent to which patient care is based on and consistent with evidence.

Fourth, it is easy to argue that achieving that goal requires EBG and other policies just as much as it requires EBID. Without EBG, physicians would have to go it alone, with grave consequences for the feasibility, quality, consistency, burden, dissemination, accountability, and preservation of the work. It is one thing to help physicians become more critical users of research papers, but quite another to expect them to conduct systematic reviews of all of the research that is pertinent to a decision, using rigorous methods. The difficulties are recognized by the fact that numerous programs have been developed to digest primary research—interpret articles, create literature reviews, and develop guidelines—to spare physicians the hard work and help ensure quality and consistency. It is also pertinent that the Users' Guides for EBIG include papers on how to use these products. But now imagine that these products were not created using evidence-based methods. EBID would teach physicians how to spot bad ones, but without EBG there would be little left that they could use. Worse, physicians would be trying to apply EBID principles in a world swirling with traditional consensus-based guidelines and policies. The conclusion seems obvious: that medicine is best served if both individual physicians and those who design guidelines and other policies follow evidence-based methods.

A fifth reason is that EBID, with its focus on physicians, misses all of the other actors—pharmacists, case managers, nurses, administrators, and public health workers. Sixth, unless EBID and EBG are integrated, there is a risk that what makes sense from the narrow viewpoint of an individual physician and patient might not make sense from the broader viewpoint of a program or population. (Concerns about costs, available resources, and efficiency are obvious examples.) A final reason to expand the definition of EBM is practical; most people who talk

about EBM are already including systematic evidence reviews, guidelines, and other evidence-based policies. Certainly everyone who is doing that work thinks that they are practicing EBM. Another clue that the current definition is too narrow is that a sizable share of the papers in this volume of *Health Affairs* are actually about EBG, not EBID.

If EBM should include EBG, there is also a strong argument for keeping EBID in the definition. Guidelines need to be tailored to individual cases, and EBID improves physicians' ability to do this. Many problems fall through the cracks of guidelines, and EBID is the only way to get evidence-based medicine to them. Physicians work on guideline teams, and the educational approach of EBID enables them to be better participants. EBID also helps physicians understand the rationale for evidence-based guidelines, which greatly improves their acceptance, especially when the evidence contradicts a time-honored practice.

It seems obvious that the current definition of *evidence-based medicine*, which focuses on individual physicians and their decisions, is too narrow.[31] It should be expanded to include not only evidence-based decision making by individual physicians, but also evidence-based systematic reviews, guidelines, and other types of policies. This would recognize that the combination moves medical practices toward evidence faster, more consistently, and more efficiently than evidence-based individual decision making alone. It would also make the definition consistent with how EBM is actually being done, bringing under the umbrella of the term the dozens of guideline programs that have been practicing the principles and developing the methods for years.

In summary, EBM is a set of principles and methods intended to ensure that to the greatest extent possible, medical decisions, guidelines, and other types of policies are based on and consistent with good evidence of effectiveness and benefit.

NOTES

1. D.L. Sackett et al., "Evidence-based Medicine: What It Is and What It Isn't" (Editorial), *British Medical Journal* 312, no. 7023 (1996): 71–72.
2. J.E. Wennberg and A. Gittelsohn, "Small Area Variations in Health Care Delivery," *Science* 82, no. 117 (1973): 1102–1108.
3. M.R. Chassin et al., "How Coronary Angiography Is Used: Clinical Determinants of Appropriateness," *Journal of the American Medical Association* 258, no. 18 (1987): 2543–2547.
4. D.M. Eddy, "Probabilistic Reasoning in Clinical Medicine: Problems and Opportunities," in *Judgment under Uncertainty: Heuristics and Biases*, ed. D. Kahneman, P. Slovic, and A. Tversky (New York: Cambridge University Press, 1982); D.M. Eddy, "Flying without Instruments: Analyzing Medical Policies by Consensus and Expert Opinion," *John A. Hartford Foundation Bulletin* (Winter 1983): 3–5; and D.M. Eddy, "Variations in Physician Practice: The Role of Uncertainty," *Health Affairs* 3, no. 2 (1984): 74–89.
5. The 15 percent figure comes an Institute of Medicine study, which in turn cited the congressional Office of Technology Assessment. See Committee for Evaluating Medical Technologies in Clinical Use, *Assessing Medical Technologies* (Washington: National Academies Press, 1985), 5.
6. A.L. Cochrane, *Effectiveness and Efficiency: Random Reflections on Health Services* (London: Nuffield Provincial Hospitals Trust, 1972).
7. See, for example, D.B. Dutton, *Worse than the Disease: Pitfalls of Medical Progress* (New York: Cambridge Univer-

sity Press, 1988).
8. D.M. Eddy, "Clinical Policies and the Quality of Clinical Practice," *New England Journal of Medicine* 307, no. 6 (1982): 343–347
9. A complete bibliography providing citations for these methods is available online at content.healthaffairs.org/cgi/content/full/hlthaff/24/1/9/DC1.
10. D.M. Eddy, "Practice Policies: Where Do They Come From?" *Journal of the American Medical Association* 263, no. 9 (1990): 1265, 1269, 1272, 1275.
11. A complete bibliography for this series of articles is available online at content.healthaffairs.org/cgi/content/full/hlthaff/24/1/9/DC1.
12. D.M. Eddy, "Guidelines for the Cancer-related Checkup: Recommendations and Rationale," *CA: A Cancer Journal for Clinicians* 30, no. 4 (1980): 193–240.
13. For example, a recommendation for an annual Pap smear became a recommendation for a Pap smear at least every three years after two negative examinations. The annual sigmoidoscopic exam became an exam every three to five years. The annual chest x-ray was no longer recommended. Breast cancer screening with mammography was recommended only for women over age fifty.
14. American College of Physicians, "CEAP Process," www.acponline.org/sci-policy/guidelines/ceap.htm (3 November 2004).
15. D.M. Eddy, *A Manual for Designing Practice Policies: The Explicit Approach* (Philadelphia: American College of Physicians Publishers, 1992).
16. Agency for Healthcare Research and Quality, "U.S. Preventive Services Task Force," www.ahrq.gov/clinic/uspstfix.htm (3 November 2004).
17. For more information, see the Cochrane Collaboration's home page, www.cochrane.org.
18. For more information, see the *Clinical Evidence* home page, www.clinicalevidence.com.
19. See AHRQ, "Evidence-based Practice Centers," March 2003, www.ahrq.gov/clinic/epc (3 November 2004).
20. For more information, see the National Guideline Clearinghouse home page, www.guideline.gov.
21. BlueCross BlueShield Association, "Technology Evaluation Center," www.bcbs.com/tec (3 November 2004); and D.M. Eddy, "Investigational Treatments: How Strict Should We Be?" *Journal of the American Medical Association* 278, no. 3 (1997): 179–185.
22. For more information, see the National Committee for Quality Assurance (NCQA) home page, www.ncqa.org.
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28. A complete listing of citations for these users' guides is available at content.healthaffairs.org/cgi/content/full/hlthaff/24/1/9/DC1.
29. See, for example, D.L. Sackett et al., *Evidence-based Medicine: How to Practice and Teach EBM*, 2d ed. (New York: Churchill Livingstone, 2000); J.P. Geyman, R.A. Deyo, and S.D. Ramsey, *Evidence-based Clinical Practice: Concepts and Approaches* (Boston: Butterworth Heinemann, 2000); and D.J. Friedland et al., *Evidence-based Medicine: A Framework for Clinical Practice* (New York: Lange, 1998).
30. Sackett et al., "Evidence-based Medicine."
31. Ibid.