The Promises And Pitfalls Of Evidence-Based Medicine

Nonadherence to practice guidelines remains the major barrier to the successful practice of evidence-based medicine.

by Stefan Timmermans and Aaron Mauck

ABSTRACT: Evidence-based medicine (EBM) aims to address the persistent problem of clinical practice variation with the help of various tools, including standardized practice guidelines. While advocates welcome the stronger scientific foundation of such guidelines, critics fear that they will lead to “cookbook medicine.” Studies show, however, that few guidelines lead to consistent changes in provider behavior. The hopes, fears, and mixed record of EBM are rooted in the traditional professional perspective of the clinician as sole decisionmaker. Multifaceted implementation strategies that take the collaborative nature of medical work into consideration promise more effective changes in clinical practice.

Evidence-based medicine (EBM) is commonly defined as “the conscientious, explicit, and judicious use of current best evidence in making decisions about the care of individual patients.” The term is loosely used and can refer to anything from conducting a statistical meta-analysis of accumulated research, to promoting randomized clinical trials, to supporting uniform reporting styles for research, to a personal orientation toward critical self-evaluation. EBM was initially defined in opposition to clinical experience, but later definitions have emphasized its complementary character and have aimed to improve clinical experience with better evidence. One common implementation of EBM involves the use of clinical practice guidelines during medical decision making to encourage effective care. The Institute of Medicine (IOM) defines clinical guidelines as “systematically developed statements to assist practitioner and patient decisions about appropriate health care for specific clinical circumstances.” An expert committee does the work of sifting through the scientific literature for clinicians and offers coherently sequenced recommendations based on the best available evidence aimed at everyday decision-making situations. Guidelines can be applied to any aspect of clinical care: how and when to order diagnostic or screening tests, when to provide certain medical services, how these should be performed, and how long...
patients should remain hospitalized following a procedure.

**Variations and guidelines.** The purpose of EBM and practice guidelines is to provide a stronger scientific foundation for clinical work, to achieve consistency, efficiency, effectiveness, quality, and safety in medical care. Besides escalating health care costs and inequality in health care access, major variation in accepted clinical practices is considered a third major issue facing the contemporary U.S. health care field, because observers agree that at least some of the variation stems from overuse, underuse, and misuse of medical care. These problems became apparent through the work of epidemiologists examining local practice variation. This work maps the frequency of a variety of medical interventions by geographical area (based, for example, on Medicare outcomes data) and has confirmed great variability depending on where the patient receives care. Several epidemiologists have suggested that one solution is to evaluate the scientific basis of medical and surgical treatments and offer population-based recommendations for professional standards of care. Accordingly, EBM experts define a focus and audience for a clinical guideline; retrieve, evaluate, and synthesize the evidence based on statistical analysis; summarize the benefits and risks; and determine the appropriateness of the intervention.

**International paradigm.** It is difficult to exaggerate the resonance of EBM in contemporary health care. Many observers have elevated EBM to a new international health care “paradigm.” Some indications of this new paradigm are the appearance of new national and international research institutions concerned with EBM; the centrality of EBM at the U.S. Agency for Healthcare Research and Quality (AHRQ); new journals and recurring editorials discussing the importance of EBM; innovations in methodologies and criteria for gathering and evaluating data; the surge of randomized controlled trials (RCTs) in medical research; and the rise of “causal pathways,” “care plans,” and “outcomes research” to streamline and evaluate every aspect of health care. In addition, EBM-based curricula have changed medical education, while EBM journal clubs have sprung up in hospitals. Evidence-based thinking has also been tied to nursing and allied health professions, nutrition, public health, justice, policy, and even hospital chaplaincy.

So many parties have jumped on the EBM bandwagon and so many clinical practice guidelines are churned out by individuals, professional organizations, insurers, and others that the benefits of uniformity may disappear in the cacophony of overlapping, conflicting, and poorly constructed guidelines. With more than 1,000 guidelines created annually, calls for “guidelines for clinical guidelines” have been issued. The quality of EBM guidelines has also been questioned. In ideal circumstances all decision points of a clinical practice guideline should be based on solid scientific evidence, preferably derived from a meta-review of large, double-blind RCTs. Because this “gold standard” of evidence is rarely available, except in industry-sponsored drug trials, researchers have come to rely on other methods for determining “best evidence,” such as small clinical trials with insufficient statistical power, studies with a nonrandomized control group, other nonrandom-
ized control studies, and, finally, studies without a control group such as case studies or testimonials. Guidelines have also been developed with the assistance of consensus meetings and focus groups. In addition, some clinical practices already largely follow the available evidence, while for others, insufficient evidence is available to evaluate any of the existing alternative interventions.

**Our purpose here.** In this paper we draw from the sociological literature on professions to situate the emergence of EBM, and specifically clinical practice guidelines, in a broader health care context. Our purpose is threefold: First, we review why EBM has been attractive to many health care players while others have approached it with mixed feelings. Second, using the example of asthma, we assess the limited success of clinical practice guidelines in changing clinical behavior. Third, we highlight a promising approach to guideline implementation. Our basic premise is that the debate over clinical practice guidelines and their mixed record at changing clinical practice result from a reliance on a traditional and narrow image of the medical professional as the sole decisionmaker in contemporary health care. A broader conception of health care professionalism might increase the success of clinical practice guidelines.

**Polarization Over Evidence-Based Medicine**

The polarization over EBM is the most current manifestation of a classic debate over the “soul” of medicine: Is medicine a science or an art?

**Supporters’ positions.** If we draw out the positions at the extremes, supporters tend to see standards as a panacea for the problems of rising costs, inequity, and variability plaguing the health care field. For this group, the promised benefits of EBM are self-evident: It ties clinical practices to scientific standards of evidence, thereby providing a means of measuring the efficacy of those practices. Instead of relying solely on accumulated personal experiences to determine which clinical techniques are most effective, individual clinicians using EBM will be able to draw upon the objective experience of many researchers working with accepted scientific standards of evidence and relate this evidence to an assessment of the patient’s circumstances and the practitioner’s clinical experience. Improved efficacy should also promote greater efficiency by allowing doctors and hospitals to filter scarce resources away from ineffective clinical practices and toward practices whose effectiveness has been conclusively shown. EBM should also promote greater uniformity by limiting idiosyncrasies in particular clinical procedures or in the rate at which procedures are performed. In addition, EBM promises to create better-informed patients and clinicians by offering collectively agreed-upon and publicly available information about treatment options. Guidelines also provide a strategic advantage by empowering clinicians to counter managerial decisions to alter their practices that may not be in patients’ best interest. More likely, however, managed care companies may regard clinical practice guidelines as tools to evaluate care and implement cost-cutting measures. Finally, EBM should provide a scientific basis for the construction of
public policy. Instead of relying on the opinions of interested parties, policymakers and insurers will be able to supplement these perspectives with objective evidence.

- **Critics’ positions.** Critics of EBM mostly come from within the medical professions. In addition to the many scientific problems of creating sound guidelines when evidence is weak, they stress the destructive effects of standards at the local level. In an age of mandated cost control and resource limitation under managed care, this group tends to see medicine in traditional terms: It is a “craft” or “art,” in which individual expertise and technique are allowed to shine through and ultimately result in a higher standard of patient care. By discouraging idiosyncrasies in clinical technique, standards introduce disincentives for individual innovations in care and healthy competition among practitioners. Instead of revolutionizing care, EBM therefore threatens to bring about stagnation and bland uniformity, derogatorily characterized as “cookbook medicine.”10 Ironically, EBM may also result in a lower standard of safety by deskilling practitioners. Instead of using clinical judgment, practitioners will be encouraged to follow protocols that treat all patients as essentially interchangeable. Providers will therefore be poorly equipped to contend with the variations between patients they will encounter in actual clinical circumstances. Even more problematically, traditional health care professionals may be replaced by less expensive, less skilled workers, who may be incapable of operating effectively in diverse situations.

- **Sociology of professions.** These opposing viewpoints can be understood from the perspective of the sociology of professions. Professionals distinguish themselves from other occupations by the special character of the knowledge required to perform their tasks: What matters most is that the profession determines and evaluates the technical knowledge used in its work.11 Proponents of EBM view practice guidelines as a solution to the persistent problem of practice variation that is attributable to clinical uncertainty. How can a profession maintain its special occupational status if practice varies seemingly randomly? From this perspective, constructing scientifically optimal clinical practice guidelines by clinicians for clinicians is necessary for the credibility of medicine, because a specialized knowledge base forms the core of professionalism. Opponents, however, are wary of the clinical transparency exposed by guidelines. They note that professions do not practice in isolation but are part of a system of countervailing powers.12 They fear the emergence of third parties using guidelines against professionals’ interests. In this professional doomsday scenario, guidelines reinforced by financial or legal incentives might become coercive tools to change clinicians’ behavior, curtail treatment choice, limit practitioners’ autonomy, and further undermine public confidence. From this viewpoint, systematically publishing scientifically sound evaluations of clinical practice is similar to revealing professional trade secrets and may result in the further “deprofessionalization” of medicine. In spite of their differences, however, critics and supporters of EBM each assume the presence of the autonomous, rational professional out to maximize jurisdiction in health care.
Implementation of EBM. As dramatic as these conflicting positions are, both appear to rely primarily on speculation concerning the potential consequences of standardization, rather than their actual consequences in the local clinical environment. Accordingly, both positions are predicated on the success of EBM: Clinical practice guidelines must be able to be unproblematically implemented for either outcome to emerge. The guidelines constitute neither a threat to professional autonomy nor a solution to the problem of practice variation if their effectiveness, in turn, is not scientifically supported. We next review the evidence on the ability of asthma clinical practice guidelines to change clinicians' behavior for this chronic condition.

Experience With Guidelines

Asthma: the “ideal” disease. Asthma is an ideal disease for clinical practice guideline development and implementation, for the following reasons. First, it is a serious public health concern: It is the most common chronic illness of early childhood, affecting an estimated seventeen million people, including 5.6 million children, in 1999. The incidence of asthma has been rising: Asthma-related disability in children increased 232 percent between 1969 and 1995. More than 5,000 people die each year from the condition, and asthma leads to an estimated 470,000 hospital admissions and 13.7 ambulatory care visits annually.13

Second, some of the old tenets of asthma treatment have been revised in recent decades.14 Thus, an easily identifiable “wrong” old practice and a better “updated” expert practice exist side by side. Third, asthma has been an early target for standardization via guidelines.15 In addition to multiple local protocols, the U.S. National Asthma Education Program of the National Institutes of Health’s (NIH’s) National Heart, Lung, and Blood Institute (NHLBI) convened expert panels to publish a first set of guidelines in 1991, a second set in 1997, and an update in 2002 (referred to, respectively, as EPR I, EPR II, and Update 2002). Finally, recent studies show that from more than 100 different pediatric guidelines, asthma guidelines are among the best known.16

Even if we take process-of-care measures rather than morbidity measures as health outcomes, the overall effect of asthma clinical practice guidelines is mixed and, considering the health stakes and spending, disappointing.17 Some examples: A 1995 self-report survey of emergency department directors indicated that four years after the first set of EPR guidelines had been published, only 45.5 percent of respondents had heard of the guidelines, about 24 percent had read the guidelines, and wide variation in asthma treatment practices persisted, which often contradicted the guidelines.18 A national survey of U.S. pediatricians showed that although about 88 percent expressed familiarity with asthma guidelines, only 35 percent followed guidelines.19 A 1999 study showed that after a hospital committee adopted the EPR guidelines, pilot-tested them for six months to neutralize any barriers, and educated the staff involved, adherence to the clinical protocol was only 68 percent.20
“Professionals treat guidelines more as options than as true standards, and professional organizations do not enforce adherence.”

Several studies indicate adherence with some tenets of the protocols but not with other critical elements, including diagnosis, spirometer tests, and asthma control. At the same time that studies confirm adherence to the current guidelines to be less expensive overall and to provide better patient outcomes, the use of anti-inflammatory medicine, for example, remains inconsistent with the national guidelines.

Lack of adherence to asthma guidelines occurs with Medicaid and managed care populations, in emergency departments and primary care settings, in the military and in civilian life, and it is independent of severity of illness, hospital size, or patient age. Internationally, asthma treatment continues to be characterized by “unacceptable” treatment variation in spite of guidelines.

- Other guidelines. The overall lack of behavioral effects from clinical practice guidelines is not limited to asthma diagnosis and treatment. A meta-analysis of various medical conditions reports a mean adherence rate of 54.5 percent. A comprehensive study in the Netherlands found adherence to guidelines hovering around an average of 67 percent. Other researchers have determined that the probability that physicians will follow practice guidelines is about 50 percent. These outcomes have been generally interpreted as disappointing and insufficient.

- Barriers to adherence. Among the barriers to increased adherence are lack of awareness, familiarity, agreement, self-efficacy, and expectations about outcomes with guidelines; the inertia of previous practices; and various guideline-, patient-, and environment-related external characteristics.

EBM proponents and opponents have presumed a strong belief in the rational power of science: If only the best evidence would be pulled together, good clinicians would automatically follow those recommendations. This assumption ignores a key characteristic of professionalism: autonomy and discretion in professional work. Being a professional implies a covenant based on trust that the expert will act in the patient’s best interest and that after years of education and credentialing, practitioners can be relied upon to make individual decisions. Practice guidelines, even if they have the authoritative imprimatur of professional organizations, remain weak tools to change professional attitudes and behavior. Professionals treat guidelines more as options than as true standards, and professional organizations do not enforce adherence to guidelines or reward guideline-following behavior. Compliance with guidelines depends upon the fit between the standards and the goals of and demands on the individual health care provider. The evidence suggests that many clinicians find such a fit wanting.
A Multifaceted Approach To EBM Implementation

The evaluation statistics of clinical practice guidelines have had a sobering effect on the attempt to standardize medicine with guidelines. Looking to explain the limited ability of practice guidelines to change clinical behavior, opponents of guidelines often emphasize the inherent weaknesses of standardized tools to capture the dynamic nature of medicine and the uncertainty of its knowledge base.30 Supporters, instead, indirectly blame the human factor: Busy or procrastinating health care providers do not take the time to incorporate guidelines into their practices.31 While opponents and supporters of EBM disagree on the source of error, their explanations remain consistent with the perspective of the autonomous professional looking to preserve professional jurisdiction.

Following the conclusion that guidelines are scientifically superior but their reception is weak, EBM supporters have shifted the focus of the field from the creation of practice guidelines to the science of implementing them. Several meta-reviews of the introduction of practice guidelines have concluded that the passive educational strategies commonly used have little effect in changing behavior.32 More promising avenues involve reliance on opinion leaders, methods of providing concurrent feedback, and academic detailing.33 These strategies work occasionally for some interventions and some groups of practitioners.

Increasingly, however, EBM researchers have reached the conclusion that one implementation model does not fit all and that, instead, a series of overlapping implementation strategies best target specific groups.34 Consequently, a multifaceted approach to guideline implementation has developed, which uses a more expansive approach that reaches out to multiple local stakeholders who are affected by the guideline, including physicians, nurses, pharmacists, equipment suppliers, administrators, and patients. Although it is still premature to attempt to disentangle which components of a multifaceted approach are most effective, reviews suggest that the successful implementation of clinical practice guidelines requires the construction of well-supported, uncomplicated guidelines with input from various stakeholders.35 In addition, the implementation of guidelines should be provided with strong leadership and sufficient resources. A multistage education campaign and the ability to select, involve, and follow up with target patients and provide feedback to providers also seem to improve the ability to change health care behavior. Guideline implementation also demands regular review of the scientific literature to implement updates if needed. Rather than a specific configuration of implementation strategies, the novelty of the multifaceted approach might reside in its inadvertent appreciation of the interdependent and collaborative nature of medicine.

Example: Royal Children’s Hospital. An example: When the Royal Children’s Hospital in Victoria, Australia, changed the asthma treatment from nebulizers to pressurized, metered-dose inhaler-spacers, they obtained a success rate of 95.5 percent during the first three months of transition.36 The change was prompted by the
“Change in health care is possible with guidelines if creation and implementation reflect the collaborative nature of health care.”

emergence of new evidence that inhaler-spacers are superior to nebulizers, and by the implementation of a clinical practice guideline. Why is their success rate so much higher than for most other published clinical practice guidelines? The answer is that this guideline was only one step in a comprehensive approach to changing the well-entrenched “nebulizer culture.”

The hospital first convened a multidisciplinary team of nurses and physicians of the most affected departments to review the scientific literature. The resulting guideline with input of local stakeholders was then modified after suggestions from hospital consultants and the hospital's clinical practice review committee. Next, a nurse and physician facilitated workshops with hospital staff members to explain the rationale behind the evidence. The message of the workshop was reinforced with written material and the involvement of pharmacists (in the hospital and community), infection-control team members, patient-support assistants, and local equipment distribution centers. The hospital then initiated a media campaign informing families of children with asthma of the impending change with newspaper articles and local television news clips. Parents were also informed during clinic visits. When the guideline was implemented, the staff conducted weekly chart reviews to evaluate the appropriate asthma treatment and provide feedback. Although no information is available on the cost of implementing the guideline, other studies report that the use of spacers is less expensive than nebulizers.

Example: hospital pneumonia guidelines. In the United States, similar positive results were obtained when a guideline was introduced in Spring 2000 to improve hospital compliance with the performance measures for community-acquired pneumonia from the Centers for Medicare and Medicaid Services (CMS, then HCFA). Health care providers reviewed available published guidelines and selected one from the Infectious Disease Society of America. They created an admission sheet and selected criteria from an influential study on the change from intravenous to oral antibiotics to decrease length-of-stay. Stakeholders from a wide variety of hospital services and hospital committees were invited repeatedly to comment on the criteria, and one criterion was changed based on local input. The involved physicians suggested that the implementation of this guideline required the hiring of an advanced practice nurse. The nurse was paid for by the hospital in which the change took place because lowering the incidence of community-acquired pneumonia might decrease length-of-stay. The nurse's task was to issue reminders to providers, discuss any deviations from the protocol, and collect data on the change. The guideline was further introduced via a mailing and a grand-rounds lecture with invitations to the nursing and pharmacy staff. Compliance with CMS indicators greatly
improved, and length-of-stay decreased without a rise in the readmission rate for discharged patients.\textsuperscript{38}

Here we have guidelines that actually work. But do the guidelines cause change or merely channel it? Examples of the multifaceted guideline-implementation approach suggest that guidelines are a necessary but insufficient step to effectively change clinical care. The important point is that change in health care is possible with guidelines if standard creation and implementation reflect the local interdependent and collaborative nature of health care delivery.\textsuperscript{39} In the Australian asthma example, the substitution of metered-dose inhalers for nebulizers was not simply imposed by health care providers out to maximize their profession’s jurisdiction, but rather involved fostering relationships and collaborations among a wide variety of relevant local stakeholders aligned with the aim of using new therapies to lower asthma morbidity. The guideline helped coordinate and strengthen these relationships. Professional goals were still served, but they were not the driving force behind the guideline.

The success of the current EBM paradigm has been constrained by its orientation toward a traditional image of the autonomous medical professional decisionmaker. The construction and implementation of clinical practice guidelines tend to remain the exclusive purview of leading experts and thereby regularly fail to take into account available resources and opinions of allied professionals, support staff, and patients who will be directly affected by the guidelines. Adherence to guidelines is also seen as a matter of individual choice. In keeping with traditional conceptions of professional autonomy, few mechanisms have been put in place to encourage the use of EBM or to discourage individual deviation from accepted guidelines. The individualistic, voluntary, and exclusive character of EBM as it is largely implemented undermines its overall effectiveness as a tool for improving care.

Addressing these difficulties does not necessarily require placing greater emphasis on enforcement but requires the introduction of explicit strategies to take advantage of the interdependent and collaborative character of medical work. A multifaceted approach tailored to local stakeholders and their differing interests is one such strategy that solves the problem of transparency without undermining professional autonomy. In the current health care climate, clinical practice guidelines used to capitalize upon interdependency and coordinate collaboration, as well as to install uniform best practices, increase the likelihood of a better standard of medical care. Rather than a final product that spontaneously propels change by the force of rationality, the guideline becomes then a scientific rallying point in a comprehensive organizational process of change. Such a comprehensive approach makes sense if we accept that health care efficiency, quality, and safety are not simply professional concerns but greatly affect patients, payers, and policymakers. Changing health practice should similarly be a joint responsibility.
The authors thank the reviewers and editors for useful comments.

NOTES

19. Flores et al., “Pediatricians’ Attitudes, Beliefs, and Practice.”


30. Charlton, “Restoring the Balance.”


