Evidence-based medicine has created a paradigm shift in medicine, away from authority-based decision making and toward decision making that is informed by research and considers patient values and preferences. Evidence-based clinical practice requires accessing and evaluating the highest-quality information that is relevant to the patient problem at hand. In light of the best evidence, clinicians can help make decisions in keeping with their patients' values and preferences. [Brief Treatment and Crisis Intervention 4:187–194 (2004)]

**KEY WORDS:** evidence-based medicine, history of medicine, decision making, mental health.

Evidence-based medicine (EBM) represents a move away from authority-based medical decision making toward a process that is informed by research and considers patient values and preferences. Evidence-based decision making requires clinicians to understand and characterize the clinical circumstances of the patient, to assess their own awareness of the benefits and risks of management alternatives, to define questions that address knowledge gaps, and to determine how available evidence applies to their patients. In light of the best available information, clinicians can help patients make decisions in keeping with the latter's values and preferences.

**The Need for Evidence-Based Decision Making**

Health care personnel consistently face difficult questions regarding patient care. Traditionally, clinicians have addressed these issues by calling on their own experience, that of respected colleagues, and traditional manuals or textbooks. Variability in clinical experience amongst individuals has often resulted in wide discrepancies in practice, without a satisfactory process for resolving these discrepancies.
Conventional textbooks and manuals are often out-of-date, and their recommendations may neither include a critical assessment of the best available research nor consider variability in values and preferences. For instance, Antman, Lau, Kupelnick, Mosteller, and Chalmers (1992) have documented recommendations for management of myocardial infarction that sometimes lagged a decade behind the evidence from randomized trials, or even contradicted the results of those trials.

With the rapid increase in important research available through journals and conferences, health care personnel face major challenges in staying up-to-date. Developing an appreciation of evidence-based resources allows clinicians the ability to track down and use secondary sources of preappraised evidence that provide immediately applicable conclusions and thus optimize clinicians’ efficiency in keeping abreast of current best evidence.

Although rigorously preappraised resources are ideal in concept, they are at times insufficient to produce consistent evidence-based care. Therefore, for optimal evidence-based practice, clinicians require the skills necessary to conduct searches for primary studies “from scratch.” This requires the development of an answerable question that centers on the patient, the problem, and the desired outcomes, followed by a thorough search of the available literature to identify relevant and up-to-date information.

Clinicians then critically appraise potentially relevant studies to determine their validity and applicability to the patient at hand. Finally, the clinician determines how to apply the evidence to generate management plans consistent with the patient’s values and preferences (see Figure 1). These skills are learned over time, by acknowledging our knowledge gaps and seeking to improve as clinicians. In this article, we will briefly address some of the background and concepts of EBM.

Development of Evidence-Based Medicine

Although the concepts of EBM have been developing since clinical trial publications became available (Chalmers & Trohler, 2000), the formal construct of devising a clinical question and searching available evidence with a critical eye toward applying it to patient problems has evolved in the last 20 years (Department of Clinical Epidemiology and Biostatistics, 1981). The term evidence-based medicine first appeared in a description of the McMaster University internal medicine residency program (Guyatt & Rennie, 2002), and the first published use of this term was in 1991 (Guyatt, 1991). Subsequently, the Journal of the American Medical Association published a series of 32 articles entitled User’s Guides that made available the fundamental concepts of evidence-based practice (Evidence-Based Medicine Working Group, 1992) and were followed by a number of texts that further developed...
A Hierarchy of Evidence

Any time we consciously observe events in the world with the purpose of making inferences about the likelihood of future events we are collecting evidence. A key feature distinguishing EBM from other approaches to clinical decision making is that EBM postulates a hierarchy of evidence (Figure 2). At the bottom of the hierarchy are unsystematic clinical observations that are subject to biases associated with intuitive inferences. We know, for instance, that clinicians (like other humans) are unduly influenced by vivid and memorable events. Thus, the unique and striking patient is likely to influence clinical decision making far beyond what a rational assessment would warrant.

While the hierarchy is not intended as a rigid structure, basic science or laboratory experiments can, in general, lead to stronger inferences than can unsystematic clinical observation. Nevertheless, while basic science experiments have resulted in countless successes, they have often proved limited in translation to patient care (Fleming & DeMets, 1996).

While observational studies often rank above basic science experiments, the varying strength of observational designs and the inconsistent rigor with which investigators carry out observational studies emphasize that one should not treat the hierarchy as a rigid series of rules. Case series generally provide an important source of hypotheses, but because of weak inferences that typically result, one must test those hypotheses in studies of stronger design (Gottlieb et al., 1981). Cohort studies can provide definitive evidence regarding disease incidence and prognosis and, along with case-control and before-after studies, suggest possible treatment benefit. But their fundamental limitations (imbalance in prognostic factors in cohort and case-control studies, uncertainties of natural history and cointervention in before-after studies) weaken inferences from observational studies.

While well-conducted observational studies often provide accurate estimates of benefit, they sometimes result in exaggerated effects or underestimate benefit (Kunz & Oxman, 1998). The history of hormone replacement therapy (HRT) as a possible treatment for a number of health problems that afflict postmenopausal women illustrates the limitations of observational studies. Well-designed and rigorously conducted observational studies demonstrated, on a consistent basis, a strong association between HRT use and a lower incidence of major cardiovascular events, as well as better cognitive function. However, randomized trials of HRT, in particular in the Women’s Health Initiative (WHI), have shown that it does not reduce the incidences of either major cardiovascular events or dementia, and may actually increase them (Rossouw et al., 2002; Shumaker et al., 2003).
Randomized controlled trials (RCTs) like the WHI, if rigorously conducted, provide stronger evidence than do observational studies. In a randomized trial, patients are assigned to treatment or control groups on the basis of chance. As sample size increases, randomization makes it increasingly likely that prognostic variables, both known and unknown, are balanced between treatment and control groups. Blinding those who enroll patients, the patients themselves, caregivers, data collectors, data analysts, and judicial adjudicators of outcomes to patients’ allocation to experimental or control treatments further decreases the likelihood of a biased estimate of treatment effect.

A systematic review and meta-analysis summarizing the results of all relevant high-quality studies can further strengthen inferences from RCTs. A systematic review of RCTs of antioxidants for the prevention of cardiovascular events provides an example of how such a review can adduce crucial information for clinical decision making. Despite variable results of individual RCTs of vitamin E, the weight of the evidence indicates that vitamin E is ineffective and that beta-carotene may be linked to increased mortality and cardiovascular events (Vivekananthan, Penn, Sapp, Hsu, & Topol, 2003).

RCTs and systematic reviews can provide surprising positive, as well as negative, results. For instance, a recent systematic review demonstrated that electroconvulsive therapy was an effective short-term treatment for depression and may be more effective than drug therapy. Systematic reviews can inform us about not only treatment benefit, but also adverse events. An example specific to mental health is a systematic review of amitriptyline versus other tricyclics for depression (Guaiana, Barbui, & Hotopf, 2003). This analysis of 184 studies indicates that amitriptyline is as efficacious as other tricyclics for depression but carries with it important side effects that may outweigh its benefit.

The highest level of evidence comes from applying systematic research methods to care of the individual patient—the N of 1 randomized trial (Guyatt et al., 1986). N of 1 trials require the clinician and patient to work together to determine the impact of a treatment in the individual. In stable chronic conditions unlikely to resolve spontaneously, patients undergo pairs of treatment periods, in which they receive one treatment during one period and a placebo or alternative during the other period, the order of periods within a pair being determined by random allocation. Inferences are further strengthened when both patient and clinician are blinded to the sequence. The trial continues until there is clear evidence establishing, or refuting, the superiority of one regimen over the other (Guyatt & Rennie, 2002, p. 277).

A series of N of 1 trials exploring the effectiveness of methylphenidate in depressed geriatric patients demonstrates the applicability of N of 1 trials in addressing treatments for mental health problems (Jansen, Olde Rikkert, Hulsbos, & Hoefnagels, 2001). This series of five trials showed significant and sustained improvement in 2 participants. Conventional RCTs of methylphenidate in elderly depressed patients have shown mixed results (Emptage & Semla, 1996; Jansen et al., 2001), most likely because some patients respond to the treatment, while others do not. The N of 1 RCT allows the clinician to prescribe treatment for those who benefit, while avoiding unnecessary use in unresponsive patients.

The hierarchy of evidence informs decision making by helping the clinician to ascertain the appropriate strength of inference associated with the evidence. Evidence from the top of the hierarchy has greater influence than lower-level observations. There are, however, instances in which low-level evidence about toxicity trumps evidence from systematic reviews of
RCTs addressing effectiveness. For instance, a 2001 systematic review demonstrated kava kava’s effectiveness for treating anxiety (Pittler & Edzard, 2001); but in 2003, case reports suggested that kava kava could cause liver failure (CDC, 2003). Given the alternative—low-toxicity agents available for treating anxiety—the potential toxicity argues strongly against the use of kava kava.

This example highlights a fundamental principle of evidence-based practice: Clinicians must use the highest-quality evidence bearing on risk and benefits of alternative therapies, even if the quality of the evidence is poor. The challenge of evidence-based practice is to ascertain the best estimates of the risk and benefits of alternative management strategies, the uncertainty associated with those estimates, and the patient’s values and preferences, and bring each of these to bear in helping patients choose treatment options that reflect their best interests.

Practicing evidence-based medicine efficiently requires rapid identification of summaries of preappraised primary studies. For busy clinicians who require up-to-date and valid information, there are sources of preappraised evidence specific to mental health. One important resource is the journal Evidence-Based Mental Health, which identifies and appraises the validity of primary studies and systematic reviews published in 41 leading journals. It is available in print form and online in a quarterly publication.

Clinicians may find other resources useful. The Cochrane Database of Systematic Reviews (http://www.cochranelibrary.com) features the work of review groups specifically devoted to mental health. Other sources, such as Clinical Evidence (http://www.clinicalevidence.com) and PIER (Physicians’ Information and Education Resource) (http://www.acponline.com), offer online summaries of the best available evidence about problems frequent in primary care and internal medicine, including regularly updated sections on psychiatry and mental health issues.

Ideally, evidence-based practitioners are able both to quickly identify preappraised sources of evidence and to appraise primary studies addressing questions of therapy, harm, diagnosis, or prognosis. Clinicians interested in acquiring these skills will find the User’s Guides to the Medical Literature (Guyatt & Rennie, 2002) a helpful resource. The text provides guides to efficiently identify key studies, determine their validity, understand their results, and judge their applicability to individual patients.

Patient Values

Determining best estimates of risk and benefits of alternative management strategies is only the first step in clinical decision making. Application of evidence must also account for the patient’s specific circumstances and values. Values and preferences determine the significance that patients place on risks and benefits and their associated uncertainties (Guyatt, 2002, p. 567).

Patients also vary in their preferred styles of decision making. At one extreme, patients prefer clinicians to make decisions on their behalf. At the other extreme, patients want to understand the risks and benefits and make their own decisions. Most patients fall in between, and prefer some form of shared decision making.

Differences in values and preferences may lead to discrepant decisions about therapy, with potential important adverse effects. Consider, for instance, patients with socially disruptive Tourette’s syndrome. Some may wish to forgo treatment due to important adverse effects (Margolese, Annable, & Dion, 2002),
while others may have no doubt that benefits outweigh potential risks.

Not only patients, but also their families may face challenging value judgments. For instance, cultural attitudes, perceptions about alternative therapies, and attitudes toward uncertainty may all influence decisions about the use of *Ginkgo biloba* in a family member suffering from dementia. A clinician’s responsibility is to avoid dismissing unconventional alternatives without considering the evidence regarding both safety and efficacy. In this case, family members may be wise to consider the treatment, as there is promising evidence that it is generally without harm (Birks, Grimley, & Van Dongen, 2002; Ernst, 2002).

For patients who are uncomfortable with, or incapable of, taking part in the decision-making process, the physician’s responsibility is to develop insight to ensure that choices made on patients’ behalf will be consistent with their values and preferences.

**Implications for Teaching**

Understanding evidence-based principles can empower clinicians, giving them confidence in using the medical literature to understand risks and benefits of interventions and to critically interpret the increasing number of guidelines and policies of administrative bodies. At the level of the student researcher, fostering the ability to critique studies and determine validity promotes higher-quality research. This will ultimately translate into better therapies, diagnostic tests, and understandings of the clinical course of health conditions. For health care administrators, developing skills in identifying and critically evaluating evidence will help bridge the gap between administrator and clinician, facilitating the development of integrated approaches to patient care (Gray, 1997). We can expect an increasingly educated and engaged public to benefit from knowledge of the basic principles of EBM. Indeed, guides for the savvy health care consumer introducing the concepts underlying EBM are becoming available (Irwig, Irwig, & Sweet, 1999).

**Conclusion**

An understanding of the principles of EBM is essential for enlightened modern health care practice. Optimal clinical decision making requires knowledge of the best available evidence regarding benefits and risk of alternative management strategies. Clinical expertise in 2004 requires both the skills to efficiently obtain and interpret this best evidence and the ability to apply the evidence in concert with patients’ values and preferences. Becoming proficient in evidence-based practice may be challenging, but ultimately enhances clinician autonomy and enriches health care practice.

**References**


