

Evidence-Based Medicine

Jennifer L Smith, NMD

*Lifestream Wellness Clinic
4921 E Bell Rd Suite 203
Scottsdale, AZ 85254
602-441-3455*

Interesting Times

The expression “May you live in interesting times” can be aptly applied to the radical paradigm shift occurring in clinical healthcare. . The doctor-patient relationship is transforming from a doctor-centered, paternalistic model of the 19th and 20th centuries to a mutually respectful, collaborative relationship. In the pre-Internet era, the practice of medicine was considered an “art form.” Doctors were granted God-like status and unilateral authority for directing patient care. The use of scientific method, biomedical research and data analysis were rare in clinical practice.¹⁵ Physicians relied primarily on clinical acumen, basing clinical judgments on personal or anecdotal experience.

Today, medical knowledge, previously available exclusively to doctors via medical schools and research libraries, is now easily accessed on the Internet. In the age of “Dr. Google,” over 80% of patients seek medical information on the web.¹² Desperate for solutions, patients endeavor to participate in their healthcare by becoming “experts” on their conditions, often consulting symptom checkers, blogs, chat rooms and Facebook groups. Armed with a stack of computer printouts, patients strive to assist their doctors by suggesting treatments based on self-sourced data that is often incomplete, inaccurate, unproven or unsuited to their condition.

Many patients, disheartened by conflicting information, skyrocketing healthcare costs, failed treatment attempts and poor rapport with past clinicians, report decreased trust in doctors and the healthcare industry. To address these concerns and optimize healthcare quality and consistency, a clinical approach known as evidence-based medicine (EBM) was introduced into the medical community in the 1990s.¹⁵ EBM is defined as “the integration of best research evidence with clinical expertise and patient values.”¹³ The physician gathers relevant, high-quality data from current studies on possible intervention strategies with similar patients, then applies clinical acumen to co-create a treatment plan with the patient.

A Brief History of EBM

EBM seeks to discover “the best course of care for a patient,” by integrating clinical wisdom and current science.² Twentieth century English epidemiologist Archibald Cochrane is acknowledged as the founder of today’s EBM, when he recognized the need for clinicians to monitor the latest medical research. In the late 1960s, an academic movement commenced to create a new form of medicine to bridge the gap between biomedical research and clinical care. In 1967, the term “clinical epidemiology” emerged as a precursor of EBM, defined by Dr. David Sackett as “the application, by a physician who provides direct patient care, of epidemiological and biomedical methods to the study of diagnostic and therapeutic process in order to affect an improvement of health.”^{5,14} Sackett, with colleagues at McMaster University Medical School, developed a method called “critical appraisal” to teach doctors how to analyze and apply research data with patients in their clinical practice. The term “evidence-based medicine” first appeared in a 1991 *ACP Journal Club* editorial, and in a 1996 article in the *British Medical Journal*, was defined as

“the conscientious and judicious use of current best evidence from clinical care research in the management of individual patients.”^{8,14}

The 5-Step EBM Approach

In contrast with the traditional doctor-centered clinical approach, the EBM clinician seeks to educate the patient and actively involve them in co-creating an optimal treatment plan. EBM clinicians follow a 5-step approach:

Step 1: Define the Problem

Ask the patient clear, answerable clinical questions, utilizing the PICO (Patient/Problem, Intervention, Comparison, Outcome) format:

P (Patient/Problem): Who is the patient (medical history, lifestyle, risk factors)? What is their problem (diagnosis, prognosis, etiology)? What is their desired outcome (cure, symptom management, palliative care)? What treatments, if any, has the patient tried, with what results? What are the patient’s treatment preferences, and their available resources or constraints (financial, insurance, caregivers, etc.)?

I (Intervention): Develop a patient-specific care plan, including types and frequencies of interventions (diagnostic tests, screening, medication, surgery, nutrition, etc.) based on the patient’s preferences and resources.

C (Comparison): Search biomedical databases for patients with a similar prognosis. Compare the proposed intervention plan to alternative or control strategies.

O (Outcome): Weigh potential benefits and consequences of the proposed intervention plan and predict a treatment outcome.

Step 2: Acquire the Best Evidence

Acquire the best evidence to address the patient’s clinical questions. Utilize reliable informational sources, including medical journals, secondary literature and biomedical databases. Evidence should be current, comprehensive (show benefits and risks of proposed treatments) and high quality.

Evidence is classified into four reliability levels, from highest to lowest. Level I evidence is considered the “gold standard” and most reliable. It comes from double-blind, randomized controlled trials (RCTs) that minimize selection bias by assigning subjects to a treatment group that receives a new intervention or a control group that receives a standard treatment or placebo (no treatment). Level II evidence derives from non-randomized or comparative (cohort) studies and aggregate patient data from multiple time series. Level III evidence includes case notes, expert committee reports, testimonies and descriptive studies with no control group. Level

IV evidence, considered the lowest quality and least reliable, originates from anecdotes, expert opinions and self-reported patient questionnaires.

Step 3. Evaluate the Evidence

Critically evaluate the data to determine the best possible intervention strategy. Examine the study's methods, conclusions and similarities (or differences) between study participants and the patient to determine data relevance and value. Clinicians must exercise caution in evaluating data reliability in an era where evidence is regularly skewed by industry-sponsored studies.³

Step 4. Apply the Evidence

Discuss relevant evidence with the patient, including cost, availability and possible benefits and risks of the proposed treatment. Involve the patient in co-creating a care plan that fits their unique needs, values, preferences and resources. Offer clinical opinions, answer questions and discuss treatment alternatives so the patient may make the best possible informed intervention choice. Jointly agree on a treatment plan.

Step 5. Evaluate Treatment Efficacy

Assess the efficacy of the intervention. Did the patient improve? If results did not meet expected projections, the physician will need to seek the source of the discrepancy (e.g., patient adherence to the prescribed treatment, missing information, etc.), adjust the treatment or consider other treatment alternatives.

Sourcing and Summarizing the Evidence

One of the strongest arguments for EBM is that access to the best possible evidence will standardize medical care at the highest level. To stay current on the latest published studies, a busy physician would have to read 19 articles per day, 365 days per year, not to mention the time it would take to critically evaluate each study. In one University of Oxford survey, general practitioners reported spending only one hour per week to review new studies.⁹ Lacking time to keep up with current research, many doctors base treatment decisions on experiential or anecdotal evidence.

A number of online databases exist to support clinicians in sourcing and evaluating research data and making point-of-care decisions. These sites offer synthesized evidence in the form of systematic reviews, meta-analyses, guidelines and critically appraised topics:⁴

- ACCESSS
- ACP Journal Club
- Cochrane Library
- DynaMed
- Embase
- Essential Evidence Plus
- Health Services/Technology Assessment Texts (HSTAT)

- PubMedHealth
- Turning Research Into Practice (TRIP)
- UpToDate

The EBM Debate

The United States is in the throes of an unprecedented healthcare crisis. In 2016, annual healthcare costs exceeded \$3.4 trillion, accounting for over 17% of the Gross Domestic Product (GDP).¹¹ To curb rising healthcare costs and increase treatment efficacy, U.S. policymakers are implementing an historic paradigm shift that employs strict EBM standards.² The Pay-for-Performance (P4P) reimbursement model is replacing the fee-for-service (FFS) model. FFS, which reimbursed providers for quantity of services performed, created incentives for clinicians to perform more tests and services, regardless of patient outcomes. In contrast, P4P provides financial incentives for providers to improve quality and efficiency of care when they meet or exceed certain pre-established performance metrics.¹⁶

The EBM debate, which has raged for over three decades, is no longer about whether to implement the paradigm. EBM is here to stay. The real question is whether evidence-based medicine can deliver on its core vision of optimizing healthcare. Proponents tout EBM's ability to increase the safety, quality, uniformity and cost-effectiveness of healthcare by creating better-informed clinicians and patients. The use of online databases and systematic clinical guidelines can help minimize treatment variations and decrease harmful interventions. For instance, EBM has improved treatment practices in areas such as stroke and myocardial infarction aftercare. Harmful practices were also reduced in post-menopausal hormone replacement therapy, when trials revealed that the risks outweighed the benefits.³

Critics argue that the use of database algorithms may instead compromise patient care by instituting a uniform, protocol-driven "cookbook approach" to medicine that treats all patients the same. Real patients rarely fit textbook descriptions of disease. Because clinical trials focus on outcomes across homogenous or narrowly focused populations, EBM critics argue that RCTs are improperly used to reach conclusions. Evidence from RCTs may not be relevant to individual patients with complex symptoms and comorbidities.²

EBM opponents also assert there is no clear way to validate evidence quality in published studies. Between two-thirds and three-quarters of all randomized trials published in major journals are funded by vested players in the drug and medical device industries. Industry-funded studies are notorious for evidence corruption, reporting bias, inappropriate control interventions and surrogate outcomes.¹⁰ Authors of industry-sponsored studies, in a drive to increase profits or funding, routinely employ "spin" in the form of misleading descriptions and exaggerated positive results to generate enthusiasm for their particular product or procedure.^{3,10} Biased reporting, including data suppression and partial reporting of trial results, has led to allegations of fraud, litigation and loss of public trust.⁸ In 2013, Johnson &

Johnson pled guilty to a criminal misdemeanor in the marketing of risperidone. The company was fined \$2.2 billion in criminal and civil fines in 2013 and \$1.2 billion in 2012 for deceptive practices, including hiding risks and exaggerating benefits.³

Healthcare policies tying insurance reimbursement to statistically “proven” treatment methods may compromise the autonomy of the doctor-patient relationship by limiting a patient’s rights to choose. Linking provider incentives to performance metrics may discourage physicians from exploring less conventional treatment options. Physician and patient may be held hostage, unable to employ a desired treatment option while waiting for appropriate statistical evidence.

To Future of EBM

For EBM to deliver the highest level of healthcare, the paradigm must evolve to implement workable strategies to address the most critical concerns and challenges. Recommendations include:

Enhance Evidence Quality and Transparency

To enhance evidence quality and transparency, the following measures are suggested:¹⁰

- Eliminate bias and spin from industry-sponsored studies.
- Eliminate selective publication by registering and reporting all clinical trials, regardless of results.
- Change funding models for clinical trials to create a firewall between profit interests and research.
- Produce evidence summaries, assessments and guidelines free of commercial and personal conflict of interest.
- Increase investment in independent research by individuals and institutions.
- Develop tools to summarize and present data in a form that is clear, understandable and easy to interpret by physicians and patients.
- Expand evidence databases to include lower level and obscure sources.

Increase Evidence Relevance

Eligibility criteria for RCTs are strictly controlled. Data from studies conducted on discrete populations may not reflect the distinctive needs of individual patients with complex diseases, circumstances and values. Though RCTs are considered the best source of available evidence, Level 1 evidence is often difficult to obtain and may not be relevant.² To serve the broadest scope of patients, the concept of “best possible evidence” must be expanded to include lower levels of evidence on a case-by-case basis.

EBM: The Next Generation

EBM has continued to evolve since its inception. Figure 1, the first EBM model published in 1996, assigns equal values to research evidence, clinical expertise and patient preferences in the clinical decision-making process.^{6,7,8}

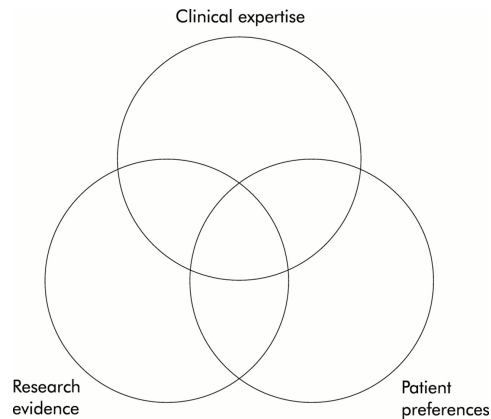


Figure 1
Early Model of the Key Elements for Evidence-Based Clinical Decisions⁶

This early model was more descriptive than prescriptive, and seemed to downplay the value of the clinician's experience. Did EBM seek to reframe the doctor's role from authoritative artist to mere data analyst? Would artificial intelligence and impersonal expert system algorithms ultimately replace a physician's human intelligence, expertise and acumen? In cases where the doctor's expert recommendations diverge from the patient's values and preferences, who chooses the course of treatment?

While both sides of the EBM debate concur that evidence alone does not provide a sound basis for clinical decisions, it has taken 20 years for a new model of EBM to emerge to better guide the clinical decision-making process.

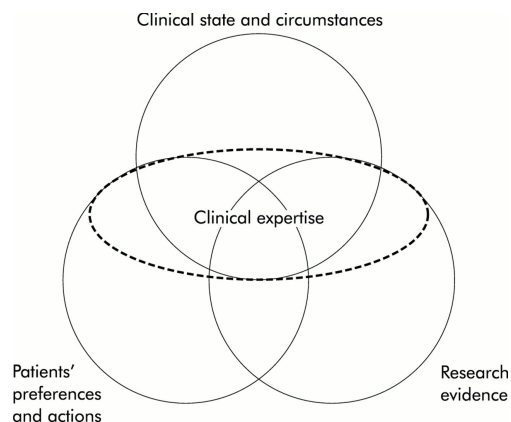


Figure 2
Updated Model for Evidence-Based Clinical Decisions⁶

In Figure 2, a patient's "clinical state and circumstances" at the time they are seeking a medical intervention replaces "clinical expertise" as a key decision driver. Two patients with the same symptoms or diagnosis and different risk factors or comorbidities will require different interventions.^{6,7}

The clinician must comprehend the patient's social and clinical context, as well as personal values and preferences. In the new model, "patient preferences" changes to "patients' preferences and actions." Patient values and preferences include the patient's unique perspectives and beliefs, expectations and goals for life and health.¹⁰ Often, a patient's stated preferences will correspond with the doctor's proposed intervention, but the patient is unable or unwilling to take necessary actions to follow the treatment plan as prescribed (e.g., quit smoking, make dietary or lifestyle changes, take prescribed medications, etc.). In this case, the clinician must adjust the treatment strategy.

In EBM's new model, the physician's clinical expertise becomes the central factor in the diagnosis and treatment process, integrating and guiding the other three factors. Rather than "dumb down" the doctor's role, EBM will require the physician of the future to enhance their clinical acumen and master an expanded clinical skill set, including:

- Keen listening and interviewing skills to ask the right questions and define the patient's problem.
- Open-minded, respectful acceptance of the patient's values, perceptions and preferences.
- Computer skills to navigate online databases and decision-support tools.
- Expertise in critical and statistical analysis to interpret data for relevance.
- Communication skills to educate the patient, explore treatment options and encourage treatment compliance.
- Humility and willingness to enlist the patient as a partner in the healing process.
- Flexibility and ability to compromise or change course of treatment when necessary.

Trust is fostered by the patient's perception of the doctor as compassionate, skilled and competent. Patients trust doctors who meet their expectations. Physicians have a moral and financial duty to provide healthcare solutions substantiated by the best available science, not myths or rumors. Physicians also have a responsibility to assume proactive leadership roles to direct the course of a paradigm shift that, in its darkest expression, threatens to relegate doctors to mindless technicians and servants of healthcare policymakers and Big Pharma.

The 21st century physician's challenge will be to walk in two worlds, to employ the best evidence when it exists, and when it does not, to boldly think outside the box and explore innovative or experimental treatment strategies with willing patients.

As clinicians conduct independent research on a greater range of patient populations, more effective treatments will become accessible for complex conditions.

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