Evidence based medicine

S J Fratesi MD
NOSM med student teaching
• Day to day clinical practice involves solving questions in a peer supported environment through evidence based medicine and involves critical appraisal of clinical papers and guidelines through the EBM process.

• EBM is the conscientious, explicit and judicious of the current best evidence in making decisions about the care of individual patients (Sackett 1996)
Fallibilism

- Fallibilism is the philosophical doctrine that all claims of knowledge could, in principle, be mistaken. Therefore, the truth is subject to change.
- Empirical knowledge can be revised by further observation, and any of the things we take as knowledge might possibly turn out to be false.
Epistemology: theory of knowledge
branch of philosophy concerned with the nature and scope (limitations) of knowledge.

It addresses the questions:
What is knowledge?
How is knowledge acquired?
What do people know?
How do we know what we know?
Why do we know what we know?
What is evidence-based medicine?

“Evidence-based medicine is the integration of best research evidence with clinical expertise and patient values”

- Dave Sackett
Evidence based medicine

- Integration of clinical expertise, patient values and the best evidence into the decision making process for patient care
- Clinical expertise refers to the clinician’s cumulated experience, education and clinical skills
- The patient brings to the encounter personal values expectations and concerns
- Best evidence is found in clinically relevant research conducted through sound technology (Sackett 2002)
### Steps in EBM process

<table>
<thead>
<tr>
<th>The patient</th>
<th>Define a clinical question or problem related to patient care</th>
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<tbody>
<tr>
<td>The question</td>
<td>Formulate a clinical question related to the case</td>
</tr>
<tr>
<td>The resource</td>
<td>Select resources and conduct a search</td>
</tr>
<tr>
<td>The evaluation</td>
<td>Scrutinize the evidence for its validity and usefulness</td>
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<tr>
<td>The patient</td>
<td>Integrate the evidence with clinical expertise and patient preferences and apply to practice</td>
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<tr>
<td>Self evaluation</td>
<td>Evaluate performance with this patient with this particular problem. The use of audit, reflections and critical case learning</td>
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Two types of evidence-based medicine

- **Evidence-based guidelines** the practice of evidence-based medicine at the organizational or institutional level. This includes the production of guidelines, policy, and regulations.

- **Evidence-based individual decision making (EBID)** is evidence-based medicine as practiced by the individual health care provider.
Clinical practice guidelines

- Documents guiding the decision making process in issues of health care
- Based on current medical evidence
- Integrate identified decision point and courses of action
- Standardize medical care
- Raise the quality of care
- Reduce risk
- Balance cost against best medical practice
Why guidelines?

Health-care is becoming more and more complicated at an astonishing rate.

Doctors are increasingly expected to take decisions balancing benefits for the patient against financial costs.

Guidelines are documents supporting health-care professionals in managing a disease in a patient to avoid substandard practices or outcomes. Their aim is to promote standards of medical care.
The proliferation of well-documented, well-founded, clear guidelines should be useful if combined with efforts to implement them and measure their effectiveness.
Guidelines

Guidelines come from a variety of sources, each with its own vision of how to proceed:

Government (federal/provincial)

Physician organizations, such as the OMA & CMA

Research groups, such as RAND, ICES

Individual health care institutions and systems

Private sector sources, such as consultants and health plans
EVIDENCE OR OPINION?

source of friction : “whether guidelines should be largely based on evidence or from opinion based on the consensus of a panel of experts. "

Guidelines
Clinical guidelines

there is a distinction between those that have a strictly defined, procedure-specific orientation (critical pathways) and those that are used to confirm diagnoses and attempt to set broad boundaries of acceptable care, within which the practitioner chooses the optimal approach (boundary guidelines).
Evidence-based medicine

Sackett

Evidence based medicine is the conscientious, explicit, and judicious use of current best evidence in making decisions about the care of individual patients integrating individual clinical expertise with the best available external clinical evidence from systematic research.
Evidence-based medicine

Using techniques from science, engineering, and statistics, such as meta-analysis of medical literature, risk-benefit analysis, and randomized controlled trials, EBM aims for the ideal that healthcare professionals should make "conscientious, explicit, and judicious use of current best evidence" in their everyday practice.
Evidence-based medicine has demoted *ex cathedra* statements of the "medical expert" to the least valid form of evidence. All "experts" are now expected to reference their pronouncements to scientific studies.
Level A: Good scientific evidence suggests that the benefits of the clinical service substantially outweighs the potential risks. Clinicians should discuss the service with eligible patients.

Level B: At least fair scientific evidence suggests that the benefits of the clinical service outweighs the potential risks. Clinicians should discuss the service with eligible patients.

Level C: At least fair scientific evidence suggests that there are benefits provided by the clinical service, but the balance between benefits and risks are too close for making general recommendations. Clinicians need not offer it unless there are individual considerations.

Level D: At least fair scientific evidence suggests that the risks of the clinical service outweighs potential benefits. Clinicians should not routinely offer the service to asymptomatic patients.

Level I: Scientific evidence is lacking, of poor quality, or conflicting, such that the risk versus benefit balance cannot be assessed. Clinicians should help patients understand the uncertainty surrounding the clinical service.
An exercise in evidence base

- It is a myth that the Scottish male wearing a kilt does not wear underwear

- A simple statement but where is the evidence?
An exercise in evidence base

- It’s all just speculation
- There are a number of possible explanations to explain the child’s reaction!
An exercise in evidence base

- Perhaps the myth is true or are they actually wearing designer thong underwear?
An exercise in evidence base

- The truth is out there
- It may be closer than you think BUT it may not be that obvious!
Two types of knowledge are involved in good medical practice.

Knowledge concerning the (patho)physiological mechanisms underlying the disease, and the way treatment influences these mechanisms (background knowledge). The knowledge involved could be causal in nature, and is an example of object-knowledge.

Knowledge concerning good practice in treatment selection; this is meta-knowledge.
Continuous medical education

- Embodies professional learning and growth and self directed
- Hope of CME is to integrate knowledge into practice
- Forms basis of knowledge translation which moves a physician from awareness to agreement, adoption, adherence to practice guidelines and a change in clinical behaviour
Does the acquisition of knowledge translate into behavioral change which improves clinical outcomes?

What is the driving force for CME?

Are physician performance indicators and patient outcome scores a measure of competence?
Conceptual change and practice

- As self directed learners, physicians must set goals and utilize appropriate educational strategies to achieve these goals.
- Physicians must recognize the need for change in their behaviour and develop the knowledge, skills and attitudes to achieve this.
- There must be an identifiable reason for change prior to implementation (Fratesi 2007).
Canadian doctors do endorse guidelines but slow to implement

Physician initiatives most acceptable

Government/third party less acceptable

Positive role of a physician champion in initiative
Guidelines must be user friendly

Guidelines must be locally acceptable and relevant

Guidelines must be better than what is already there
Prior to 1990’s most guidelines by Delphi principle

Heavy reliance on clinical experience, recent journals and textbook

Education by lecture and pamphlet

Guidelines must be valid, reliable and feasible

Major reason for failure is poor dissemination and implementation
Barriers to guideline use

- Too rigid
- Challenge physician autonomy
- Oversimplified (cookbook)
- Legal liabilities/use in disciplinary action
- Local environment does not allow implementation
- Don’t believe in them
- Don’t understand them
- Don’t like who developed them
- Don’t agree with them (specialists)
Barriers (continued)

- Might be implemented here but not there
- Lack of awareness
- Lack of familiarity
- Practice inertia
- Personal/patient preferences
- Lack of resources
- Bias in the guidelines
Medical guidelines

- Identify, summarize and evaluate best evidence about prevention, diagnosis, therapy, risk/benefit ratios and cost effectiveness
- Defines the questions in clinical practice and identifies the clinical options and their outcomes
- If they contain decision or computational algorithms, decision point and course of action are integrated with the physician clinical judgment and experience
Which doctor do you want?

William Osler, 1900

Smart young doctor
Which doctor do you want?

Wise & experienced smart young doctor
The Prognosis of Ignorance is Poor
Worse with "duration in practice"

Figure 2. Distribution of study results relating physician age to clinical performance in various domains.

- Studies in which length of time in practice or age was associated with lower performance for all outcomes.
- Studies in which length of time in practice or age was associated with lower performance for some outcomes; no effect was found for other outcomes.
- Studies in which there was a concave relationship between length of time in practice or age and performance.
- Studies in which no association was found between length of time in practice or age and performance.
- Studies in which length of time in practice or age was associated with higher performance for some outcomes; no effect was found for other outcomes.
- Studies in which length of time in practice or age was associated with higher performance for all outcomes.
Rule 31 – Review the World Literature Fortnightly*

*"Kill as Few Patients as Possible" - Oscar London

Medical Articles Per Year

- Biomedical: 25,000,000 per year
- MEDLINE: 1,500,000 per day
- Trials: 95 per day
- Diagnostic?: 0
Is keeping up to date Mission Impossible?
Coping with the overload: three *possible* things you might try

A. Read an evidence-based abstraction journal (and cancel other journals)

B. Keep a logbook of your own clinical questions

C. Run a case-discussion journal club with your practice
Recognize important knowledge gaps

- Keep a logbook of questions
- Answer a few important questions
- Discuss evidence with colleagues
"Nurse, get on the internet, go to SURGERY.COM, scroll down and click on the 'Are you totally lost?' icon."
The 4 steps of EBM

1. Formulate an answerable question
2. Track down the best evidence
3. Critically appraise the evidence
4. Individualize, based clinical expertise and patient concerns
Formulate an answerable clinical question

- Structure of researchable questions – PICO-T
  - Population/Patients
  - Intervention
  - (Comparison)
  - Outcome
  - (Time)
- The “best” research type depends on the question type
Appraise the evidence

Did you find good quality studies?

Two steps
• PICO
• RAMMbo
Applying to the individual

What do the results mean on average? What do they mean for this individual?
Alternative Approaches to Medicine

**Basis of clinical practice**

<table>
<thead>
<tr>
<th>Basis for clinical decisions</th>
<th>Marker</th>
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<tbody>
<tr>
<td>Evidence</td>
<td>Randomised controlled trial</td>
</tr>
<tr>
<td>Eminence</td>
<td>Radiance of white hair</td>
</tr>
<tr>
<td>Vehemence</td>
<td>Level of stridency</td>
</tr>
<tr>
<td>Eloquence (or elegance)</td>
<td>Smoothness of tongue or nap of suit</td>
</tr>
<tr>
<td>Providence</td>
<td>Level of religious fervour</td>
</tr>
<tr>
<td>Diffidence</td>
<td>Level of gloom</td>
</tr>
<tr>
<td>Nervousness</td>
<td>Litigation phobia level</td>
</tr>
<tr>
<td>Confidence*</td>
<td>Bravado</td>
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*Applies only to surgeons.*
Clinical effectiveness through EBM

- Evidence through research and scientific reviews
- Evidence based clinical guidelines
- Implementation through education and change management
- Audit compliance and outcomes
Evidence –based medicine

- Level I: Evidence obtained from at least one properly designed randomized controlled trial.
- Level II-1: Evidence obtained from well-designed controlled trials without randomization.
- Level II-2: Evidence obtained from well-designed cohort or case-control analytic studies, preferably from more than one center or research group.
- Level II-3: Evidence obtained from multiple time series with or without the intervention. Dramatic results in uncontrolled trials might also be regarded as this type of evidence.
- Level III: Opinions of respected authorities, based on clinical experience, descriptive studies, or reports of expert committees.
Qualification of evidence

- Categorizes different type of medical evidence

- Ranks according to freedom of bias and validity

- Stratified by quality ...US Level 1-111
Categories of recommendations

Classified by the risk versus benefit of the service or treatment AND the level of evidence on which the evidence is based
Grading recommendations

- **Level A**: good scientific evidence shows clinical intervention substantially outweighs risks. Discuss with patient.
- **Level B**: fair scientific evidence of benefit. Discuss with patient.
- **Level C**: fair scientific evidence of benefit but the risk benefit ratio too close to call to make a general recommendation. Do not offer unless there are individual considerations.
- **Level D**: fair scientific evidence shows risks outweigh benefits. Usually not offered to asymptomatic patients.
Critical appraisal in EBM

- Ask patient specific questions
- Search the literature
- Critically appraise the literature by evaluating clinical trials and using critical appraisal tools
- Apply the evidence and integrate clinical experience and patient values
Clinical questions/tasks

- Therapy...more good than harm
- Diagnosis...select and interpret
- Prognosis...estimation of the clinical course
- Etiology..identification of harm and cause
PICO format

- Population, patient or problem
- Intervention (therapy, diagnostic, exposure or risk factors)
- Comparison to the intervention or control
- Outcomes
More often you will need information about:

- choice of therapeutic agents
- the best diagnostic test for a disease
- or the best treatment strategy for a particular patient
The Question?

Defining the question is an essential early stage in the search process. Try to divide your questions into four components: THE PICO question

- who is affected,
- what is being done,
- how are effects being measured,
- and, if necessary, how to compare the effectiveness of different approaches.
Why EBM varies

High levels of evidence will not exist for all clinical questions because of the nature of medical problems and research and ethical limitations. Randomized controlled studies cannot be done in certain situations. For example, it would be unethical to measure the effect of exposure to dioxane on health by asking one group to be exposed to the chemical while another group is not exposed, since the dioxane group would be subject to unnecessary harm.

For ethical reasons, the research to answer the question would come from observational studies (cohort studies, case control studies or case series). Observational studies follow patients over time who presently have experienced the exposure/condition (for dioxane, it could be military personnel exposed to the chemical during the Viet Nam war) and compare them with another matched group without the condition or exposure.
Test run: structure the question in PICO format

In patients with (Population) does (Intervention) or (Comparison, if any) affect (Outcome)?

What is the best first-line therapy for treating high blood pressure in the elderly?
EBM analysis

PICO ANALYSIS

• **Patients or Problem:** Who is affected? Describe the specific patient population and/or problem context. For our question, the population is hypertensive elderly patients.

• **Intervention(s):** What is being done? Define the interventions. We are interested in finding out which drug is the most effective monotherapy for treating hypertension in elderly people.

• **Comparison:** How effective are different interventions? How do diuretics compare with beta-blockers or other drugs for treating the elderly with hypertension?

• **Outcomes** Define the outcomes you wish to assess. Focus on outcomes that are important to patients like morbidity and mortality rather than intermediate surrogate endpoints like lowering blood pressure. What the patient is interested in is whether the intervention will help him/her live longer with good functional capacity and few undesirable side effects.
**EBM analysis**

**Question:** *Are parachutes effective in preventing trauma related to gravitational change?* Smith & Pell BMJ 2003

"as with many interventions to prevent ill health, the effectiveness of parachutes has not been subject to a randomized controlled study. Advocates of EBM have been critical of using observational study in making a recommendation. The most radical EBM protagonist should take part in a double blind randomized controlled study to settle the issue about the effectiveness of the parachute when jumping from the plain to prevent trauma. Any volunteers?"
EBM analysis

Question:

"assessing the value of using antibiotic therapy in the treatment of neonatal meningitis"

"use of thrombolytic therapy in the treatment of acute MI"
Evidence Pyramid

- Randomized Controlled Double Blind Studies
- Cohort Studies
- Case Control Studies
- Case Series
- Case Reports
- Ideas, Editorials, Opinions
- Animal research
- In vitro ('test tube') research

Systematic Reviews and Meta-analyses

From: Medical Research Library of Brooklyn
Fundamental to evidence based health care is the idea of a "hierarchy of evidence". This is a model for grading the evidence. Evidence grading is based on the idea that different grades of evidence (study designs) vary in their ability to predict the effectiveness of the health practices. Higher grades of evidence are more likely to reliably predict outcomes for your patients than lower grades.

Thus, a systematic review of randomized controlled trials that show consistent results would be graded as providing higher quality evidence than a review of randomized controlled trials that show variable results without a good explanation of the variability (heterogeneity). Also, a systematic review of several randomized controlled trials provides a higher level of evidence than a single well-designed randomized controlled trial.
The Evidence Pyramid illustrates the evolution of the literature. The base of the pyramid is where information usually starts with an idea or laboratory research. As you move up the pyramid the amount of available literature decreases, but increases in relevance to the clinical setting. (McKinnell and Elliott, 1997).
To search for the best published studies on a clinical question, drill down the Evidence Pyramid to rapidly identify high-quality information that you can apply in clinical practice. Search appraised resources that are likely to contain reviews of the kinds of clinical research listed in the top four levels of the pyramid. It is research design studies at these levels that will most likely be clinically relevant.
Critical appraisal of the literature

• The essence of the appraisal is the validity of the study

• Validity: the degree to which the inference drawn from a study especially generalizations which extend beyond the study sample are true taking into account study methods, representativeness of the study and the nature of the population drawn
3 key questions in appraisal of an article

- What is the PICO of the study?
- Is the study a good one?
- What do the results mean and are they due to chance?
BICCEP appraisal

- Bias: has the study been skewed by design or conduct
  - Internal validity: are the measurements accurate?
  - Chance: are results due to chance?
  - Confounding: something affected with and associated with the outcome but NOT measured
  - External validity: relevance of the study to the question
- Power: capacity of study to demonstrate association
Critical appraisal: RAMMBO

- **Recruitment**: were subjects representative of target population?
- **Allocation or adjustment**: were groups comparable at start of trial and was allocation concealed before randomization?
- **Maintenance**: was comparable status maintained throughout (management and followup)?
- **Measurement**: how were outcomes measured
- **Blinded subjects/assessors?/Objective outcomes?**