

FROM EMINENCE-BASED TO EVIDENCE-BASED MEDICINE AND VICE VERSA

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To improve is to change; to be perfect is to change often.

Winston Churchill

In clinical practice nowadays, many healthcare professionals address most health problems using an evidence-based approach (evidence-based medicine) rather than a more traditional approach known as eminence-based medicine. This transition from eminence-based to evidence-based medicine, as well as its popularity in recent decades, can be traced to the superiority of the systematic evidence-based approach in promoting better health care. This real paradigm shift has now been fully realized, ensuring that the preferred medicine is based on shareable clinical practices that are evidence-based and risk-assessed and are evaluated according to the principles of the scientific method complemented with significant statistical inferences.

Evidence-based medicine involves integrating clinical knowledge and expertise with the best available evidence, which is critically evaluated while properly considering the patient's reference values and preferences to make the best possible clinical decision according to science and conscience¹. The scientific evidence in the literature is growing exponentially and can result in major changes in clinical rationale and therapeutic practice. Evidence-based practice inherently cannot disregard this dynamic reevaluation of the conclusions obtained. Therefore, healthcare professionals must continually reassess the appropriateness of a particular intervention based on the recent and robust evidence available. Practicing evidence-based medicine means relying on results obtained according to the rules of controlled clinical trials, which are known to consider a pyramidal hierarchy of evidence following categories generated by the statistical power of studies. This is effectively the dominant cultural paradigm that has become an established component of how we care for patients today from whatever perspective we engage in a direct or indirect relationship with them.

Eminence-based medicine is a practice whereby a clinical decision is made solely on a physician's opinion. Epistemologically, this approach has increasingly been defined as the opposite alternative to evidence-based medicine. The medical specialist, the prominent healthcare professional, or the reputable academic is increasingly known to the public, as well as to a large proportion of healthcare professionals, as someone who could make decisions without regard to any scientific evidence but only based on their own experience, which can possibly be fallacious.

Over time, a radical view has emerged in favor of evidence-based medicine. According to this view, while acknowledging the value of experience and the resulting increase in credibility, even an expert's opinion is invalid if not based on scientific evidence. Similarly, there has been a growing body of those believing that the credibility of scientific research conducted by experts should be mistrusted, as the evidence presented could still derive from research funded, even indirectly, by pharmaceutical companies that can influence the choice of research topics and the outcomes while favoring the dissemination of evidence favorable to the product studied.

To summarize, does the E in the acronym EBM still stand for evidence only, or may it also be interpreted as E for eminence? Let us try to answer this provocative question by thinking about what is happening in the medical world today regarding the topic of rare diseases (by definition, prevalence <1:2,000) and especially ultra-rare diseases (suggested prevalence one-thousandth of rare disorders corresponding <1:2,000,000).

There is no doubt that interest in rare diseases has grown enormously in recent years, as shown by the interests of pharmaceutical companies and also the agendas of politicians and health authorities. At the same time, attention to ultra-rare diseases is still too scarce. Medical science has yet to focus on all the possible scenarios when faced with such infrequent, often exceptional if not “private”*, clinical situations. Clearly, in such unusual scenarios, not even large-scale collaboration instruments can be considered suitable. It will be necessary to exponentially increase tools, such as e-mail consulting, virtual centers of excellence, Wiki (Wiki is derived from the Hawaiian phrase “quick” and is used as an online collaborative resource for compiling information from numerous authors), and support groups². However, none of this will be enough, and we must rediscover the great value of expertise, which should be called again and without hesitation by its proper name: the value of eminence.

Drug approval regulations have also adapted to these new scenarios. Conventional randomized controlled trials (RCTs) comparing phase III treatment efficacy to provide a basis for evidence-based treatments applicable to the average patient do not always provide useful evidence for treating single patients with ultra-rare or non-rare diseases with uncommon omics profiles. To resolve this impasse, an alternative approach has been proposed to identify optimized treatments using experimental designs focusing on individuals. This approach involves customized designs, or n-of-1 designs, which still provide both a functional analysis of effectiveness and a comparative analysis of possible treatments. Such an approach is relevant in the personalized medicine zeitgeist and offers clinicians a paradigm to study optimal treatments for rare diseases where RCTs are not feasible. This will make it possible to identify optimal treatments for patients with comorbidities excluded from most RCTs and manage clinical situations where patients respond idiosyncratically (positively or negatively) to treatment³.

This is the sense of the *vice versa* we intended in the title. The evolution of the omics sciences, particularly the topic of ultra-rare diseases, restores centrality and dignity to the competencies of the expert, the eminence. The modern physician must not shirk the responsibility to assert their knowledge where evidence cannot be reached according to the scientific method. Therefore, under these extreme conditions, the eminent clinician should make choices based on their knowledge and experience (science) using their self-awareness and perception of the external world they relate to, their identity, and the complexity of their inner activities (consciousness). Such choices will be good to the extent of their capacity to be shared with the patient, who has the right to express their usefulness by being involved in the treatment project within the doctor-patient relationship that medicine for humans must never allow anything or anyone to overshadow.

*Private mutation: a rare gene mutation that is usually found only in a single family or a small population. A private mutation occurs and is passed to a few family members, but not to future generations.

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