EBM: Evidence-based Medicine or Experience-based Medicine?

Evidence based medicine (EBM) is the practice of medicine based as much as possible on double blind, placebo controlled data. But it will never be possible to test every treatment in every clinical situation. When should trials be done when the preliminary, but not objective, controlled, data suggest that the intervention is actually either not going to work or will make matters worse? The importance of addressing this question arose in my mind only in recently when I read an article on treating one aspect of Parkinson's disease (PD). It mentioned that a single drug had been shown effective for a problem, that two others had been shown ineffective and the rest had only been the subject of case reports and open label series. Hence, the article concluded, suggestions related to the use of these drugs had to wait until evidenced based medicine would point to their being useful or not. This would have attracted my attention anyway, but it raised my interest because this implication was present in two other articles I read on the topic, namely that recommendations would have to wait for controlled clinical trials.

Last year a renowned stroke expert neurologist was asked to give a plenary session to the several thousand member audience of the American Academy of Neurology on the pitfalls of relying too heavily on EBM. He presented a case of a person who didn't fit exactly into the criteria addressed by the various clinical trials in treating stroke. The general recommendations derived from EBM would be that she not be treated. In fact, several branch points in decisions about her care put her into the “don't treat” category, but close inspection would reveal that these recommendations didn't really apply to her since her medical profile put her outside the inclusion criteria for the studies from which EBM could make recommendations. And, of course, it is obvious on even minimal inspection, that many patients we see in everyday life would not have qualified for the various trials upon which EBM is based. On the other hand, studies do need to restrict inclusion and exclusion criteria or each study could be reduced to absurdities. Pity the poor FDA. If a study excluded diabetics, the FDA would clearly state that a treatment has not been proven for diabetics, but if a study excluded several smaller populations, it is unlikely that the FDA would limit use of the drug strictly to the population studied.

EBM is a useful construct, but it is, in fact, a construct, a set of guidelines, not a mandate. When deviating, one must justify why the choices were made rather than citing the “expert recommendations” derived from EBM. In the publications which upset me the authors were unwilling to make commonsense recommendations. Let me be specific. A relatively common side effect of the drugs to treat motor symptoms of PD cause patients to develop hallucinations and less commonly paranoid delusions. It has become accepted therapy to treat these with antipsychotic drugs. Two of these drugs don't cause motor symptoms to worsen but all the others do. Of the ones which do cause this problem only one has been studied in controlled trials, which earned it a “black box” warning as contraindicated in PD. The other drugs were not subjected to such trials, hence spared the black box warning. Interestingly however, several small series of isolated cases have been published in which PD patients did well on these drugs. Which is understandable if one realizes that there is a wide spectrum of sensitivities to the drug throughout the population. On the other hand, all specialists in the field have seen these drugs cause parkinsonism in otherwise healthy schizophrenics. It is a well known and universally accepted observation, so that the jump from observing this in those with normal mobility to expecting worsened motor function in those already parkinsonian is not a leap of faith. It is expected. In addition, the authors of these sanguine reports have not been recognized experts in the field.

My question is, when does one require level 1 evidence of a problem before advising colleagues to avoid certain treatments? It is surely not possible to study every intervention in every scenario and if a drug causes a certain side effect in a population at low risk, one can reasonably infer that it will cause the same side effect, or worse, in a vulnerable population. We cannot study everything. Medicine, despite the various advances is still an art based on science and the science has its limitations. Not only that, but it will always have these limitations, now matter how closely we come to the Star Trek model of whole body scans for all ailments.

At a recent lecture I heard strong evidence suggesting that certain identified genes play a role in causing disease and in drug response. These are not yet available for testing, but probably will be soon, but who is going to pay for this? Some insurers won't even cover gene testing for Huntington's disease, which would not only improves quality of life for a family, but saves a far greater amount spent on unnecessary testing. How can we think they'll pay for testing to allow us to choose one symptomatic, non-curative therapy over another?

There will always be a need for experience based medicine as well as evidenced based medicine and the two must go hand in hand and will continue to do so until the end of time, or at least until there isn't any more disease needing treatment. Good clinical judgment represents the proper amalgam of the two EBM's.

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Disclosure of Financial Interests

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