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RESEARCH NOTES

HEALTH TECHNOLOGY ASSESSMENT AND CLINICAL DECISION MAKING:

Which Is the Best Evidence?

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Abstract

This paper examines the rationality of the concepts underlying evidence-based medicine and health technology assessment (HTA), which are part of a new current aimed at promoting the use of the results of scientific studies for decision making in health care. It describes the different approaches and purposes of this worldwide movement, in relation to clinical decision making, through a summarized set of specific HTA case studies from Catalonia, Spain. The examples illustrate how the systematic process of HTA can help in several types of uncertainties related to clinical decision making.

Keywords: Evidence-based medicine, HTA, Decision making, Health policy

Health care is subject to increasing scrutiny. The ever-growing awareness about variability in clinical practice, uncertainties regarding the real health effect of the multiple preventive, diagnostic, and therapeutic alternatives available for the same clinical condition, and the worries caused by the fast growth of health care expenses are the reasons for the appearance of new paradigms in health care.

Evidence-based medicine (EBM) is one of these paradigms. The term, in itself, comes from the initiative of clinical practice, epidemiology, and biostatistics professionals, but its conceptual basis and methodological tools to promote a scientific reorientation of decision making in health care has been widely developed and used by several health technology assessment (HTA) groups and institutions all over the world for more than 30 years. The clinical origin of the term EBM is determining its rapid diffusion and acceptance among health care professionals worldwide, because EBM is viewed as an alternative to other trends in current health care systems, such as health care reforms, changes in managerial models, or standardization of clinical practice trends, which are under suspicion of having cost-containment as their sole objective.

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The EBM movement promotes the conscious, explicit, and sensible utilization of the best clinical scientific evidence available to make decisions about the care of individual patients (12); that is, it promotes clinical excellence. However, EBM can be also seen as an intellectual, scientific, and professional movement promoting the use of the results of scientific research for decision making in health care. The different terms and approaches used to define this movement are determined by the different levels of methodological specialization of the groups involved. Thus, EBM, HTA, outcomes research, and economic evaluation are, to different extents, part of the same current. Moreover, the different emphasis given to the use of the available sources of scientific evidence may influence the final approach.

CLINICAL DECISION MAKING: WHICH IS THE BEST EVIDENCE?

Decisions in medicine are not easy, and no one said they would be. The decision takes place after physicians have implicitly integrated different elements, such as evidence, experience, uncertainties, the available resources, and their own individual values as well as those of both patients and their families. The relative weight of each of these elements in their decisions will determine the outcomes, as will the interpretation of which is the best evidence.

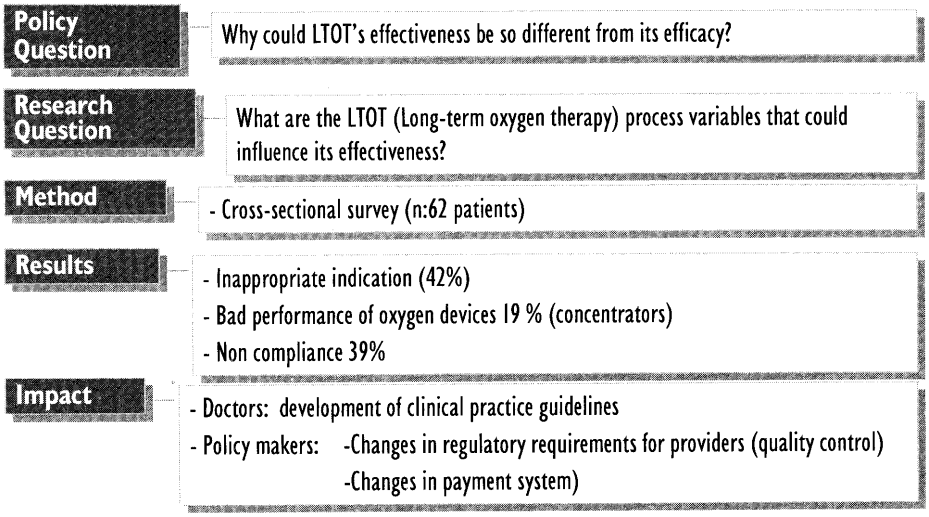
The term "best evidence" (11) has been defined, for the use of a therapy, as that coming from randomized controlled trials (RCTs) demonstrating their efficacy and safety, and for a diagnostic technique, as that evidence coming from studies that measure the precision and accuracy of these techniques. The evidence from systematic reviews of these studies using meta-analytic techniques is also considered the best evidence.

However, the use of experimental designs alone to inform clinical decisions has advantages, limitations, and implications both for the patient and the health care system as a whole, which must be taken into consideration. One of the advantages of RCTs is that this design is the most valid and objective means to demonstrate that the obtained effect can be attributed to the assessed therapy—provided the same conditions as defined in the study protocol take place, that is, type and features of the patient, clinical condition, type, dose, and duration of the therapy, among others.

Among the disadvantages of experimental designs, the most important is the low external validity, that is, its low generalizability. Thus, the effect shown in experimental conditions (efficacy) is not always maintained in everyday clinical practice (effectiveness). Finally, many other questions other than efficacy, such as effectiveness, appropriateness, needs, equity, and efficiency, may not be answered by this type of design.

Therefore, a more holistic and complementary approach means considering the best evidence as that which gives the most valid and objective answer to the different types of questions arising both in clinical practice and in health care resources management, and coming from the synthesis and integration of the results of scientific studies from different knowledge fields aside from health sciences—such as social, economic, and political sciences.

In the HTA framework the best evidence should actually be the one able to answer the uncertainties of decision makers (1;5). The main decision makers in the health care systems—doctors—face different types of uncertainties regarding the care of their patients. The ceaseless increase of the available technological options, the indiscriminate inflation of information surrounding them, the plural values



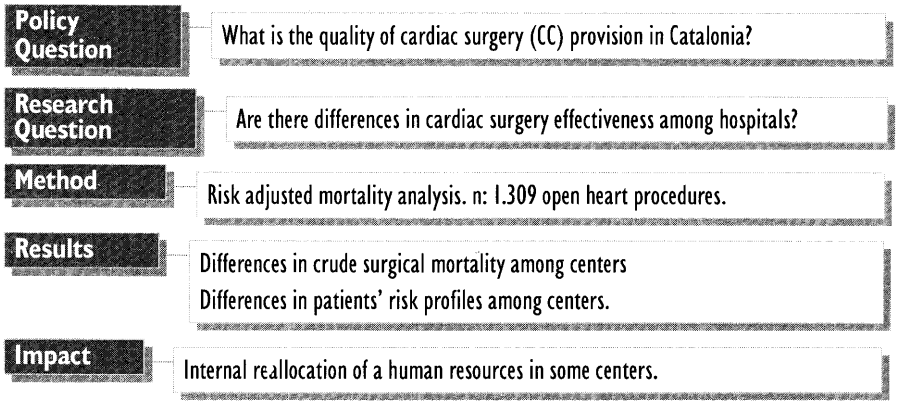
Adapted from Granados A, Escarrabill J, Borràs JM, Rodríguez-Roisín R, 1997(4).

Figure 1. Assessment of long-term oxygen therapy.

produced by sociodemographic changes, and the ever-growing pressure of cost-containment are the source of these uncertainties. Answers seem to require more than RCTs, precision and accuracy studies, or meta-analyses.

The results of well-designed effectiveness or cost-effectiveness studies need not be of poorer quality than of those produced by efficacy studies. As mentioned above, the quality of scientific evidence is determined by its ability to give plausible answers to a policy question when this question has been translated into an adequate research question. At this point, it is worth recalling that effectiveness is context-dependent. Therefore, evidence of efficacy is necessary but not sufficient to support some clinical decisions.

Sometimes the relevant question in the decision is why the effectiveness of a treatment is so different from its efficacy, and how to fill the gap. In those cases, well-designed observational studies may be the best means to answer it, as, for example, has been reported after a local assessment of long-term oxygen therapy for chronic obstructive pulmonary disease and chronic respiratory failure (4), summarized in Figure 1, suggesting the different interventions needed to improve the therapeutic process. Sometimes groups of clinicians wonder whether their clinical outcomes (effectiveness) are related to differences in the patients' risk factors or to other elements. This was a specific assessment done at the request of the Catalan Study Group on Open Heart Surgery (10) that provided outcomes information and led to changes in practices (Figure 2). Clinicians are increasingly asking themselves how to choose the most efficient option among different diagnostic alternatives for a given condition, and the best way to answer this question is through a sound cost-effectiveness study. Figure 3 shows a specific assessment process carried out upon request of a group of radiologists (13). The results of this assessment allowed reallocation of resources in a specific center. Incidentally, we should not consider as being of inferior quality studies that provide sound evidence, for instance, of the patients' quality of life (8) or preferences. In Figure 4 the outline of another



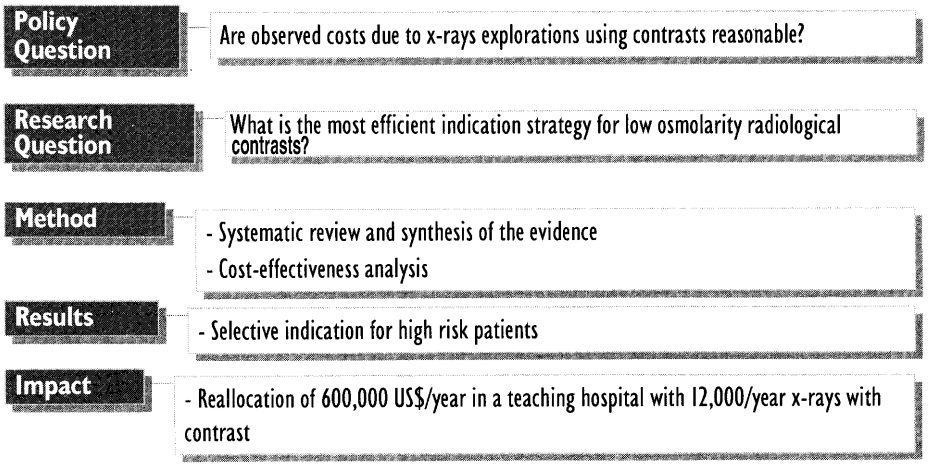
Adapted from Pons JMV, Granados A, Espinàs JA, et al., 1997(10).

Figure 2. Assessment cardiac surgery in Catalonia.

HTA process illustrates how survival data are insufficient to evaluate patients with liver transplantation.

Systematic reviews of the scientific literature using scales or checklists to classify and rate the quality of the evidence are undoubtedly a milestone in the assessment of the rigor of the scientific evidence regarding many currently used health technologies (6;16). However (and this should be understood as a challenge), the design of these scales or checklists should be adapted to the fact that research methods must be at the service of the problems to be solved, and their quality should be judged and rated after considering in depth what question they are answering.

This approach does not intend to question the value of recommendations issued by evidence-based clinical practice guidelines. If they have been elaborated after



Adapted from Sampietro-Colom L, Granados A, 1993(13).

Figure 3. Assessment of low osmolarity contrast media.

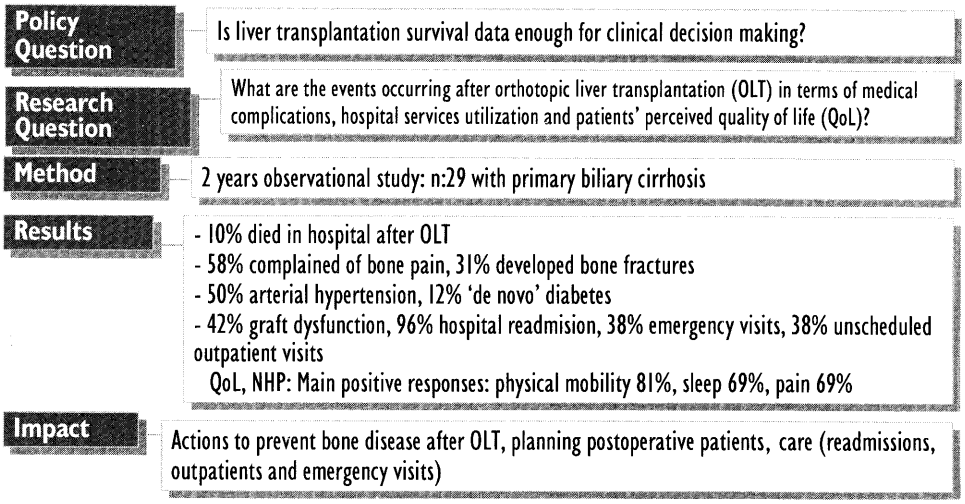


Figure 4. Assessment of liver transplantation.

a systematic review of the scientific literature, these guidelines make it possible to narrow the gap between the effectiveness of health care interventions and their efficacy.

But searching and using only the best evidence to achieve clinical excellence—as defined by some of the followers of evidence-based medicine—is the same as assuming that practitioners have only efficacy uncertainties; thus, it is of no use that they also search for and use whatever evidence may help them to make efficient decisions or to learn patients' health status perceptions or preferences.

EVIDENCE-BASED CLINICAL PRACTICE, BUT EVIDENCE-BASED HEALTH CARE?

Decisions regarding groups of patients or populations—i.e., services, health care centers management, and public health—determine the availability of resources in a health care system. As mentioned above, available resources are another element that increasingly influences clinicians in their decisions about individual patients. Basing decisions regarding health care on scientific knowledge is again approximating excellence to health care policy and management. Therefore, to guarantee that clinical practice will be evidence-based, decisions on the provision and distribution of resources should also have a scientific basis (1;7).

However, if physicians on the way to clinical excellence tend to maximize their sense of responsibility toward individual patients, providing what clinicians consider the best therapeutic option based on the best evidence of the efficacy of a therapy, then they could discard other types of information that might nonetheless allow them to appraise the cost-opportunity of their decision. On the other hand, if policy makers in a limited budget public health care system also proceed toward excellence, their sense of social responsibility should be maximized, and consequently, the efficiency of their decisions should be emphasized. Thus, it will be necessary to find the right balance to avoid an insoluble conflict among the different decision makers in a health care system.

THE VALUES: ONLY EVIDENCE-BASED MEDICINE?

Let us suppose that we reach the ideal status of evidence-based medicine, that is, that there is scientific evidence on the efficacy and safety of most of the diagnostic and therapeutic options, and this evidence comes from well-designed clinical trials, using the adequate outcome measure with numerous precise and accurate studies of sufficient quality. Even so, there will still be multiple available alternatives for a single clinical condition, with different degrees of efficacy, safety, effectiveness, and efficiency. What criteria should be used to choose among alternatives? Clinical excellence, patients' preferences, efficiency, justice toward the individual patient, or social justice?

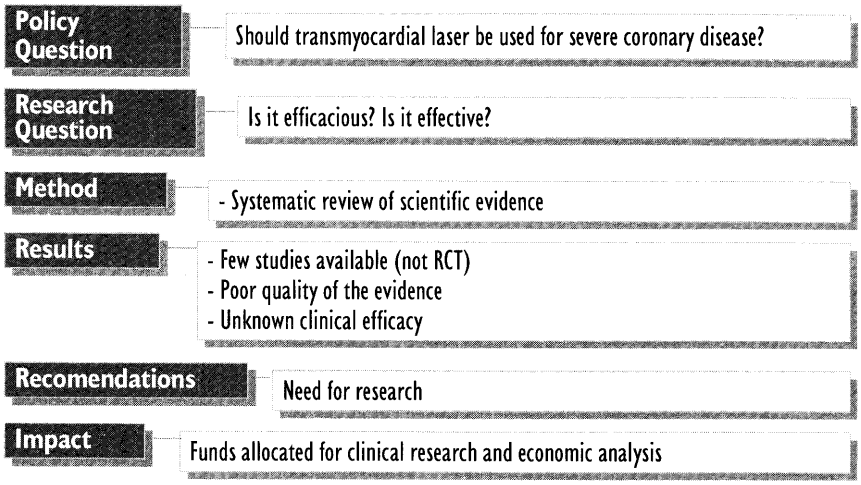
The use of scientific evidence to inform decisions at any level in a health care system makes it possible to strengthen the rationality of the decision-making process and helps the decision makers to face responsibilities with solid arguments, that is, with a scientific approach. However, direct scientific evidence rarely provides decision makers with information on the appropriateness, cost, or social, legal, and ethical implications of the adoption and use of medical techniques and practices in the real world.

Due to all of the above, several groups and organizations operating internationally devote their resources and professional experience to assess medical techniques and procedures, analyzing direct or indirect scientific evidence, synthesizing it, and spreading it actively, integrating in their recommendations, however, other types of information useful for the different decision makers of a health care system. They study the implications of the local and international legal frameworks regarding medical techniques and their influence on the availability of resources, as well as the ethical, economic, and organizational consequences of the adoption and use of techniques, both new and commonly used, and remain alert to the importance of patients getting involved in decisions regarding their health (14).

HEALTH TECHNOLOGY ASSESSMENT: A GOOD TOOL TO IDENTIFY CLINICAL RESEARCH AND TRAINING NEEDS

HTA organizations also promote the local production of scientific evidence (15) because in that crucial step in the HTA process, which consists of reviewing the quality of scientific evidence available in relation to a specific technique, quite often there is not sufficient rigorous evidence available as a main finding. This is a good starting point to identify research needs in a concrete health care context. This was the case in the assessment of transmyocardial laser revascularization to treat severe coronary disease (Figure 5) (9). Figure 6 illustrates the role of HTA in synthesizing or producing information for decision making in health care and identifying health care knowledge needs.

HTA organizations are increasingly involved in the design and implementation of education and training programs (2) for under- and postgraduate students, which help foster critical thinking among the main decision makers in the health care system. The global aim of these actions is to provide specific methodological tools to learn how to search, evaluate, interpret, and use the best scientific evidence to answer several types of uncertainties related to clinical decision making. Moreover, an open discussion is encouraged on the ethical, economic, and organizational conflicts that are part of everyday life in health care.



Adapted from: Pons JMV, 1996(14).

Figure 5. Assessment of transmyocardial laser.

CONCLUSION

Decisions in clinical practice and in health care resources management do not only determine results in terms of patients' health and quality of life, but also determine the economic sustainability of health care systems. Basing medicine on scientific evidence can improve the rationality of the decisions made at the different levels of the health care system, provided the question of what is the best evidence is correctly answered.

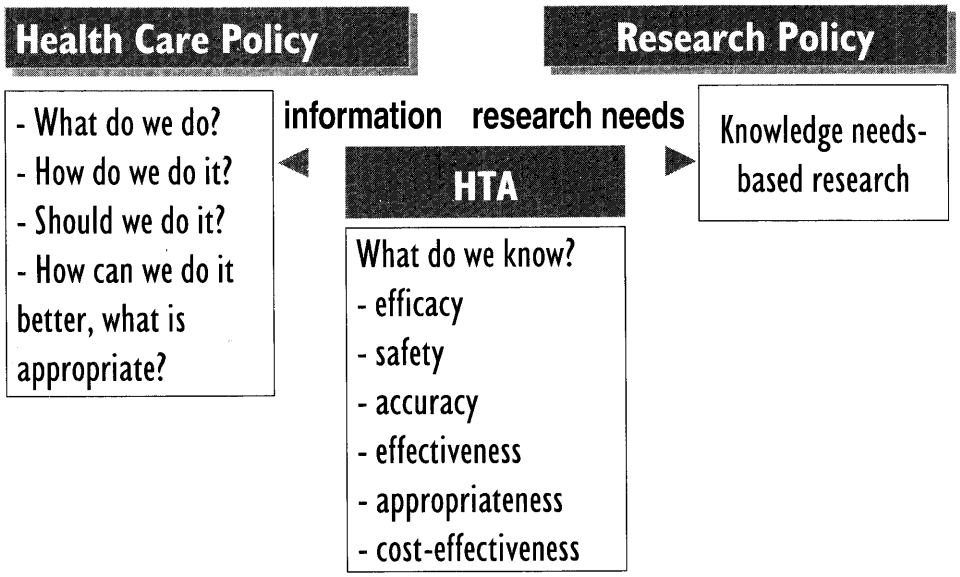


Figure 6. The role of HTA in the Catalan health care system.

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Achieving clinical excellence should not be in conflict with the respect for patients' autonomy nor with the achievement of efficiency in health care. Understanding the advantages, the limitations, and the implications of the use of scientific studies for the practice of medicine becomes more and more unavoidable every day. Public health care systems should also be able, today and in the future, to shoulder the burden of ethical and economic challenges. HTA is prepared in many countries to meet these challenges.

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