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What is the impact of disease prevalence upon health technology assessment?

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A B S T R A C T

As national budgets for health care will remain under stress for the foreseeable future, health technology assessment (HTA) aimed at offering guidance to policy-making will have an increasing role to play in optimizing resources. The emergence of new treatment paradigms and health technologies, and the prevalence studies which determine when a disease is a current or future burden for patients and the community are in the roots of the HTA process. Analysing studies on screening test strategies and health care policy, this paper revisits two key concepts in epidemiology, prevalence and incidence, in order to show their major impact upon HTA. Utilization of the predictive values of screening tests that include prevalence in their calculations, and analysing all options for screening strategies are necessary in HTA. Cost-effectiveness analyses and statistical models should include potential externalities, especially the impact of prevention and treatment on infectious disease prevalence. Beyond estimates of cost-effectiveness ratios, decision makers also need to know by how much their annual health care budget is likely to increase or decrease in the years following the emergence of new technologies: hence the importance of incidence- or prevalence-based economic evaluations. As new paradigms are occurring, especially in the field of oncology, with treatments targeted to 'small' groups of patients identified through genetic testing, prevalence data are strongly needed. Precise estimates of disease prevalence, in general populations as well as in risk or targeted groups, will therefore be necessary to improve HTA process.

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Until recently and for decades, the decision to use an emergent health technology has been left to physicians who offered it to patients on the basis of their interpretation of published studies or reports, as determined by their personal training, skills, and experience. Then patients accepted (or did not) according to their own convictions and knowledge. Thus health technology assessment occurred at an individual level. Developments and technological innovations in the life and health sciences have a huge potential on the one hand to improve (or impair) the health of populations, and, on the other hand, to have a dramatic impact upon all functions of social life, such as economics, health care policy, ethics, and law. Consequently both decision makers and health professionals had to be helped by further decision making tools. While health professionals consider only the benefit for the patient under their care, the issue for decision makers is to find a relative balance of costs (including harm to patients) and health benefits in the population. The first step of this historical process consisted in a systematic evaluation of evidence for new technologies. The Evidence-Based Guidelines (EBG), and more generally Evidence-Based Medicine (EBM), were born. EBG have been used unevenly by health professionals, and it is quite difficult to thoroughly evaluate their impact on medical practice and populations' health. EBG have been increasingly used by health policy decision makers, but they proved to be insufficient to make appropriate decisions. Hence, the birth of the Health Technology Assessment (HTA), defined by the International Society of Pharmacoeconomics and Outcomes Research (ISPOR) as 'a form of policy research that examines short- and long-term consequences of the application of a health care technology. Properties assessed include evidence of safety, efficacy, patient-reported outcomes, real world effectiveness, cost and cost-effectiveness as well as social, legal, ethical, and political impacts' [1]. HTA is defined by its aim of offering guidance to policy-making [2].

While in the past HTA has often been limited to assessing 'hard' medical technologies, such as computer tomography scanners or robots, it has increasingly expanded its reference to include 'applied knowledge' used in the health care sector, and consequently encompassing pharmaceuticals, medical devices, screening programs, organizational interventions, and health promotion initiatives [3]. HTA can lead to coverage of the technology concerned or to a refusal of reimbursement by health insurance systems; more often it leads to restrictions of access to technologies (e.g. second- or third-line use, limitation of availability to certain patient groups, etc.). Sometimes the mere intention to conduct an HTA can be expected to have an impact upon the use of health products. For the last fifteen years, HTA has played an increasing role in public health decision making, and, in many Western countries, organizations devoted to HTA have been created such as the UK National Institute for Care and Health Excellence (NICE), the German Agency of Health Technology Assessment (DAHTA), the International Network of Agencies for Health Technology Assessment (INAHTA) and the European Network on Health Technology Assessment (EuHTAnet), among others. These organizations have greatly contributed to health policy in their respective countries and worldwide, and the current economic crisis in Europe has only increased the role of HTA in public health.

The first outstanding point when dealing with the issue of HTA is that it is multiform: it implies multidisciplinary tasks and it varies across countries, depending tremendously on national frameworks, health organizations, and social and political concerns. 'Full HTA' can be presented as a program of five stages, the first being evidence analysis, the second an outcomes analysis resulting in benefit-risk ratios, and the third and fourth stages being analysis of costs and cost-effectiveness; the last stage considers the ethical and legal implications of technology [1]. HTA is thus recognized as 'a multidisciplinary field of policy analysis studying the medical, economic, social and ethical implications of the development, diffusion and use of health technologies' [4]. The two pillars which sustain any HTA according to this definition are the evidence of the clinical efficacy and safety of the treatments under review on the one hand, and the burden assessment of the considered disease on the other hand. This paper will focus on the burden assessment, and will question the role and importance of disease prevalence in HTA.

Prevalence and incidence in HTA

Prevalence, defined as a measure of disease frequency, is a key concept in epidemiology. It refers to the proportion of individuals in a population who have a disease or condition [5]. While incidence