Improving patient access to novel medical technologies in Europe

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The European Society of Cardiology (ESC) organized a one-day workshop with clinicians, health economic experts, and health technology appraisal experts to discuss the equity of patient access to novel medical technologies in Europe. Two index technologies were considered: implantable cardioverter defibrillators (ICDs) and drug-eluting stents (DES). The use of ICDs range from 35 implants/million population in Portugal to 166 implants/million population in Germany, whereas for implants of DES (as percentage of total stents) it is lowest in Germany at 14% and high in Portugal at 65%. These differences can in part be explained by a lack of structured implementation of guidelines, the direct cost in relation to the overall healthcare budget, and to differences in procedures and models applied by Health Technology Assessment (HTA) agencies in Europe. The workshop participants concluded that physicians need to be involved in a more structured way in HTA and need to become better acquainted with its methods and terminology. Clinical guidelines should be systematically translated, explained, disseminated, updated, and adopted by cardiologists in Europe. Clinically appropriate, consistent and transparent health economic models need to be developed and high-quality international outcome and cost data should be used. A process for funding of a technology should be developed after a positive recommendation from HTA agencies. Both the ESC and the national cardiac societies should build-up health economic expertise and engage more actively in discussions with stakeholders involved in the provision of healthcare.

Introduction

The Cardiovascular Round Table Taskforce 2 of the European Society of Cardiology (ESC) organized a one-day workshop in December 2004 to discuss the issue of patient access to novel medical technologies. Representatives from seven national societies’ members of the ESC, health technology experts, ESC clinical guidelines experts, and representatives from health technology assessment (HTA) and appraisal agencies were invited to the workshop. In order to focus the discussion, two index technologies were selected, implantable cardioverter defibrillators (ICDs) and drug eluting stents (DES). It is assumed that the principles discussed here are applicable to other medical technological innovations.

After a review of the adoption in the EU of the two index technologies, the process of guidelines preparation was discussed, followed by a review of the principles of HTA, and the appraisals in two example countries, the UK and Germany. The present article is a summary of the main issues discussed during the presentations and of the joint recommendations from the discussions and breakouts.

Medical technologies in Europe

A recent survey showed that implantation rates of ICDs varied across a selection of EU countries from 35 implants/million population in Portugal to 166 implants/million population in Germany (unpublished data on file). There was a steeper uptake of ICDs from 2001 to 2003 in those countries with higher baseline levels as compared with countries having lower baseline levels. This suggests that differences
between countries persist and continue to diverge. Relative healthcare spend per citizen only poorly predicts the uptake of newer technologies such as cardiac resynchronization therapy with defibrillator (CRT-D) usage in Europe, but it does correlate positively with established technologies such as ICDs. Tax-based health care funding is generally associated with lower implant rates as compared with social insurance-based systems. In contrast, the penetration of drug-eluting stents (DES) is (as a percentage of total stents) lowest in Germany at 14% and rising to 75% in Switzerland. Portugal has the second highest implant rate at 65% (unpublished data on file). This indicates that the uptake of new technologies is not country-specific but varies with the technology in question. The decision to reimburse the DES technology has a great impact on the adoption of the technology as can be seen from a steep uptake after the decision to reimburse in, for instance, Belgium and France. Interestingly, this is less obvious in the UK where no such immediate increase was observed after the UK National Institute for Health and Clinical Excellence (NICE) issued a positive recommendation. In summary, there is a great variation in the adoption of medical technology for approved indications despite the evidence provided by large clinical trials and the availability of ESC guidelines for clinical practice. This variation can only in part be explained by differences in per capita spend on healthcare and the healthcare funding system. A survey of the ESC among seven national cardiac societies that is, Spain, Sweden, the UK, Poland, Germany, and France indicated that direct cost in relation with the overall healthcare budget is the main limiting factor for patient access to the two medical technologies (ICDs and DES) in any particular country. The survey indicated a lack of structured implementation of ESC guidelines in the countries suggesting the need for guidelines to be systematically translated, explained, disseminated, updated, and adopted by a majority of cardiologists. Finally, there was overall no clear link between the clinical guidelines development and implementation, clinical practice, and reimbursement guidance. Clearly, the profession needs to be involved in both HTA and appraisals across all countries, as this is systematically the case only in the UK and in Sweden at present.

**Clinical guidelines development in the ESC**

Medicine is continuously evolving because of developments in basic and clinical science as well as in technology. Guidelines are then used to show how and when these developments should be implemented in clinical practice. The development of clinical guidelines is a well-defined process of reviewing and evaluating clinical evidence based on published (and other) literature. The ESC established in 1994 the Committee for Practice Guidelines (CPG) which identifies topics, proposes task forces to review the literature, assess and grades the evidence according to a standardized protocol, and then issues the guidelines. The process for developing guidelines has now reached a mature phase—ESC Guidelines are endorsed by the majority of European National Societies of Cardiology—and the real challenge lies in their implementation and incorporation into reimbursement policies of the national health care systems and from there into clinical practice. Dissemination of the recommendations included in the Guidelines should not be limited to those cardiologist involved in interventional sub-specialties such as implant of stents and ICDs, but they should reach the larger platform of cardiologists involved in patients care. These clinicians in fact play a major role in the acceptance and advocacy of novel therapies including devices. The limited target audience of the educational message derived from Guidelines is a potentially important reason as to why the message of Guidelines has limited impact on practice. ESC clinical guidelines are primarily concerned with safety and efficacy and do not incorporate a cost-efficacy assessment. Given that the guidelines are intended to apply throughout Europe, and that cost issues vary widely across the various countries, and there is little value in providing 'average' figures. Therefore, it is fundamental that the dissemination of ESC guidelines at the national level be accompanied by discussions on aspects that are unique for a given medical system (reimbursement/availability/priorities/social and ethical aspects) and that reimbursement agencies and local health economists are involved in the process.

**Health technology assessment**

HTA primarily considers the effectiveness, appropriateness, cost, and broader implications of technologies. Effectiveness refers to the fact that the technology is evaluated not only to see whether it works (i.e. its efficacy) or is safe, but also to see whether it provides ‘added value’ over and above treatments that are already available, and whether that additional benefit is worth the additional cost. In this way, HTA assesses both the clinical effectiveness and the cost-effectiveness of a given technology. One of the main outcomes of the HTA is the incremental cost-effectiveness ratio (or ICER). The ICER is a measure of the additional cost of the new technology over and above the existing technologies as compared with the difference in outcome between the new and existing technologies. In other words, what is the additional cost per unit of health care gained? In determining the expected health care gain, quality adjusted life years (QALYs) are generally used. QALYs take into account health-related quality of life by assigning to each year a weight ranging from 1 to 0, i.e. optimal health to death. Economic models can be difficult to interpret and their cost-effectiveness estimate for a given technology can vary widely depending upon model assumptions. For instance, the median cost-effectiveness of cardiac resynchronization therapy (CRT) to medical therapy for patients with severe heart failure was US$107 800 per QALY gained in an early meta-analysis by Nicholson et al. Recent randomized controlled trials have shown an ICER of US$19 600–$43 000 per QALY gained for CRT alone and CRT-ICD (with defibrillator), respectively, in COMPANION, and a median of €19 319 (–US$23 000) per QALY gained for CRT and CRT-ICD combined in CARE-HF. International healthcare policy-makers are increasingly using HTA and in particular cost-effectiveness data to underpin their decisions on access to innovative technologies. At present, the procedures and processes of European HTA agencies and systems vary considerably. In order to minimize inappropriate geographical variation in access to innovative cardiac technologies, HTA must increasingly use consistent methods, high quality international outcome, and cost data and clinically appropriate and transparent economic models.
Health technology appraisal

The UK experience

The National Institute of Health and Clinical excellence (NICE) was set-up in 1999 to provide national guidance to the National Health Service in England and Wales on best practice based on both clinical and cost-effectiveness. It is an independent, government-funded organization. Its technology appraisal process is initiated by the UK Department of Health, and takes about 12 months from start to finish. NICE relies on independent advisory committees, each made up of about 30 persons meeting on a monthly basis. Members of the Appraisal Committees are appointed for a 3-year term and are drawn from the NHS, patient/carer organizations, relevant academic disciplines, and the pharmaceutical and medical devices industries. Members of the appraisal committees act in their personal capacity and not as representatives of their respective organizations. A comprehensive process is in place to resolve conflicts of interest of committee members. The deliberations of the technology appraisal committees are assisted by clinical specialists and patient experts who do not participate in the decision-making process, but provide advice and advocacy.

NICE uses an extensive evidence base including evolutions in clinical practice, patient experience and views, and industry evidence and data to answer questions such as how innovative is this technology, what is the clinical need of the particular population with the condition in question, what are clinical specialists and patient experts saying about that technology, both in terms of its benefits and risks. Ultimately, a judgement has to be made, on the basis of all of these factors, and on the basis of comparisons with other programmes that are currently funded. There is no fixed floor or ceiling of cost per QALY but rather a band or range. That band lies around £20 000–£30 000 (€30 000–€45 000) per QALY. A technology with higher cost per QALY may nevertheless be recommended but in such a case the reasons for recommendation will need to be specified.

To date, NICE has assessed and appraised about 90 technologies. The outcome of the process is a recommendation regarding the clinical and cost-effectiveness of the technology within the NHS in England and Wales, and the NHS is then required to make funds available for technologies recommended in NICE technology appraisal guidance within a defined period of time. All appraisals are regularly updated and each one contains a date at which the Institute will review the need to update the recommendations.

The German experience

The German health system is highly decentralized, with a sharing of decision-making powers. The three main players are the Federal Ministry of Health, the 16 State (or Lander) ministers of health, and the Federal Joint Committee (G-BA: Gemeinsamer Bundesausschuss) consisting of the national associations of doctors and dentists, the German Hospital Federation, and the health insurance funds. The G-BA plays a pivotal role in the appraisal process and it must give a positive evaluation of a new method or drug before it can be paid for by the statutory health insurance funds.

HTA data is provided by the Agency for HTA (DAHTA@ DIMDI) which is part of the Federal Ministry of Health and which has access to extensive databases. Requests for HTA can be submitted by anyone, including the public, and are prioritized by a board of trustees which includes all decision-making bodies in German Health Care. HTA analyses are carried out by experts outside DIMDI from German-speaking countries, and the process is regulated by a number of protocols and standardized operating procedures reducing the time frame to about 12 months. The reports are published by the electronic journal GMS-HTA (www.egms.de) and the DIMDI website free of charge. To date, DIMDI has published about 60 HTA reports (including one on DES), with more than 30 reports ongoing. The impact of such HTA reports on the take up of new technologies such as DES is still unknown, and will be the subject of further study. Positive recommendations issued from DIMDI do not lead to compulsory funding of the technology by health-funding agencies.

Recently, the Centre for Quality in Health Care (IQWIG) has been created. It is an independent organization with a main focus of supporting the Federal Joint Committee (G-BA). Its aim is to investigate the evidence base, assess the quality of health care, review guidelines, determine the effectiveness of pharmaceuticals, and prepare user-friendly information for consumers on the quality and efficiency of health care. IQWIG carries out the assessment of medical efficacy and effectiveness for the reimbursement process of statutory health insurance, whereas the decision including appraisal and economic evaluation is taken by the G-BA. The assignment of two agencies, IQWIG and DAHTA, ensures the appropriate support required by the reimbursement process of statutory health insurance and provides enough room for the needs of all other decision makers in health care concurrently.

Germany has now adopted the diagnosis related groups (DRG) system with respect to hospital funding. Once a DRG covers a particular technology, it will generally be available in most hospitals. However, incorporating new technologies into a DRG is a very time-consuming process. Presently, any new technology has to be budget-neutral as the relevant budget is not necessarily increased when a new technology is added. Additional payments are possible through a supplementary reimbursement system (Zusatzzegelte), which was launched in 2004. However, the advantages of this system has yet to be demonstrated as the overall budget available for healthcare continues to remain the same.

HTA and appraisal from a physician’s perspective

The economic and societal impact of new technologies needs to be considered in addition to the clinical evidence base. However, HTA may lead to the perception that choice has been removed from patients and doctors because an outside agency has deemed what is appropriate treatment in a given situation. As such, it could potentially change the dynamics of the doctor–patient relationship. There are currently three main limitations on the HTA process. First, the time-frame for the process does not keep pace with rapidly evolving technologies. In particular, some agencies will only consider published (and therefore relatively out of date) data. Industry-sponsored studies which often constitute the greater part of the evidence base are sometimes ignored. The assessment therefore may not reflect the state-of-the-art knowledge on a given technology. Secondly, broadly constituted appraisal committees (such as those involved in the NICE process) need to ensure that they supplement their general expertise with
specific understanding of the clinical practice in question and the practical value of an innovation. The committees can sometimes be suspicious of the evidence base and may be wary that political or vested interests are playing a role in the views others hold about the value of a given technology. Third, even when a technology is recommended by an appraisal committee, local budget restrictions often mean that the technology is not automatically authorized. In addressing these issues, the development of an HTA governance code would be a valuable adjunct. This should include a process for ensuring that funding for a technology is identified when it is approved by an HTA. In addition, physicians should be involved in all stages of the process. Physicians involved in the HTA process also need to develop expertise in cost-effectiveness assessments, an area in which they are often lacking. In parallel, they should receive further communications training to assist them in getting their messages across to the HTA experts and policy-makers.

Conclusions/Recommendations

HTA will be of increasing importance in determining the appropriate use of medical technology in all European countries. Countries such as Spain, Sweden, and the UK enjoy the most developed systems, followed by France. The planned European Network of HTA (EUNetHTA, start foreseen in 2006) will be a first step to facilitate EU collaboration and standardization in this area. The profession should play a role in all stages of the HTA process but currently, in most European countries, the profession is generally involved on the basis of ad hoc and non-standardized principles. A more structured approach is therefore needed and the profession itself needs to develop a better grasp of HTA, and become more comfortable with the terminology and vocabulary used.

Clinical guidelines play a crucial role in establishing best practice and equity in healthcare provision across Europe. Guidelines need to be systematically translated, explained, disseminated, updated, and adopted by cardiologists in Europe. In addition, they might be reformulated to, for instance, a short synopsis or fact sheet making them more accessible and engaging for government agencies and other payers. By providing a broader level of data presentation, i.e. include health economic considerations, availabilities, and so on, these derivatives of the guidelines would become of greater relevance and interest to other audiences.

Care must be taken to ensure that HTA does not lead to a constraining of innovation. There is a concern about the accuracy of health economic models with respect to device therapies when compared with models having drugs, particularly when considering the short life-cycle of medical devices. The process could be improved by designing clinical trials with the subsequent HTA in mind. There should also be an agreement that funding for a technology is implemented after a positive recommendation from HTA. The profession should play a leading role in the decision to refer technologies to HTA, as practitioners are ideally placed to identify developing technologies very early in the process. New technologies could be made available very early in their development on a limited basis for clinical assessment, in trials or in registries, in order to allow for proper collection of evidence before an HTA is carried out. In such cases, it is necessary to ensure that the process is insulated from any industry bias.

The process of HTA needs to provide information to all stakeholders involved in the provision of healthcare. These are physicians, policy-makers, payers, patients (i.e. through heart foundations), general practitioners, and public health specialists. The value of a given innovation may need to be explained to the public and to policy makers through a structured communication campaign. In particular, such communication might emphasize the social value of a technology that goes beyond its immediate medical benefits. The health care system is a leading contributor to the economy in terms of job creation, and generation of revenues and income taxes, and this point is often ignored by other stakeholders. It is recommended that both the ESC and national societies should build up health economic expertise, and engage in discussions about the above with the national stakeholders involved in the provision of healthcare.

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List of attendees

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References