HTA in Europe: Approaches, benefits and challenges

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Overview

- Why do we need HTA?
- What is HTA?
- HTA systems
  - Scope and approach
  - Methods
  - Example of NICE in England
- Impact of HTA
- Concluding remarks

Trends in healthcare and innovation systems

- Healthcare system
  - Ageing populations and increases in diseases of affluence
  - Increasing consumer expectations and demands
  - Increasing opportunities
  - Increasing costs
  - Health care spending limited by financial crisis and budgets under extreme pressure
- Innovation system
  - Exciting scientific advances and clinical applications
  - Increasing costs of bringing regular technologies to market
  - Personalized medicine and genetic therapies likely to be even more expensive to bring to market

The need to prioritise

- No health care system can afford to fund everything for everyone
- There is a need to prioritize - and that need is growing
- Governments and health systems are increasingly turning to HTA to help them focus funding on treatments or programmes of most value in a way that is rational, fair and defensible

Health Technology Assessment

- HTA involves systematic evaluation of the nature, benefits, risks, costs and wider impacts of health technologies in the real life setting
- “health technologies” is used here to refer to any intervention in health and health care – from drugs and devices and equipment, to procedures, systems, and models of care delivery and financing. But the focus is typically on drugs and frequently also medical devices

Regulation and HTA

- Regulators consider safety and clinical effectiveness, to determine if clinical benefits outweigh risks. Their evaluation is often based on short to medium term clinical outcomes (often surrogate outcome measures – eg disease progression). They do not consider costs and they typically do not consider comparative effectiveness. The goal is to judge whether a technology should be granted access to market.
- HTA systems aim to assesses the overall benefits and costs of technologies to inform decisions about whether a technology which has market access is worth paying for. So HTA seeks information on
  - Long term health outcomes (eg survival and quality of life)
  - The costs of using the technology
- HTA systems therefore often require information in addition to that required by regulators.

Assessment and appraisal

- The purpose of HTA is to inform policy and decision making
- A distinction may be drawn between:
  - Assessment – the systematic analysis by experts of information on the benefits, costs and wider impacts of technologies, and the presentation of that information in a form accessible to decision makers
  - Appraisal – the review of information from assessments, alongside other relevant information such as priorities and other policy considerations (eg equity), to arrive at decisions on policy or practice by those accountable for such decisions

HTA systems: scope and approach

HTA systems vary in:

- The technologies covered
  - Eg drugs, devices, equipment, procedures, system organisation
- The timing of assessment
  - Eg pre market launch and/or later (eg 2/3 years post-launch)
- The perspective taken: costs and benefits for
  - the health care system, the whole of government, the whole of society
- Whether and how they consider budget impact and affordability as well as value
- The extent of stakeholder involvement
  - Eg clinicians, patients, industry
- The division/allocation of the assessment and appraisal/decision making functions
- The extent of transparency and accountability in assessment and decisions, and of challenges allowed

HTA systems: methods

HTA systems vary in methods used:

- Approaches to assess and compare benefits and costs include:
  - Assessment of "quality-adjusted life years" (QALYs), and cost per QALY
  - Assessment of overall "clinical added benefit" (CAB), and consideration of cost in relation to added benefit
  - Use of multi-criteria decision analysis (MCDA) to assess benefits and costs
- Willingness to accept data from different study designs (eg RCT, case control studies, real world evidence on outcomes)
- Systems doing assessment pre-launch may use Managed Entry approaches to manage uncertainty in benefits and costs
  - Eg restricting use to specific patient groups; capping volume and/or costs; linking payment to outcomes achieved in real life; collecting evidence on effectiveness

QALY systems (1)

- The incremental QALY gain of the new treatment is calculated. For example, if a new treatment gives a patient 10 more years of life in full health than the current treatment, the incremental gain is 10 QALYs. If those 10 years of extra life are associated with pain or functional impairment, the incremental QALY gain will be reduced – say to 5 QALYs
- The incremental cost of delivering the new treatment is then calculated, ie the increase in costs over the current treatment.
- The incremental cost is then divided by the incremental QALY gain to give a cost per QALY. For example, if a treatment has an incremental QALY gain of 5 QALYs at an incremental cost of £100,00, the cost per QALY is £20,000
- The cost per QALY is then compared with a "threshold" which is considered to indicate that a treatment is "cost-effective" or a good use of health care resources. This should reflect the resources available to the system
- A decision is then made on coverage/price based on the position of the incremental cost per QALY relative to the threshold, and other factors

QALY systems (2)

- Advantages
  - Relatively objective
  - QALYs can be calculated for all patients and diseases, so all health systems users can be given the same opportunity to benefit from limited resources – hence popularity with fixed budget “national health services” (where spending on one patient affects what can be spent on others)
- Challenges
  - How is the “threshold” set for what is and is not value for money or “cost-effective”? (NICE uses around UD $30k to $50k per QALY – with various “modifiers”)
  - Complex methods and models are often needed to calculate cost per QALY
  - Decisions still need to take account of factors not in the QALY
  - QALYs calculated from generic quality of life/utility measures which may be insensitive to the full patient experience for some diseases
  - QALYs are inherently “ageist” - saving a younger life produces more QALYs than saving an older one

Clinical Added Benefit systems (1)

- CAB systems compare the clinical benefits of a new treatment with those of the current treatment to place it in one of a number of pre-defined categories of Clinical Added Benefit (CAB), eg (for Germany):
  - major; considerable; minor; unquantifiable; none; less
  - [note that each of these category is defined in more detail, eg: “major added benefit” means “recovery from disease, significant extension of life, long-term freedom from severe symptoms”]
- They then consider/negotiate price according to the category (and other relevant factors), eg:
  - no CAB: same price as current treatment
  - considerable/major CAB: higher/premium price

Clinical Added Benefit systems (2)

- Advantages:
  - Allow assessments to use disease-specific QoL measures, and hence to take better account of the full patient experience
  - Combine objectivity and a degree of consistency (pre-defined categories of CAB) with pragmatism and flexibility
- Challenges
  - Leave much of the decision on actual price to judgment/negotiation (though may also include provisions for more formal cost-effectiveness analysis)
  - Less helpful to decision makers than cost per QALY for making trade-offs between funding for treatments for different conditions (but in national insurance based systems this may be less critical)

Example: NICE in England

- QALY-based system
- Covers selected drugs, devices, procedures and public health interventions
- Assessment is undertaken by industry (dossier) and commissioned academic teams; appraisal and decision by NICE Appraisal Committees
- Prospective assessment (note recent suggestions for change)
- “Patient Access Schemes” (managed entry) play a role
- Methodologically flexible (eg may accept observational data)
- Strict timelines
- Highly consultative and transparent
- Resource intensive (note recent proposals from NICE for “Fast Track”)
- Has rejected some high profile drugs, but approves (in part or full) most technologies it considers
- “Threshold”(£20,000 to £30,000 per QALY) seen as too low by patients and industry, and too high by some health service managers and economists – who see it as a pressure on budgets

Impact of HTA

- HTA-based assessment may result in technologies not being adopted, and manufacturers getting lower prices than they seek
- The approach taken to HTA by a system, together with its budget constraints and priorities, affect the decisions made, eg:
  - England rejects more drugs than Germany
  - Germany and France use similar approaches and have similar levels of spending, but do not always make the same decisions
- Is HTA impacting health system spending?
  - Questionable impact on overall spending in “developed” countries (general price negotiations probably more impactful in reducing spending); concerns in England that it creates a cost pressure
  - Should be resulting in resources being allocated to technologies of highest value
  - Increasing interest in affordability as well as value for money (eg HCV)
- Is HTA impacting on innovation?
  - May be reducing industry revenues
  - May be focusing innovation efforts on areas of most value

Concluding remarks

- HTA systems can provide an objective and accountable way to set priorities for spending in some areas of health care, and to respond to innovation
- The approach adopted to assessment affects the resources required and the outcomes achieved – you need to choose methods and system architecture that are appropriate to your health system, skills base and societal values
- HTA-based assessment does not solve all the problems health systems are facing. It does not remove budget pressures or challenges around affordability. But it provides an important tool for responding to them in a way that is objective and accountable