Challenges for Highly Specialised Technology Evaluations at NICE - The assessment of OMPs

Ultra-orphan drugs: establishing a fair and reasonable priority setting process

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Guidance Teams
- Technology Appraisals
- **Highly Specialised Technologies**
- Diagnostic Assessment Programme
- Medical Technologies Evaluation Programme
- Interventional procedures

Further programmes
- Patient Access Scheme Liaison Unit
- Scientific Advice
- Research and Development
What I will cover

• A brief outline of the NICE highly Specialised Technology (HST) Evaluation process
• Summary of the challenges faced in HST Evaluations
• How to overcome the challenges
HST remit

• To evaluate the benefits and costs of “technology x” within its marketing authorisation for the treatment of “disease y” for national commissioning by NHS England.
HST Evaluation Committee

• Chair
• 2 clinicians (including a geneticist)
• 1 public health physician
• 1 NHS finance/management
• 3 lay members (including someone with knowledge/experience of ethical issues)
• 2 health services researchers (including a health economist)
• 1 health care industry
• 1 pharmacist
• 1 commissioner (NHS England)
Evaluation

- Independent Assessment
- Company submission
- Patient & professional submissions
- Topic experts
- Public consultation/appeal
- Guidance
Challenges associated with economic evaluation of orphan drugs

- Orphan drugs do not usually prove to be cost-effective based on HTA methods designed for conventional diseases

- Rarity means weight of evidence is not the same as for conventional diseases
  - Small, heterogeneous populations
  - Short duration of follow-up of studies
  - Limited scientific understanding/consensus on clinical endpoints
  - Limited hard clinical outcomes such as survival
  - Limited natural history data
  - Lack of consensus/data on comparators

- More to decision-making rather than strict application of cost-effectiveness methods
  - Societal value
  - Seriousness of the condition
  - Availability of alternative treatment options
  - Cost to the patient if the drug is not reimbursed
  - Technical versus allocative efficiency
Evaluation - Challenges

• Defining the patient population
  – Population defined in Marketing Authorisation
  – Clarity on the patient population that will most benefit

• Uncertainty on outcomes
  • Create solutions to bridge gap
  • Give assurance to NICE that these will be addressed
Evaluation - Challenges

• Impact on carers/ family members
  – Quantify this impact in submission

• Lack of Natural History Data
  – Use of surveys/ interviews
  – Patient group data
Evaluation - Challenges

• Value Proposition
  – Creative / adaptive pricing
  – Incremental evidence creation
  – Clear data collection requirements
  – Collective responsibility
  – Implications of non delivery/compliance
  – Ways to minimise budget impact on the NHS
  – Early thinking/ discussion
  – Transparency
HST: other considerations

- Para 41…
  When evaluating cost to the NHS and PSS, the Committee will take into account the total budget for specialised services, and how it is allocated, as well as the scale of investment in comparable areas of medicine. The committee will also take into account what could be considered a reasonable cost for the medicine in the context of recouping manufacturing, research and development costs from sales to a limited number of patients.
HST : Managed Access Agreements (MAA)

• 2 technologies have been approval with a managed access arrangement

• A MAA scheme facilitates access to ultra-OMPs, whilst generating valuable evidence in collecting ‘real-world’ data.

• All stakeholders agree on a set of criteria and conditions that need to be fulfilled by patients, clinicians and industry.

• There may be some additional financial arrangements between payers and the relevant pharmaceutical company. At the end of the MAA period, the product is re-evaluated via the HST process. If no benefit is gained, the ultra-OMP will no longer be available to any patient via the NHS.
HST : Managed Access Agreements (MAA)

• All stakeholders agree on a set of criteria and conditions that need to be fulfilled by patients, clinicians and industry.

• There may be some additional financial arrangements between payers and the relevant pharmaceutical company.

• The product is re-evaluated via the HST process at the end of the MAA period. If no benefit is gained, the ultra-OMP will no longer be available to any patient via the NHS.
Further Questions

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