NICE’s Highly Specialised Technologies (HST) evaluation committee

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EUCOPE P&R / Market Access Working Group Meeting

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Remap Consulting is a specialist pricing and market access consultancy focused on three core areas:

- Pricing and Market Access Strategy Development and Implementation
- Enhancing Organisational Market Access Effectiveness
- Training and Capability Development
Founded in 1999, NICE is an independent body whose aim is to ensure that NHS resources in England and Wales are optimally utilised.

NICE base their recommendations on a review of clinical and economic evidence:

- **Clinical evidence**: how well the treatment works.
- **Economic evidence**: does it represent value for money?

NICE have seven different approaches when developing its guidance:

- Technology appraisals
- Diagnostic technologies guidance
- Medical technologies guidance
- Interventionsal procedures
- Clinical guideline development
- Public health guidance
- Highly specialised technologies
The highly specialised technologies evaluation committee evaluates ultra-orphan diseases

When

• Since May 2013 NICE has been given the responsibility of determining whether very rare disease treatments should be recommended for NHS-wide commissioning in England

Why

• NICE recognised that its current assessment processes were not suitable for assessment of very rare diseases (<1 in 50,000)

How

• Building on the decision-making framework developed by the Advisory Group for National Specialised Services (AGNSS) it has developed an interim methodology

What’s next

• NICE hope to finalise this methodology, following a public consultation, in 2015
The interim highly specialised technologies evaluation committee process is resource and time consuming

- Process takes 17 - 27 weeks from receipt of submissions to guidance publication!
- Process can be started 20 months prior to marketing authorisation
What do the HST committee consider in their decision making?

1. Nature of the condition
2. Impact of the new technology
3. Cost to the NHS and Social Services
4. Value for money
5. Impact of technology beyond direct health benefits
6. The impact of the technology on the delivery of the specialised service
What do the HST committee consider in their decision making?

Nature of the condition

- Disease morbidity and patient clinical disability with current standard of care
- Impact of the disease on carers’ quality of life
- Extent and nature of current treatment options
What do the HST committee consider in their decision making?

Nature of the condition

Impact of the new technology

• Clinical effectiveness
• Overall magnitude of health benefits to patients and, when relevant, carers
• Heterogeneity of health benefits within the population
• Robustness of the current evidence
• Treatment continuation rules
What do the HST committee consider in their decision making?

Nature of the condition

Impact of the new technology

Cost to the NHS and Social Services

- Budget impact in the NHS and social services
- Robustness of costing and budget impact information
- Patient access agreements
What do the HST committee consider in their decision making?

Value for money
- Technical efficiency
- Productive efficiency
- Allocative efficiency
What do the HST committee consider in their decision making?

- Nature of the condition
- Impact of the new technology
- Cost to the NHS and Social Services
- Value for money
- Impact of technology beyond direct health benefits
  - Are there significant benefits other than health
  - Is a substantial proportion of the costs or benefits incurred outside the NHS and social services
  - The potential for long-term benefits to the NHS of research and innovation
What do the HST committee consider in their decision making?

The decision is made by gaining consensus agreement from the members of the committee.
To date only one product, Soliris, has been through the whole HST process

- NICE evaluated Soliris (eculizumab) for the treatment of atypical hemolytic uremic syndrome (aHUS) drug, a life-threatening blood disorder that can cause organ failure

March 2014: Draft guidance

NICE rejects Soliris due to its high budget impact and called on Alexion to justify the cost of Soliris; £340,200 per patient for their first year of treatment in respect to the development and commercialisation costs
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Final guidance: September 2014

From the available evidence and from the testimony of clinicians and patients, families and carers …eculizumab is a significant breakthrough in the management of aHUS. The drug is, however, very expensive.
Unsurprisingly the positive NICE opinion for Soliris comes with some conditions

1. NHS England and Alexion should consider what opportunities might exist to reduce the overall cost of eculizumab to the NHS.
2. Use must be through an expert centre.
3. Monitoring systems to record the number of people who receive eculizumab and at what dose.
4. A research programme be set up to evaluate when Soliris treatment could be stopped or adjusted.
In summary, whilst the HST process is still being refined, demonstrating incremental clinical benefit is key

Process still needs refinement
- Soliris example shows that there is still teething problems
- NICE uncomfortable with moving away from cost-effectiveness

Clinical benefit is key
- If the product is addressing a high unmet need and can demonstrate clinical benefits it is hard for NICE to say no
- Patient support is critical

Budget impact is a concern
- NICE are concerned over the budget impact of these treatments
- Expect further attempts by NICE to minimise budget impact through use of patient access schemes
THANK YOU

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