



Highly Specialised Technology Evaluations at NICE

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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

NICE National Institute for
Health and Care Excellence

Highly Specialised Technologies

What I will cover

- Role of HST at NICE
- HST Evaluation process
- HST Committee
- Challenges Associated with HST Evaluations
- HST Criteria considered by Committee
- The role of the patient in evaluations
- Evaluation examples

HST at NICE

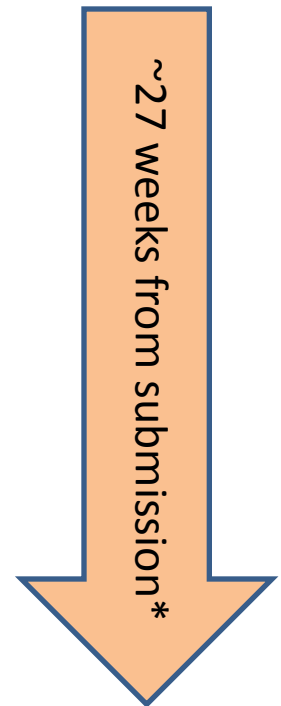
- NICE took over the responsibility of the evaluation of very high cost drugs for patients with rare conditions in April 2013
- This was to ensure there was a robust, objective, independent and transparent assessment of these drugs.

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.

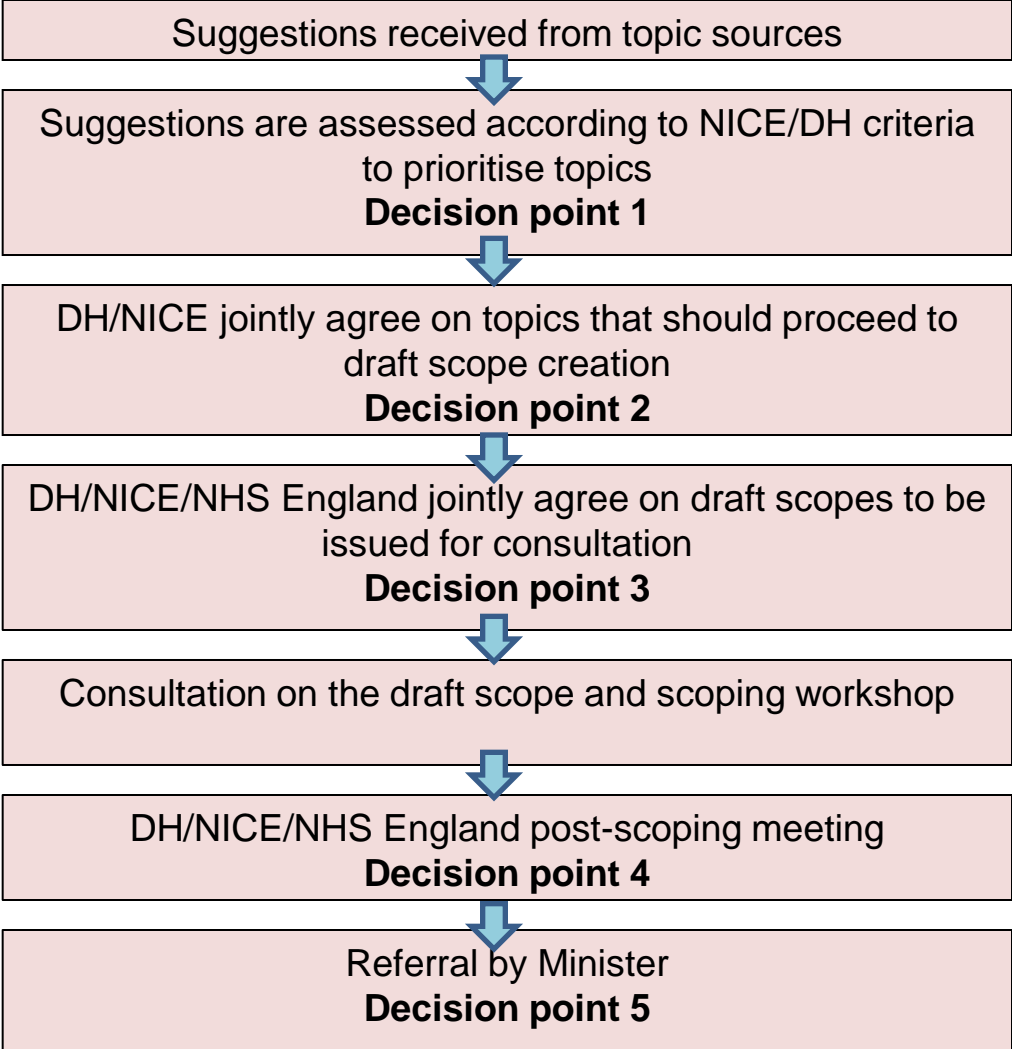
Highly specialised technologies programme: process

- NICE produces provisional list of topics
- Consultees identified
- Scope prepared and consulted on
- Topics referred by Minister to NICE
- Evidence submitted by manufacturer and other consultees, comments invited on potential clinical effectiveness and value
- Evidence review group (ERG) report independently commissioned and prepared
- Committee papers prepared: Evidence submissions from manufacturer, patients, clinical specialists and NHS England, ERG report
- Evaluation committee considers all evidence
- Evaluation committee document (ECD) produced only if recommendations are more restrictive than license; public consultation for 4 weeks
- Evaluation committee considers responses to public consultation
- Final evaluation determination (FED) produced; any appeals considered
- Guidance issued



Topic Selection & Scoping

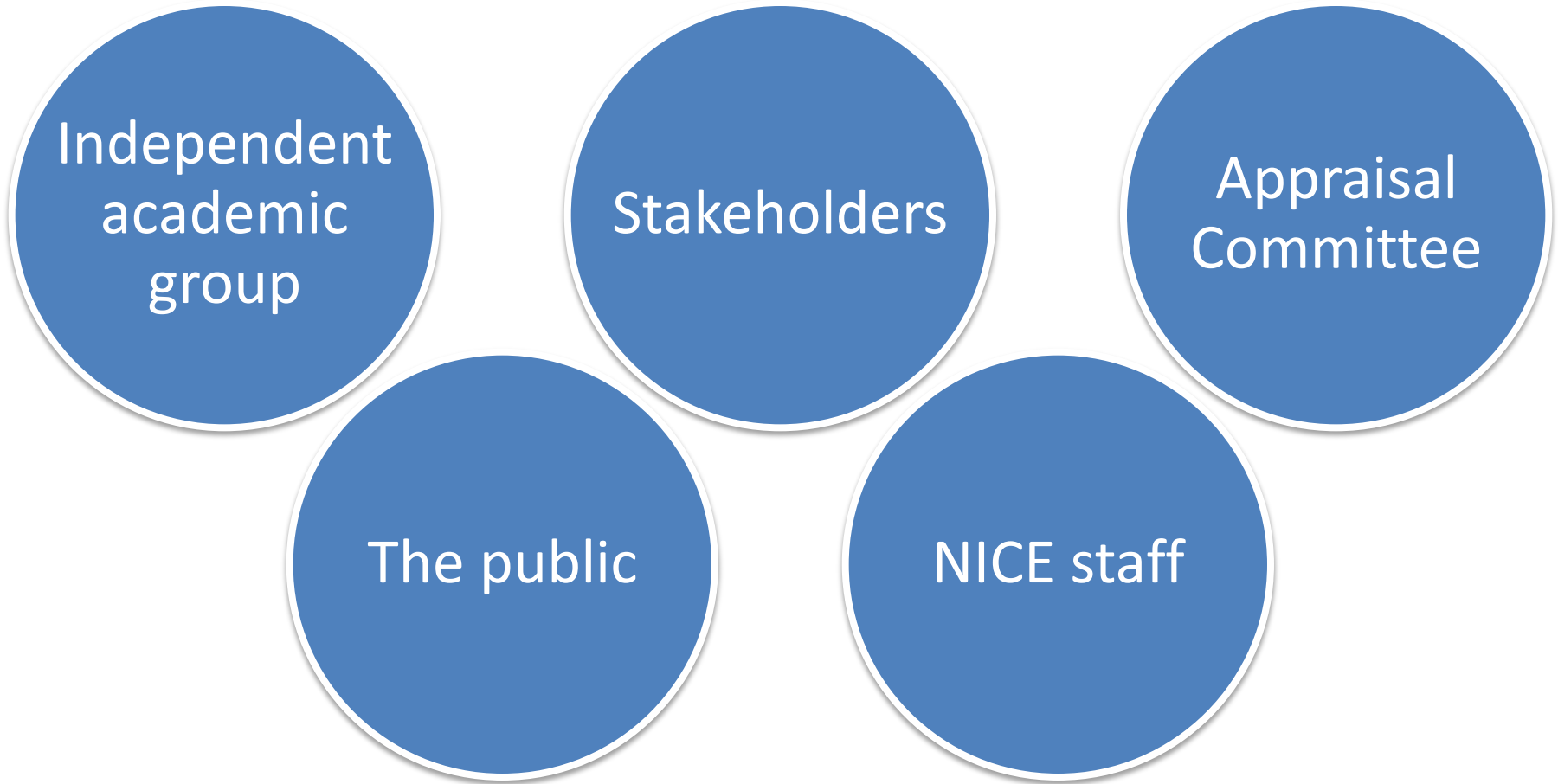
Reject or refer elsewhere
e.g.
National Screening Committee



Process starts approximately 2 years before a drug is licensed

Ideally 12–15 months before a drug is licensed

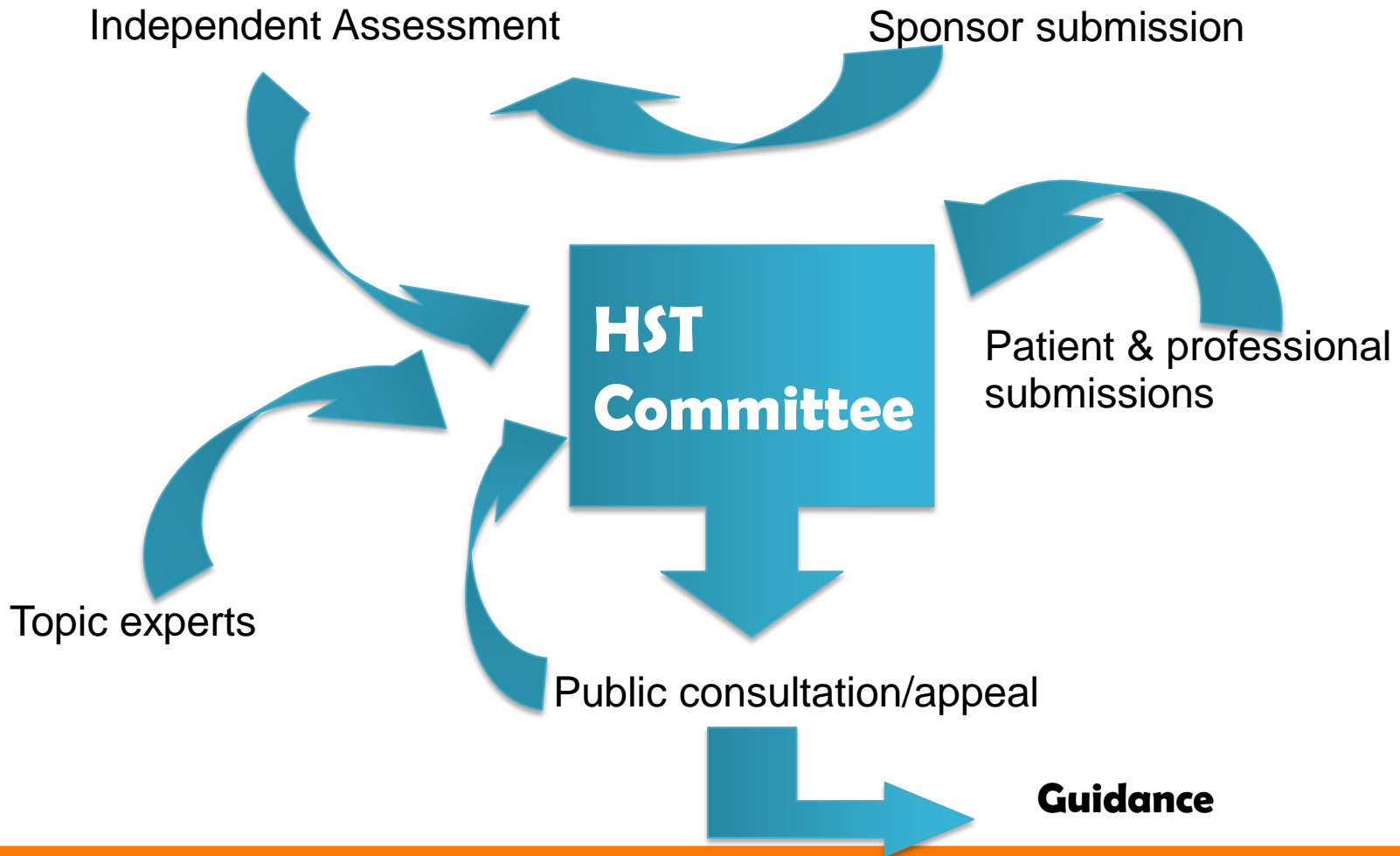
Groups involved in an appraisal



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



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

Highly specialised technologies programme: prioritisation criteria

- The target patient group for the technology in its licensed indication is so small that treatment will usually be concentrated in very few centres in the NHS
- The target patient group is distinct for clinical reasons
- The condition is chronic and severely disabling
- The technology is expected to be used exclusively in the context of a highly specialised service
- The technology is likely to have a very high acquisition cost
- The technology has the potential for life long use
- The need for national commissioning of the technology is significant

HST evaluation criteria

- Nature of the condition
- Impact of the new technology
- Cost to the NHS and Personal Social Services
- Value for money
- Impact of the technology beyond direct health benefits
- Impact of the technology on the delivery of the specialised service

HST criteria

Criterion	Factors considered
Nature of the Condition	<ul style="list-style-type: none">• Disease morbidity/mortality• Patient clinical disability with current standard care• Impact of the disease on family/carers' quality of life• Extent and nature of current treatment options
Impact of the New Technology	<ul style="list-style-type: none">• Clinical effectiveness• Overall magnitude of health benefits to patients, and where relevant, their families/carers• Heterogeneity of health benefits within the population• Robustness of the current evidence base and anticipated contribution the guidance may make to strengthen it• Treatment continuation rules (if applicable)

HST criteria

Criterion

Factors considered

Cost to the NHS and PSS

- Budget impact of technology in the NHS and PSS
- Robustness of costing and budget impact information
- Patient access schemes

Value for Money

- Incremental benefit of the new technology compared with current treatment options (technical efficiency)
- Nature and extent of the other resources needed to enable the new technology to be used (productive efficiency)
- Impact of the new technology on the budget available for specialised commissioning (allocative efficiency)
- Opportunity cost of the technology (effect of investing in this technology rather than in another specialised service)

HST criteria

Criterion

Factors considered

Impact of the technology beyond direct health benefits

- Significant benefits other than health
- Whether a substantial proportion of the costs (savings) or benefits are incurred outside of the NHS and PSS
- Potential for long-term benefits to the NHS and society of research and innovation

Impact of the technology on the delivery of the specialised service

- Staffing and infrastructure requirements
- Training requirements and need to plan for expertise
- Best clinical practice in delivering the service

HST: other considerations

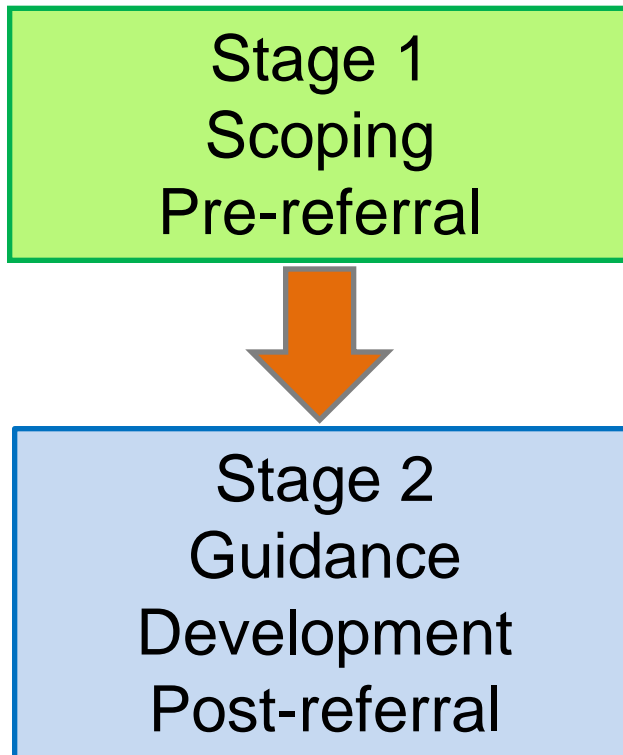
- Para 41...

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.

How patients and carer organisations can contribute to a NICE Highly Specialised Technology Evaluation



Overview of patient involvement



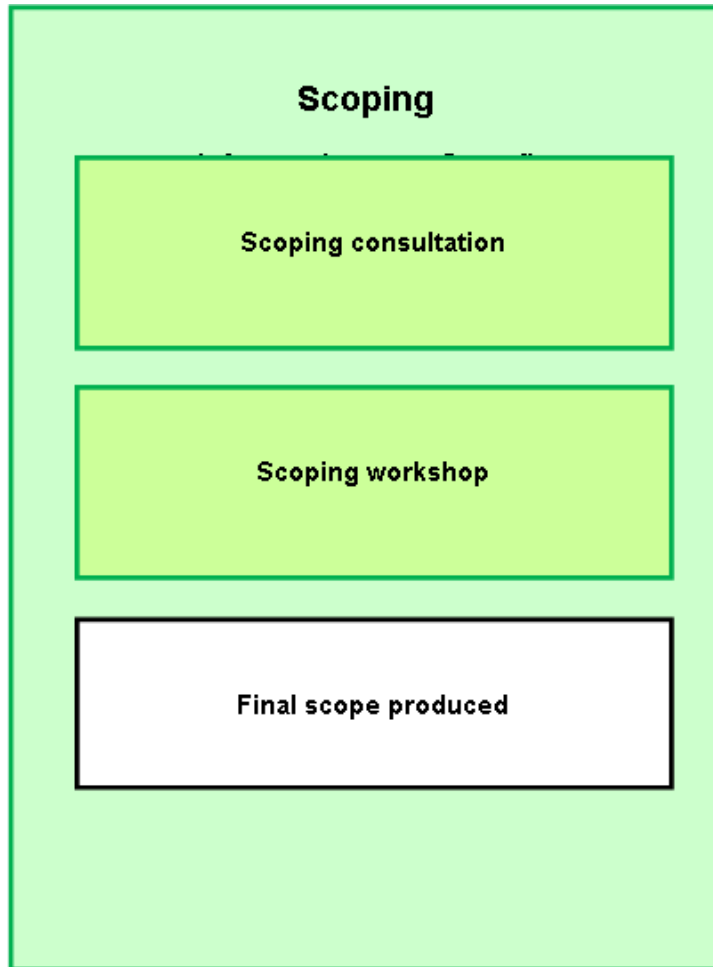
Scoping usually occurs:

1. before a licence has been granted for a technology
2. Before the topic has been referred

Guidance development is usually:

1. After referral
2. As close to issue of licence as possible
(the licence has to be issued before the evaluation consultation can begin)

Scoping (pre-referral)



Patient organisations can:

1. Comment in writing on the draft
 - remit
 - scope
 - matrix (stakeholder list)
2. Participate in scoping workshop

Patient input

- Limited evidence base means patient evidence is particularly important for HST evaluations
 - Patient numbers
 - Burden of disease
 - Impact of treatment
 - Likely uptake
- NICE team proactively identify and support patient groups
- Report commissioned on role of patient evidence and support requirements
 - Will inform methods and process review

The role of patient experts

Patient Experts

- provide statements which will help the Committee consider key criteria such as the nature of the condition
- attend Committee meetings as individuals

They will have

- experience of the broader patient population relevant to the evaluation and/or
- relevant personal experience

HST Guidance Published

- HST has published guidance on 2 topics
 - Eculizumab for atypical Haemolytic Uremic Syndrome (aHUS)
 - Elosulfase alfa for Mucopolysaccharidosis (type IVA)

HST in action: eculizumab for aHUS

- Key considerations included:
 - **Severity** of atypical Haemolytic Uremic Syndrome (aHUS)
 - Eculizumab as **innovative** - step-change in treatment for aHUS
 - **Limitations and uncertainties** in the evidence base
 - **Very effective** treatment – substantial QALY gains
 - **High-cost** per patient
 - **Substantial budget-impact** with uncertainty about projected figures

Eculizumab for aHUS: recommendation

- To fund Eculizumab (Soliris) treatment of atypical Haemolytic Uraemic Syndrome (aHUS) only if the following are in place:
 - coordination through an expert centre
 - monitoring systems
 - national protocol for starting and stopping eculizumab for clinical reasons
 - a research programme with robust methods to evaluate when stopping treatment or dose adjustment might occur.
- The budget impact is uncertain but will be considerable. NHS England and the company should consider what opportunities might exist to reduce the cost of eculizumab to the NHS.

HST in action: Mucopolysaccharidosis (type IVA) - elosulfase alfa

- Key considerations included:
 - **Severity** of Mucopolysaccharidosis (type IVA)
 - elosulfase alfa as **innovative** - step-change in treatment for MPS
 - **Uncertainties** in the evidence base – clinical trial data could not be reconciled with patient testimonies
 - **High cost** - Committee was not satisfied that the high cost of elosulfase alfa was fully justified
 - **Effective** treatment - Committee concluded that the health and quality of life of some patients improved significantly on treatment.

Mucopolysaccharidosis (type IVA) - elosulfase alfa: recommendation

To fund elosulfase alfa (Vimizim) treatment of Mucopolysaccharidosis (type IVA) according to the conditions in the managed access agreement

Committee considered the cost of elosulfase alfa incorporating the patient access scheme too high to be recommended outside the context of a managed access agreement. However, it was satisfied there was sufficient evidence some patients did well on elosulfase alfa to justify further exploration of costs and benefits in routine clinical practice, within the context of a managed access agreement, to inform a future review of this guidance.

Questions