

HIGHLY SENSIBLE TACTICS: STRATEGIC INSIGHTS FOR NAVIGATING HIGHLY SPECIALISED TECHNOLOGY (HST) TOPIC SELECTION

Soboil J¹, Barker-Yip J¹, Mohindru B¹, Orchard M¹

¹Cogentia (contact: enquiries@cogentia.co.uk)

Cogentia[®]

WWW.COGENTIA.CO.UK

BACKGROUND

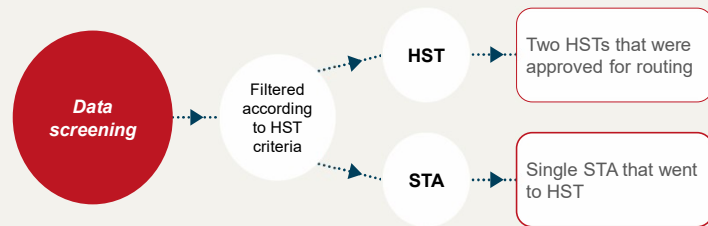
- Highly specialised technologies (HST) are interventions used for treatment of very rare conditions. The HST programme was passed on to the National Institute for Health and Care Excellence (NICE) in 2013, through the Health and Social care Act 2012. The significance of the programme is that it allows for a generous cost-effectiveness threshold of up to £100,00 per QALY gained. The HST programme is specifically designed for use in exceptional circumstances, with eligible technologies being evaluated based on the following, strict criteria: the target population includes a small number of patients and is clinically distinct, the condition is chronic and severe, there is limited or no treatment options, and there is difficulty in collecting evidence, and there is potential for life-long use of the technology.

OBJECTIVE(S)

- This study analysed publicly available HST checklists to assess the extent to which checklist criteria were met, identify if any themes emerged and how HST/STA routing decisions were impacted.

METHODS

- Available HST checklists, downloaded from the NICE website, were reviewed quantitatively, and assessed against final routing outcomes/status. Researchers evaluated the findings to interpret whether patterns exist in relation to decision-making against the four HST criteria.



RESULTS

- As of February 2023, 9 HST checklists were available on the NICE website. Of these nine appraisals, only two were deemed eligible for HST; five proceeded as STA and two were suspended (one due to manufacturer decision following STA-routing decision; one due to EMA-license issues).
- Checklist evaluation showed that 56% of appraisals met the first three HST criteria.
- However, only 33% of appraisals met the last criterion (i.e., benefit over existing treatment options).
- Criteria stratification, based on HST/STA outcome, showed that HST appraisals met all four criteria. However, for the seven STA-routed appraisals, criteria decisions were deemed 'met' or (at the other extreme) 'unmet' in equal measure (n=11 times), with only two 'partially met' or four 'unclear' decisions made.

DISCUSSION

- Often treatment for rare or very rare conditions represent a unique challenge to decision makers. Due to the rare nature of the disease, data which drives the health technology assessment (HTA) is scarce. This results in a cost effectiveness which is subject to a high degree of decision uncertainty.
- In such contexts, routing through HST provides greater potential for successful approval of treatments. However, since the introduction of new regulations and charges that came into effect in 2019 for HST routing, it is important for companies to evaluate the trade-offs between the costs and risks of being rerouted to an STA.

REFERENCES

- Health and Social Care Act 2012. [cited 2023 Oct 26]. Available from: <http://www.legislation.gov.uk/ukpga/2012/7/contents/enacted>
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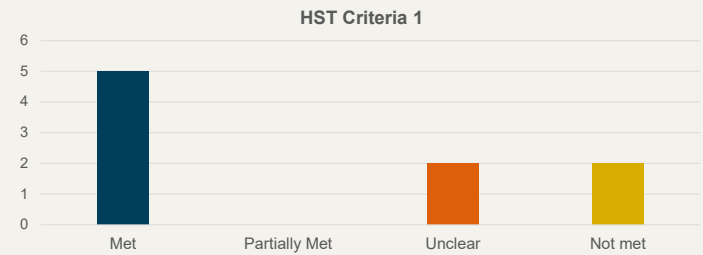


Figure 1 a) Criteria 1: The condition is very rare defined by 1:50,000 in England or about 1,100 people

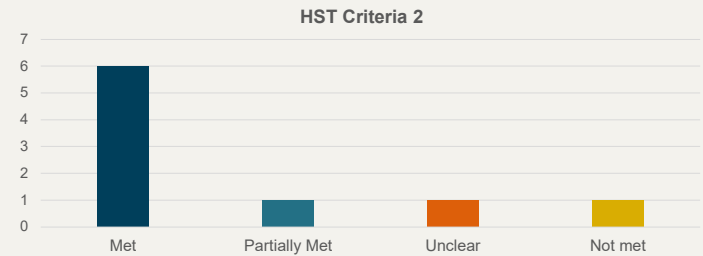


Figure 1 b) Criteria 2: Normally no more than 300 people in England are eligible for the technology in its licensed indication and no more than 500 across all its indications

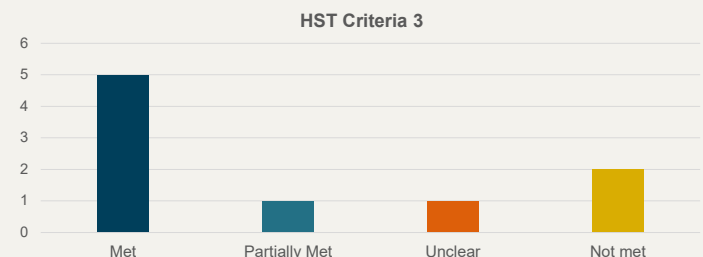


Figure 1 c) Criteria 3: The very rare condition significantly shortens life or severely impairs its quality

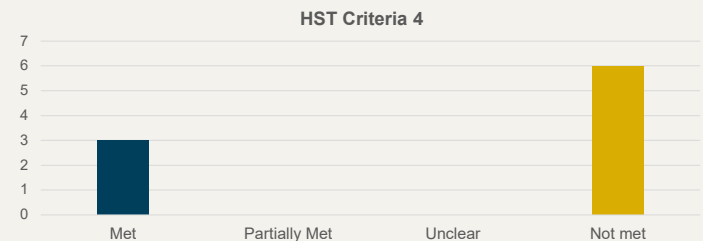


Figure 1 d) Criteria 4: There are no other satisfactory treatment options, or the technology is likely to offer significant additional benefit over existing treatment options.

Abbreviations:

CONCLUSIONS

- Our analysis strongly indicates that the evaluation of criteria assessment decisions for HST routing are polar extreme.
- The frequency of decisions for each criteria are largely concentrated as either 'met' or 'unmet'.
- HST routing approval is only consistently achieved when all checklist criteria are met.
- Overall, this suggests firm decision-making by TSOP against the checklist criteria.
- A critical criterion is benefit over existing treatment options, which was mostly unmet. We believe that this highlights product innovation within a sparse competitor landscape as the biggest determination for HST routing success.