CONSISTENT DECISION-MAKING BY NICE'S TSOP HIGHLIGHTS PRODUCT INNOVATION AS CRITICAL FOR HST ROUTING SUCCESS

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BACKGROUND/INTRODUCTION

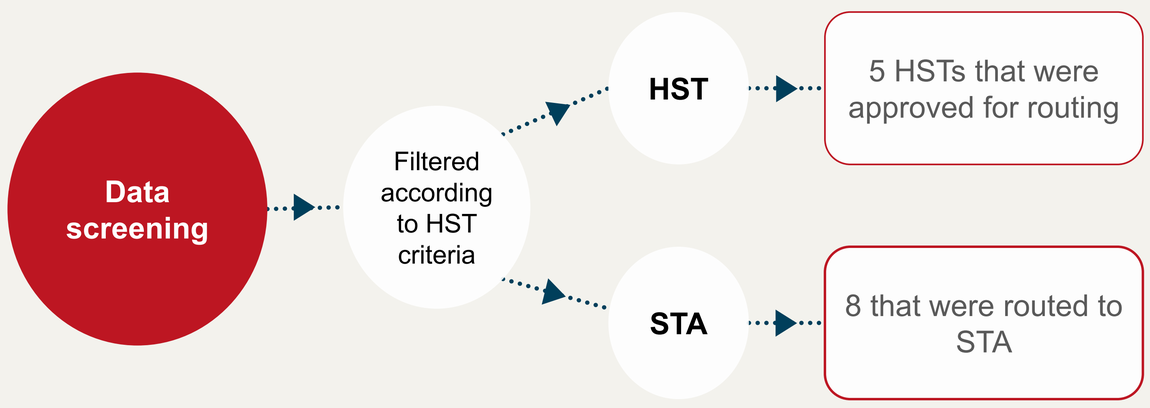
- Highly specialised technologies (HST) are interventions used for treatment of very rare conditions. The HST programme was passed on to the National Institute for Health and Care Excellence (NICE) in 2013, through the Health and Social Care Act 2012. The significance of the programme is that it allows for a generous cost-effectiveness threshold of up to £100,000 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 condition is very rare, the target population is small, the condition severely impacts life expectancy and or severely impairs HRQoL, and there is a clear unmet need; the technology offers significant additional benefit over existing standard of care (SoC).

OBJECTIVES

- To analyse the key determining criteria for HST/STA routing decisions from publicly available HST checklists.
- Assess the average time to reimbursement, overall and HST vs. STA.

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. Time to reimbursement was also assessed between HSTs and STAs.



RESULTS

- As of June 2024, we identified 13 published HST checklists publicly available on the NICE website. Of these 13, we found that only 5 ultimately became HST, 6 routed to STA and 2 STAs were suspended (one due to manufacturer decision following STA-routing decision; one due to EMA-license issues). In this update, 4 new topics were identified for inclusion in addition to the original 2023 analysis.
- Checklist evaluation showed that, on average, HST criteria were 'met' 9/13 times (69%). The last criterion (regarding benefit over existing treatment options) was split between 'met' 7/13 times (54%) and 'not met' 6/13 times (46%). Criteria stratification, based on HST/STA outcome, showed that HST appraisals met all four criteria.
- However, for the six 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' and four 'unclear' decisions made.
- Lastly, evaluation of time to reimbursement shows that, on average, HSTs take longer than STAs (1.3 years more) to achieve final reimbursement

DISCUSSION

- Treatment for rare or very rare conditions represent a unique challenge to decision makers.
- Data which drives the health technology assessment (HTA) is often scarce. This results in a high degree of decision uncertainty.
- 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.
- NICE's update of HST criteria is due in April next year; they plan to launch public consultation of these criteria in December 2024.
- We will be keeping an eye on criterion 2 regarding small target population, which has faced criticism from industry.

REFERENCES

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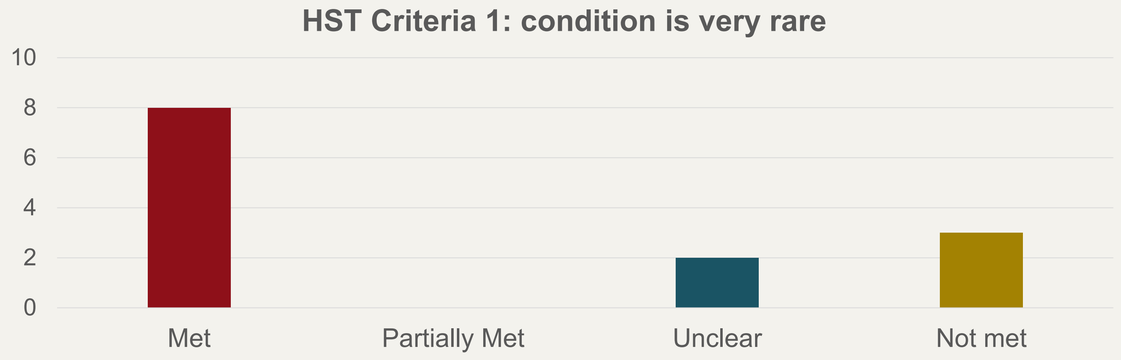

Figure 1 a) Criteria 1: The condition is very rare defined by 1:50,000 in England or about 1,100 people

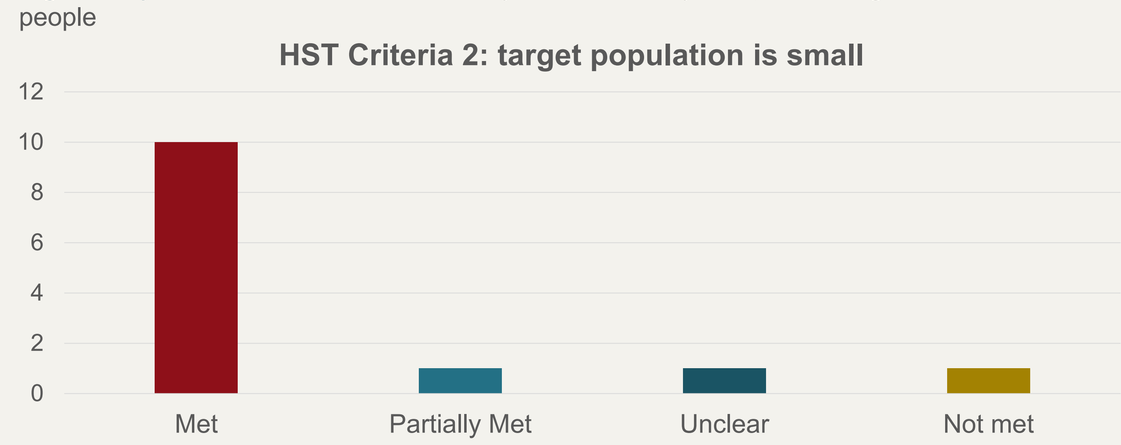

Figure 1 b) Criteria 2: Normally no more than 300 people in England are eligible for the technology in its licensed indication and no more than 500 across all its indications

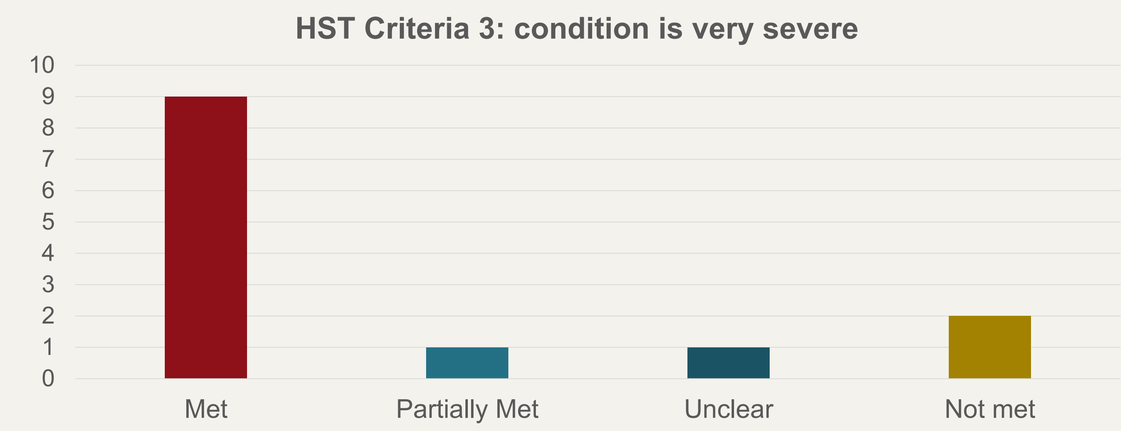

Figure 1 c) Criteria 3: The very rare condition significantly shortens life or severely impairs its quality

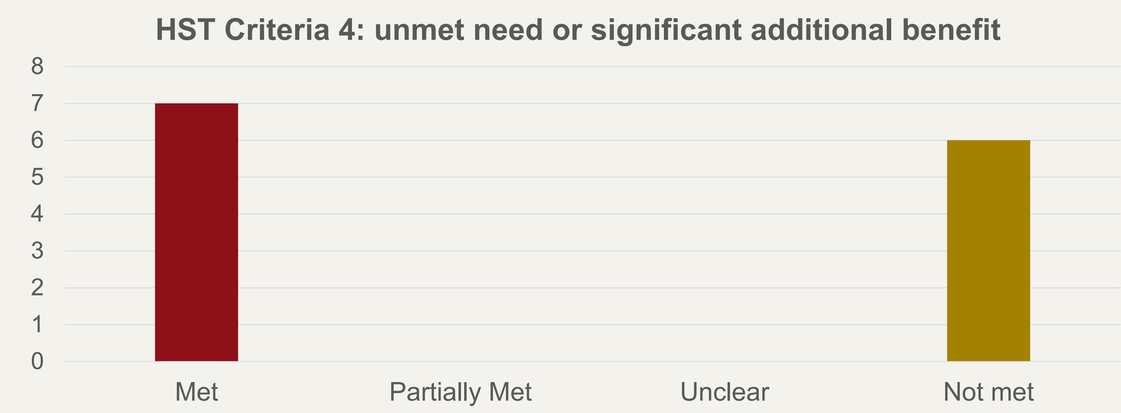

Figure 1 d) Criteria 4: There are no other satisfactory treatment options, or the technology is likely to offer significant additional benefit over existing treatment options.

Time to reimbursement

Routing (STA vs. HST, overall)	Time (years)
STA	1.66
HST	2.98
Overall	2.25

Table 1): Average time to reimbursement based on routing.

CONCLUSIONS

- Our analysis strongly suggest that criteria assessment decisions are generally polar extreme, indicating consistent decision-making by TSOP against HST checklist criteria.
- HST routing approval is only consistently achieved when all checklist criteria are met.
- This signals that product innovation is a key determinator for successful HST routing.
- Markedly, the last criterion (benefit over existing treatment options) was unmet 5/6 times (83%) for technologies that failed to qualify for HST according to TSOP.
- A critical criterion is benefit over existing treatment options, which was mostly unmet. We believe that this highlights product innovation within a sparce competitor landscape as the biggest determination for HST routing success.