HTA326

The Phantom Menace: How Consultations and Appeals During NICE HST Appraisals Contribute to Delays in Patient Access

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Aim

This study evaluated the impact of consultations, appeals, and extra committee meetings on the time to final guidance in NICE HST appraisals which resulted in a positive recommendation for the appraised technology.

Background

The National Institute for Health and Care Excellence (NICE) Highly Specialised Technology (HST) programme is designed to evaluate innovative technologies that address the high unmet need in ultra-rare diseases with debilitating symptoms.

These conditions have an exceptional negative impact and burden on the people living with them, and on their carers and families, for whom timely access to new treatments is key.

However, events that extend the health technology assessment (HTA) process such as consultations, appeals, and additional committee meetings can push back publication of NICE final guidance, delaying patients' access to treatment.

Methods

- Publicly available data on HST appraisals were collected from programme inception to September 2025 (N = 30).
- The dataset comprised the time from Final Scope (FS) to Final Evaluation Determination (FED) and the number of appeals, consultations, and committee meetings—referred to herein as additional events.
- We initially explored these variables with descriptive statistics and Wilcoxon Mann-Whitney tests, to assess the significance of the time differences observed.
- Binary variables for analysis were created based on thresholds of ≥1 appeal, ≥1 consultation, and ≥2 committee meetings.

"How do additional events occurring during NICE HST appraisals impact timelines and delay patient access?"

Results

- Of the 30 HST appraisals published between January 2015 and May 2025, 28 (93%) resulted in the appraised technology being recommended.
- The mean duration of positive appraisals was 81 weeks (range: 39–318).

***Figure 1**. Time from FS to FED, by additional events (box plots depict the distribution and median, with the mean indicated by an X)*

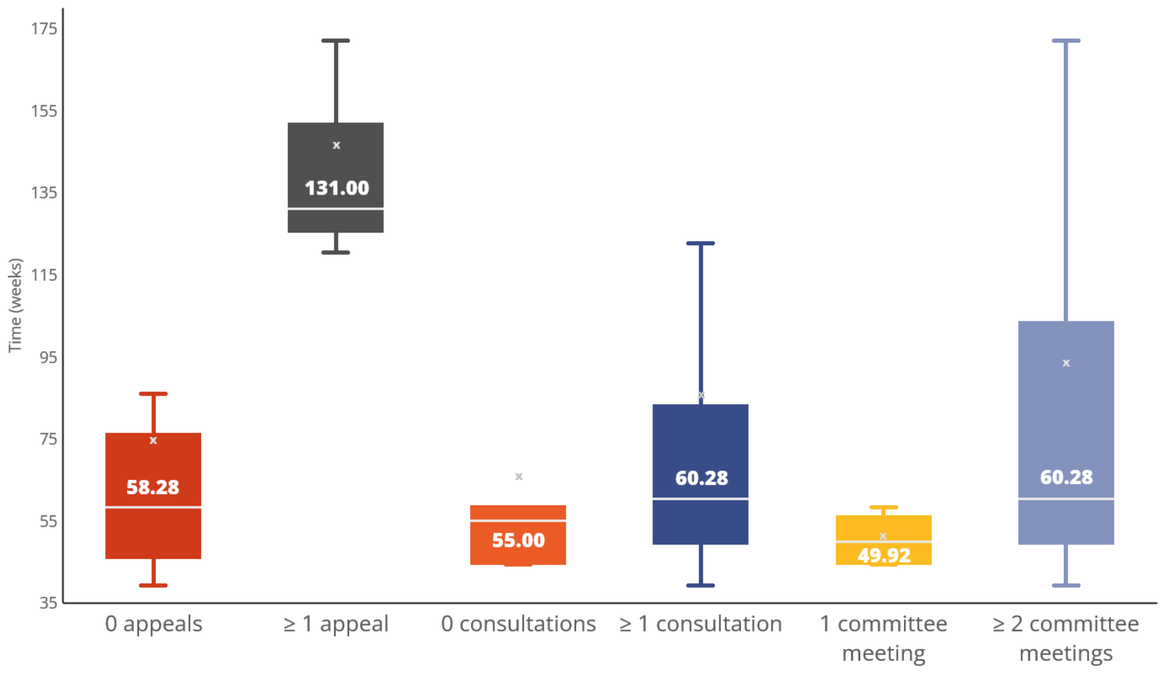

***Figure 2**. Impact of additional events on time to FED*

- Appraisal duration was notably shorter for appraisals with no additional events (n=4) compared to those with ≥1 additional event (n=24), at 51 weeks (range: 44–58) and 86 weeks (range: 39–318), respectively.
- Consultations were held in 23 of the positive appraisals (82%), four of which required a second consultation.
- Appeals were less frequent, occurring in only three (11%) appraisals.
- The number of committee meetings could be identified for 27 of the positive appraisals, of which 23 (85%) had ≥1 (range: 2–5).[1]
- The Wilcoxon Mann-Whitney tests showed a statistically significant increase of 67.0 weeks for appraisals with at least one appeal versus those with no appeals (p=0.021) (Figure 2).
  - While not statistically significant, consultations, committee meetings, and any additional event were also associated with mean time increases of 17.8, 36.9, and 35.8 weeks, respectively.

Discussion

Conclusions: Delays from appeals, consultations, and extra committee meetings in the HST pathway can significantly push back market access (up to 67 weeks). This not only incurs significant financial burdens from lost revenue, increased operational costs, and extended resource use, but also postpones critical treatment access for patients in need.[2]

Implications for Practice: To avoid significant delays, companies are advised to take the following 3 steps:

I. Engage early with HTA bodies and payers to understand requirements and tailor the evidence package and dossiers to each target market.

II. Consider how technical engagement, when provided by the HTA agency, may be leveraged to discuss evidence gaps prior to submission.

III. Develop a cross-functional evidence generation plan to address gaps and support robust economic models with validated assumptions.

Limitations: Limited materials were used in the analyses, as potentially relevant components of the HST dossier, such as appendices and technical engagement documents (when applicable), are not publicly available.

Due to a small sample size, this analysis had low statistical power. We expect the observed trend will persist and that our non-significant results may reach statistical significance as more data become available.

"Our advice: engage early, consider technical engagement, and develop a cross-functional evidence generation plan"

References

1. National Institute for Health and Care Excellence (NICE) *Published: Guidance, quality standards and advice* Accessed February 15, 2025 https://bit.ly/NICE_HST
2. Getz, *Appl Clin Trials* 2024; 33(6):12-13

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