Key Drivers Underlying Positive and Negative Decisions for NICE TAs and HST Appraisals in 2023

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Introduction

- NICE is responsible for HTA in England, providing recommendations for new and existing treatments through TA and HST assessments.
- There is a need to identify and mitigate potential barriers to access to increase the likelihood of a positive recommendation.
- This research aimed to identify trends underlying positive and negative decisions for emerging treatments in England.

Methods

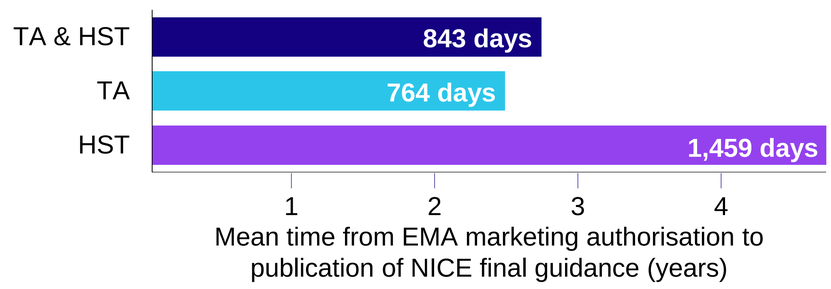
- NICE TA and HST published in 2023 were identified. Terminated/withdrawn submissions were excluded.
- The EMA website was searched to identify marketing authorisation of the treatments assessed in the identified TAs and HSTs.
- Pre-defined topics, including NICE recommendation, clinical and economic evidence submissions, and decision drivers, were extracted with 14% quality checked by a second reviewer.

Results

NICE treatment recommendations in 2023

- NICE published 81 TA and HST in 2023 (72 TA and 9 HST).
- Time from EMA marketing authorisation to publication of NICE final guidance was longer for HSTs than TAs (Figure 1).

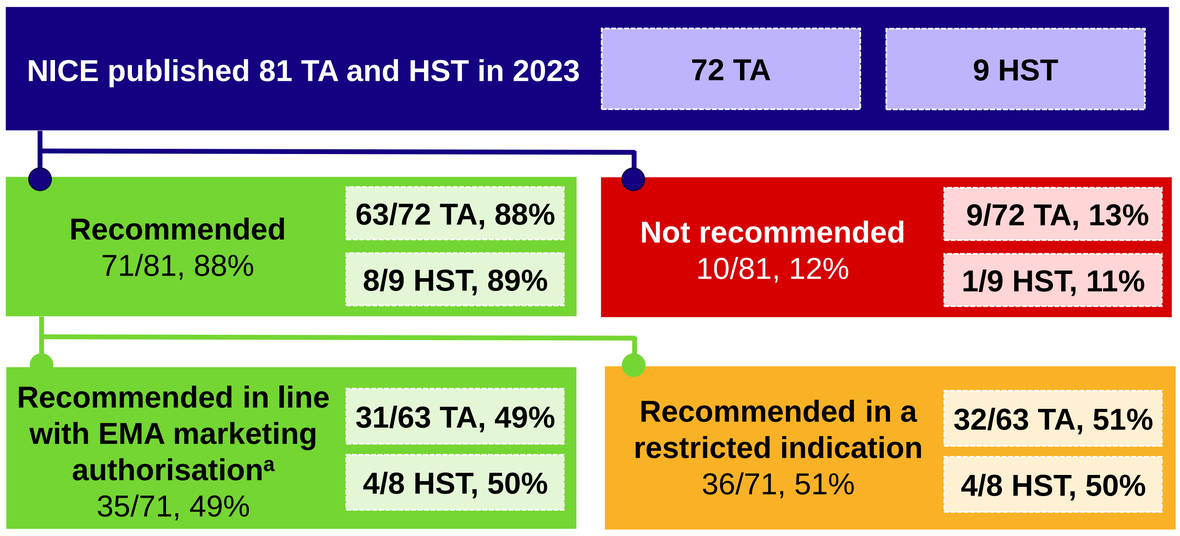
Figure 1: Time from EMA marketing authorisation to publication of NICE final guidance



Note: n=79: 2 TA were not included in the analysis as EMA marketing authorisation was not identified (TA943[1]: device; TA934[2]: authorised by MHRA).

- 88% (71/81) of treatments received a positive recommendation (Figure 2).
- However, 32% of positive recommendations (23/71) were dependent on a MAA or funding via the CDF, and only 49% were recommended in line with EMA marketing authorisation.

Figure 2: Outcomes for treatments assessed by NICE in 2023



Note: Percentages may not add up to 100% due to rounding. [a]Includes 1 TA for a device (no marketing authorisation identified) which was recommended through a 5-year phased roll out[1].

Restrictions compared to marketing authorisation

- Of the treatments recommended in a restricted indication, most were restricted to a specific patient subgroup (Table 1).
- All restrictions to HST (4 HST) were to a specific patient subgroup.

Table 1: Indication restrictions applied compared to marketing authorisation

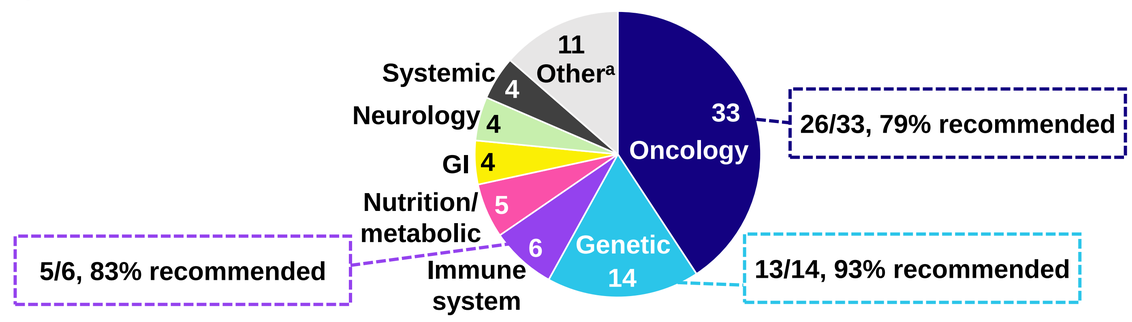
Restriction applied	Number of TA and HST (%)
Patient subgroup[a]	28 (78)
Treatment line	7 (19)
Stopping rule	6 (17)
Add-on to SoC only	4 (11)
Only if a specific treatment would otherwise be offered[b]	1 (3)

Note: Groups not mutually exclusive. Percentages represent a proportion of the TA and HST recommended in a restricted indication (out of 36). [a]Age, treatment history/contraindications, biomarkers, disease-specific measures, performance indicators. [b]Intervention was only considered cost-effective compared to a specific alternative treatment.

Appraisals according to disease area

- TA and HST in oncology were most frequent (41%), followed by genetic conditions (17%) and immune system disorders (7%; Figure 3).
- Genetic conditions had the largest proportion of positive recommendations, all of which were HST.
- Although oncology therapies had a high (79%) rate of positive recommendations, 81% of these depended on funding via the CDF.

Figure 3: Number of TA and HST in each disease area



Note: [a]Disease areas with ≤3 TA and HST: cardiovascular (n=3), dermatology (n=2), nervous system (n=2), respiratory (n=2), infections (n=1), renal (n=1). Groups are mutually exclusive: TA and HST which could be categorised as oncology and another disease area (e.g., NSCLC can be classified as respiratory and oncology) were categorised as oncology.

Clinical evidence included in submissions

- 78% (63/81) of TA and HST relied on evidence from Phase 3 clinical trials.
- There were 3 TAs recommended based on evidence from a Phase 1/2 trial. These were either restricted by treatment line (1), orphan medicines (2), considered innovative by NICE (1), supported with RWE (2), or relied on funding within the CDF (2).[3-5]
- An HST gene therapy was recommended (following review as part of a MAA) based on RWE to estimate treatment benefit vs. BSC. The committee considered that the RWE presented more patient-relevant endpoints and longer-term data than the clinical trial data from the MAA.[6]

Key decision drivers

- Treatments recommended in a restricted indication were frequently critiqued for uncertainty in clinical data and CE estimates (Table 2).

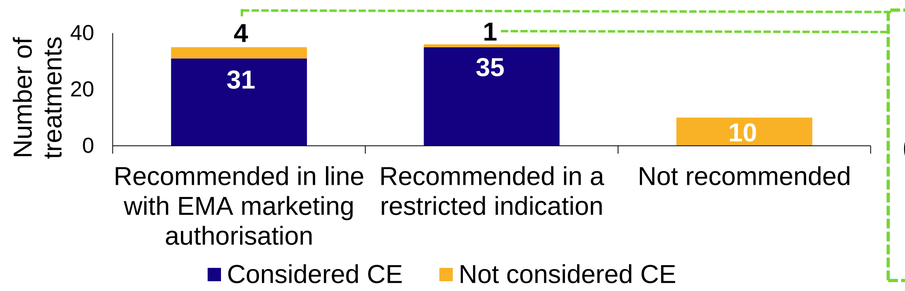
Table 2: Frequent critiques for treatments recommended in a restricted indication

Critique	Number of TA and HST (%)
Uncertainty in CE estimates	27 (75)
Uncertainty in clinical data[a]	25 (69)
Lack of direct comparative clinical effectiveness	21 (58)

Note: Percentages represent a proportion of the TA and HST recommended in a restricted indication (out of 36). [a]Uncertainty in long-term benefit, size of treatment benefit, or indirect treatment comparison.

- All 10 treatments that received a negative recommendation were critiqued for lack of CE, compared to 7% of recommended treatments (Figure 4).

Figure 4: Treatments considered cost-effective according to NICE recommendation



Conclusions

- The longer time to recommendation for HSTs reflects the complexity in value assessment within rare diseases, however, the recommendation using RWE over clinical data to determine treatment benefit demonstrates openness to accept innovative approaches for conditions facing data generation challenges.
- Data uncertainty was a key critique contributing to restricted indications. However, positive recommendations based on early-Phase studies suggest a willingness to accept uncertainty to accelerate patient access, with use of MAA and the CDF allowing access to treatments that do not meet HST criteria.
- Manufacturers should consider innovative data sources to optimise the evidence base, particularly for key CE model inputs, to reduce payer risk and increase the likelihood of unrestricted patient access.

Abbreviations: BSC: best supportive care; CDF: Cancer Drugs Fund; CE: cost-effectiveness; EMA: European Medicines Agency; GI: gastrointestinal; HST: highly specialised technology; HTA: health technology assessment; MAA: managed access agreement; MHRA: Medicines and Healthcare products Regulatory Agency; NICE: National Institute for Health and Care Excellence; NSCLC: non-small cell lung cancer; RWE: real-world evidence; SoC: standard of care; TA: technology appraisal; vs.: versus.

References: 1. TA943, NICE (2023). 2. TA934, NICE (2023). 3. TA927, NICE (2023). 4. TA911, NICE (2023). 5. TA872, NICE (2023). 6. HST22, NICE (2023). Included TA and HST are detailed in supplementary materials.

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