Trends Underlying Positive and Negative Decision-Making for New Oncology Treatments Appraised by NICE in 2023

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Introduction

- Oncology is a rapidly evolving therapeutic area: NICE published guidance on 81 treatments (TAs and HSTs) in 2023, with 41% in oncology.[1]
- With many new treatments and escalating costs, there is a need to effectively determine the best value for money in a crowded market.
- This research aimed to identify trends in decision-making for emerging oncology treatments in England.

Methods

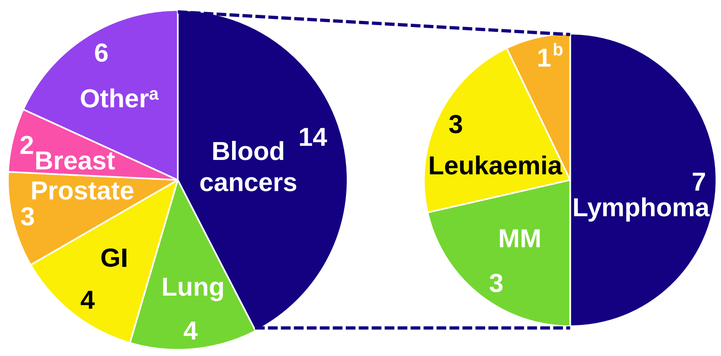
- NICE TAs and HSTs for oncology indications published in 2023 were identified. Terminated/withdrawn submissions were excluded.
- The EMA website was searched to identify marketing authorisation of the treatments assessed in the identified TAs and HSTs.
- Pre-defined topics, including NICE recommendation, clinical and economic evidence submissions, and decision drivers, were extracted from the TAs and HSTs, with 12% quality checked by a second reviewer.

Results

Overview of oncology indications and treatments

- NICE published 33 TA for oncology treatments in 2023. No HST in oncology indications were identified.
- TAs in blood cancers were most common, followed by lung cancer and GI cancers (Figure 1).

Figure 1: Number of TA in each cancer type



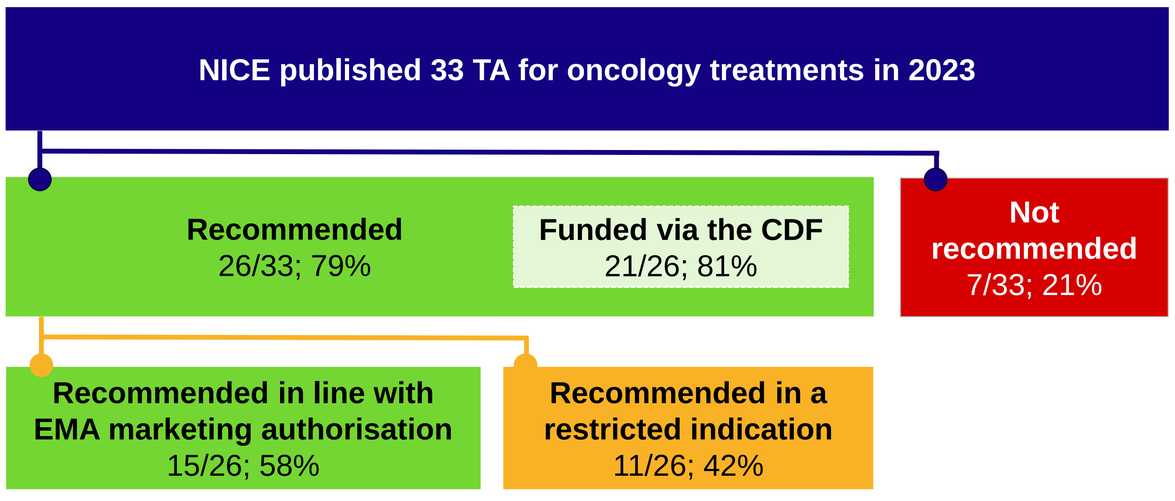
Note: [a]Cervical, endometrial, ovarian, renal, thyroid, and other/mixed (all n=1). [b]Polycythaemia vera.

- Most frequent treatments were monoclonal antibodies (12/33, 36%) or kinase inhibitors (10/33, 30%, groups not mutually exclusive).

NICE recommendations for oncology treatments

- 79% (26/33) of treatments received a positive recommendation (Figure 2).
- However, over 80% (21/26) of these positive recommendations required funding via the CDF and only 58% (15/26) were recommended in line with EMA marketing authorisation (Figure 2).

Figure 2: Outcomes for oncology treatments assessed by NICE in 2023



Restrictions compared to marketing authorisation

- Of the treatments recommended in a restricted indication (11 TA), most were restricted by treatment line or to a specific patient subgroup (Table 1).

Table 1: Indication restrictions applied compared to marketing authorisation

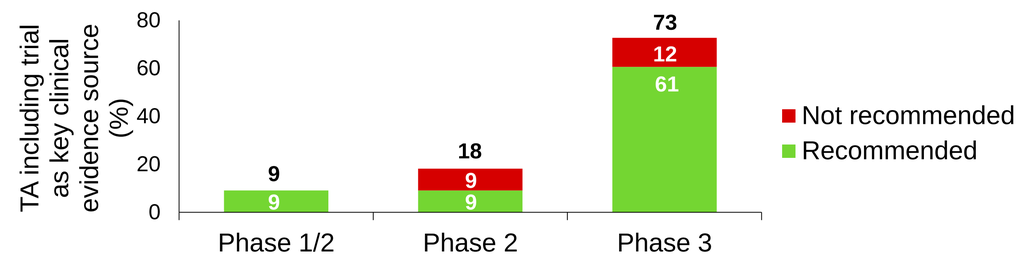
Restriction applied	Number of TA (%)
Treatment line	6 (55)
Patient subgroup[a]	6 (55)
Stopping rule	2 (18)
Only when a specific treatment would otherwise be offered[b]	1 (9)

Note: Groups not mutually exclusive. Percentages represent a proportion of the TA recommended in a restricted indication (out of 11). [a]Biomarker, prognostic index, treatment history/contraindications. [b]Intervention was only considered cost-effective compared to a specific alternative treatment.

Clinical evidence included in TA submissions

- Almost all submissions included Phase 3 clinical trial evidence (Figure 3).

Figure 3: Outcomes for TA according to Phase of clinical trials included in submission



Note: 4 TA included evidence from multiple clinical trials, including at least 1 Phase 3 trial. These have been included in the Phase 3 group. Percentages represent a proportion of total TA (out of 33).

- There were 3 (9%) submissions that relied on evidence from a Phase 1/2 clinical trial as the key clinical evidence source (Figure 3).
- All 3 were recommended and were either: restricted by treatment line, orphan medicines, considered innovative by the committee, supported with RWE, or relied on funding within the CDF (Table 2).

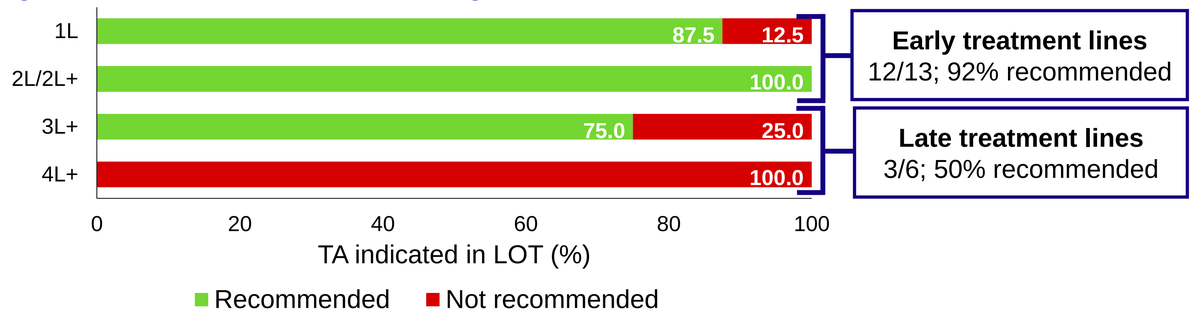
Table 2: Submissions including a Phase 1/2 clinical trial as key clinical evidence source

Indication	Restricted indication	Funded via CDF	Orphan medicine	Considered innovative	Supported with RWE
B-cell lymphoma[2]	✗	✓	✓	✗	✓
B-cell lymphoma[3]	✗	✗	✓	✗	✓
NSCLC[4]	✓	✓	✗	✓	✗

Recommendations according to LOT

- For TA specifying a LOT (19 TA), more early LOT treatments were recommended compared to those in later LOT, despite the committee's recognition of the unmet need in later LOT indications (Figure 4).

Figure 4: Outcomes for TA according to indicated LOT

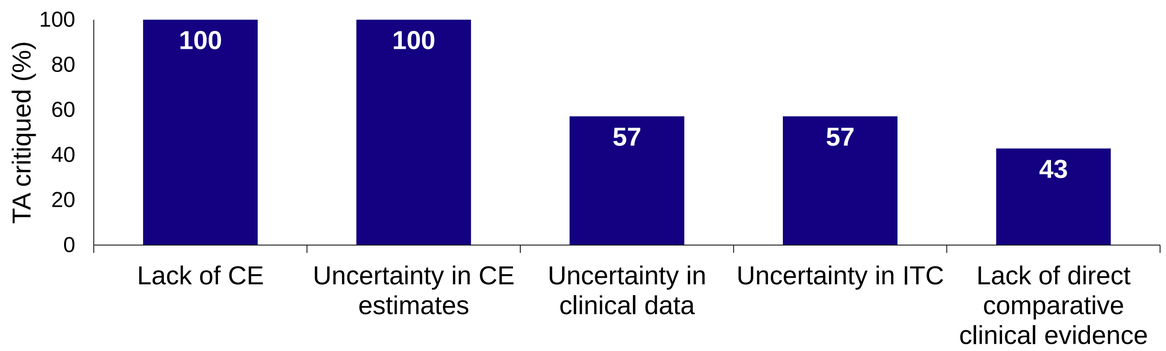


Note: Percentages represent a proportion of the TA indicated in each treatment line (1L: 8; 2L/2L+: 5; 3L+: 4; 4L+: 2).

Key drivers of negative recommendations

- Overall, negative recommendations (7 TA) were primarily driven by lack of CE and data uncertainty (Figure 5).

Figure 5: Key decision drivers for negative recommendations



Note: Percentages represent a proportion of the TA that received a negative recommendation (out of 7).

Conclusions

- England is a CE-driven market, and, unsurprisingly, lack of CE was a key driver in all negative decisions with data uncertainty impacting the reliability of CE estimates. Despite significant unmet need, price justification in later LOT indications can be difficult due to the challenge in demonstrating survival benefit.
- While most treatments received a positive recommendation, many faced indication restrictions which ultimately creates a barrier to patient access.
- Despite restrictions, early-Phase data is becoming more acceptable, and lack of direct comparative data is rarely a critique in negative decisions. Therefore, for treatments with clear patient benefit, earlier access is possible, and manufacturers should consider innovative approaches to mitigate data uncertainties.

Abbreviations: 1L: first line; 2L: second line; 3L: third line; 4L: fourth line; CDF: Cancer Drugs Fund; CE: cost-effectiveness; EMA: European Medicines Agency; GI: gastrointestinal; HST: highly specialised technology; ITC: indirect treatment comparison; LOT: line of therapy; MAA: managed access agreement; MM: multiple myeloma; NICE: National Institute for Health and Care Excellence; NSCLC: non-small cell lung cancer; RWE: real-world evidence; TA: technology appraisal.

References: 1. Stothard CA et al. Key Drivers Underlying Positive and Negative Decisions for NICE TAs and HST Appraisals in 2023. Poster presented at: ISPOR EU; November 2024; Barcelona. 2. TA927. NICE (2023). 3. TA872. NICE (2023). 4. TA911. NICE (2023). See supplementary materials for details of included TA.

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