Review of HTA Decisions for Oncology Treatments by NICE and the NCPE

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01. Objectives

Understanding the decision-making processes of Health Technology Assessment (HTA) agencies is crucial for developing successful submission strategies that maximise the likelihood of approval for new and innovative healthcare technologies. Oncology treatments face complex assessments due to their high costs and often limited long-term survival data at the time of appraisal.

This study aimed to comparatively analyse recent HTA decisions for oncology treatments made by two key agencies: the National Institute for Health and Care Excellence (NICE) in England and the National Centre for Pharmacoeconomics (NCPE) in Ireland. By examining the trends and differences in the recommendations provided by these agencies, the study aims to generate insights that can inform future strategic planning for market access and contribute to a more nuanced understanding of how oncology treatments are evaluated across different healthcare systems.

02. Overview of NICE and NCPE

The NCPE is an independent organisation commissioned by the Health Service Executive (HSE) to evaluate the clinical and cost-effectiveness of treatments seeking reimbursement in Ireland. NICE is an executive non-departmental public body sponsored by the Department of Health and Social Care in the United Kingdom (UK). It provides national guidance and advice aimed at improving health and social care. NICE plays a crucial role in the UK's healthcare system by ensuring that treatments, medical devices, and clinical practices are safe, effective, and cost-efficient.

NICE and NCPE conduct HTAs and provide evidence-based recommendations to promote cost-effective healthcare decisions, ensuring that treatments are clinically beneficial and economically viable for their healthcare systems. However, differences in healthcare structures, cost-effectiveness thresholds, local needs, and negotiation power can result in variations in their recommendations for new therapies despite their shared goal of evidence-based decision-making.

NCPE provides one of four possible recommendations for HTA:

- Reimbursement recommended
- Reimbursement is recommended if cost-effectiveness is improved
- Reimbursement is not recommended unless cost-effectiveness is improved
- Reimbursement not recommended

If cost-effectiveness improvements are required, the applicant enters price negotiations with the HSE. Based on the outcome of these negotiations, final recommendations are then made.

In contrast, NICE in England provides recommendations that differ slightly from those of the NCPE. NICE can provide one of five possible recommendations:

- Recommended
- Optimised
- Recommended for use in the Cancer Drugs Fund (CDF) – for cancer treatments only
- Only in research
- Not recommended

The outcomes 'recommended' and 'not recommended' are common in NICE and NCPE. However, the other outcomes (‘optimised,' 'recommended for use in the CDF’, and ‘only in research’) are specific to NICE and differ from those used by NCPE. These outcomes are explained below.

'Optimised' indicates that a drug or treatment is recommended for a smaller subset of patients than initially specified by the marketing authorisation, meaning the treatment is only cost-effective for a specific subgroup. ‘Recommended or optimised within CDF’ indicates that some treatments are recommended for inclusion in the CDF based on early evidence suggesting clinical benefits for cancer patients. However, further data collection is needed to reduce uncertainties in the case of cost-effectiveness.

CDF: Cancer Drugs Fund; HSE: Health Service Executive; NCPE: National Centre for Pharmacoeconomics; NICE: National Institute for Health and Care Excellence

03. Methods

The dataset pertaining to HTA submissions reported to the NCPE was extracted from the publicly available NCPE website and consolidated into a *Microsoft Excel* spreadsheet for further analysis. The dataset included a range of relevant variables, such as the HTA identification number, the name of the drug and its corresponding indication, the assessment status, and the date of assessment completed by the NCPE.

To ensure the focus remained on the most relevant oncology submissions, filters were applied to both the category and the submission date, thus including only oncology-related submissions reported after 2022. Following this filtering process, a total of 29 oncology-specific HTA submissions to the NCPE since 2022 were identified.

For each submission, the NCPE assessment outcome and date of negotiations (if applicable) were recorded. Of these, 16 treatments successfully achieved reimbursement. The NCPE has completed the assessments for the remaining 13 treatments, but the final decisions are still pending approval from the HSE.

In the next step, equivalent submissions for these 29 oncology treatments were located on the NICE website, where the same data points were extracted, including reference number, drug name, indication, submission date, and final outcome, allowing for a direct comparison between NICE and the NCPE assessments.

The analysis identified similarities as well as differences between the evaluation processes employed by NICE and the NCPE, particularly in terms of clinical evidence, cost-effectiveness thresholds, and patient access considerations.

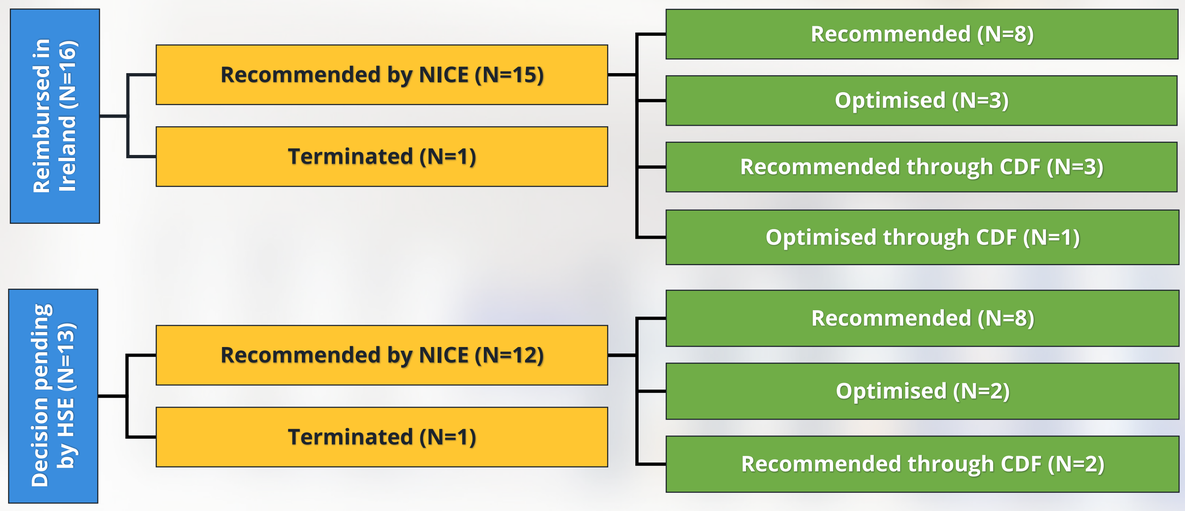
Additionally, a detailed comparison of the timelines for submissions that received positive reimbursement statuses from both NICE and the NCPE was conducted to understand the relative duration taken by each agency to issue its recommendations. Specifically, this involved analysing the date of negotiations recorded by the NCPE and comparing it to when NICE issued recommendations. This comparison helps to elucidate any differences in the speed and efficiency of the recommendation processes between the two agencies, providing insights into how each organisation manages and resolves reimbursement decisions for new treatments.

HSE: Health Service Executive; NCPE: National Centre for Pharmacoeconomics; NICE: National Institute for Health and Care Excellence

04. Results

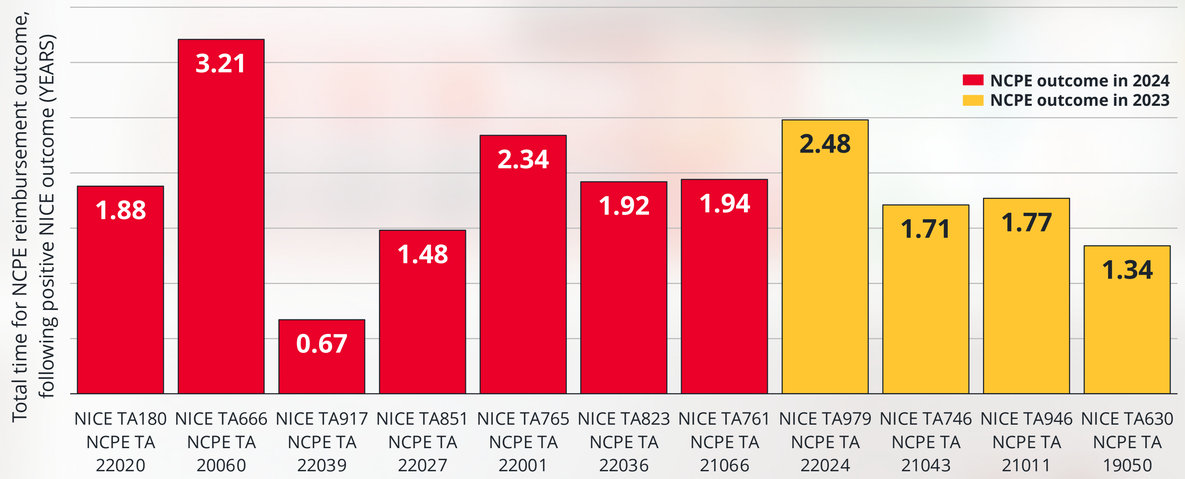
Of the 29 oncology HTA submissions that completed the NCPE assessment process, 16 were reimbursed. When comparing these to the equivalent NICE appraisals, 15 HTAs recommended in Ireland received a 'recommended' status from NICE, with three accessed through the CDF. Additionally, four HTAs reimbursed in Ireland received an 'optimised' status from NICE, while only one HTA achieved this status when including those assessed via the CDF (Figure 1). Notably, one HTA was recommended by the NCPE but not by NICE due to a termination by the company (Figure 1).

Figure 1: Overview of the results



A total of 11 submissions were reimbursed by both the NCPE and NICE (classified as either 'reimbursed' or 'optimised'). In all cases, NICE made its recommendations before the NCPE. The time taken by the NCPE to make a recommendation after NICE ranged from a minimum of 0.67 years to a maximum of 3.21 years, with an average delay of 1.88 years (Figure 2).

Figure 2: Additional time to reimbursement for NCPE compared to NICE



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05. Conclusions and Recommendations

The analysis highlights significant differences between NICE's and NCPE's reimbursement processes for oncology treatments. NICE employs a structured approach, utilising 'recommended' and 'optimised' statuses and mechanisms like the CDF, which manages uncertainties in the long-term survival outcomes for oncology treatments through real-world data collection. However, the primary goal remains to secure direct reimbursement without needing the CDF.

For both NICE and NCPE, leveraging RWE and external literature should be a priority to resolve uncertainties. However, while NICE has the CDF as an additional option for collecting long-term data, NCPE lacks such a mechanism. As such, it becomes even more critical for stakeholders to engage early with the NCPE informally during the submission process to discuss evidence requirements and address potential concerns early. Nonetheless, NCPE's process is generally characterised by longer decision timelines compared to NICE.

Based on this analysis, the following recommendations can be made to help stakeholders better navigate these differing processes and improve submission strategies for both agencies.

1. **Engage early with NCPE**. Given that NCPE’s decision timelines are significantly longer than NICE’s, engaging with the NCPE process by submitting a Rapid Review as early as possible, ideally during or shortly after CHMP approval is crucial. This proactive approach will help minimise delays and ensure timely patient access.
2. **Direct reimbursement should be prioritised in NICE submissions**. While the CDF offers a pathway for treatments with unresolved uncertainties, the goal should be to obtain full reimbursement without relying on it. Submissions should be designed to address clinical and economic uncertainties upfront and reduce the need for CDF involvement.
3. **Leverage alternative mechanisms for NCPE submissions**. In the absence of a structured mechanism like the CDF, stakeholders should utilise RWE, external literature, and indirect treatment comparisons to pre-emptively address potential concerns. Additionally, engaging informally with NCPE during submission can help clarify evidence requirements and mitigate issues early on.
4. **Encourage further research across therapeutic areas**. Additional research is needed across other therapeutic areas to understand if the patterns observed in oncology apply more broadly. This will help refine submission strategies for different conditions, ensuring they align with each agency's requirements.
5. **Monitor the long-term impact on patient access**. It is essential to continuously evaluate how the differences in NICE and NCPE processes affect patient access to innovative treatments. Monitoring timelines and access levels will provide valuable insights to guide future recommendations and inform potential policy changes.

By tailoring submission strategies to the specific frameworks of NICE and NCPE, stakeholders can enhance the likelihood of timely and successful reimbursement, ensuring earlier patient access to innovative treatments.

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