Insights into NICE Technical Appraisal Outcomes in Oncology

HTA19

Champsi S, Howard D, Ali A, Sus J

Amgen Limited, Cambridge, United Kingdom

Introduction

In January 2021, the Medicines and Healthcare products Regulatory Agency (MHRA) introduced the Innovative Licensing Access Pathway (ILAP), and in January 2022 the National Institute for Health and Care Excellence (NICE) replaced the end of life (EOL) criteria with a severity-based decision modifier. [1,2]

Objectives

This analysis aims to describe the outcomes of NICE oncology single technology appraisals (STAs), including those entering Cancer Drug Fund (CDF) managed access agreements (MAAs), following recent changes in regulatory and reimbursement processes.

Methods

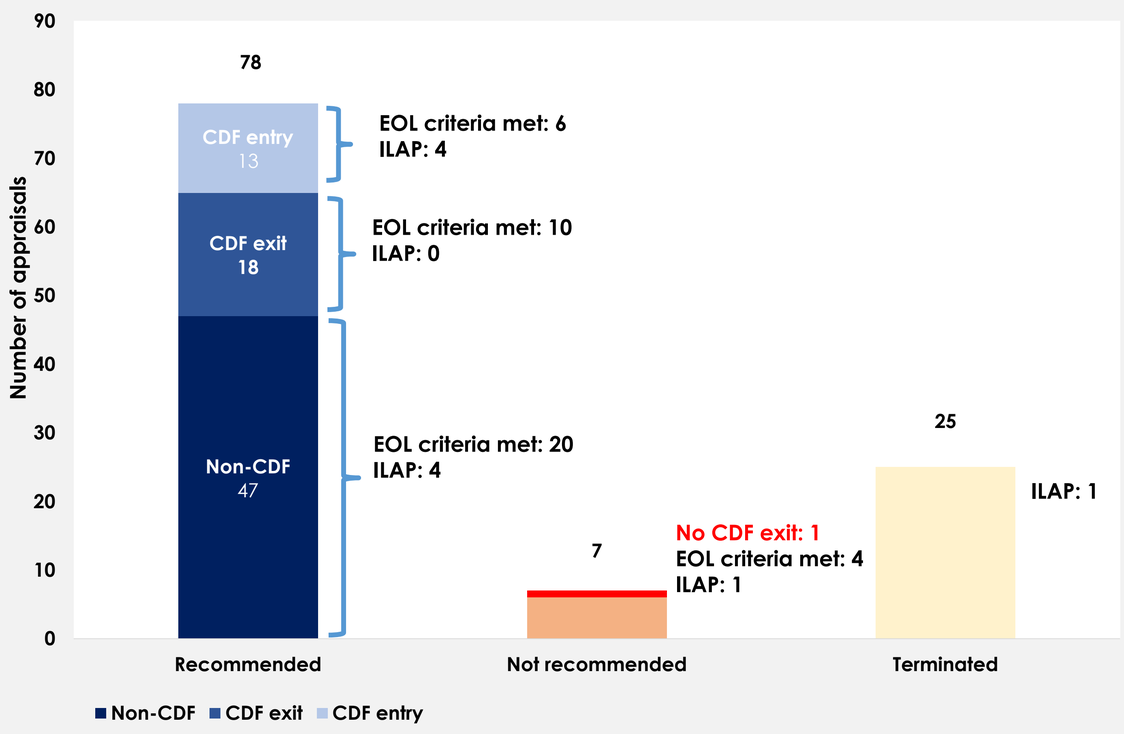
All oncology drug STAs from January 2021 to May 2023 were identified, and key information relating to reimbursement processes and decisions were extracted, including ILAP status, NICE recommendations, and EOL criteria.[3] Severity modifier was estimated for appraisals where standard of care quality-adjusted life years (QALYs) were unredacted.

Results

Oncology technical appraisal outcomes

A total of 110 published technology appraisals were reviewed. Sixty-five appraisals were recommended for routine commissioning (of which 18 exited the CDF) and 13 appraisals were recommended via the CDF. Seven appraisals were not recommended, one of which was previously recommended via the CDF, and 25 appraisals were terminated (Figure 1).

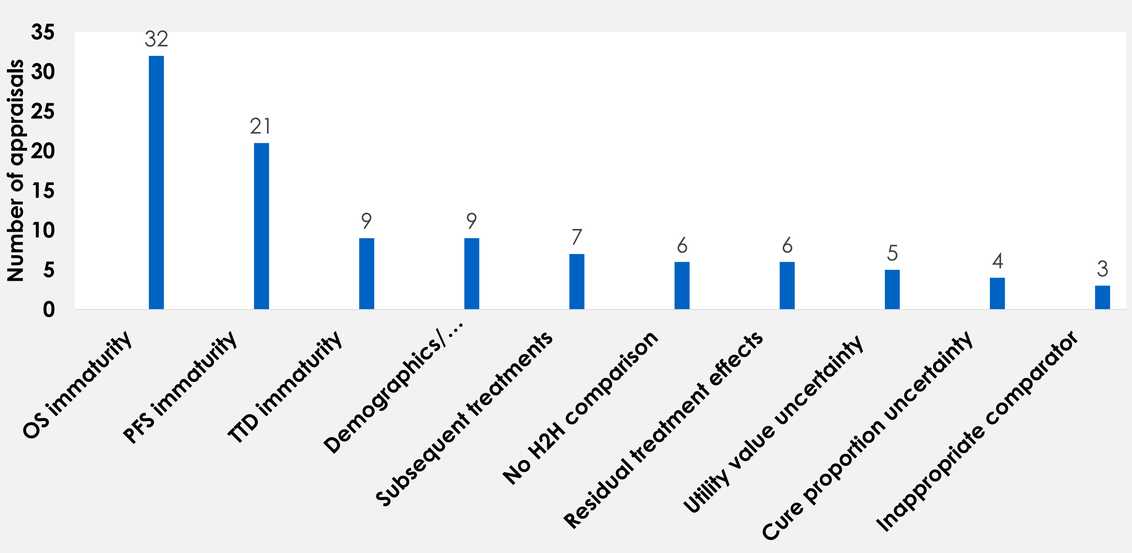
Figure 1: Outcomes of NICE appraisals for oncology drugs from January 2021-May 2023



CDF uncertainties in managed access agreements (MAAs)

Amongst appraisals that entered into CDF MAAs, the most frequently cited uncertainties are related to immaturity of trial data, representativeness of trial data to the UK population, followed by issues with uncertainties around subsequent treatments (Figure 2).

Figure 2: Frequency of main CDF uncertainties described in managed access agreements



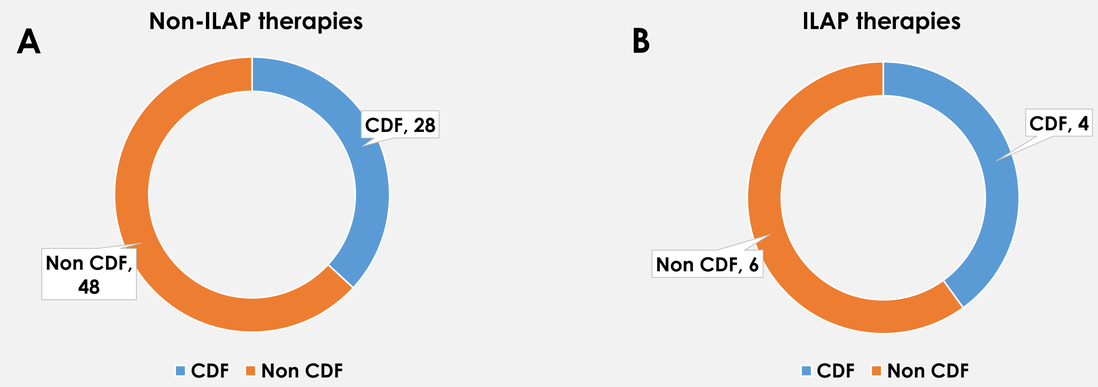
100% (n=32) of appraisals that entered into CDF MAAs cited overall survival as an area of clinical uncertainty

Abbreviations: *OS, overall survival; PFS, progression-free survival; TTD, time to treatment discontinuation; H2H, head-to-head*

Potential impact of ILAP process on CDF entry

Since the introduction of the ILAP process in January 2021, 10 oncology drugs were approved as ILAP therapies. For non-ILAP therapies (Figure 3A), the proportion of TAs which entered the CDF was similar to that for ILAP therapies (Figure 3B); 36.8% (28/77) versus 40% (4/10) respectively, although the ILAP sample size was small.

Figure 3: CDF entry status for non-ILAP versus ILAP therapies

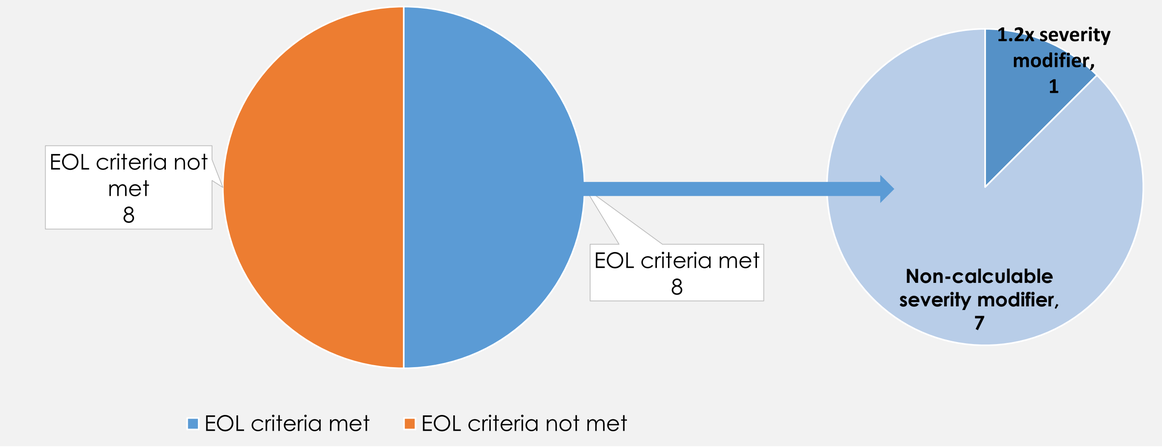


Replacement of EOL criteria with a severity-based decision modifier: implications for CDF appraisals (February 2022 to May 2023)

Since the NICE Methods update in 2022, 16 appraisals entered the CDF, of which half met the EOL criteria (Figure 4). While these oncology therapies are expected to exit the CDF in upcoming years, it is unclear under which threshold they will exit as severity modifiers were not estimable for most appraisals due to commercial-in-confidence (CIC) or academic-in-confidence (AIC) redactions. Of the 8 appraisals which met the end-of-life criteria, only one appraisal had a calculable severity modifier (a severity modifier of 1.2) (Figure 5).

Figure 4: End-of-life criteria status for treatments which entered the CDF between February 2022 - May 2023

Figure 5: Severity-modifier status for treatments that entered CDF with end-of-life criteria



Conclusions

This study identified ten main uncertainties cited by NICE in CDF MAAs, with the most-frequently-cited uncertainties being immaturity of OS and PFS data. Earlier routes to regulatory approval (i.e. ILAP) which are likely to rely on less-mature clinical data for their HTA submissions are likely to be subject to many of the identified uncertainties. It seems logical that this may increase the probability of ILAP treatments entering the CDF rather than baseline commissioning. However, the small number of oncology ILAP appraisals identified within the time horizon (n=10) means it is not possible to draw conclusions about the relationship between ILAP status and CDF entry (Figure 3B). Further research is recommended as more oncology treatments are granted ILAP status.

The impact of the introduction of the disease severity modifier could not be evaluated due to the large amount of CIC and AIC redactions of QALY data present in most NICE submissions; of the 8 CDF HTA submissions that met the EOL criteria, a severity modifier was only calculable for 1 of them. To better understand the impact of disease severity modifiers on NICE appraisal outcomes, a new approach should be considered on how we can transparently assess consistency in how modifiers are being calculated across new treatments and their impact on the cost-effectiveness threshold.

The full impact of changes to the UK regulatory and reimbursement landscape remains uncertain. Redactions in appraisals make it difficult to ascertain near-term implications of ILAP and NICE methods update, as ILAP status was not readily available for terminated appraisals (and therefore may be underestimated in our analysis) and severity modifiers were not estimable for most appraisals. It therefore remains unclear whether drugs that entered CDF with EOL criteria would exit with a similar threshold.

References

1. UK Government (2021). *Innovative Licensing and Access Pathway*. Available at: https://www.gov.uk/guidance/innovative-licensing-and-access-pathway (Accessed June 2023)
2. National Institute for Health and Clinical Excellence (2022). *NICE Health Technology Evaluations: The Manual.* Available at: https://www.nice.org.uk/process/pmg36/resources/nice-health-technology-evaluations-the-manual-pdf-72286779244741 (Accessed October 2023)
3. National Institute for Health and Clinical Excellence (2022). *Guidance, NICE advice and quality standards.* Available at: https://www.nice.org.uk/guidance/published?ngt=Technology%20appraisal%20guidance&ndt=Guidance&ps=9999 (Accessed May 2023)

Disclosures: This study was funded by Amgen Ltd. SC, DH and JS are employees of Amgen and hold Amgen shares