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Recent trends from the UK's Cancer Drugs Fund—key drivers of acceptance

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Introduction

With accelerated regulatory pathways, many products now undergo health technology assessment (HTA) evaluation with immature or single-arm study data.

Managed entry agreements (MEAs) provide an important access route for such treatments where there is uncertainty in the clinical- or cost-effectiveness of products for conditions with the highest unmet need.

The Cancer Drugs Fund (CDF) is one of the two dedicated sources of funding for MEAs in the UK and allows for a temporary period of reimbursement while additional data are collected to address the key uncertainties in the data package (Table 1). 73% of products reimbursed initially via the CDF are subsequently recommended for routine reimbursement once the MEA period has ended,[1] highlighting the importance of this route for oncology products with immature data packages.

In recent years, there has been a growing disconnect between regulatory and HTA decisions, where products with expedited approval are being rejected for reimbursement due to uncertainties in the data package.[2]

Table 1: Overview of the UK's Cancer Drugs Fund

Annual budget	£340 million
Timeframe	The shortest period possible to address the uncertainties in the data package, but no more than five years
Key criteria	• A new oncology product cannot be recommended as the evidence is deemed too uncertain • Has the potential to be cost effective at the currently agreed price if new evidence is provided from ongoing or planned clinical trials or could be collected from patients having the medicine in clinical practice • These data could feasibly be collected within a reasonable timeframe (ie, within five years)
Number of products funded	58 (since 2000)

Methods

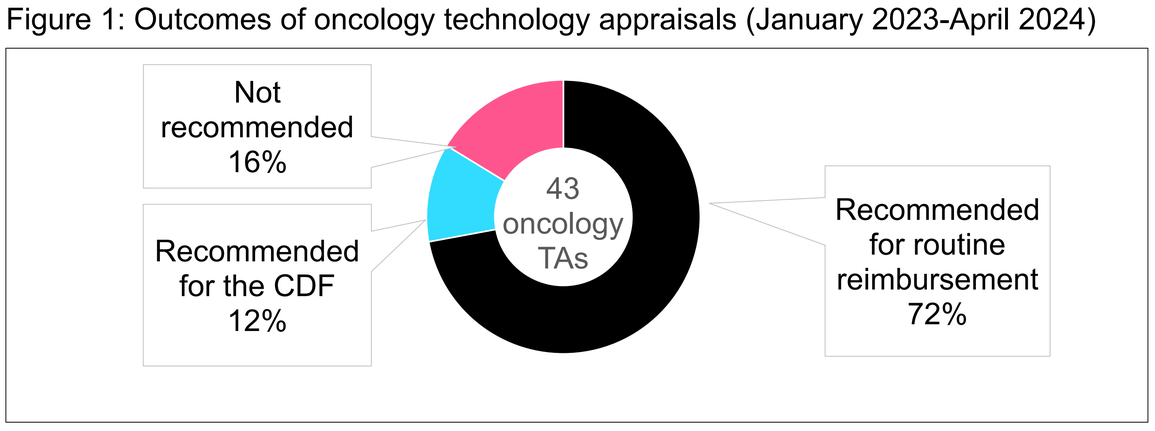
To understand the key requirements to gain access to the CDF, we undertook a review of recent National Institute for Health and Care Excellence (NICE) technology appraisals, from January 2023 to April 2024.[5]

Appraisals were categorized into recommended for routine reimbursement, recommended for the CDF, and not recommended. For those not recommended for the CDF and not recommended, appraisal reports were screened for commentary on the reasons for the decision. Terminated appraisals were not included in the analysis.

Results

From January 2023 to April 2024, we identified 43 completed oncology appraisals, 31 of which were recommended for routine reimbursement. Of the remaining appraisals, five were accepted into the CDF and six were not accepted. One appraisal was not recommended without any accompanying commentary on the CDF (Figure 1; Table 2).

Figure 1: Outcomes of oncology technology appraisals (January 2023-April 2024)



CDF, Cancer Drugs Fund; TA, technology appraisal.

Table 2: Assessments considered for the CDF

Appraisal Name	ID	Recommended for the CDF
Dostarlimab with platinum-based chemotherapy for treating advanced or recurrent endometrial cancer with high microsatellite instability or mismatch repair deficiency	TA963	Yes
Lutetium-177 vipivotide tetraxetan for treating PSMA-positive hormone-relapsed metastatic prostate cancer after two or more treatments	TA930	No
Cabozantinib for previously treated advanced differentiated thyroid cancer unsuitable for or refractory to radioactive iodine	TA928	No
Selpercatinib for untreated RET fusion-positive advanced non–small-cell lung cancer	TA911	Yes
Lorlatinib for untreated ALK-positive advanced non–small-cell lung cancer	TA909	No
Axicabtagene ciloleucel for treating relapsed or refractory follicular lymphoma	TA894	No
Axicabtagene ciloleucel for treating relapsed or refractory diffuse large B-cell lymphoma after first-line chemoimmunotherapy	TA895	Yes
Brexucabtagene autoleucel for treating relapsed or refractory B-cell acute lymphoblastic leukemia in people 26 years and above	TA893	Yes
Mosunetuzumab for treating relapsed or refractory follicular lymphoma	TA892	No
Tafasitamab with lenalidomide for treating relapsed or refractory diffuse large B-cell lymphoma	TA883	No
Trastuzumab deruxtecan for treating HER2-positive unresectable or metastatic breast cancer after one or more anti-HER2 treatments	TA862	Yes

ALK; anaplastic lymphoma kinase; CDF, Cancer Drugs Fund; HER2, human epidermal growth factor receptor 2; PSMA, prostate-specific membrane antigen; RET, receptor tyrosine kinase; TA, technology appraisal.

Having a single-arm trial as the evidence base was not a major driver of entry into the CDF: Of the five assessments that were recommended for the CDF, two did not provide head-to-head data, whereas three of the six that were rejected did not provide direct comparative data (Table 3).

For such products without head-to-head data, acceptance into the CDF was typically contingent on upcoming Phase 3 trials being planned that could address the uncertainty generated through filling based on a single-arm study alone (Table 3).

Conversely, having no planned comparative data or only providing comparative data against products that were not relevant to UK practice were the main drivers of rejection (Table 3).

Table 3: Key drivers for the CDF recommendation

ID	Recommended for the CDF	Head-to-head data provided	Trial comparator considered relevant	Further data cut planned	Phase 3 trial planned
TA963	Yes	X	X	X	
TA911	Yes		N/A	X	X
TA895	Yes	X	X	X	
TA893	Yes		N/A	X	
TA862	Yes	X	X	X	
TA930	No	X			
TA928	No	X	X		
TA909	No	X		X	
TA894	No		N/A	X	X
TA892	No		N/A	X	
TA883	No		N/A	X	

CDF, Cancer Drugs Fund; TA, technology appraisal.

Interestingly, one assessment (TA893) was recommended for the CDF based on a single-arm study, without a confirmatory Phase 3 trial planned. Crucially, evidence from a later data cut was considered likely to sufficiently address uncertainty up to a point where the product could be considered cost effective.

For all appraisals not recommended for the CDF, NICE commented that no plausible scenarios existed where the cost per quality adjusted life year (QALY) gained could be considered as cost effective.

Discussion

To enhance access to innovative medicines for patients with the highest unmet needs, there have been an increase in products gaining regulatory approval based on single-arm or immature studies, particularly in oncology.[6,7] Single-arm studies or immature data inevitably introduce additional uncertainty into cost-effectiveness estimates, which can decrease the likelihood of NICE recommending a treatment for reimbursement.

Overall, despite a trend of an increase in products seeking reimbursement based on the single-arm studies,[2] there does not appear to have been an overall change in the rate of oncology products gaining entry into the CDF. Since 2000, 12% of oncology appraisals have been recommended for the CDF and 19% have not been recommended,[1] which are in line with the most recent rates (Figure 1).

Despite the increase in uncertainty, applying for reimbursement based on a single-arm trial did not appear to be a major driver of inclusion into the CDF. Instead, having a data generation plan with comparative data versus locally relevant comparators is crucial for products launching with single-arm or immature data.

This analysis also suggests that manufacturers that may be targeting temporary reimbursement via the CDF are still required to approach assessment with a costing strategy that allows NICE to recognize the cost-effectiveness of their product.

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Poster presented at ISPOR EU, 17–20 November 2024, Barcelona, Spain