NICE National Institute for Health and Care Excellence

HTA241

Rare Diseases: The role of Managed Access

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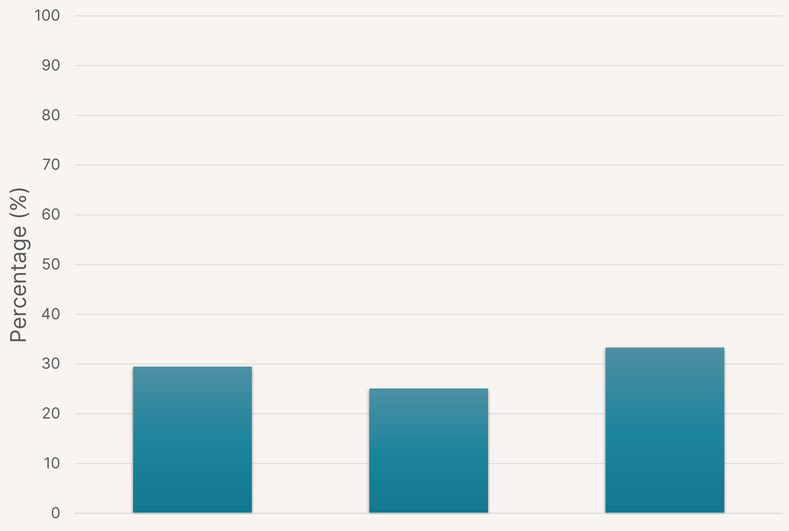
Background

NICE evaluates new active substances; since 2016 the Cancer Drugs Fund (CDF) has supported earlier access to promising cancer treatments, whilst further data is collected to address evidential uncertainties[1]. The Innovative Medicines Fund (IMF) extended this managed access (MA) approach to non-cancer. Previous analysis undertaken by NICE assessed the impact of the CDF on the appraisal of treatments for rare cancers and demonstrated that with the CDF there was an increase in the number of appraisals for rare cancers, and in the proportion resulting in a positive recommendation[2]. We present a comparative analysis of recommendations by rarity of disease to provide insight about appraisal of all rare diseases by NICE (Jan 2021-April 22) including cancer and other conditions.

Methods

Recommendations from NICE's technology appraisal (TA) and highly specialised technology (HST) programmes were reviewed from January 2021-April 2022 . Rare cancers were defined as ≤6 cases per 100,000/year[3] and rare non-cancer conditions as ≤ 1 per 2000[4]. Recommendations and publication dates were extracted. For the final analysis we removed appraisals that had been terminated because the manufacturer did not submit evidence and recommendation updates.

Figure 1: Percentage of topics reviewed which are 'rare'



Results

There were 102 appraisals producing 109 recommendations: 47.7% for cancers. Overall, 29.4% of all conditions appraised by both programmes were 'rare': 25.0% of cancers and 33.3% of other conditions. Overall, 90.6% of all rare conditions were recommended for reimbursement (i.e., routine commissioning or with managed access) compared with 88.3% for non-rare.

92.3% of rare cancers received reimbursement recommendations compared with 89.7% of non-rare cancers. For conditions other than cancer, 89.5% of rare conditions received reimbursement recommendations and 86.8% for non-rare conditions. The HST programme reviews treatments for very rare conditions, six of these 'other' conditions were reviewed by HST and all received reimbursement recommendations.
Of those cancer appraisals recommended for reimbursement, 16.7% of rare cancers were recommended with MA compared with 22.9% for non-rare.

Figure 2: Recommendations for rare vs non-rare topics overall (cancer and other)

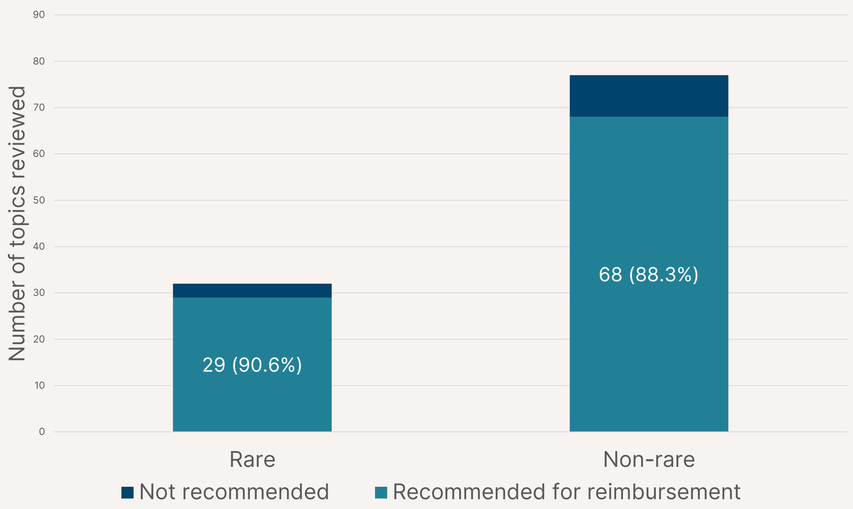
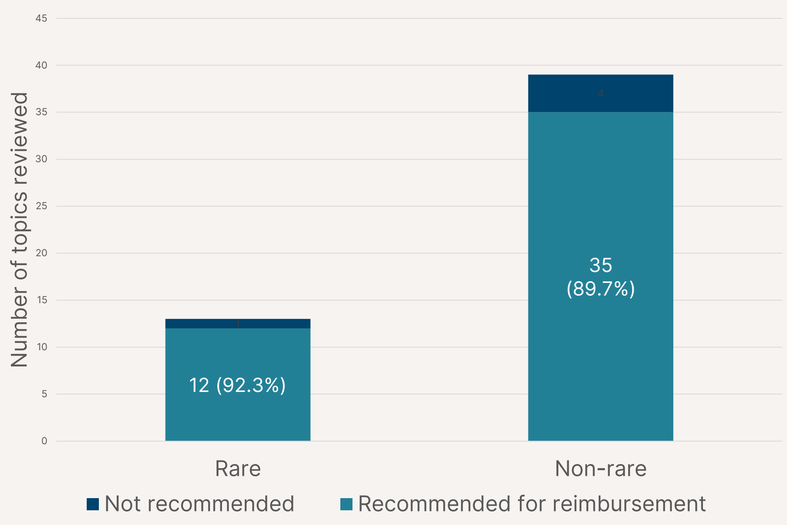


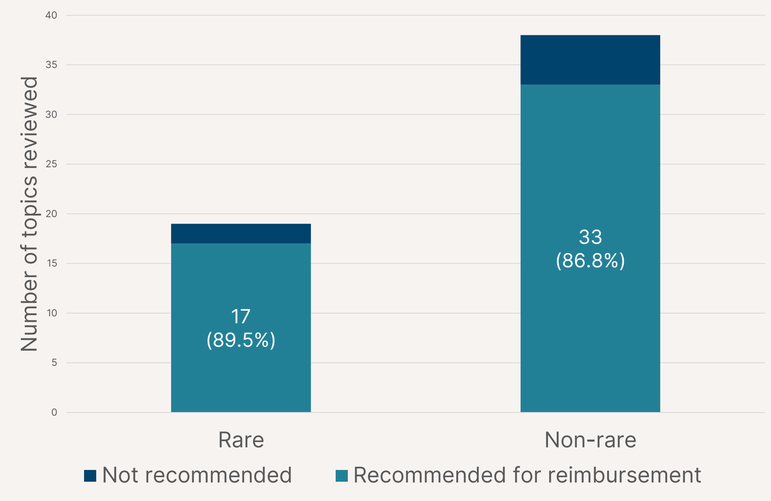
Figure 3: Recommendations for rare vs non-rare cancer topics



Conclusions

This review has found that rare conditions (cancer and others) are more likely to be recommended for routine use compared to non-rare conditions. Recommendations with managed access provide an invaluable secondary route to reimbursed patient access where there is significant evidential uncertainty. Managed access has provided a reimbursement mechanism for cancer drugs through the CDF which allows access to promising drugs for both rare and other cancers. The IMF now provides this route for other rare and non-rare conditions.

Figure 4: Recommendations for rare vs non-rare 'non-cancer' topics



The Cancer Drugs Fund and the Innovative Medicines Fund

- An independent NICE Appraisal committee will assess the available evidence for a particular drug. Where there are key uncertainties about the clinical effectiveness of a particular drug, the committee may recommend that it is made available via the Cancer Drugs Fund or Innovative Medicines Fund.
- It must be plausible that the drug is cost-effective and that further data collection will address the clinical uncertainties in the evidence.
- This enables access to promising new drugs with a managed access agreement containing a data collection arrangement and a commercial agreement.

References

1. NHS England (2016) Appraisal and funding of cancer drugs from July 2016
2 Edwards, H.K. et al. PCN239 - NICE and the cancer drugs fund: appraising treatments for rarer cancers in England. Value in Health, Volume 21, S54
3. RARECARE, Rare cancer list http://www.rarecare.eu/rarecancers/rarecancers.asp Accessed 31/05/2022
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