NICE National Institute for Health and Care Excellence

Utility of Early Access to Medicines Scheme data for NICE decision-making

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Background

The Early Access to Medicines Scheme (EAMS) gives patients in the UK with life threatening or seriously debilitating conditions early access to new medicines that do not yet have a marketing authorisation, when there is clear unmet clinical need. The primary benefit of EAMS is regarded as early patient access to new medicines, with a secondary benefit being potential opportunities for real world data collection to support market access.

What we did and why

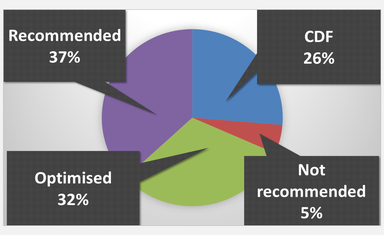
The UK Office for Life Sciences conducted an industry survey on EAMS which showed that most pharmaceutical companies considered EAMS to be a useful vehicle to collect real-world evidence but that the perceived value of this data for NICE HTA was uncertain.

NICE guidance on EAMS medicines was therefore reviewed to assess whether data collected during the EAMS period would have made a difference to NICE decisions where these were not recommended, optimised[1], or recommended in the Cancer Drugs Fund (CDF)[2].

[1]Recommendations are optimised if the technology is recommended for a smaller group of patients than originally stated by the marketing authorisation in the UK

[2]New treatments can be recommended for use in the CDF if there is early evidence that a drug has clinical benefits for cancer patients but still needs more evidence to prove its cost-effectiveness

To date, NICE has appraised 19 EAMS medicines; 18 via the single technology appraisal process and 1 through highly specialised technology evaluation. Only 1 of these was not recommended; 7 were recommended and 11 were recommended with restrictions (either optimised or in CDF):



What we found

Analysis showed that EAMS data would not have significantly impacted the overall NICE decisions:

Decision	Rationale	Could EAMS data have made a difference?
Not recommended (n=1)	No reliable cost-effectiveness estimate	Further data unlikely to make a material difference because of fundamental issues with the structure of the economic model.
Optimised: cancer drugs (n=4)	2 year stopping rules for immunotherapies Evidence not presented for a subgroup	EAMS data collection periods too short to produce data on treatment duration beyond 2 years. EAMS data unlikely to make a difference where evidence has not been submitted to make a case for reimbursement.
Optimised: non-cancer drugs (n=2)	To reflect NHS treatment pathway	How treatment is used during the EAMS period is likely to reflect how it would be used in the NHS and therefore would not provide data on its use in line with the full marketing authorisation.
CDF (n=5)	Data needed on: •Long term survival •Treatment duration •Relative benefit estimates •Subgroups •Patients more reflective of those seen in NHS	EAMS data collection periods too short to produce data on long term survival and treatment duration. Comparative data cannot be collected during EAMS. However, real world evidence from the EAMS data collection period may provide useful data on baseline characteristics for specific groups of patients (such as defined subgroups, or patients more reflective of those seen in the NHS compared with those in the clinical trials), and HRQOL benefit.

What we learnt

The analysis shows that data collected during the EAMS period would not have made a significant difference to negative, optimised or CDF decisions about EAMS medicines. It therefore appears that overall, EAMS data collection is unlikely to have a direct impact on HTA recommendations, based on the way the scheme runs currently. However, EAMS data could provide important information to support committee decision-making. For example, it could provide data to validate economic modelling assumptions or the generalisability of trial findings, or to supplement the trial information used in HTA (for example, HRQOL data). This could potentially reduce uncertainty in some appraisal decisions and thereby enable more accurate estimates of cost-effectiveness.

Abbreviations
EAMS Early Access to Medicines Scheme
HTA Health Technology Assessment
CDF Cancer Drugs Fund
HRQOL Health-related Quality of Life

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