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Examining the impact of the Early Access to Medicines Scheme (EAMS) on the National Institute for Health and Care Excellence (NICE) recommendations over the past 4 years

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Background

The primary objective of the United Kingdom (UK) Early Access to Medicines Scheme (EAMS), which was launched in 2014/2015, is to provide patients suffering from life-threatening or severely debilitating conditions access to medicines that have not yet received marketing authorization but address a clear unmet medical need. Within the EAMS framework, the Medicines and Healthcare Products Regulatory Agency (MHRA) evaluates the benefit-risk profile of the medicine by issuing a scientific opinion, considering the available data at the time of EAMS submission.

During the EAMS, the product is supplied at no cost from the start of the scientific opinion until the marketing authorization is granted. Patients who initiated treatment during the EAMS period will continue to receive the product free of charge until a positive recommendation from the National Institute for Health and Care Excellence (NICE) is issued.

Objective

Analysis of EAMS products and their NICE appraisal outcomes over the past 4 years.

Methods

Analyze all products for which the EAMS scientific opinion[1] expired between January 2020 and December 2023 and the corresponding NICE recommendation[2], if available.

Results

Of the 25 EAMS products, the average EAMS period was 8.7 months (range: 1.2–39.7 months). This is twice as long as the average for EAMS products between 2015 to 2019 (average: 3.8 months).

Of the 25 EAMS products, 60% (N=15) were oncology treatments. Among the 19 EAMS products that underwent a single technology (N=18) or highly specialized technology (N=1) appraisal by the NICE, approximately 58% (11) received a positive recommendation, while 32% (6) were optimized, and 11% (2) were not recommended. In contrast, of the 232 non-EAMS products appraised by NICE from 2020 to 2023, 43% (99) were positive, 48% (111) were optimized, and 9% (22) were not recommended.

Out of the 19 EAMS products that underwent a NICE appraisal, 11 (58%) of these appraisals cited qualitative and/or quantitative data collected during the EAMS program, which was considered by NICE. The data was submitted by the company and/or other stakeholders, including treating clinicians. The cited qualitative/quantitative data included the following findings:

- Uptake supports unmet need
- Use supports product positioning
- Clinical benefits were observed shortly after administration
- Clinicians who have experience with the product through EAMS can help train other clinicians
- Observed efficacy and safety data validate the benefits demonstrated in the trials
- Because of EAMS, more centers are treating patients, which reduces the time to diagnosis
- Confirmed infusion time
- Cost data collected during EAMS were included in the health economic models

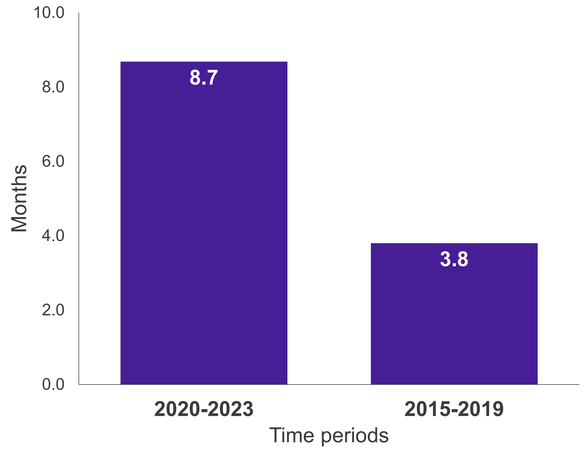
Conclusions

While the duration of EAMS has doubled over the past 4 years, it remains uncertain whether this is a broader trend or if it was influenced by COVID-19-related delays.

The duration of EAMS is also highly variable, ranging from just over 1 month to over 1.5 years. However, when excluding 3 products—raxone, cipaglucosidase alfa, and avalglucosidase alfa—from this analysis, all remaining 22 products (88%) were available for ≤12 months, thereby drastically reducing variability regarding the length of EAMS. The reason for the long EAMS periods in the case of raxone and cipaglucosidase alfa was due to delays with the NICE appraisals.

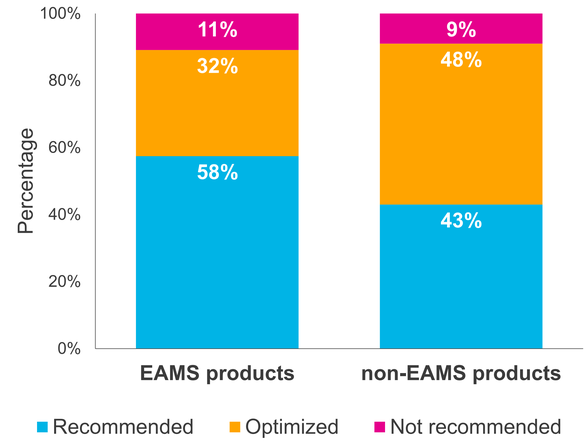
This analysis also reveals that EAMS products are more likely to receive a positive NICE recommendation (58% vs 43%) than products appraised via standard pathways. Evidence collected during EAMS can support a product's value proposition, even if the data are only qualitative in nature. Consequently, when preparing for EAMS, it is crucial for manufacturers to thoroughly assess the data that can be collected to address uncertainties.

Figure 1. Average EAMS length (months)



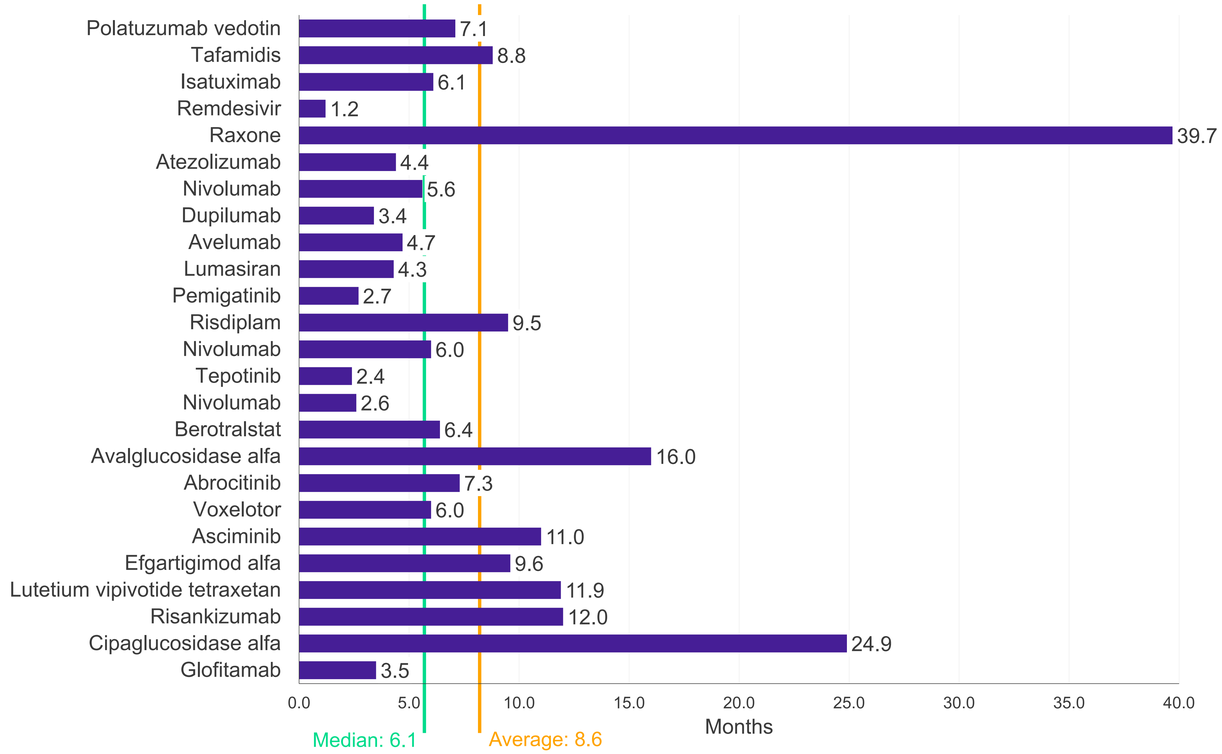
Key: EAMS – Early Access to Medicines Scheme.

Figure 2. NICE appraisal outcomes



Key: EAMS – Early Access to Medicines Scheme; NICE – National Institute for Health and Care Excellence.

Figure 3. EAMS length per product between 2020-2023



Key: EAMS – Early Access to Medicines Scheme.

References: 1. Medicines and Healthcare products Regulatory Agency (MHRA). Apply for the early access to medicines scheme (EAMS). Page last updated April 18, 2023. Accessed January 2024. https://www.gov.uk/guidance/apply-for-the-early-access-to-medicines-scheme-eams **2.** National Institute for Health and Care Excellence (NICE). Guidance, NICE advice, and quality standards. Accessed January 2024. https://www.nice.org.uk/guidance/published?sp=on

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