Medicines & Healthcare products Regulatory Agency

Board Meeting – public session

The Early Access to Medicines Scheme (EAMS) – 5-years on

15 April 2019

Issue/ Purpose: Update to the Board at the 5-year milestone for EAMS
Summary: The UK Early Access to Medicines Scheme (EAMS) gives patients with life threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorisation when there is an unmet medical need. Unmet medical need means a condition for which there exists no satisfactory method of treatment or, if such a method exists, the medicinal product will be of major therapeutic advantage to those affected. The MHRA is responsible for the scientific aspects of the scheme and a benefit risk scientific opinion is issued after a two-step evaluation process; step I, the Promising Innovative Medicine (PIM) designation and step II, the Early Access to Medicines Scheme scientific opinion. Since launch in April 2014, over 1500 patients have been treated with EAMS medicines in a variety of conditions.
Resource implications: Existing resource
EU Referendum implications: N/A
Implications for patients and the public: EAMS offers a route to medicines before they are licensed, in areas of unmet medical need
Timings: Ongoing
Action required by Board: For information
Links: https://www.gov.uk/guidance/apply-for-the-early-access-to-medicines-scheme-eams
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Which of the themes in the Corporate Plan 2018/2023 does the paper support? 2a: We will support innovation and growth in Life Sciences 2a(ii) further enhance the Early Access to Medicines Scheme (EAMS)
CET Sponsor: Dr Siu Ping Lam

UK Early Access to Medicines Scheme (EAMS) – 5-years on

Promising Innovative Medicine (PIM) designation – Step I
A Promising Innovative Medicine Designation is an early indication that a medicinal product is a potential candidate for the EAMS, intended for the treatment, diagnosis or prevention of a life-threatening or seriously debilitating condition, with the potential to address an unmet medical need. The designation is issued to a new chemical or biological entity or for a new indication for an established medicine in a defined disease area after a MHRA designation scientific meeting. Applicants may apply from early stages in a clinical development programme. Since launch (April 2014) there have been 97 applications, 70 granted, 17 refused, 4 withdrawn and 6 pending (at the time of this report). The PIM designation gives a company reassurance that its clinical development is on 'track' by having an early review of its data by the UK medicines regulator, and the grant letter contains specific NHS/HTA contacts in the UK nations, facilitating opportunities to engage earlier with other stakeholders on patient access issues.

Early Access to Medicines Scheme Scientific Opinion - Step II
The scientific opinion describes the risks and benefits of using the medicine. The opinion supports the prescriber and patient to make a decision on whether to use the medicine before its licence is approved. A positive scientific opinion is only issued if the criteria for the EAMS are fulfilled. The positive scientific opinion is published on the MHRA webpage alongside a public assessment report and the EAMS Treatment Protocols (provides more detailed information to the patient and the physician). The opinion is valid for one year and lapses at this time or at the time of the grant of a marketing authorisation. Since launch there have been 33 applications, 23 opinions awarded, 3 refused, 4 withdrawn and 3 pending at the time of this report. Opinions have been issued in a variety of conditions, examples include Duchenne Muscular Dystrophy, severe atopic dermatitis, different cancers (e.g. breast, lung, kidney, bladder, melanoma), heart failure, amyloidosis, haemophilia A and chronic hepatitis.

EAMS publications
There have been a number of publications on EAMS including an MHRA case study, a comparison between the PIM designation and the EMA's PRIME designation and an independent review commissioned by the government.

https://www.gov.uk/government/case-studies/innovation-over-500-uk-patients-gain-early-access-to-new-skin-cancer-treatment
https://www.gov.uk/government/publications/independent-review-of-early-access-to-medicines-scheme-eams
https://journals.sagepub.com/doi/full/10.5301/maapoc.0000009
https://embed.topra.org/sites/default/files/regrapart/1/6579/2_focus_-_real_world_experience_of_prime_and_pim_applications.pdf

Implications for patients and the public

EAMS and medicines access
Over 1500 patients have been treated by EAMS medicines in a variety of conditions and EAMS has been described as a world-leading example of how healthcare agencies and industry can work together to get treatments to patients more quickly (see innovation case study).

EAMS and patient engagement
During the renewal procedural for an EAMS medicines to treat boys with Duchene Muscular Dystrophy, a Patient Group Meeting was held to gain a fuller understanding of the patient perspective. The patient meeting was attended by Duchenne Muscular Dystrophy patients, patient representatives, Commission on Human Medicines (CHM) Commissioners and representatives from the MHRA. Acknowledging the uncertainties, the CHM advised that the EAMS scientific opinion should be upheld and renewed, taking into consideration the high level of unmet need as clearly described in the patient group meeting.
https://assets.publishing.service.gov.uk/government/uploads/system/uploads/attachment_data/file/718185/Final_ANNEX_to_PAR_Raxone_EAMS_Renewal.pdf

Evolving EAMS
Since launch, the EAMS Government-Industry Stakeholder Task Group has regularly brought together key stakeholders from the bio-pharmaceutical industry, NHS and arms' length bodies to inform the development of EAMS.

Real-world data generation

The importance of supporting innovation and early access to medicines have been recognised. In the Life Science Sector Deal 2 published in December, it was stated that the MHRA will continue to pioneer fast-tracked and safe patient access to ground-breaking treatments, benefiting both patients through early access to medicines and the sector, as they are able to better gather evidence for the medicines licensing process. The MHRA will work with industry and other partners to define a supportive framework for the collection of real-world data during the scheme. This will give industry greater support to gather the evidence they need to underpin licensing applications, in addition to early engagement with MHRA, NICE and the health system. Patients will continue to benefit from earlier access to medicines, often for conditions with unmet clinical need.

https://www.gov.uk/government/publications/life-sciences-sector-deal/life-sciences-sector-deal-2-2018

Summary
The Early Access to Medicines scheme addresses a public health need to improve access to important innovative medicines and has successfully facilitated the access to new medicines and new indications for hundreds of patients with unmet medical need. A number of proposals to strengthen the scheme since launch have been implemented in the last five years and exciting developments are to be expected, as the scheme matures and recommendations from the EAMS task force, government response to the Accelerated Access Review and Life Science deal are applied, further benefiting patients and supporting innovation.