Medicines & Healthcare products Regulatory Agency

MHRA
Regulating Medicines and Medical Devices

Session 4: Listening to the users of innovative medicinal products National authority early access scheme

UK Early Access to Medicines Scheme (EAMS)

Daniel O'Connor - Medical Assessor

UK Early Access to Medicines Scheme (EAMS)

EAMS Milestones

- Ministerial Industry Strategy Group

- The Prime Minister's Strategy for UK Life Sciences
  - Early Access to Medicines Scheme Consultation
  - Expert group on the innovation in the regulation of healthcare

- Early Access to Medicines Scheme launch
  - Step I: the Promising Innovative Medicine (PIM) Designation
  - Step II: the EAMS Scientific Opinion

- Summary and update

Ministerial Industry Strategy Group (MISG)

- The MISG brings together government and the bio-pharmaceutical industry to promote a strong and profitable UK-based pharmaceutical industry
- In 2008, a proposal for an early access scheme was developed as part of a series of MISG events
- The Regulatory Working Group forum considered there was support from stakeholders for earlier access to medicines
- The Working Group developed an early framework for early access
- Acknowledging that whilst access to such medicines will – at least in most cases – be towards the end of the formal development stage, the scheme could still provide potentially life-saving treatments around one year earlier than at present

Strategy for UK Life Sciences – Public Consultation

- In December 2011 the Prime Minister announced a new Strategy for UK Life Sciences
- The publication detailed actions aimed at maintaining the UK's world-class reputation in life sciences, improving patient health and acting as a catalyst for economic growth
- One of the commitments was that the MHRA will bring forward for public consultation proposals for a new 'Early Access Scheme'
- The Strategy set out the guiding principles for the scheme and included:
  - Eligible products will be determined by a scientific opinion
  - The likely clinical benefits outweigh the risks identified to date
  - There is high unmet need
  - NHS funding must be cost effective

Strategy for UK Life Sciences – Public Consultation

- The MHRA and Department Health launched a joint public consultation from July to October 2012
- There were 26 questions including should a scheme be established
- 52 responses were received from a variety of stakeholders, including patient groups
- Overall, there was overwhelming support for a scheme
- The Government, in considering EAMS, stated that:
  - EAMS addresses a public health need to improve access to important innovative medicines for patients with life threatening or seriously debilitating conditions without adequate treatment options
  - EAMS demonstrates a commitment from the UK to pharmaceutical innovation, through the Promising Innovative Medicine designation and earlier patient uptake of new innovative medicines in the health service

Strategy for UK Life Sciences – UK Expert Group

- Another commitment from the strategy was for the creation of an 'Expert Group on innovation in the regulation of healthcare'

- A group of experts drawn from government, regulators, the NHS, industry and the academic and third sector communities met quarterly to discuss healthcare regulation issues, including the development of new initiatives and innovations…

- The group included representatives from Parkinson's UK, Cancer Research UK and the Tuberous Sclerosis Association

- The group considered maximising the impact of, and learning from, the EAMS consultation

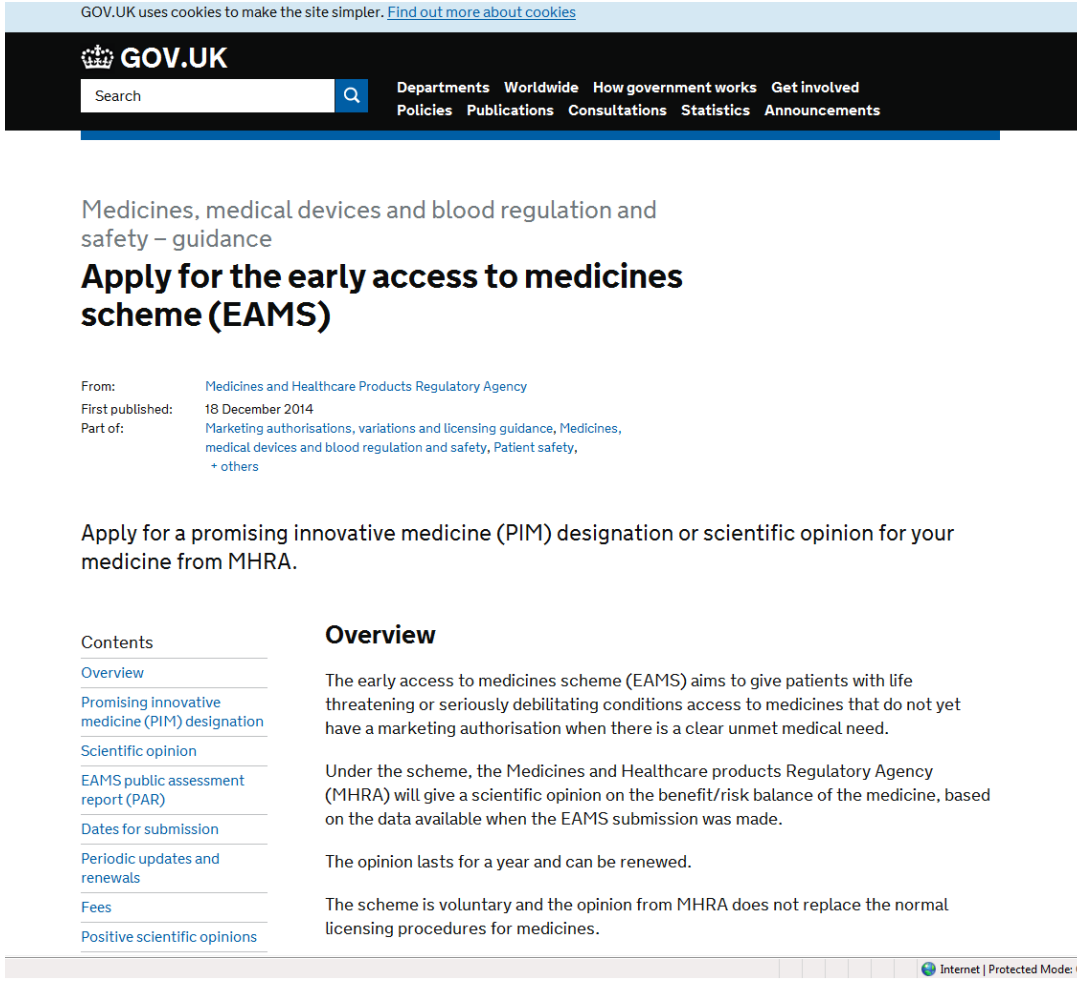
- The Expert Group report published in September 2013:

  – Welcomed the proposal for a UK EAMS and endorsed the draft Government response to the consultation

  – Advised that the scheme should be launched as soon as cross-Government agreement was obtained

Early Access to Medicines Scheme - launch

- A month after the responses to the consultation were published, the MHRA launched the scheme in April 2014
- Dedicated MHRA webpage with detailed guidance and application forms/ templates
- EAMS coordinator to ensure swift and efficient operation of the scheme: eams@mhra.gsi.gov.uk
- https://www.gov.uk/apply-for-the-early-access-to-medicines-scheme-eams



Early Access to Medicines Scheme

- The scheme covers medicines that are not yet available as licensed treatments
- The scheme is not a substitute for appropriate clinical development and inclusion of patients in well designed clinical studies remains the preferred option, if available in the UK
- Primarily aimed at medicines that have completed Phase III trials, but may be applied to completed Phase II trials in exceptional circumstances
- The medicine is to be provided for free by the company during the scheme
- EAMS is a two step process:
  - Step I: the Promising Innovation Medicine (PIM) Designation
  - Step II: the EAMS Scientific Opinion

The EAMS criteria

The criteria of suitability for an EAMS application are:

- Life threatening or seriously debilitating conditions, without adequate treatment options – high unmet need. This could include drugs intended for the treatment, prevention or diagnosis of diseases
- The medicinal product offers promise - that it is likely to offer benefit or significant advantage over and above existing treatment options
- Potential adverse effects likely to be outweighed by benefit. i.e. the benefit: risk ratio is concluded as being positive
- The Applicant is able to supply the product and to manufacture it to a consistent quality standard (GMP)

Step I: Promising Innovation Medicine (PIM)

- A PIM Designation is an early indication that a medicinal product is a potential candidate for the EAMS
- The PIM will be issued after an MHRA scientific designation meeting on the basis of non-clinical and clinical data available on the product, in a defined disease area
- A PIM designation is a prerequisite to enter the EAMS scientific opinion assessment (step II)
- 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
  - Opportunities to engage with NICE (HTA) and the NHS (National Health Service) on patient access issues
  - Opportunities to engage with the MHRA’s Innovation Office and scientific advice service

Step II: EAMS Scientific Opinion

- The scientific opinion describes the benefits and risks of the medicine and supports both the prescriber and patient in making a decision on using the medicine before its licence is approved

- EAMS applications are reviewed by our independent advisory committees (rules on interests observed), including the Commission on Human Medicines (CHM)

- CHM's role is to advise the MHRA in relation to the quality, safety and efficacy of human medicinal products

- CHM is supported in its work by Expert Advisory Groups (EAGs) covering various therapeutic areas (for example Biologicals, Oncology, Paediatric)

- https://www.gov.uk/government/organisations/commission-on-human-medicines/about/membership

EAMS Scientific Opinion

- A scientific opinion is issued after assessment if the criteria for the EAMS are considered to be fulfilled and the benefit risk is positive
- Current EAMS SO are published in the format opposite
- Expired opinions are also listed on the EAMS webpage and include the Public Assessment Report

Information on the EAMS scientific opinion given to pembrolizumab (MK-3475), including the public assessment report.

Documents

Pembrolizumab (MK-3475) EAMS public assessment report

PDF, 78.6KB, 3 pages

This file may not be suitable for users of assistive technology.
Request a different format.

Treatment protocol for patients

PDF, 94.2KB, 5 pages

This file may not be suitable for users of assistive technology.
Request a different format.

Treatment protocol for healthcare professionals

PDF, 180KB, 13 pages

This file may not be suitable for users of assistive technology.
Request a different format.

Treatment protocol on the pharmacovigilance system

PDF, 80.9KB, 3 pages

This file may not be suitable for users of assistive technology.
Request a different format.

Further consultation on EAMS

- A Government-Industry Stakeholder EAMS Task Group has been established to bring together key stakeholders from the bio-pharmaceutical industry, government and arms' length bodies to:
  - Inform the development of EAMS procedures
  - Establish consistent lines of communication between stakeholders
  - To clarify and resolve emerging issues since launch

- The UK Government's Accelerated Access Review (AAR) aims to speed up access to innovative drugs, devices and diagnostics for NHS patients

- EAMS is specifically included as part of the review (due to report Q2 2016):
  - '*Consider how we might strengthen the Early Access to Medicines Scheme, taking into account how this fits with the Adaptive Pathways Pilot, NICE Technology Appraisal, the NICE Implementation Collaborative and other schemes such as Evaluation through Commissioning*'

- https://engage.dh.gov.uk/acceleratedaccess/

EAMS Summary

- Aim to give patients access to medicines that do not yet have a marketing authorisation when there is a clear unmet medical need

- The scientific opinion is provided after a two-step evaluation process:
  - PIM designation 11 awarded (18 applications)
  - EAMS SO 5 awarded

- Patients are able to access important medicines before they are licensed and prescribers have greater confidence in the safety and efficacy of prescribing

- In developing the scheme, users of innovative medicines have been consulted at different stages, including at public consultation and in the Expert Group

- The assessment process involves consulting our Expert Committees which includes practicing clinicians on CHM and EAG, and input from lay members

Thank You

daniel.oconnor@mhra.gsi.gov.uk