Access to Medicines:
UK Early Access to Medicines Scheme (EAMS)
Action Duchenne conference: 11th of November

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Drug regulation

- Government agencies throughout the world have responsibility for supervising medicinal products and regulating the activities of the pharmaceutical industry.

- Member States (MS) have Competent Authorities which have these responsibilities.

- The European Medicines Agency (EMA) has responsibility for some types of products (e.g. rare diseases, cancer) and also coordinates activities across MS.

- Individual MS authorities and the EMA work together in a regulatory network.

- In the UK, the Competent Authority is the Medicines and Healthcare products Regulatory Agency (MHRA).

- We protect and improve health through the effective regulation of medicines and medical devices, underpinned by science and research.
  - We are one of the largest regulators in Europe.
  - We aim to be an accessible, pragmatic and efficient medicines regulator.
Earlier access to medicines

- A key challenge confronting regulators and other stakeholders is earlier patient access to innovative medicines, particularly in areas of unmet medical need.

- Ultimately there is often a fine balance between ‘denying’ patients potentially useful drugs and approving products for which the drug development is considered as immature.

- However, it is recognised that with greater medical needs e.g. life threatening conditions with no adequate treatments, it is acceptable to make decisions based on a greater degree of uncertainty in the data.

  - ‘Evidence versus access’ balance
Earlier access to medicines

• In the last 2-3 years, 3 initiatives have been launched to support earlier patient access on a UK and EU level

• Common to all these initiatives is early engagement with the regulator and other stakeholders

  – A UK initiative, Early Access to Medicines Scheme (EAMS), which aims to give access to medicines that do not yet have a marketing authorisation but meet an unmet medical need

  – A European initiative, Adaptive Pathways, a concept of ‘staggered marketing authorisation approval’, using existing regulatory tools and involving more stakeholders

  – A European initiative, Priority Medicines (PRIME), supporting development of priority medicines for unmet medical needs
UK Early Access to Medicines Scheme (EAMS)

‘EAMS aims to give patients with life threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorisation when there is a clear unmet medical need’
EAMS - a brief history
EAMS milestones

• 2008: Recommendation from the Ministerial Industry Strategy Group (MISG) for an early access scheme

• 2011: The Prime Minister’s Strategy for UK Life Sciences brings forward:
  – 2012: EAMS Public Consultation
  – 2013: Formation expert group on the innovation in the regulation of healthcare

• 2014: The MHRA launch two step Early Access to Medicines Scheme
  – Step I: the Promising Innovative Medicine (PIM) Designation
  – Step II: the EAMS Scientific Opinion

• 2014: First PIM designation awarded in September

• 2015: First EAMS scientific opinion approved in advanced melanoma in March
The Ministerial Industry Strategy Group (MISG) brings together government and the 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 a 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.

https://www.gov.uk/government/groups/ministerial-industry-strategy-group
Strategy for UK Life Sciences – Public Consultation

- In December 2011 the Prime Minister announced a new Strategy for UK Life Sciences

- One of the commitments was that the MHRA would bring forward for public consultation proposals for a new ‘Early Access Scheme’

- The consultation ran from July to October 2012
  - 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

- Conclusion: 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

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, NHS, industry and academia to discuss healthcare regulation issues….

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

- The group reviewed and welcomed the proposal for an early access scheme and endorsed the draft Government response to the consultation

EAMS launch & overview
Early Access to Medicines Scheme - launch

- Dedicated MHRA webpage launched April 2014 with detailed guidance and application forms/ templates
- [https://www.gov.uk/guidance/apply-for-the-early-access-to-medicines-scheme-eams](https://www.gov.uk/guidance/apply-for-the-early-access-to-medicines-scheme-eams)

**Apply for the early access to medicines scheme (EAMS)**

Apply for a promising innovative medicine (PIM) designation or scientific opinion for your medicine from MHRA.

**Overview**

The early access to medicines scheme (EAMS) aims to give patients with life threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorisation when there is a clear unmet medical need.

Under the scheme, the Medicines and Healthcare products Regulatory Agency (MHRA) will give a scientific opinion on the benefit/risk balance of the medicine, based on the data available when the EAMS submission was made.

The opinion lasts for a year and can be renewed.

The scheme is voluntary and the opinion from MHRA does not replace the normal licensing procedures for medicines.
Early Access to Medicines Scheme - overview

• EAMS is a two step process:
  – Step I: the Promising Innovative Medicine (PIM) Designation
  – Step II: the EAMS Scientific Opinion

• 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 towards the end of their development
  – What is required is evidence to support a positive benefit risk profile and fulfilment of EAMS criteria

• The Company supplies the product free of charge during the EAMS period
The four 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 Innovative 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 with 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
  - Specific NHS/HTA contacts in the UK nations, with opportunities to engage on patient access issues
Step II: EAMS scientific opinion (SO)

- During the benefit risk assessment, EAMS applications are reviewed by our independent advisory committees (CHM, EAG), include practicing clinicians.

- A positive scientific opinion is issued after a Day 75/90 timetable if the criteria for the EAMS are considered to be fulfilled and the benefit risk is positive.

  ➢ EAMS positive SO = patient access in the NHS before marketing authorisation.

- The scientific opinion describes the benefits and risks of the medicine and supports both prescriber and patient in making a decision on using the medicine before its licence is approved.
EAMS & HTA / NHS in England

- PIM designated products are prioritised through the NICE Topic Selection process.

- NICE’s Office for Market Access offers a meeting to discuss the company’s data collection plans during the EAMS period.

- Data on clinical / cost effectiveness may need to be generated during the EAMS period to address uncertainties - data may inform subsequent submission.

- EAMS products are planned as a priority in the work programme:
  - NICE starts the evaluation during the EAMS period.
  - First committee decision published within 3 months of marketing authorisation, (usual 6 months).
  - NICE recommended products commissioned by NHS England in 30 days for EAMS products (normally commissioned within 3 months).

What has happened in two+ years since launch?
The first EAMS scientific opinion published

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

- EAMS SO are published in the format opposite

- The opinion is valid for one year, renewable and expires at the point of MA

- Expired opinions are also listed on the EAMS webpage and include the Public Assessment Report

## EAMS applications – PIM designation

### EAMS step I PIM designations - April 2014 to October 2016

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- The therapeutic areas we have had interest in are in oncology, anti-infective, central nervous system, dermatology, blood disorder, cardiovascular and ophthalmology.
EAMS applications – EAMS SO

EAMS step II - April 2014 to September 2016

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- To date the majority of EAMS SO have been in the oncology setting: lung cancer, melanoma, renal cell carcinoma
MHRA published an EAMS case study

• Pembrolizumab was the first medicine to be awarded an EAMS positive scientific opinion

‘EAMS is an important step in ensuring patients gain access to innovative medicines as soon as possible, improving health outcomes in patients that urgently need new treatments’

Case study

Innovation: over 500 UK patients gain early access to new skin cancer treatment

History: Published 17 December 2015

MSD successfully put their advanced new melanoma treatment through MHRA’s early access to medicine scheme (EAMS).
Government EAMS task force

- Post-launch, an EAMS Government-Industry Stakeholder Task Group was established to bring together key stakeholders to:
  - Inform the development of EAMS procedures
  - Establish consistent lines of communication between stakeholders
  - To clarify, address and accelerate the resolution of emerging issues since launch

- The stakeholder group has developed additional supporting material to help explain the scheme:
  - An agreed EAMS ‘principles’ document (across the UK)
  - An operational guidance and a schematic showing the relationships between MHRA, NICE, NHSE and the company
  - Devolved administrations annexes in development

Independent review – the recommendations

The government commissioned PwC to conduct an independent review of EAMS (published March 2016)

Short term recommendations
- Provide updated guidance on the benefits and entry requirements of EAMS
- Provide easier industry access to MHRA, NICE and NHS
- Track patient access of approved products during the EAMS period

Medium term recommendations
- Earlier HTA of EAMS-approved products
- Rapid NHS uptake following a positive HTA to provide smooth transition to access
- Offer funding via application

Long term recommendations
- Use existing databases to collect real world data

Accelerated Access Review (AAR)

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

• The review makes recommendations to government on reforms to accelerate access – published 24th October 2016

• EAMS is specifically included as part of the terms of reference:
  – Patient access can be brought forward by up to four years where an EAMS scientific opinion is used (saving 12-18 months)
  – SMEs and not-for-profit organisations with products on the EAMS pathway should, in some cases, receive some funding

• Patient involvement is a key cornerstone of AAR, with a goal to give patients and service users a say at every stage of innovation

11 December 2015 — Authored article

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

How should we involve patients in research and innovation? (https://www.gov.uk/government/speeches/how-should-we-involve-patients-in-research-and-innovation)

Hilary Newiss, Chair of National Voices, asks for your opinion on how to give patients and service users a say at every stage of innovation.
Early Access to Medicines Scheme summary

• EAMS gives patients earlier access to medicines that do not yet have a marketing authorisation when there is an unmet medical need

• Patients are able to benefit from 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 an Expert Group

• The assessment process involves consulting our Expert Committees – includes practising NHS clinicians & input from lay members

• EAMS continues to evolve – MHRA, NHS and HTA processes:
  – Independent review has made a number of recommendations
  – Specific NICE EAMS guidance including data collection aspects
  – Accelerated Access Review report and OLS task force
Thank you

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