Regulation of ATMPs in the UK – ‘With or Without EU’

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Disclaimer

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Mission and Overview

“We protect and improve the health of millions of people every day through the effective regulation of medicines and medical devices, underpinned by science and research”

• Government trading fund and an executive agency of the Department of Health and Social Care (DHSC)
• Around 1350 staff
UK Rapporteurships for centralised applications - 2016

From EMA Annual Report 2016
The future:

Brexit timeline: 23:00 GMT on 31 October 2019

If the UK and EU ratify the withdrawal agreement before then, the UK will leave on the first day of the following month

Scenarios:

1. **DEAL** - Ratification of withdrawal agreement
   UK and MHRA in Transition Period until 31 December 2020

2. **NO DEAL** – MHRA as freestanding regulator from November 2019
No deal preparations:

- Statutory instruments passed by parliament
- Grandfathering of EMA licenses
- Transitional arrangements: Different options for ‘in-flight’ procedures
- CAPconversion@mhra.gov.uk for Brexit enquiries
No deal - guiding principles at MHRA

- Alignment with European timelines: Renewals, orphan designations etc.
- Orphan products: there will be an Orphan scheme European decision taken into consideration
- Plasma Master Files: Recognised
- Biosimilars: Reference product from EU possible
- European Guidelines continue to apply as they stand on exit day

Underpinned by MHRA and EU mutual recognition agreements and continued participation in ICH, PIC/S, ICMRA
No deal - New Assessment Routes:

- **Targeted assessment**
  - MHRA will evaluate the marketing authorisation application together with the Committee for Medicinal products for Human Use (CHMP) assessment reports submitted by the applicants, and will reach its opinion on approvability within 67 days of submission of a valid application to the MHRA.
  - Available for products containing new active substance and biosimilars.

- **Accelerated assessment Pathway**
  - MHRA will evaluate the marketing authorisation application and will reach its opinion on approvability within 150 days of submission of a valid application.
  - Available for all products containing new active substances, including biologicals for whom the applicants wish to obtain a marketing authorisation in the UK.
No deal - New Assessment Routes ctd:

- **Rolling Review pathway**
  - new route for marketing authorisation applications intended to enhance development of novel medicines. It does this by offering on-going regulatory input and feedback enabling the applicants to ‘get it right first time’ and reduce attrition due to avoidable regulatory pitfalls.
  - available for all products containing new active substances, including biologicals for whom the applicants wish to obtain a marketing authorisation in the UK

DEAL: Transition period

Details under negotiation

- No UK Rapporteurships
- UK recognises decisions taken at EMA level
- UK to participate in EMA committees if it is in the interest of patient safety for EU or UK
Brexit-independent offerings from MHRA
MHRA Innovation Office

- Launched March 2013
- Encourage early dialogue with researchers and companies
- Help clarify regulatory requirements
- Applicable to all of the MHRA product areas
- Provide regulatory / informal advice or scientific advice
- Request at any stage of product development
- Irrespective of existing guidelines
- Case studies published to encourage enquiries

- Since its introduction, the Innovation Office has responded to ~800 queries and held ~200 regulatory meetings.
Innovation Office - enquiry types and users

Innovation Office Enquiry Source

- Academic Institution: 28%
- SME: 37%
- Other: 8%
- Consultancy: 5%
- Not-for-profit Organisation/Charity: 2%
- Pharmaceutical/Biotechnology Company: 12%
- NHS Organisation: 7%
- Catapult Centre: 1%

Innovation Office Enquiry Type

- Devices: 32%
- Other: 7%
- ATMP: 23%
- Manufacture: 12%
- Chemical drug: 6%
- Novel non-clinical/clinical method or approach: 8%
- Novel formulation: 4%
- Biological Drug/Vaccine: 8%
Regulatory Advice Service for Regenerative Medicine (RASRM)

- Also known as the ‘One Stop Shop’ – unique in providing access to all national regulators
- Launched in 2014 and received approx. 75 enquiries to-date
- Provides consolidated advice on Advanced Therapy medicines
- Other agencies such as the Health & Safety Executive (HSE) and Department for Rural Affairs (Defra) give advice when relevant
- RASRM enquiries are submitted via the usual Innovation Office online form: https://info.mhra.gov.uk/forms/innovation_form.aspx
Early Access to Medicines

- The MHRA launched the scheme 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

- medicines that are still being developed but cannot yet be made available as licensed treatments
- not a substitute for appropriate clinical development and inclusion of patients in well designed clinical studies remains the preferred option
- Primarily aimed at medicines that have completed Phase III trials, but may be applied to completed Phase II trials in exceptional circumstances

The criteria of suitability:

✓ High unmet need (life-threatening, seriously debilitating)
✓ product offers benefit or significant advantage over and above existing treatment options
✓ Potential adverse effects likely to be outweighed by benefit
✓ The Applicant is able and willing to supply the product and to manufacture it to a consistent quality standard (GMP)
PIMS and EAMs statistics since 2014
Scientific Advice

- The MHRA can provide both regulatory and scientific advice to companies on any type of medicinal product;
- It can provide advice on all aspects of development (regulatory, non-clinical, quality and clinical);
- Approx. 300 scientific advice meetings are held per year;
- Companies can request to have a scientific advice meeting at any stage of development; they are most useful before submission of a Marketing Authorisation Application (MAA) but can also be after an MAA is granted e.g. concerning a variation to an existing product licence;

- See MHRA website for the on-line request form at:
MHRA broader scope advice

- Useful for innovative approaches that are not tied to just one development programme
  - e.g. novel trial design, new manufacturing process
- As approaches are new formal written answers are not given
- Informal feedback given at a face-to-face meeting

Parallel scientific advice from the MHRA and NICE

- Since April 2010.
- After a joint scientific advice meeting the MHRA and NICE will produce separate documents to answer the respective questions raised by the Company.
Thank you!

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