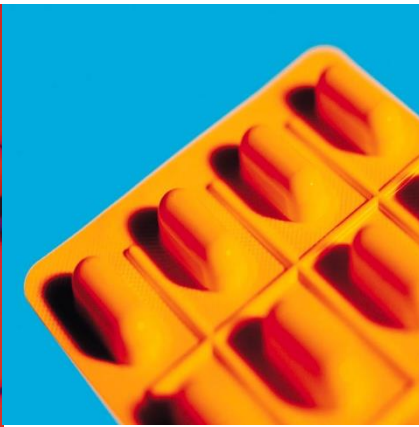


Partnering well together – creating a global ecosystem for advanced therapies

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BMA, London
Date 14th November 2017



ATMPs as cures – the opportunities and challenges for Europe

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Disclaimer



Opinions expressed in this presentation are my own, are not necessarily shared by other assessors at MHRA and can not be considered to be MHRA policy.

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Brexit

- for now, MHRA continues in the regulatory systems as they are and which will continue in the EU after UK has left, whatever the MHRA's future role is



Jeremy Hunt & Greg Clark, UK Secretaries of State for Health & for Business



Whatever the outcome of Brexit negotiations, we are clear that should we not achieve our desired relationship with the EU, we will set up a regulatory system that protects the best interests of patients and supports innovation – we'll seek to process licenses as quickly as possible; and our fee pricing will be competitive. However, our door will always be open to a deep and special relationship with the EU to promote improved patient outcomes both in Europe and globally
(FT, 4 July 2017)



Key questions



Why are UK patients not getting treated with ATMPs supplied on a commercial basis?

&

Are regulators being unreasonable in licensing decisions?

&

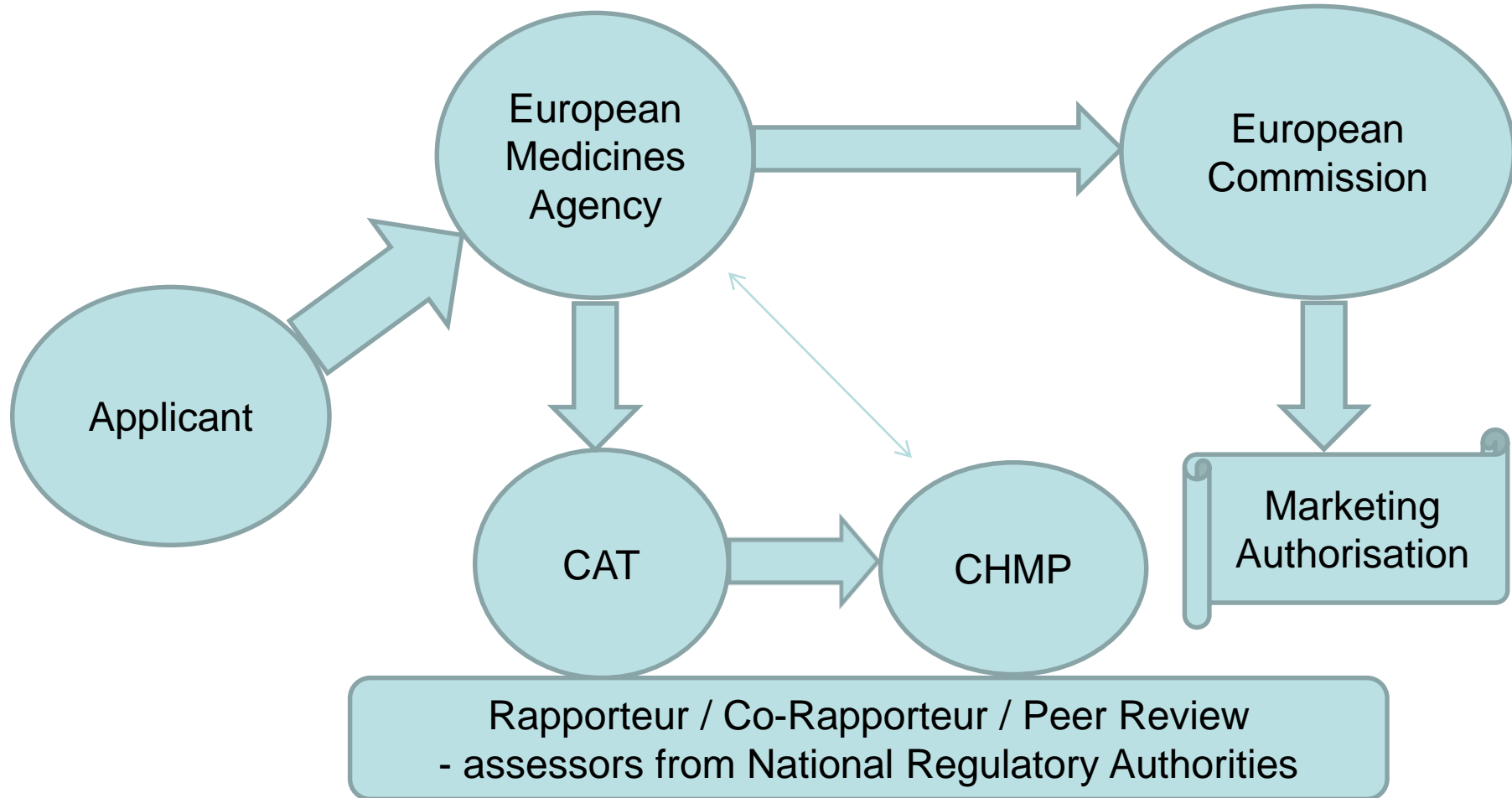
What can be done to supply pts?



Regulation 1394/2007

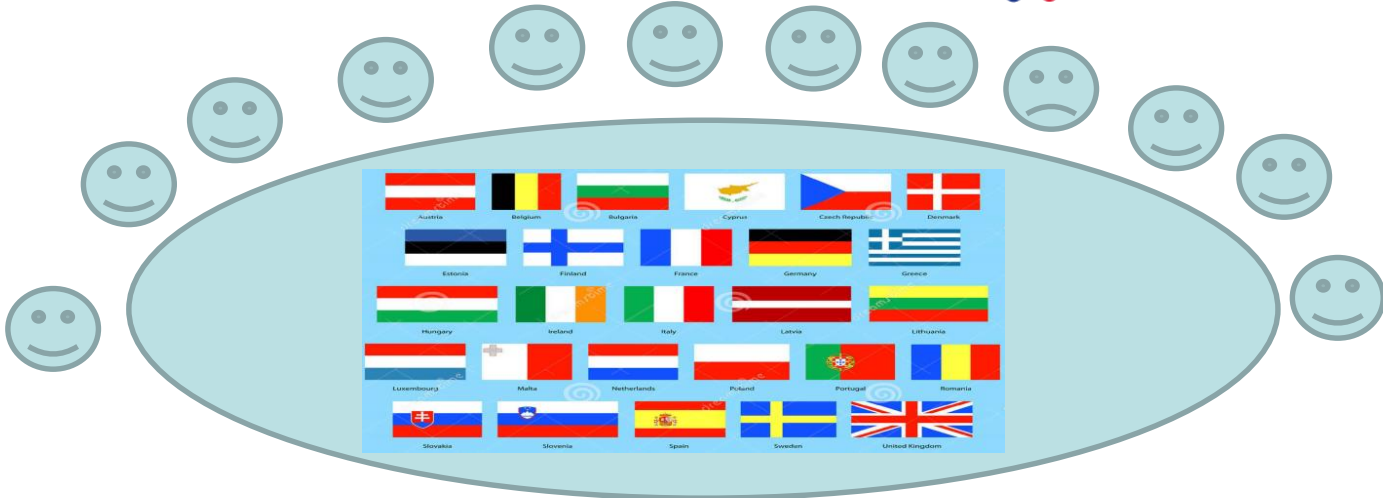


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Regulating Medicines and Medical Devices

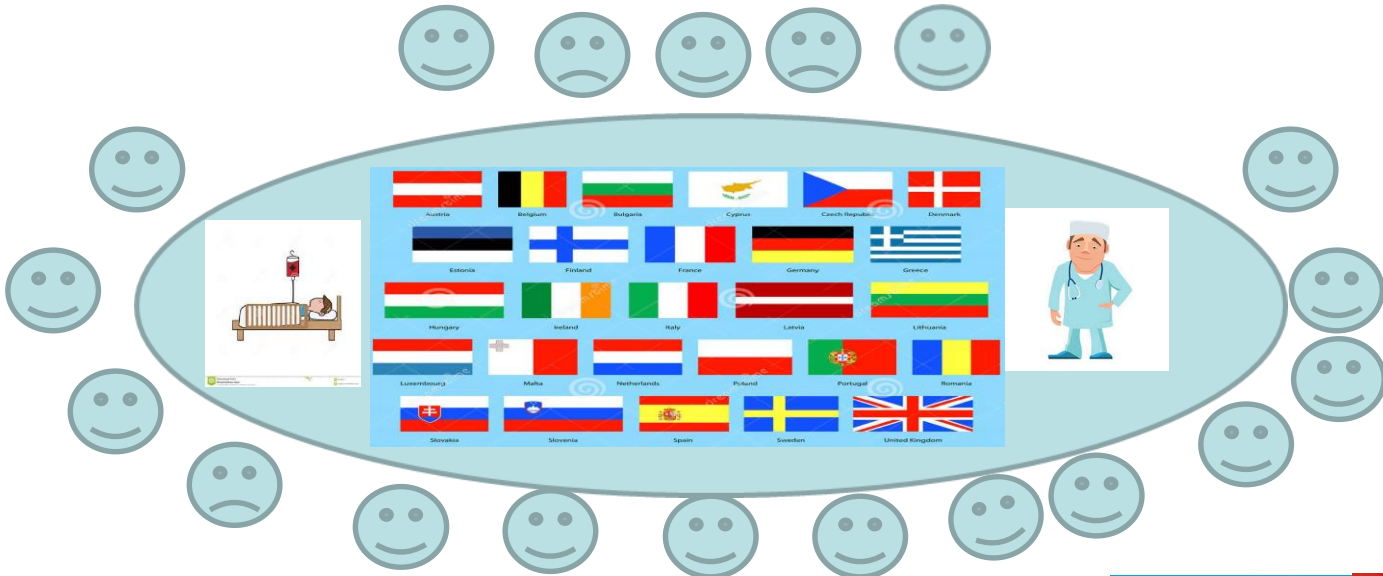




CHMP



CAT



Directive 2001/83 and Regulation 1394/2007



- 2001/83 and 1394/2007: legislation detailing market access
- other means of patient access include:
 - clinical trials
 - Early Access to Medicines scheme (UK only)
 - Hospital Exemption
 - Specials scheme (UK)



Patient access to ATMPs

- the evidence base to support use of licensed ATMP products as compared to use of products by Hospital Exemption and through the UK Specials Scheme represents a large hurdle
- why license a medicinal product if it can be supplied under these other schemes?



Regulatory Hurdle



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How to bring your ATMP product to the market?



The most useful suggestion I have is for you is to engage with regulators

Engage \neq partner: regulators are not your partner in drug development

But, if you don't engage you will fail



How to bring your ATMP product to the market?



Scientific advice: - national and CHMP processes

- to aim to present data that enables the regulators to agree that the product is safe enough for the intended use, efficacious and is made consistently to an acceptable standard →→→ approval

eg

- comparability plans post manufacturing change?
- design of (or no need for ?) in vivo tumorigenicity study?
- design of PIII study – comparator? / 1° and 2° endpoints? / numbers to be treated?



I think ...

UK patients not getting treated with ATMPs because

- products are not (shown to be) efficacious
- small patient numbers & high cost of ATMP production pose challenges for returns on investment
- for cell products, there are challenges in supply issues to due to short shelf-life

&

Are regulators being unreasonable in licensing decisions?

- CHMP forms the final scientific opinion and in the last 10 years has approved umpteen new products that are being used in the UK





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[ENDS
&
THANKS]



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Additional slides



EMA's Innovation Task Force



The Innovation Task Force (ITF) is a multidisciplinary group that includes scientific, regulatory and legal competences. It was set up to ensure coordination across the European Medicines Agency and to provide a forum for early dialogue with applicants.

Its objectives are to establish a discussion platform for early dialogue with applicants, in particular small and medium sized enterprises (SMEs) to proactively identify issues relating to emerging therapies; identify any need for specialised expertise at an early state; provide advice on the eligibility (ie is this an (AT) MP?); advise on cases where a medicinal substance is incorporated in medical devices

Recent areas of ITF engagement have included nanomedicines, pharmacogenomics, synthetic biology, biomaterials, modelling and simulation



EMA's Innovation Task Force



For further details see:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000334.jsp

You do not need to consult with ITF – it is available if you wish



How to bring your ATMP product to the market?



PRIME: PRiority MEdicines

http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000660.jsp&mid=WC0b01ac058096f643

PRIME is a scheme launched by the European Medicines Agency (EMA) to enhance support for the development of medicines that target an unmet medical need. This voluntary scheme is based on enhanced interaction and early dialogue with developers of promising medicines, to optimise development plans and speed up evaluation so these medicines can reach patients earlier.



PRIME



- If a candidate medicine has been selected for PRIME, the EMA will:
- appoint a **rapporteur** from the CHMP or from the CAT (if an ATMP) to provide continuous support and **help to build knowledge ahead of a marketing-authorisation application**;
- organise a **kick-off meeting** with the CHMP/CAT rapporteur and a multidisciplinary group of experts, so that they provide **guidance on the overall development plan and regulatory strategy**;
- assign a **dedicated contact point**;
- provide **scientific advice at key development milestones**, involving additional stakeholders such as health-technology-assessment bodies, to facilitate quicker access for patients to the new medicine;
- confirm potential for accelerated assessment at the time of an application for marketing authorisation



EAMS



- EAMS = Early Access to Medicines Scheme
- this is specific to the UK
- EAMS is not a licence / marketing authorisation
- See: <https://www.gov.uk/guidance/apply-for-the-early-access-to-medicines-scheme-eams>



EAMS – early access



- 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.
- in response to a request, the MHRA gives a scientific opinion on the benefit/risk balance of the medicine
- is this a promising innovative medicine (PIM)?
- if no, procedure ends: if yes, product is eligible for the EAMS
- then, company applies for an EAMS scientific opinion
- if the product is judged suitable based on scientific grounds, the MHRA issues an EAMS Public Assessment Report
- in principle, EAMS aims to provide patients with access to products that are in late stage development: payment for the product is not addressed



SAWP



- SAWP – Scientific Advice Working Party
- a multidisciplinary committee supported by EMA that give scientific advice on drug development plans on behalf of CHMP
- See:
http://www.ema.europa.eu/ema/index.jsp?curl=pages/contacts/CHMP/people_listing_000022.jsp

