Understanding EU regulation of cell based medicinal products

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Medicines and Healthcare Products Regulatory Agency

Regulatory framework for medicinal products

- what regulatory systems apply to medicinal products?

- any medicinal product must act by exerting a pharmacological, immunological or metabolic action (EC Directive 2001/83)

- Regulation 1394/2007 established that gene therapy, somatic cell therapy and tissue engineered products, insofar as they act by pharmacological, immunological or metabolic means, are biological medicinal products and defines these as advanced therapy medicinal products (ATMPs)

Regulatory framework for advanced therapy medicinal products

- 1394/2007 requires CHMP, in assessing an application for a marketing authorisation for an ATMP, to consult CAT

- CHMP - Committee for Human Medicinal Products - is the final scientific decision maker for centralised marketing authorisation applications in the EU

- CAT - Committee for Advanced Therapies – is a Europe-wide body with specialist expertise in ATMPs, charged with overseeing assessment of the application

- CAT can also offer:
  - classification – is 'this' an ATMP (or not)?
  - certification – review of quality / preclinical data

Role of CHMP, EMA and EC

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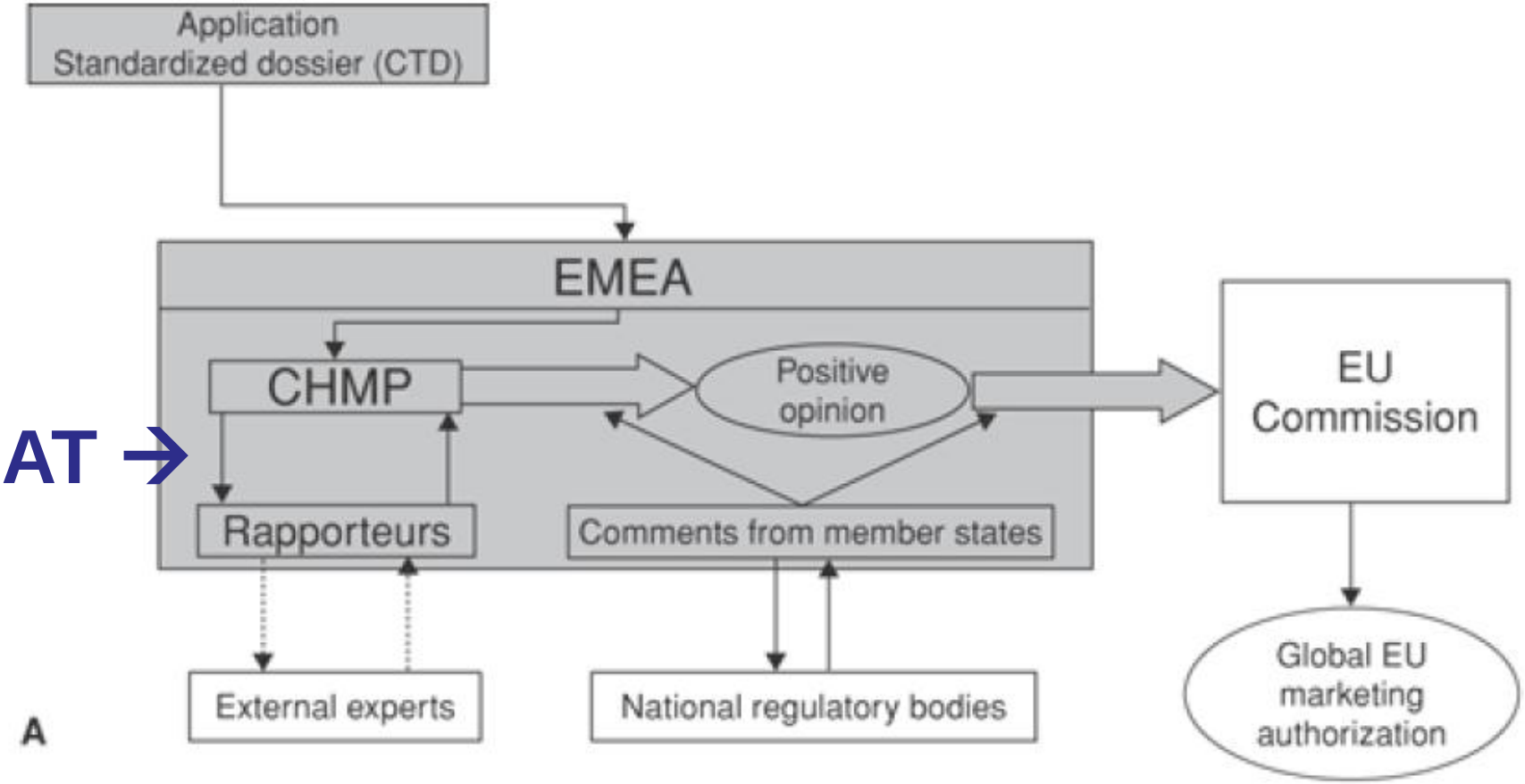
CAT →



Supranational and national roles

- whereas marketing authorisation applications are addressed at EU level in line with 1394/2007

- clinical trials are handled at national level in accordance with Directive 2001/20

- BUT!

- from 2016 onwards clinical trials will be handled somewhat differently with one administrative process in the EU, following a Regulation that is being implemented …. even as I speak ….

MHRA approach to review of clinical trial applications

- assign to 3 assessors
    - 1 quality / pharmaceutical
    - 1 preclinical / pharmacotoxicological
    - 1 medical / clinical

- meet weekly to discuss those where this is the first trial in the UK for that drug

- assessor is asked, in essence
- can I with right and conscience approve/deny this trial?

Support available …

If you are stuck ….

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… advice may be available

- EMA / CHMP offer advice on wider development plan
- via Scientific Advice Working Party (SAWP); referral to CAT

- MHRA offers regulatory / scientific advice
- on preparing for a specific trial, or wider development plan

- 5 methods
- email / phone your contact at the agency (x)
- email / phone the clinical trial helpline (www.mhra.gov.uk)
- seek advice from the Innovation Office (‘’)
- have a face-to-face meeting with assessors - written reply

Thanks for your attention!

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