EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Update on Post-Authorisation Collaboration - PAES, Other

PCWP HCPWP joint meeting
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Developments

- Outline expanded framework for post-authorisation efficacy studies (PAES)
- Highlight upcoming work on a scientific guideline for PAES.
- Meeting data needs in the context of health technology assessment (HTA).
- The The European Network of Centres for Pharmacoepidemiology and Pharmacovigilance (ENCePP) HTA working group experience
- Other aspects

PAES: not an entirely new concept

legal frameworks existed for PAES in the context of

- Conditional Marketing Authorisation (MA)
- MA in exceptional circumstances
- MA for Advanced Therapy Medicinal Products (ATMPs)
- The paediatric use of a medicinal product
- Referral procedures

New legal tool

- Publication, and now in force as of April 2014

- Expands framework for PAES imposition beyond existing legal frameworks
- Specific situations within Delegated regulation

  - Delegated Regulation (EU) No 357/2014

PAES Delegated Regulation

- **At the time of granting the marketing authorisation**: concerns relating to some aspects of the efficacy of the medicinal product are identified and can be resolved only after the medicinal product has been marketed

- **After granting the marketing authorisation**: the understanding of the disease or the clinical methodology or the use of the medicinal product under real-life conditions indicate that previous efficacy evaluations might have to be revised significantly

Imposed PAES – direction of travel

- **Exceptional instrument** for 'standard' MA
- Not be used as justification for premature granting of a MA
- **Context**: efficacy evaluation
- **Identified concern** – well-reasoned scientific uncertainty
- **Justified** on a case-by-case basis and **feasible** to conduct
- **Goal**: address concerns with tangible results with direct impact on the maintenance of the marketing authorisation
- **Design**: appropriate to answer the scientific question – focal point: supplementing efficacy data

Mandate for PAES scientific guidance

In order to facilitate the performance of pharmacovigilance activities within the Union, the Agency shall**, in cooperation with competent authorities and other interested parties,** draw up:

(a) guidance on good pharmacovigilance practices for both competent authorities and marketing authorisation holders;

(b) **scientific guidance on post-authorisation efficacy studies**.

Rapporteur Planning Group

- Agency (Jane Moseley, Kevin Blake)
- CHMP (Pierre Demolis, Robert Hemmings)
- PRAC (Stephen Evans, Almath Spooner)
- CMDh (Peter Bachmann, Jascha Hoernisch)

Regulatory & procedural guidance/Question & Answer document will be developed/updated in parallel

Indicative timing to be confirmed

- ✓ July 2014: Planning Group finalised with Committee leads
- ✓ July 2014: setting up drafting group
- ❑ Q3/4 2014 preparation and drafting relevant parties
- ❑ Q2/3 2015: Committees/CMD(h) adopt draft for public consultation
- ❑ 3 month public consultation
- ❑ Finalise late 2015

PAES -data needs in the context of health technology assessment (HTA)?

- Benefits of a medicinal product demonstrated in clinical trials are significantly affected by the use of the medicinal product under **real-life conditions**, or, in the case of vaccines, protective efficacy studies have not been feasible"
- Exceptionally
- Can explore how in such context the study programme can be designed to also meet data needs in the context of HTA.
- Opportunity for open dialogue on the imposed studies but also on other studies listed in the RMPs, collaboration at an early stage can be of help.
- To be discussed further at the EUnetHTA EMA joint meeting

ENCePP

- The European Network of Centres for Pharmacoepidemiology and Pharmacovigilance (ENCePP)is a network of over 170 research centres, existing networks and providers of healthcare data, which is coordinated by the European Medicines Agency.
- Its goal is to strengthen post-authorisation monitoring of medicines by facilitating the conduct of multicentre, independent studies using available European research expertise

ENCePP HTA Working Group

- In light of growing interest in HTA related outcomes being incorporated into post-authorisation studies of medicines, a HTA WG of ENCePP has been established.

- To map current capacity, a survey of ENCePP has been conducted (July 2014) to identify current HTA research experience, relevant resources and skills development requirements to undertake research that might the needs of regulatory and HTA stakeholders.

- Preliminary results based on responses from 35 centres show that HTA supportive studies are already part of everyday activity of some centres.

EMA EUnetHTA joint 3 year workplan

http://www.ema.europa.eu/docs/en_GB/document_library/Other/2013/11/WC500154588.pdf

Thank You

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