Das Committee for Orphan Medicinal Products

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COMP - Members

- One member nominated by each member state
- Three members nominated by the Commission to represent patients organisations
- Three members nominated by the Commission on the basis of EMA recommendation
- Non-voting members
  - Representatives from Norway and Iceland
- European Commission representative
- General observer

COMP - Tasks

- Examine applications for orphan drug designation
- Advice Commission on the establishment and development of a policy for orphan drugs
- Assist Commission in liaising …
  - … internationally on matters relating to orphan drugs
  - … with patient support groups
- Assist Commission in drawing up detailed guidelines
- NOT: Authorisation of orphan drugs
  - Scientific advice etc

Double Membership

- Link between Committees: Double membership
- CHMP
  - David Lyons
- PDCO
  - Janos Borvendég
- SAWP
  - Kerstin Westermark
  - Brigitte Blöchl-Daum
  - Rembert Elbers

Interaction COMP-CHMP (Authorisation)

- COMP is formally not involved into the authorisation procedure
  - Review of orphan designation criteria in parallel to CHMP assessment (including significant benefit)
- CHMP assessment: Safety and efficacy
- Protection of orphan drugs includes similar products
  - Derogation: Clinical superiority
- Superiority and similarity assessed by CHMP
  - Not COMP
  - “Superiority” versus “significant benefit”

Interaction COMP-CHMP (Advice)

- Scientific advice for orphan drugs: Protocol assistance
  - Responsible: SAWP (finally approved by CHMP)
  - COMP not involved into the procedure
- Several COMP members are in the SAWP
  - But protocol assistance does not formally require involvement of these members (nor approval by the COMP)
- Case study:
  - Protocol assistance for designated orphan product
  - Medical plausibility questioned by SAWP/CHMP
  - No clinical strategy could be agreed: Development currently on hold

Interaction COMP - CAT

- Similar to CHMP
- Most products reviewed by CAT are “ultra-orphan”
  - No particular definition of ultra orphan in the EU
  - No specific legislation
- COMP not involved into the procedure

Interaction COMP – PDCO (1)

- Orphan products are not excluded from the paediatric regulation
  - Contrast to US legislation (PREA)
- Accordingly all paediatric age groups have to be involved into development
- Fundamental hurdle in the development of orphan products: Lack of patients
  - Frequently even less paediatric than adult patients exist
  - PDCO is reluctant with waiver based on rarity
  - Consequence: Particular challenge for sponsors

Interaction COMP – PDCO (2)

- Orphan designation needs to cover whole condition
  - It should not be possible to define subset as orphan population
  - But development can be limited to indication as addressed in pivotal trials
- PIP has to cover full condition (rather than adult indication only)
- Potential issue: Lack in harmonisation of the condition for the PIP/Orphan Designation
  - Broader PIP condition could result in non-orphan population
  - Applicant looses designation or has to file separate applications

Interaction COMP – PDCO (3)

- Harmonisation required (e.g. of terminology)
- Example “Significant benefit”
  - has to be demonstrated for orphan products (if an authorized treatment exists for the targeted condition)
  - “Lack of significant benefit” is a potential justification for a waiver in the context of the paediatric regulation
- Different definitions of “significant benefit”

Some Light at the End of the Tunnel?

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

25 April 2012
EMA/274306/2012
Press Office

Press release

European Medicines Agency's Scientific Coordination Board starts reflection on best cooperation between scientific committees

Executive Director formalises new high-level group

The European Medicines Agency has formally launched its new Scientific Coordination Board. The mission of the group will be to ensure that there is sufficient coordination between the committees, so that the standards they set for the development of medicines are consistent across the whole product life-cycle, for increased robustness and predictability of benefit-risk assessment.

Thank you

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