Medicines and Healthcare Products Regulatory Agency

PCWP/HCPWP Joint meeting

COMP update March 2016

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On behalf of the COMP






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- The Committee for Orphan Medicinal Products (COMP) is the committee at the EMA responsible for reviewing 'orphan-medicinal-product designation‘
- The 2016 work plan activities include:

  - Consider whether “Points to Consider on the calculation and reporting of the prevalence…” (COMP/436/01) should be updated
  - Systematically involve patients’ representatives in COMP discussions on significant benefit based on major contribution to patient's care
  - Establish a working group on Protocol Assistance that will meet monthly to discuss any topics on significant benefit/ prepare recommendations
  - Publication of a comprehensive article that summarises the conclusions of the Working Group on Significant Benefit
  - Update the “COMP recommendation on elements required to support the medical plausibility and the assumption of significant benefit for an orphan designation, EMEA/COMP/436/01”

http://www.ema.europa.eu/docs/en_GB/document_library/Work_programme/2016/01/WC500200369.pdf

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- The workshop on significant benefit was held in December 2015:
  - ‘Demonstrating significant benefit of orphan medicines: concepts, methodology, and impact on access’

- Attended by representatives of EU regulators, HTA bodies, the pharmaceutical industry, payers, patients, health care professionals and academics
- A further 443 participants from 32 countries accessed the event via a live webcast

- To explore concepts and demonstration of significant benefit of orphan medicines over existing treatments;
- To discuss existing methodologies for comparative efficacy and effectiveness, and for major contribution to patient care, and how they could be applied to the demonstration of significant benefit at marketing authorization;
- To discuss the impact of significant benefit on HTA assessment, pricing decisions, and access to orphan medicines
  - Slides and other resources available:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/events/2015/09/event_detail_001195.jsp&mid=WC0b01ac058004d5c3

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Commission guidance

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- Revision of the EC's 2003 Communication on Orphan Medicinal Products - to be replaced by a Notice from the Commission
  - Current version includes sections on designation criteria, procedures for designation and removal from the register & market exclusivity

- Item 1: Clarification of the definition of "significant benefit"
- Item 2: Encourage development of medicines for communicable diseases
- Item 3: Simplifying procedure for the reassessment of orphan criteria when two procedures are pending in parallel for two products
- Item 4: Introduce reassessment of orphan criteria for a new subset
- Item 5: Clarifications on process of the transfer of orphan designations between sponsors

- Period of consultation has now closed

  http://ec.europa.eu/health/human-use/orphan-medicines/developments/index_en.htm

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Thank You

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