Medicines & Healthcare products Regulatory Agency

PCWP plenary: COMP update November 2016

Dr Daniel O'Connor (UK representative)
On behalf of the COMP

CPRD NIBSC MHRA

COMP

- The Committee for Orphan Medicinal Products (COMP) is the committee at the EMA responsible for reviewing 'orphan-medicinal-product designation
  - Finalising work plan for 2017
    - Implement changes following new Commission Notice
    - Consider principles and practices for determining significant benefit, defining orphan conditions and prevalence criteria

- COMP has an interest in the challenges of developing medicines in small populations
  - Initiative for patient registries launched in September 2015, with a cross-committee task force on registries

  - Patient registries workshop (28/10/2016)

    Identify the challenges faced by registries and industry when collaborating and understand the technical challenges - identify concrete solutions

  - A video recording and meeting report will be made available
- http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/events/2016/08/event_detail_001315.jsp&mid=WC0b01ac058004d5c3

New ‘Notice’ on orphan medicines

- ‘Communication’ now replaced by a ‘Notice’ from the EC:
  - Where prevalence in the EU is currently approximately zero, account should be taken of the risk that persons in the EU may become affected
  - ‘Subsetting’ a condition with the use of biomarkers will not be acceptable unless the sponsor provides solid scientific evidence that the activity of the product would not be shown on the larger population
  - In certain cases, medicinal products prepared for an individual patient in a pharmacy may be considered as satisfactory treatment if they are well known and safe and this is a general practice
  - http://eur-lex.europa.eu/legal-content/EN/TXT/PDF/?uri=OJ:JOC_2016_424_R_0003&from=EN

New ‘Notice’ on orphan medicines

- A new pharmaceutical form, a new strength or a new route of administration, should bring a major contribution to patient care - in all cases relevant data showing meaningful benefits for patients should be provided

- Extensions of the therapeutic indication are encouraged for the benefit of patients, the competent authorities may need to ascertain the significant benefit of this major change as compared with existing treatments in order to ensure that the variation remains within the terms of the orphan marketing authorisation

- http://eur-lex.europa.eu/legal-content/EN/TXT/PDF/?uri=OJ:JOC_2016_424_R_0003&from=EN

Thank You

daniel.oconnor@mhra.gsi.gov.uk