8th ENCePP Plenary meeting
EMA, 23 November 2011

Feedback from Rare Diseases Workshop

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European Union Committee of Experts on Rare Diseases

EUCERD/EMA WORKSHOP REPORT

Towards a public-private partnership for registries in the field of rare diseases

London, 4 October 2011

Participants

- EMA – COMP
- European commission
  - DG Sanco; DG Research
- Industry representatives
- Patient representatives
- Experts & Academic registry leaders
- EUCERD Scientific Secretariat

Objectives

(from Ségolène Aymé presentation)

- To foster the establishment of quality data repositories
  - to facilitate clinical research in the field of rare diseases (RD) and orphan drugs (OD)
  - to provide data to regulatory / reimbursement bodies
- to avoid duplication of efforts and waste of resources
- to provide unified sources of data where several products are available (to favor disease registries over product registries)

Orphanet Report Series
"Disease registries in Europe" (Jan 2011)

3- Distribution of registries by institution

INSTITUTION	NUMBER OF REGISTRIES
ACADEMIC	490
PATIENT ORGANISATION	8
PRIVATE COMPANY	16
TOTAL	514

From Carla Hollak presentation

Registries as a resource for post marketing studies

- European Orphan Drug Legislation (2000)
- 40% of drugs approved under exceptional circumstances
- Need to gain more valid and long term data
  - Clinically meaningful outcome data, including QoL
  - Safety
  - Prognostic factors

Disease registries vs product registries

- The example of enzyme replacement therapies for Fabry disease was given
- Two enzymes were authorised in the EU in 2001, both under exceptional circumstances
- Two post-marketing registries were established
- Difficulties in comparing
  - Appropriateness
  - Long term safety and effectiveness

Discussion/Suggestions

- Rare disease registries represent an important resource for post marketing studies
- To allow comparisons, disease registries rather than product registries need to be promoted
  - potential discrepancy with regulatory requirements
- Importance of high standards for quality
- Interaction with companies: the importance of ENCePP
  - code of conduct
  - guide for methodological standards