European Union Committee of Experts on Rare Diseases

EUCERD/EMA Workshop : Towards a public-private partnership for registries in the field of rare diseases

4 October 2011, EMA London

09:00-16:30

Tentative Agenda

9:00 Session 1: Overview of the current situation and identified issues for discussion between stakeholders

Co-Chairs: Kate Bushby & Lesley Greene

- State of the art of disease and product registries in the EU – *Ségolène Aymé* (10 mins)
- Issues identified:
    - Point of view of academic registry leaders – *Carla Hollak* (15 mins)
    - Point of view of Industry – *TBI* (15 mins)
    - Point of view of patient representatives – *To be nominated* (15 mins)
    - Point of view of regulatory agencies – Stella Blackburn (15 mins)
- Discussion (30 mins)

10:40 – 11:00 Coffee break

11:00 Session 2: Usefulness of registries for regulatory purposes: Opportunities and challenges

Co-Chairs: Kerstin Westermark + Nancy Dreyer/Rich Gliklich

- Overview of the issues – *Nancy Dreyer/ Rich Gliklich* (20mins)
- Experiences from rheumatology registers and outcomes of a Swedish EU presidency project. *Nils Feltelius* (20mins)
- Roundtable (20 mins): *Meindart Boysen + Industry representative TBI + Hanns Lochmüller*
- Discussion (30 mins)

12:30 -13:30 Lunch (cafeteria)

13:30 – 15:00 Session 3: EC Initiatives to provide high quality and unified sources of data to all stakeholders

Co-Chairs: Catherine Berens & Antoni Montserrat

- Roundtable: Areas for action at EU level
    - The EUCERD perspective – *Kate Bushby* (15 mins)
    - The IRDiRC perspective – *Stuart Tanner* (10 mins)
    - The EPIRARE perspective – *Domenica Taruscio* (20 mins)
    - The Joint Action on Patient Registries (PARENT) perspective – *Matic Meglic* (10mins)
- Discussion (30 minutes)

15:00 – 15:30 Coffee break

15:30 Session 4: Conclusions and points for action

Co-Chairs: Catherine Berens & Antoni Montserrat

- Roundtable with representative of each group of stakeholders
    - EMA representative – *Stella Blackburn* (5 mins)
    - Academic registry leader representative – *Hanns Lochmüller* (5 mins)
    - Industry representative – *To be identified* (5 mins)
    - Patient representative – *Fabrizia Bignami* (5 mins)
    - National regulatory agency representative – *Patrick Salmon* (5 mins)
- General discussion (35 mins)

16:30 End of workshop