"Retooling the European Orphan Drug Regulation and US Orphan Drug Act for Better and Faster Development of Rare Disease Therapies"

June 18th, 2010  
Barcelona, Spain

Draft Agenda

8:30 Welcome & coffee

MORNING: 9:00 - 12:30
Chairpersons:  
Josep Torrent i Farnell, COMP- European Medicines Agency (EMA)  
Antoni Montserrat, representative from DG SANCO, European Commission

9:00 – 9:15: Opening speech


9:45 – 10:15: “What have we learnt from the EU Paediatric and Advanced Therapy Regulations that could be used to foster orphan drug development?”, Jordi Llinares Garcia, European Medicines Agency (EMA)

10:15 – 10:45 COFFEE BREAK


11:15 – 11:45: “US perspectives on strategies to stimulate and improve orphan product development: results from the Focus Group Research”, Peter Saltonstall, National Organization for Rare Disorders (NORD)

45’ discussion

12:30 – 14:00: LUNCH
**AFTERNOON: 14:00 -17:00**

*Chairpersons:*
*Eric Abadie, CHMP, European Medicines Agency (EMA)*
*Yann Le Cam, EURORDIS*

**14:00 – 14:15:** “Introduction to the parallel breakout sessions and presentation of the 5 discussion topics”, *Fabrizia Bignami, EURORDIS*

**14:15 - 15:30:** parallel breakout sessions

**Topics/groups:**

1. New medicinal products for rare diseases without an orphan designation on the EU market?
   *Rapporteurs: Bettie Voordouw (COMP, European Medicines Agency) and Ruediger Gatermann (CSL Behring)*

2. Linking significant benefit to effectiveness and relative effectiveness. Which Benefit Management Plan for monitoring the “real life” value of orphan drugs?
   *Rapporteur: Catarina Edjaell (Celgene International)*

3. Mechanisms to facilitate the development of off-label and off-patent drugs for orphan indications, taking examples of useful incentives from other legislations (e.g. PUMA and certification procedure)
   *Rapporteurs: Josep Torrent i Farnell, (COMP- European Medicines Agency) and Michael Bone (EFGCP)*

4. How to identify and address the “rare” unmet medical needs? Importance of an early dialogue among all stakeholders to better determine research strategies.
   *Rapporteurs: Ségolène Aymè (ORPHANET) and Lesley Greene (COMP/EURORDIS)*

5. Global orphan drug development and EU-US collaboration: state of the art, limits and how to retool the process.
   *Rapporteurs: Peter Saltonstall (NORD) and Jutta Ulbrich (Bayer Schering Pharma)*

**15:30 – 16:15:** Feedback from the parallel groups presented by the 5 rapporteurs

**16:15 – 17:00** Discussion

**17:00**  
*End of Workshop*