12th Workshop
Eurordis Round Table of Companies

"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*

9:15 – 9:45: "The EU Regulation EC 141/2000 after 10 years of implementation - Strengths & Limitations", *Kerstin Westermark, COMP, European Medicines Agency (EMA)*

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

10:45 – 11:15: "Industry's 10-year experience in Europe and perspectives for improvement of the EU Orphan Drug Regulation", *Erik Tambuyzer, EuropaBio/EBE Orphan Drug Task Force*

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 Edfjaell (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 Aymé (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**