



12<sup>th</sup> 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 18<sup>th</sup>, 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 Westermarck, 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**