



9<sup>th</sup> Workshop  
Eurordis Round Table of Companies

*“Significant Benefit of Orphan Drugs:  
Impact on Clinical Development and Assessment”*

*December 12th, 2008  
Les Salons de l'Aéro-Club de France  
Paris, France*

Programme

8:30 Welcome & coffee

**MORNING: 9:00 - 13:00**

*Chairpersons:*

**Eric Abadie (Chair of the CHMP, EMEA) – Josep Torrent-Farnell (COMP member, EMEA)**

**9:00 – 9:20:** *Welcome address and presentation “From EU Policy Framework to Public Awareness: Rare Disease Day” (Yann Le Cam, Eurordis)*

**9:30 – 9:50:** *“Presentation of the Revised Guideline on elements required to support the medical plausibility and assumption of Significant Benefit for orphan designation” (Kerstin Westermark, COMP, EMEA)*

10' questions

**10:00 – 10:20:** *“The Significant Benefit Criterion: the Experience gained at the Time of Designation and of Marketing Authorisation” (Jordi Llinares-Garcia, EMEA)*

10' questions

**10:30 – 11:00: COFFEE BREAK**

**11:00 – 11:20:** *“Is there an Optimum Clinical Trial Design for Significant Benefit Demonstration? The Experience of the Scientific Advice Working Party (SAWP)” (Mira Pavlovic, SAWP)*

10' questions

**11:30 -12:45: Panel discussion: Real-life Experiences of Significant Benefit (90')**

Panel includes all morning speakers, chairpersons and three contributors from the pharmaceutical industry: Alex H. West, GSK – Catarina Edjfaëll, Celgene – Franck Rauschen, Shire.

**12:45 – 14:15: LUNCH**

**AFTERNOON: 14:15 -16:30**

*Chairpersons:*

**Kerstin Westermark (Chair of the COMP, EMEA) – Peter Saltonstall (CEO, NORD)**

**14:15 – 14: 35: “What is the Potential Impact of Significant Benefit in the Field of Medical Oncology?” (Kateřina Kubáčková, COMP, EMEA)**

**10’ questions**

**14:45 – 15:05: “Why and How to Communicate on Significant Benefit?” (Yann Le Cam, Eurordis)**

**10’ questions**

**15:15 – 15: 35: “Significant Benefit and “Relative Effectiveness” (Hans-Georg Eichler, EMEA)**

**10’ questions**

**15: 45 – 16:30: Discussion with panel and all stakeholders**

Panel includes all afternoon speakers, chairpersons and two contributors from the pharmaceutical industry: Wills Hughes-Wilson, Genzyme – Karin Blumer, Novartis.

**16:30**

**End of Workshop**