



7th Workshop
Eurordis Round Table of Companies

"Proof of Concept and Level of Evidence in Orphan Drug Development"

December 14th, 2007
Paris, France

La Maison des Arts & Métiers
9 bis Avenue d'Iena – 16^{ème}

Programme

8:30 Welcome & coffee

MORNING - 9:00 - 12:30

Chairpersons:

Eric Abadie (Chair of the CHMP) – Kerstin Westermark (Chair of the COMP)

9:00 - 9:15 Welcome address (Yann Le Cam, Eurordis)

Same language but different definitions of «proof-of-concept».

9:15 - 9:35 *"Proof-of-Concept: Univocal Concept or Shared Ambiguity?"*
(Rembert Elbers, COMP member nominated by Germany)

Learning from experience: analysis of the levels of evidence in the US and European designation and marketing authorization processes:

9:35 -10:00 *"Proof-of-Concept in the European Orphan Drug Designation Process"*
(Fabrizia Bignami, Eurordis)

10:00 -10:25 *"Proof-of-Concept in the US Orphan Drug Designation Process"*
(Tan Nguyen, FDA)

10:30 – 11: 00 – COFFEE BREAK

11:00 - 11:25 *"From Proof-of-Concept to Evidence-based Marketing Authorisation"*
(Marco Cavaleri, EMEA, SAWP)

11:25 – 11:40 *"From Proof-of-Concept to Evidence-based Marketing Authorisation, the CHMP Perspective"*
(Pierre Demolis, AFFSSaPS, CHMP)

11:40 – 12:30 *Discussion (50')*

12:30 -13:45 – LUNCH

AFTERNOON - 13:45 -16:30

Chairpersons:

Tan Nguyen (OOPD -FDA) – Josep Torrent-Farnell (COMP member)

13:45 -14:25 “Proof-of -Concept as a Green Light for Further Development”
(Detlef Niese, Novartis Pharma AG and Louis-Christian Claus, Baxter)

14:25 -14:45 “Proof- of -Concept as a Green Light for Regulators”
(Kerstin Westermark, Chair of the COMP)

14:45 -15:15 “Proof- of- Concept as a Green Light for Ethics”
(Olivia Niclas, patient representative AFDE and Jean-Claude Ameisen, Inserm)

15:15 -15:45 “Case Studies” by 2 industry representatives
(Bonnie Mills, IDM Pharma, Inc. and Andreas Orfanos, Neurochem, Inc.)

15:45 -16:30 Discussion with panel and all participants (45’)

Panel members: afternoon speakers

16:30

End of Workshop