EURORDIS
ROUND TABLE OF COMPANIES

17th Workshop

EURORDIS Round Table of Companies (ERTC)

The Value of Orphan Drugs: Efficient for the Patients, Useful to Society?

September 28, 2012 – 9:00 to 17:30 - Barcelona, Spain

AGENDA

Chairpersons: - Prof. Josep Torrent-Farnell (Member of the Committee of Orphan Medicinal Products (COMP), EMA, Spain & Director of Fundació Doctor Robert, Autonomous University of Barcelona (UAB)) - Mr Yann Le Cam (Chief Executive Officer of EURORDIS, EU)	
9:00 – 9:15	Welcome address: Mr Yann Le Cam
9:15 – 11:00	*Patients' challenges to gain recognition of the utility of their medicines by society: case studies by rare diseases patient advocates*
(15' + 5' Q & A)	Case study 1 – Ms Ulrike Pypops (Cystic Fibrosis Europe, BE)
(15' + 5' Q & A)	Case study 2 – Ms Ramunė Šliuožaitė & Ms Eglė Zikiene (National Association of MPS & OGMD, Lithuania) by video presentation
(15' + 5' Q & A)	Case study 3 – Dr Bernd Quadder (German Sarcoidosis Association)
10:15 – 11:00	Panel discussion with speakers
11:00 – 11:30	COFFEE BREAK
11:30 – 13:00	*Experience, challenges and reflection by the assessors of the utility of medicines for rare diseases*
(20' + 10' Q & A)	Challenges to assess the utility of rare disease therapies for society – Dr Francois Meyer (HAS, France; EUnetHTA)
(20' + 10' Q & A)	Perspective on reimbursement for Fabry and Pompe treatments from the Dutch Health Care Insurance Board – Dr. Martin van der Graaff (CVZ, The Netherlands)
(20' + 10' Q & A)	Rare diseases, costs per QALY, and priority setting. How to take into account the patients' and public preferences? – Dr Erik Nord (Norwegian Institute of Public Health)
13:00 – 14:30	LUNCH

Chairpersons: - Dr Catherine Berens (DG Enterprise & Industry, European Commission) - Ms. Flaminia Macchia (EU Public Affairs Director, EURORDIS)	
14:30 – 16:15	*How is industry innovating to address the challenges of rare disease utility demonstration?*
(15' + 5' Q & A)	Demonstrating the value of orphan drugs – Ms Martina Garau (Senior Economist, Office of Health Economics, UK)
(15' + 5' Q & A)	The AGNSS multi-criteria decision analysis approach to HTA to demonstrate the value of orphan drugs - Mr Francis Pang (Senior Director, Market Access and Public Affairs, Shire, CH)
(15' + 5' Q & A)	Positioning orphan drugs in sustainable healthcare systems – time to implement a new approach – Dr Geoffrey McDonough (President & CEO, SOBI, Sweden)
15:30 – 16:15	Panel discussion with speakers and a discussant: Dr Jon Beauchamp (VP Medical Affairs for EMEA, Alexion Pharmaceuticals, UK) on the Soliris case
16:15 – 17:30	*How can innovative approaches impact on the future evaluation of utility?*
(20' + 10' Q & A)	Adaptive Licensing – Prof. Hans-Georg Eichler (Senior Medical Officer, European Medicines Agency, UK)
(20' + 10' Q & A)	Reflections of the EU Working Group on a Mechanism of Coordinated Access to Orphan Medicinal Products – Dr. Catherine Berens (Policy Officer, Unit Food & HealthCare Industry & Biotechnology, DG Enterprise & Industry, European Commission)
17:15 – 17:30	Open discussion with audience and conclusions
17:30	Meeting ends