EURORDIS
ROUND TABLE OF COMPANIES

19th Workshop

EURORDIS Round Table of Companies (ERTC)

"Practical aspects of Progressive Patient Access to orphan medicinal products, post-marketing evidence generation & related pricing questions for rare disease patients "

Tuesday 15 October, 2013 (9:00 – 16:00)

Barcelona, Spain
Fundació Doctor Robert, Universitat Autònoma de Barcelona, C/Sant Antoni Maria Claret, 171

AGENDA

Morning session ***State of play and key challenges of progressive patient access to orphan medicinal products for rare disease patients*** Chairpersons: Dr Hans-Georg Eichler (Senior Medical Officer, EMA) and Dr Richard Bergström (Director General, EFPIA)	
9:00 – 9:20	Welcome address: Mr Yann Le Cam (CEO, EURORDIS) 20'
9:20 – 9:40	EFPIA's perspective on Progressive Patient Access: Magda Chlebus (Director of Science Policy, EPFIA, BE) - 20' including Q&A
9:40 – 10:00	EMA's perspective on Progressive Patient Access: Dr Hans-Georg Eichler (EMA) - 20' including Q&A
10:00 – 10:20	Patients' and COMP's Perspective on Progressive Patient Access: Dr Pauline Evers (COMP member Netherlands and Policy Officer NKF) - 20' including Q&A
10:20 – 10: 50	30' COFFEE BREAK
10:50 – 11:35	Panel discussion led by Dr Eichler and Dr Bergström, involving all morning speakers, as well as Dr Paolo Siviero (Head of the Pharmaceutical Policy dept., AIFA, IT) & Dr. Anton Hoos (Senior VP & Acting Head, Global Rare Disease Unit, GSK, UK) – 45'
11:35 – 11:40	Introduction to the topics of the afternoon parallel sessions Dr Maria Mavris (Therapeutic Development Director, EURORDIS) 5'
11:45 – 13:00	1h15' LUNCH

Afternoon session ***Progressive access to OMPs - optimising key decision-making processes with patient involvement*** Chairpersons: Ms Wills Hughes-Wilson (VP External Affairs, Chief Patient Access Officer at SOBI/EBE-EuropaBio) and Mr Yann Le Cam (EURORDIS)	
13:15 – 14:45 *Each group is assigned a leader & rapporteur*	***Parallel breakout sessions:*** *(including preparation of a short report) – 1h30'* **Topic 1: Concrete approaches to the first EMA pilots on Progressive Patient Access:** *Questions to address include:* • What would you propose for the first pilots for PPA by EMA in terms of timing and process? • How can PPA be applied to therapies for rare diseases? **Topic 2: Aligning Progressive Patient Access with EU collaboration on HTA & access:** *Questions to address include:* • What are the best mechanisms to take advantage of early dialogue and optimise the parallel EMA/HTA scientific advice? • What concrete actions can be proposed to enhance the dialogue between EMA & HTA at the time of marketing authorisation assessment for PPA by CHMP, in order for HTA to trust the value of rare diseases therapies? • How can the PPA approach fit in with the *Mechanism* for *Coordinated Access to Orphan Drugs* (MoCA) and Managed Entry Agreements?
14:45 – 15:30	Feedback from each breakout sessions
15:30 – 16:00	Key challenges and success factors of PPA for RD therapies: Mr Le Cam, Ms Hughes-Wilson & Dr Eichler
16:00	Meeting ends