EURORDIS
ROUND TABLE OF COMPANIES

23rd Workshop

EURORDIS Round Table of Companies (ERTC)

"Patient Relevant Outcome Measures & Patient Reported Outcomes"

29 September, 2015 (9:00 to 16:00) - UAB - Casa Convalescència, Barcelona, Spain

AGENDA

Morning session co-chaired by: Dr. Alicia Granados (President of the Scientific Advisory Council of AQuAS, Spain) Prof. Bruno Sepodes (Chairperson of the COMP, EMA & Professor of Pharmacology at Lisbon Univ.)	
9:00 – 9:30	**Welcome & Introduction** - Yann Le Cam (Chief Executive Officer, EURORDIS) Definition of the terms PROMs & PROs and how we are using them.
9:30 – 13:00	***Morning Session:*** ***State-of-the-Art of Patient Relevant/Reported Outcomes (PROMs & PROs)***
9:30 – 9:50	**A validated and recognised approach on how to develop PROMs: the contribution of PCORI** - Sharon F. Terry (Genetic Alliance & IRDiRC)
9:50 – 10:05	**EMA's approach & experience with PROMs** – Dr. Maria Isaac (Scientific Advisor, EMA) - *Interviewed by Lesley Greene* (Patient rep. at COMP, EMA)
10:05 – 10:20	**What data is required by regulators in a dossier?** Prof. Bruno Sepodes (Chairperson of the COMP, EMA) - *Interviewed by Flaminia Macchia* (Vertex Pharmaceuticals)
10:20 – 10:40	**Q & A**
10:40 – 11:10	Coffee break
11:10 – 11:30	**Who uses PROs and potential value for industry -** Benoît Arnould (Mapi)
11:30 – 11:45	**The Core Outcome Measures in Effectiveness Trials (COMET) Initiative** – Heather Bagley (University of Liverpool)
11:45 – 12:00	**Case study 1: How to integrate PROs in clinical development?** Samantha Parker (Lysogene)
12:00 – 12:15	**Case study 2: Impact of lack of PROMs for innovative drugs & current actions in Duchenne** - Elizabeth Vroom (Dutch Duchenne Parent Project; United Parent Projects Muscular Dystrophy (UPPMD))
12:15 – 12:30	**Case study 3: The Italian CF experience of PROs** – Mario Ricciardi (Cystic Fibrosis Europe)
12:30 – 13:00	**Panel discussion with morning speakers**
13:00 – 14:15	LUNCH

Afternoon session co-chaired by: Dr. Fabrizia Bignami (Global Medical Affairs & Patient Relations Director, GSK Rare Diseases) & Solange Rohou (Director Regulatory Affairs, AstraZeneca)	
14:15 – 16:00	***Afternoon Session:***
14:15 – 15:00	**Group discussions per table in plenary: three principal barriers & benefits of PROs** (3 max. per table)
15:00 – 15:45	**Feedback from group discussions** (5' per table) & **Questions**
15:45 – 16:00	**Conclusions**
16:00	Meeting ends