24th Workshop of the EURORDIS Round Table of Companies (ERTC)

"Bringing Solutions to Young Rare Disease Patients"

Let's discuss the paediatric regulation

Tuesday 27 September, 2016 (8:30 to 17:00) - UAB - Casa Convalescència, Barcelona, Spain

PROGRAMME

Morning session co-chaired by:

Tsveta Schyns-Liharska, PhD., *Secretary General, European Network for Research on Alternating Hemiplegia (ENRAH), Belgium/Bulgaria, patient representative on the PDCO for EURORDIS*

Dr Mark Turner, *Chair, European Network for Paediatric Research at the European Medicines Agency, Advisor on International Liaison, NIHR Children's Theme and Speciality Group and Consultant in Neonatology at the University of Liverpool, UK*

8 :30 - 9:00	**Welcome introduction – Mr Yann Le Cam**, Chief Executive Officer, EURORDIS, France/Belgium
	Morning Session: ***State-of-play and identification of challenges***
9:00 – 9:30 (incl. 10 mins Q&A)	**The Patient Data Platform – a promising patient-centered approach:** **Mr Julian Isla**, Chairman, Dravet Syndrome European Federation & Dravet Syndrome Foundation, Spain & **Prof Bruno Sepodes**, Prof of Pharmacology and Pharmacotherapy, University of Lisbon/ Chair of the COMP, CHMP Member, EMA
9:30 - 10:00 (incl. 10 mins Q&A)	**Taking stock of the experience gained in the field of paediatric rare diseases studies:** Impact of the paediatric legislation, new methods used, specific issues of rare diseases with PIPs and their implementation **Dr Emilie Desfontaine**, Paediatric Coordinator (rare diseases procedures), European Medicines Agency (EMA)
10:00 - 10:30	Coffee break
10:30 - 11:00	**How to make the best use of the Orphan & Paediatric legislations + Scientific Advice?** **Dr Segundo Mariz**, Scientific Administrator, European Medicines Agency (EMA)
11:00 - 11:30	**Two case studies (15' each):** **Dr Martine Zimmermann**, Senior Vice President, Head of Global Regulatory Affairs, Alexion Pharma International, Switzerland & **Dr Geneviève Le Visage**, Head EU Regulatory Intelligence & Policy, Novartis Pharma AG, Switzerland
11:30 - 12:30	**Panel discussion: Issues raised by the Paediatric Regulation in Therapeutic Development for rare paediatric diseases.**

	Panellists: morning session chairpersons, **Peter Lack**, CEO of Childhood Cancer Switzerland; **Dr James Barnes**, Director of Regulatory Policy & Advocacy, Vertex Pharmaceuticals, UK; **Ms Tamara Lewicky**, Director, Global Regulatory Affairs, Quintiles, Spain; **Mr Robert Morgan**, Senior Regulatory Affairs Director, Shire Plc, Ireland and **Dr.med. Klaus Rose**, klausrose Consulting, Switzerland
12:30 - 14:30	LUNCH, including a one hour guided tour of Sant Pau Recinte Modernista building from 13:30 – 14:30

	Afternoon session co-chaired by: ***Dr.med. Immanuel Barth,*** *Member of PDCO, Paul-Ehrlich-Institut, Federal Institute for Vaccines and Biomedicines, Germany* ***Dr Alexander Natz,*** *Attorney at law, Secretary General, EUCOPE*
14:30 – 17:00	***Afternoon Session: Exploring concrete solutions***
14:30 – 15:00	Introduction on the breakout sessions by the afternoon chairpersons
15:00 – 16:00	**Breakout session 1: Patient engagement of parents and young patients** (conditions of success and type of training needed) Moderator and rapporteur: **Ms Kerry Leeson-Beevers**, Alternate patient representative on the PDCO for EURORDIS, National Development Manager & Project Lead for Breaking Down Barriers, Alström Syndrome UK & **Mr Olav Veldhuizen**, Project Manager, John Walton Muscular Dystrophy Research Centre, Newcastle Univ., UK
15:00 – 16:00	**Breakout session 2: How extrapolation and innovative small populations designs (SPCT) can help?** Moderator and rapporteur: **Dr Cécile Ollivier**, Paediatric Scientific Officer, European Medicines Agency (EMA) & **Prof Gérard Pons**, Head Clinical Paediatric Pharmacology, Inserm UMR1129 - CEA, Gif sur Yvette, MSWG & Chair Extrapolation WG –EMA, France
15:00 – 16:00	**Breakout session 3: How to make the PIP work for rare diseases? What can industry, in conjunction with regulators and paediatricians, do to perform better studies on children?** Moderator and rapporteur: **Peter Lack**, CEO of Childhood Cancer Switzerland & **Dr Solange Rohou**, Director Regulatory Affairs, AstraZeneca, UK
16:00 – 16:45	**Feedback session with panel of discussants** Led by the afternoon chairpersons
16:45 - 17:00	**Concluding remarks : Prof Bruno Sepodes**, Prof of Pharmacology and Pharmacotherapy, University of Lisbon/ Chair of the COMP, CHMP Member, EMA
17:00	Meeting ends