**25th Workshop of the EURORDIS Round Table of Companies (ERTC)**

*Healthcare Companies & European Reference Networks: Expectations & Potential for Collaboration*

Tuesday 26 September, 2017 (09:00 to 17:00) - UAB - Casa Convalescència, Barcelona, Spain

### PROGRAMME

**Morning session co-chaired by:**

*Chairpersons: Ms. Claudia Crocione, Managing Director, HHT Europe, Italy & Pr. Josep Torrent-Farnell, Head of Medicines Division, CatSalut (Catalan Health Service), Generalitat de Catalunya, Spain*

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| 09:00 – 09:20 | Welcome introduction, setting the scene & goals for the day: A reminder of why we are all here. Our shared ambition for ERNs.  
Mr. Yann Le Cam, Chief Executive Officer, EURORDIS, France/Belgium |
| 09:20 – 09:35 | State of the art of ERNs: What they are (core responsibilities), how they are structured and organised and their overall vision  
Mr. Jaroslaw Waligora, Policy Officer, European Commission |
| 09:35 – 09:45 | Board of Member States Reflections  
Prof. Nicoline Hoogerbrugger, Chair, EC ERN Committee on Data Protection, Ethics & Informed Consent, The Netherlands |
| 09:45 – 09:55 | From concept to reality: European Reference Network for Hereditary Metabolic Diseases, MetabERN  
Dr. Maurizio Scarpa, Clinical lead for Rare Metabolic Diseases, Director of the Centre for Rare Diseases Helios Dr Horst Schmidt Clinic, Germany |
| 09:55 – 10:05 | Round table Q&A: Current state of play, needs and expectations, looking ahead  
Moderator: Pr. Josep Torrent-Farnell, Head of Medicines Division, CatSalut (Catalan Health Service), Generalitat de Catalunya, Spain  
Panelists:  
Mr. Jaroslaw Waligora, Policy Officer, European Commission  
Dr. Maurizio Scarpa, ERN Chairperson, Clinical lead for Rare Metabolic Diseases, Director of the Centre for Rare Diseases Helios Dr Horst Schmidt Clinic, Germany  
Prof. Nicoline Hoogerbrugger, Professor in Hereditary Cancer, Radboud University Medical Center, The Netherlands & Chair, EC ERN Committee on Data Protection, Ethics & Informed Consent |
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| 10:05 – 10:30 | What can ERNs bring to therapeutic development? - Patient Expectations: mapping of drug development pipeline in relation to ERN ‘touch points’ & The Patients’ Role & Expectations in ERNs  
Mr. Matt Bolz-Johnson, Healthcare & Research Director, EURORDIS, Germany (10’)
Dr. Virginie Hivert, Therapeutic Development Director at EURORDIS, France (10’)
Q&A 5’                                                   |
| 10:30 – 11:00 | Coffee break                                                                                       |
| 11:00 – 12:15 | Two case studies (20’ presentation + 15’ Q&A each):                                               |
|              | 1) Reflections on a European network and registry. Key learnings from a Company perspective on a public private partnership on Homocystinurias and Methylation Defects (E-HOD)  
Dr. Céline Plisson, Medical Director, Orphan Europe, France |
|              | 2) The collection of data through collaborative platforms (EUPID)                                  
Prof. Ruth Ladenstein, Professor of Paediatrics, Senior Consultant in Paediatric Oncology, Children’s Cancer Research Institute, Austria |
| 12:15 - 13:15 | Crossfire panel discussion: Expectations & Opportunities                                           
Moderator: Ms. Claudia Crocione, Managing Director, HHT Europe, Italy  
Panelists: Dr. Robert Bez, Director Medical, SMA Lead Europe and Canada, Biogen, Switzerland  
Dr. Massimo Boriero, Head of Europe - Commercial, Spark Therapeutics, Italy  
Dr. Paul Robinson, Executive Director, Scientific Medical & Patient Perspective, MSD, UK  
Mr. Dion Warren, Vice-President, Head of Europe Oncology Business Unit, Takeda, Switzerland  
Dr. Hartmann Wellhoefer, Vice President and Head Rare Diseases & Internal Medicine, Shire, Switzerland |
| 13:15 - 14:30 | Lunch                                                                                              |
| 14:30 – 17:00 | Afternoon Session: Potential for collaboration                                                       
Afternoon session co-chaired by:  
Chairpersons: Mr. Juan Fuertes, Advocacy and Access Manager, PHA Europe, Spain &  
Dr. Alexander Natz, Secretary General, EUCOPE, Belgium |
| 14:30 – 14:50 | Introduction to the breakout sessions                                                                 
Moderators: Mr. Juan Fuertes, Advocacy and Access Manager, PHA Europe, Spain &  
Dr. Alexander Natz, Secretary General, EUCOPE, Belgium  
Chairs of the breakout sessions (5’ each) |
| 15:00 – 16:00 | Breakout session 1: Key questions around disease understanding and data generation                   
Moderator: Ms. Victoria Hedley, RD-ACTION Thematic Coordinator, Newcastle University John Walton Muscular Dystrophy Research Centre, MRC Centre for Neuromuscular Diseases, Institute of Genetic Medicine, UK  
Rapporteur: Ms. Avril Daly, Chief Executive Officer, Retina International, Ireland |
| 15:00 – 16:00 | **Breakout session 2:** Key questions around sustainability  
Moderator: **Ms. Samantha Parker**, Chief Patient Affairs/Health Policies Officer, Lysogene, France  
Rapporteur: **Mr. Dimitrios Athanasiou**, Muscular Dystrophy Association Hellas, Greece |
| 15:00 – 16:00 | **Breakout session 3:** Key questions around exchange of expertise between healthcare companies and ERNs  
Co-Moderators: **Mr. Maarten Kraaijenhagen**, Regional Rare Disease Medical Lead & **Ms. Katherine Beaverson**, Patient Advocacy Lead, Rare Disease Research, Pfizer  
Rapporteur: **Mr. Chris Sotirelis**, UK Thalassemia Society, UK |
| 16:00 – 16:50 | **Feedback session with panel of rapporteurs**  
Moderated by the afternoon chairpersons  
Rapporteurs:  
Breakout session 1: **Ms. Avril Daly**, CEO, Retina International, Ireland  
Breakout session 2: **Mr. Dimitrios Athanasiou**, Muscular Dystrophy Association Hellas, Greece  
Breakout session 3: **Mr. Chris Sotirelis**, UK Thalassemia Society, UK |
| 16:50 - 17:00 | **Concluding remarks:**  
**Mr. Yann Le Cam**, Chief Executive Officer, EURORDIS, France/Belgium  
**Dr. Maurizio Scarpa**, ERN Chairperson, Clinical lead for Rare Metabolic Diseases, Director of the Centre for Rare Diseases Helios Dr Horst Schmidt Clinic, Germany  
**Dr. Diego Ardigo**, Project Leader of advanced therapy medicinal products (ATMP) and biologics, Chiesi Farmaceutici S.p.A., Italy |
| 17:00 | Meeting ends |