



## 25<sup>th</sup> 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*

##### *Morning Session: Exploring opportunities*

09:00 – 09:20	<p><b>Welcome introduction, setting the scene &amp; goals for the day:</b> A reminder of why we are all here. Our shared ambition for ERNs.</p> <p><b>Mr. Yann Le Cam</b>, Chief Executive Officer, EURORDIS, France/Belgium</p>
09:20 – 09:35	<p><b>State of the art of ERNs:</b> What they are (core responsibilities), how they are structured and organised and their overall vision</p> <p><b>Mr. Jaroslaw Waligora</b>, Policy Officer, European Commission</p>
09:35 – 09:45	<p><b>Board of Member States Reflections</b></p> <p><b>Prof. Nicoline Hoogerbrugger</b>, Chair, EC ERN Committee on Data Protection, Ethics &amp; Informed Consent, The Netherlands</p>
09:45 – 09:55	<p><b>From concept to reality: European Reference Network for Hereditary Metabolic Diseases, MetabERN</b></p> <p><b>Dr. Maurizio Scarpa</b>, Clinical lead for Rare Metabolic Diseases, Director of the Centre for Rare Diseases Helios Dr Horst Schmidt Clinic, Germany</p>
09:55 – 10:05	<p><b>Round table Q&amp;A:</b> Current state of play, needs and expectations, looking ahead</p> <p>Moderator: Pr. Josep Torrent-Farnell, Head of Medicines Division, CatSalut (Catalan Health Service), Generalitat de Catalunya, Spain</p> <p>Panelists:</p> <p><b>Mr. Jaroslaw Waligora</b>, Policy Officer, European Commission</p> <p><b>Dr. Maurizio Scarpa</b>, ERN Chairperson, Clinical lead for Rare Metabolic Diseases, Director of the Centre for Rare Diseases Helios Dr Horst Schmidt Clinic, Germany</p> <p><b>Prof. Nicoline Hoogerbrugger</b>, Professor in Hereditary Cancer, Radboud University Medical Center, The Netherlands &amp; Chair, EC ERN Committee on Data Protection, Ethics &amp; Informed Consent</p>



10:05 – 10:30	<p><b>What can ERNs bring to therapeutic development? - Patient Expectations:</b> mapping of drug development pipeline in relation to ERN ‘touch points’ &amp; The Patients’ Role &amp; Expectations in ERNs</p> <p><b>Mr. Matt Bolz-Johnson</b>, Healthcare &amp; Research Director, EURORDIS, Germany (10’)</p> <p><b>Dr. Virginie Hivert</b>, Therapeutic Development Director at EURORDIS, France (10’)</p> <p>Q&amp;A 5’</p>
10:30 – 11:00	<i>Coffee break</i>
11:00 – 12:15	<p><b>Two case studies (20’ presentation + 15’ Q&amp;A each):</b></p> <ol style="list-style-type: none"> <li>1) <b>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)</b> <b>Dr. Céline Plisson</b>, Medical Director, Orphan Europe, France</li> <li>2) <b>The collection of data through collaborative platforms (EUPID)</b> <b>Prof. Ruth Ladenstein</b>, Professor of Paediatrics, Senior Consultant in Paediatric Oncology, Children’s Cancer Research Institute, Austria</li> </ol>
12:15 - 13:15	<p><b>Crossfire panel discussion: Expectations &amp; Opportunities</b> Moderator: Ms. Claudia Crocione, Managing Director, HHT Europe, Italy Panelists: <b>Dr. Robert Bezar</b>, Director Medical, SMA Lead Europe and Canada, Biogen, Switzerland <b>Dr. Massimo Boriero</b>, Head of Europe - Commercial, Spark Therapeutics, Italy <b>Dr. Paul Robinson</b>, Executive Director, Scientific Medical &amp; Patient Perspective, MSD, UK <b>Mr. Dion Warren</b>, Vice-President, Head of Europe Oncology Business Unit, Takeda, Switzerland <b>Dr. Hartmann Wellhoefer</b>, Vice President and Head Rare Diseases &amp; Internal Medicine, Shire, Switzerland</p>
13:15 - 14:30	<i>Lunch</i>
	<p style="text-align: center;"><b>Afternoon session co-chaired by:</b></p> <p><i>Chairpersons: Mr. Juan Fuertes, Advocacy and Access Manager, PHA Europe, Spain &amp; Dr. Alexander Natz, Secretary General, EUCOPE, Belgium</i></p>
14:30 – 17:00	<i>Afternoon Session: Potential for collaboration</i>
14:30 – 14:50	<p><b>Introduction to the breakout sessions</b> Moderators: Mr. Juan Fuertes, Advocacy and Access Manager, PHA Europe, Spain &amp; Dr. Alexander Natz, Secretary General, EUCOPE, Belgium Chairs of the breakout sessions (5’ each)</p>
15:00 – 16:00	<p><b>Breakout session 1: Key questions around disease understanding and data generation</b> Moderator: <b>Ms. Victoria Hedley</b>, RD-ACTION Thematic Coordinator, Newcastle University John Walton Muscular Dystrophy Research Centre, MRC Centre for Neuromuscular Diseases, Institute of Genetic Medicine, UK Rapporteur: <b>Ms. Avril Daly</b>, Chief Executive Officer, Retina International, Ireland</p>



15:00 – 16:00	<p><b>Breakout session 2: Key questions around sustainability</b></p> <p>Moderator: <b>Ms. Samantha Parker</b>, Chief Patient Affairs/Health Policies Officer, Lysogene, France</p> <p>Rapporteur: <b>Mr. Dimitrios Athanasiou</b>, Muscular Dystrophy Association Hellas, Greece</p>
15:00 – 16:00	<p><b>Breakout session 3: Key questions around exchange of expertise between healthcare companies and ERNs</b></p> <p>Co-Moderators: <b>Mr. Maarten Kraaijenhagen</b>, Regional Rare Disease Medical Lead &amp; <b>Ms. Katherine Beaverson</b>, Patient Advocacy Lead, Rare Disease Research, Pfizer</p> <p>Rapporteur: <b>Mr. Chris Sotirelis</b>, UK Thalassaemia Society, UK</p>
16:00 – 16:50	<p><b>Feedback session with panel of rapporteurs</b></p> <p>Moderated by the afternoon chairpersons</p> <p>Rapporteurs:</p> <p>Breakout session 1: <b>Ms. Avril Daly</b>, CEO, Retina International, Ireland</p> <p>Breakout session 2: <b>Mr. Dimitrios Athanasiou</b>, Muscular Dystrophy Association Hellas, Greece</p> <p>Breakout session 3: <b>Mr. Chris Sotirelis</b>, UK Thalassaemia Society, UK</p>
16:50 - 17:00	<p><b>Concluding remarks:</b></p> <p><b>Mr. Yann Le Cam</b>, Chief Executive Officer, EURORDIS, France/Belgium</p> <p><b>Dr. Maurizio Scarpa</b>, ERN Chairperson, Clinical lead for Rare Metabolic Diseases, Director of the Centre for Rare Diseases Helios Dr Horst Schmidt Clinic, Germany</p> <p><b>Dr. Diego Ardigo</b>, Project Leader of advanced therapy medicinal products (ATMP) and biologics, Chiesi Farmaceutici S.p.A., Italy</p>
17:00	Meeting ends