

**5<sup>TH</sup> EUROPEAN CONFERENCE ON RARE DISEASES – ECRD 2010 IN KRAKOW**
**SECOND DAY (MAY 14TH)**

Time	Sessions
8.30 to 9.30 am	<b>Registration</b>
9.30 am to 10.30 am session 1 5 languages	Opening Session <ul style="list-style-type: none"> <li>▪ <i>Mr Torben Grønnebaek, co-chair of the programme committee</i></li> <li>▪ <i>Mr Marek Twardowski, Undersecretary of State, Ministry of Health Poland</i></li> <li>▪ <i>Mrs Consuelo Sanchez Naranjo, Undersecretary of State, Ministry of Health, Spain (invited)</i></li> <li>▪ <i>Dr Andrzej Rys, Director of Public Health, European Commission</i></li> </ul>
	<ul style="list-style-type: none"> <li>▪ <b>Welcome Coffee</b></li> </ul>
11.00 am to 1.00 pm session 2 5 languages	Theme 1: National Plan and Strategies for Rare Diseases  <i>The Dynamic of National Initiatives for Rare Diseases</i>  Co-chairs: <ul style="list-style-type: none"> <li>▪ Prof Josep Torrent i Farnell, COMP, Fundacio Doctor Robert</li> <li>▪ Mrs Avril Daly, Genetic and Rare Disorders Organisation, Ireland (invited)</li> </ul> Where are we? <ul style="list-style-type: none"> <li>▪ Terkel Andersen, President of Eurordis</li> </ul> State of the art of services in Europe: where are the problems? <ul style="list-style-type: none"> <li>▪ Dr Ségolène Aymé, Leader of the Task Force on Rare Diseases</li> </ul> The German plan for rare diseases: a development in progress <ul style="list-style-type: none"> <li>▪ Prof Dr. Johann Matthias Graf von der Schulenburg, Director, Forschungsstelle für Gesundheitsökonomie, Leibnitz Universität</li> </ul>
1.00 pm to 2.00 pm	<ul style="list-style-type: none"> <li>▪ <b>Lunch break</b></li> </ul>
2.00 pm to 3.30 pm session 3 5 languages	Theme 2: Centres of Expertise and European Reference Networks  <i>The Added Value of Centres of Expertise</i>  Co-chairs <ul style="list-style-type: none"> <li>▪ Dr Edmund Jessop, Medical adviser, NHS National Commissioning Group, United Kingdom</li> <li>▪ Jose Robalo, DGS, Portugal (invited)</li> </ul> Introduction <ul style="list-style-type: none"> <li>▪ Yann Le Cam, C.E.O Eurordis: Definitions, expectations and roles of Centres of Expertise</li> </ul> Round table Panellists: <ul style="list-style-type: none"> <li>▪ Prof Fons Gabreels, Department of Child Neurology, Radboud University Hospital, The Netherlands</li> <li>▪ Stein Are Aksnes, Directorate for Health and Social Affairs, Norway</li> <li>▪ Guillaume Le Henanff, Ministry of Health, France (invited)</li> <li>▪ Cristina Rusu, University of Medicine and Pharmacy, Lasi, Romania</li> </ul>

<p>2.00 pm to 3.30 pm session 9 English and Polish</p>	<p>Theme 3: Science from the bench to the bed side</p> <p><i>Improving Access to Orphan Drugs</i></p> <p>Co-Chairs:</p> <ul style="list-style-type: none"> <li>▪ Prof Hans Georg Eichler, Senior Medical Officer, European Medicines Agency</li> <li>▪ Lia van Ginneken, EMP, Netherlands (invited)</li> </ul> <p>Speakers</p> <ul style="list-style-type: none"> <li>▪ Dr Claudia Wild, Ludwig Boltzman Institute of Health Technology Assessment, Vienna, Austria : EUnetHTA working group on Monitoring emerging/new technology development and prioritisation of HTA:</li> <li>▪ Dr Andras Fehervary, Novartis: The point of view of the industry</li> <li>▪ Prof Hans Georg Eichler, Senior Medical Officer, European Medicines Agency</li> </ul>
<p>2.00 pm to 3.30 pm session 5 English and Polish</p>	<p>Theme 4: Information and Medical Education</p> <p><i>Help Lines for Rare Diseases</i></p> <p>Chairperson: Rosa Sanchez Vega, FEDER, Spain</p> <ul style="list-style-type: none"> <li>▪ Thomas Heuyer ,MRIS, France</li> <li>▪ Henriette Hutters, CSH, Denmark</li> <li>▪ Peter Saltonstall, NORD, USA (invited)</li> </ul>
<p>2.00 pm to 3.30 pm Session 18 English and Polish</p>	<p>Theme 7: Rare Diseases in Central / Eastern Europe</p> <p><i>Focus on national plans and Centres of Expertise in Eastern countries</i></p> <p>Co-chairs</p> <ul style="list-style-type: none"> <li>▪ Jakub Adamski, Ministry of Health, Poland</li> <li>▪ Rumen Stefanov, Bulgarian Association for Promotion of Education and Science (BAPES)</li> </ul> <p>Speakers:</p> <ul style="list-style-type: none"> <li>▪ Gabor Pogany – Hufendis, Hungary</li> <li>▪ Dorica Dan – Prader Willi, Romania</li> <li>▪ Dr Mirando Mrcic, Croatian Society for Patients with RD</li> </ul>
<p>3.30 pm to 4.00 pm</p>	<ul style="list-style-type: none"> <li>▪ <b>Coffee break</b></li> </ul>
<p>4.00 pm to 5.30 pm session 7 English and Polish</p>	<p>Theme 2: Centres of Expertise and European Reference Networks</p> <p><i>The Added Value of European Reference Networks?</i></p> <p>Co-chairs:</p> <ul style="list-style-type: none"> <li>▪ Antoni Monserrat, DG Sanco, EC</li> <li>▪ Dr Frits Lekkerkerker, WGM, Netherlands</li> </ul> <p>Speakers</p> <ul style="list-style-type: none"> <li>▪ Dr Frederic Sicard (Co-chair High Level Group on Care and Medical Services)</li> <li>▪ Prof Krystyna Chrzanowska, Department of Medical Genetics, The Children's Memorial health Institute Poland : the Dyscerne Project</li> <li>▪ Prof Thomas Wagner, Johann-Wolfgang Goethe University, Frankfurt, Department of Internal Medicine : ECORN-CF</li> </ul>

<p>4.00 pm to 5.30 pm session 8 5 languages</p>	<p>Theme 3: Science from the bench to the bed side</p> <p><i>Making the Best use of Funds for Genetic Testing</i></p> <p>Co-chairs</p> <ul style="list-style-type: none"> <li>▪ Pol Gerits, Federal Service of Health, Food Chain Safety and Environment, &amp; Europlan, Belgium (invited)</li> <li>▪ Prof Milan Macek, Department of Biology and Medical Genetics, Charles University Prague, Czech Republic</li> </ul> <p>Speakers</p> <ul style="list-style-type: none"> <li>▪ Prof Bruno Dallapiccola, CSS Mendel Universita di Roma: Not all tests are useful. Are we wasting resources?</li> <li>▪ Prof David.E Barton, National Centre for Medical Genetics, Ireland: Cross Border genetic testing</li> <li>▪ Prof. Anil Mehta, University of Dundee, UK: Undiagnosed rare diseases, the EuroCareCF project</li> </ul>
<p>4.00 pm to 5.30 pm session 4 English and Polish</p>	<p>Theme 3: Science from the bench to the bed side</p> <p><i>Orphan Drug Development, Paediatric Investigation Plans and Advanced therapies</i></p> <p>Co-chairs:</p> <ul style="list-style-type: none"> <li>▪ Michele Lipucci di Paola, Committee of Advanced Therapies, European Medicines Agency, and Eurordis</li> <li>▪ Prof Josep Torrent i Farnell, COMP, Fundacio Doctor Robert</li> </ul> <p>Speakers</p> <ul style="list-style-type: none"> <li>▪ Dr Kerstin Westermark, COMP, European Medicines Agency: the EMEA workshop May 2010 for the 10th anniversary of the Orphan Drug Regulation and challenges for the future.</li> <li>▪ Dr Fernando Andrés Trelles , PDCO, European Medicines Agency: Paediatric Committee and Paediatric Investigation Plan: What is relevant to rare diseases?</li> <li>▪ Dr Fabrizia Bignami, Eurordis : Committee for Advanced Therapies: What is relevant to rare diseases?</li> </ul>
<p>4.00 pm to 5.30 pm Session 18 bis English and Polish</p>	<p>Theme 7: Rare Diseases in Central / Eastern Europe</p> <p><i>Focus on access to cross border care</i></p> <p>Co-chairs:</p> <ul style="list-style-type: none"> <li>▪ Miroslaw Zielinski (National Forum for therapies of rare diseases Poland, President)</li> <li>▪ Prof Anna Tylki Szymanska, Children's Memorial Health Institute, Metabolic Diseases</li> </ul> <p>Speakers</p> <ul style="list-style-type: none"> <li>▪ Prof Paola Facchin, Veneto Region</li> <li>▪ Prof Jolanta Sykut-Cegielska, The Childrens Memorial Health Institute</li> <li>▪ Silvia Stuppäck, Central European Countries Plan Extensive Cooperation for Rare Diseases</li> </ul>
<p>5.30 pm to 6.30 pm session 11</p>	<p>Theme 8: Services to Patients, families and carers</p> <p><i>Poster session</i></p> <p>Co-chairs:</p> <ul style="list-style-type: none"> <li>▪ Dr Frits Lekkerkerker, WGM, Netherlands</li> <li>▪ Mrs Dorica Dan, PW Romania</li> </ul>

6.30 pm to 7.30 pm Session 12 5 languages	Welcoming Reception  <i>Mrs Maria Kaczynska (invited)</i> <i>Mrs Bozenna Dembowska, Polish member of the COMP, European Medicines Agency</i> <i>Mr Pawel Wojtowicz, Cystic Fibrosis Poland</i> <i>Mr Terkel Andersen (invited)</i>
8.30 pm to 22.30	▪ Welcome diner

### THIRD DAY (MAY 15TH)

9.00 am to 10.30 am Session 13 5 languages	Theme 3: Science from the bench to the bed side  <i>Research and Research Policy</i>  Co-chairs: <ul style="list-style-type: none"> <li>▪ Mr Patrick Kolar, Head of Unit DG RTDF.4 "Genomics and systems biology", EC</li> <li>▪ Dr Birgit Wetterauer, Federal Ministry of Education and Research, Germany (invited)</li> </ul> Speakers <ul style="list-style-type: none"> <li>▪ <a href="#">Determinants for research on rare diseases</a> Prof Francesc Palau, Scientific Director CIBERER, Spain</li> <li>▪ <a href="#">Predictors of orphan drug approval in the European Union</a> Dhr. drs Harald E. Heesmtra, Division of Pharmacoepidemiology and Pharmacotherapy, Utrecht University</li> <li>▪ <a href="#">Funding opportunities and impact on orphan drug development</a> Dr Sophie Koutouzo, Programme Coordinator, E-Rare</li> </ul>
9.00 am to 10.30 am Session 14 English only	Theme 3: Science from the Bench to the Bedside  <i>The International Classification of Diseases Revision</i>  Co-chairs: <ul style="list-style-type: none"> <li>▪ Dr Ségolène Aymé, Director of Orphanet</li> </ul> Speakers <ul style="list-style-type: none"> <li>▪ Dr Peter Robinson, Institute for Medical Genetics, Humboldt University, Berlin</li> <li>▪ Dr Jakob Robert, OMS Genève</li> <li>▪ Ana Rath, Orphanet</li> </ul>
9.00 am to 10.30 am Session 15 English and Polish	Theme 6: Policy Scenarii for Rare Diseases (Polka project)  <i>PlayDecide Session (interactive session with voting devices)</i>  Co-chairs: <ul style="list-style-type: none"> <li>▪ Lene Jensen, Rare Disorders Denmark</li> <li>▪ Anna Kole, Public Health Project Manager, Eurordis</li> </ul> "PlayDeciders" <ul style="list-style-type: none"> <li>▪ Anna Kole, Eurordis</li> <li>▪ Aiki Giannakopoulou, Ecsite</li> <li>▪ Louise Taylor, Orphanet</li> <li>▪ Matteo Merzagora</li> <li>▪ The audience</li> </ul>

<p>9.00 am to 10.30 am</p> <p>Session 16</p> <p>English only</p>	<p>Theme 6: Policy Scenarii for Rare Diseases (Polka project)</p> <p><i>PlayDecide Session (interactive session with voting devices)</i></p> <p>Co-chairs:</p> <ul style="list-style-type: none"> <li>▪ François Houÿez, Health Policy Director, Eurordis</li> </ul> <p>“PlayDeciders”</p> <ul style="list-style-type: none"> <li>▪ Andrea Bandelli</li> <li>▪ Michael Creek</li> <li>▪ Marta Fikus-Krynska, Kopernicus Science Centre, Poland</li> <li>▪ The audience</li> </ul>
<p>10.30 am to 11.00 am</p>	<ul style="list-style-type: none"> <li>▪ <b>Coffee break</b></li> </ul>
<p>11.00 am to 12.30 pm</p> <p>Session 17</p> <p>5 languages</p>	<p>Theme 2: Centres of Expertise and European Reference Networks</p> <p><i>Cross-border activities of Centres of Expertise</i></p> <p>Co-chairs:</p> <ul style="list-style-type: none"> <li>▪ John Dart, Debra, United Kingdom</li> <li>▪ Yolande Wagener, Ministry of Health, Luxembourg (invited)</li> </ul> <p>Speakers:</p> <ul style="list-style-type: none"> <li>▪ Antoni Montserrat Moliner, Policy Officer for Rare and Neurodevelopmental Diseases, European Commission</li> <li>▪ Dr Pohla Gubo The Epidermolysis Bullosa House in Salzburg</li> <li>▪ Dr Kate Bushby, TREAT NMD, Newcastle</li> </ul>
<p>11.00 am to 12.30 pm</p> <p>Session 19</p> <p>English and Polish</p>	<p>Theme 6: Policy Scenarii for Rare Diseases (Polka project)</p> <p><i>PlayDecide Session (interactive session with voting devices)</i></p> <p>Co-chairs:</p> <ul style="list-style-type: none"> <li>▪ Lene Jensen, Rare Disorders Denmark</li> <li>▪ Anna Kole, Public Health Project Manager, Eurordis</li> </ul> <p>“PlayDeciders”</p> <ul style="list-style-type: none"> <li>▪ Anna Kole, Eurordis</li> <li>▪ Aliki Giannakopoulou, Ecsite</li> <li>▪ Louise Taylor, Orphanet</li> <li>▪ Matteoa Merzagora</li> <li>▪ The audience</li> </ul>
<p>11.00 am to 12.30 pm</p> <p>Session 20</p> <p>English only</p>	<p>Theme 6: Policy Scenarii for Rare Diseases (Polka project)</p> <p><i>PlayDecide Session (interactive session with voting devices)</i></p> <p>Co-chairs:</p> <ul style="list-style-type: none"> <li>▪ François Houÿez, Health Policy Director, Eurordis</li> </ul> <p>“PlayDeciders”</p> <ul style="list-style-type: none"> <li>▪ Andrea Bandelli</li> <li>▪ Michael Creek</li> <li>▪ Marta Fikus-Krynska, Kopernicus Science Centre, Poland</li> <li>▪ The audience e</li> </ul>
<p>12.30 pm to 1.30 pm</p>	<ul style="list-style-type: none"> <li>▪ <b>Lunch break</b></li> </ul>

<p>1.30 pm to 3.00 pm</p> <p>Session 21 5 languages</p>	<p>Theme 2: Centres of Expertise and European Reference Networks</p> <p><i>Ultra Rare Diseases</i></p> <p>Co-chairs:</p> <ul style="list-style-type: none"> <li>▪ Peter Saltonstall, NORD</li> <li>▪ Yann Le Cam, CEO Eurordis</li> </ul> <p>Speakers</p> <ul style="list-style-type: none"> <li>▪ Marjet Stamsnijder, Progeria Family Circle, The Netherlands</li> <li>▪ Prof Hennekam, Marshall Smith group in the Netherlands</li> <li>▪ Annet van Betuw: Q11 network</li> </ul>
<p>1.30 pm to 3.00 pm</p> <p>Session 22 English only</p>	<p>Theme 3: Science from the bench to the bed side</p> <p><i>Clinical Trials, Involvement of Patients in Clinical Trials</i></p> <p>Co-chairs:</p> <ul style="list-style-type: none"> <li>▪ Dr Erik Tambuyzer, EBE/EuropaBio</li> <li>▪ Alastair Kent, Genetic Interest Group, United Kingdom</li> </ul> <p>Speakers</p> <ul style="list-style-type: none"> <li>▪ Richard West, Behcet United Kingdom</li> <li>▪ Greetje Goossens, European Myeloma Platform</li> <li>▪ Dr Erik Pelkmans, Tibotec</li> </ul>
<p>1.30 pm to 3.00 pm</p> <p>Session 23 English only</p>	<p>Theme 3: Science from the bench to the bed side</p> <p><i>Databases and Registries</i></p> <p>Co-chairs</p> <ul style="list-style-type: none"> <li>▪ Torben Groennebaek, Rare Disorders Denmark</li> <li>▪ Helmut Brand NLD Dg Sanco Public Health Genomic European Network (invited)</li> </ul> <p>Speakers</p> <p><a href="#">Introduction</a></p> <ul style="list-style-type: none"> <li>▪ Stuart Tanner, EuroWilson: Summary of EPPOSI, RDTF and ENCPP meetings (invited)</li> </ul> <p><a href="#">Three panellists</a></p> <ul style="list-style-type: none"> <li>▪ Prof. G.B.Landwehrmeyer, European Network for Huntington Disease</li> <li>▪ Elizabeth Hernberg-Stahl, Shire</li> <li>▪ Cees Smit, European Haemophilia Consortium</li> </ul>
<p>1.30 pm to 3.00 pm</p> <p>Session 24 English only</p>	<p>Theme 1: National Plan and Strategies for Rare Diseases</p> <p><i>Comparison of measures: what is happening?</i></p> <p>Co-chairs:</p> <ul style="list-style-type: none"> <li>▪ Dr Domenica Taruscio, Europlan leader, ISS, Italy</li> <li>▪ Dr Manuel Posada de la Paz, Instituto de Salud Carlos III, Spain (invited)</li> </ul> <p>Speakers</p> <p><a href="#">Evaluation of results</a></p> <ul style="list-style-type: none"> <li>▪ Laura Fregonese, Europlan and Rare Disease Task Force working group (invited)</li> </ul> <p><a href="#">Recommendations for national plans</a></p> <ul style="list-style-type: none"> <li>▪ Dr Edmund Jessop, National Commissioning Group NCG NHS, UK</li> </ul> <p><a href="#">Recommendations for national plans</a></p> <ul style="list-style-type: none"> <li>▪ Albert van der Zeijden, International Association of Patient Organisations and Europlan</li> </ul>

<p>1.30 pm to 3.00 pm session 10 English only</p>	<p>Theme 4: Information and Medical Education</p> <p><i>Medical Education: the Role of Patients' Organisations</i></p> <p>Co-Chairs:</p> <ul style="list-style-type: none"> <li>▪ Prof Tomasz Grodzicky, Dean of the Faculty of Medicine, Krakow</li> <li>▪ Marie Louise Borg, Ministry of Social Policy, Malta (invited)</li> </ul> <p>Speakers</p> <ul style="list-style-type: none"> <li>▪ Rainald von Gizycki, Retina Europe</li> <li>▪ Prof. Wojciech Cichy, Medical University in Poznan.</li> <li>▪ Christel Nourissier, Prader Willi France</li> </ul>
<p>3.15 pm to 4.15 pm Session 25 5 languages</p>	<p>Theme 5: the European Committee for Rare Diseases</p> <p>Co-chair:</p> <ul style="list-style-type: none"> <li>▪ Mr Terkel Endersen, President Eurordis</li> </ul> <p>Speakers</p> <ul style="list-style-type: none"> <li>▪ Dr Andrzej Rys, Director of Public Health, European Commission</li> <li>▪ Antoni Montserrat Moliner, Policy Officer for Rare and Neurodevelopmental Diseases, European Commission</li> <li>▪ Members of the Committee of Experts for Rare Diseases</li> </ul> <p>Final talk: Steffen Sucher, Germany</p>
<p>4.15 pm</p>	<ul style="list-style-type: none"> <li>▪ <b>Conference closes</b></li> </ul>