Thursday 30 May

**Satellite Meetings**

10:00 - 12:30 - Room Boreas
Meeting of patient organisations from Croatia and neighbouring countries
Organised by the Croatian Alliance for Rare diseases
Language: Croatian

14:00 - 18:00 - Room Lavantera
CNA Meeting
(closed meeting – by invitation only)

14:00 - 18:00 - Room Mistral A
Learning from Each Other Workshop
Social Media Best Practices & RareConnect
- RareConnect Update
- RareConnect Social Media and Communication Outreach
- Moderator Presentation
- Round Table with current members and moderators
- Future RareConnect upgrades

**Coffee break**
- Case study: best practice use of social media by a rare disease patient group
- Discussion on benefits of social media to your patient group or a family living with a RD
- Capacity Building: Hootsuite for Twitter, Facebook interests lists, use social media to follow clinical trials
- How to use information gathered on social media
- Wrap Up

Friday 31 May - Room Mistral B+C

09:00 - 10:30 EURORDIS General Assembly (members only)
Business Agenda & Election of Directors

10:30 - 11:00 Coffee break

Plenary session
National Plans: the state of the art in 2013 across Europe
Chairman: John Dart, Debra International & EURORDIS Deputy General Secretary

11:00 - 11:10 Opening speech, Mirando Mrsic, Minister of Labour & Pension System, Croatia

11:10 - 11:20 Welcome speech, Terkel Andersen, President EURORDIS

11:20 - 11:45 Progress Report: the National Plan in Croatia
Prof. Dr Ingeborg Barišic, President of Croatian National Plan Committee

11:45 - 12:10 Making progress in National Plans: Comparison of different approaches in Denmark, Germany, and the UK
Lene Jensen, Rare Disorders Denmark
On behalf of the EURORDIS-EUROPLAN Advisor Committee.

12:10 - 12:35 Moving forward: Key common issues that need to be taken into account in National Plans
Christel Nourissier, EUCERD patient representative & EURORDIS, France
On behalf of the EURORDIS-EUROPLAN Advisor Committee.

12:35 - 13:00 Best practices in the Management, Funding, Indicators and Monitoring of a National Plan
Dorica Dan, EUCERD patient representative & EURORDIS, Romania
On behalf of the EURORDIS-EUROPLAN Advisor Committee.

13:00 - 14:30 Lunch, Mozaik restaurant
**Workshop 1** Room: Mistral A

**Introduction to National Plans**

Moderator: Simona Bellagambi, UNIAMO, F.I.M.R, Italy

Developing National Plans for Rare Diseases is a long lasting dynamic process. Each European country has a different story to tell. Listen to first-hand experiences from fellow patients active in shaping National Plans in their countries. In spite of the differences, lessons can be learned across the spectrum on why National Plans are needed, how they can be implemented, and what role patients can play in their development & management. This workshop is especially intended for those patients who are not yet involved in National Plans development & management and wish to do so.

*Short presentations of national experiences with National Plans*

Lene Jensen, Rare Disorders Denmark

Vlasta Zmazek, Croatian Alliance for Rare Diseases

**Workshop 2** Room: Mistral B

**Funding for National Plans**

Moderator: Valentina Bottarelli, EURORDIS

Funding Rare Disease National Plans in times of economic crisis causes headaches. This workshop will shed light on the current state of play (how National Plans are actually funded) and the potential sources of funding. Speakers will also help the audience think creatively about the funding of their Plan, including looking into realistic solutions to make a Plan work without a dedicated budget.

*EU sources of funding: opportunities to support National Plans for RD*

Antoni Montserrat, European Commission, DG SANCO

*Squaring the circle: how EU Member States fund National Plans*

Domenica Tarascio, ISS, Italy

**Workshop 3** Room: Mistral C

**Newborn screening & Genetic testing**

Moderator: Dimitrios Synodinos, Greek Alliance for Rare Diseases, Greece

Newborn screening and genetic testing have become a topic of controversy, because of the costs involved, the follow up & treatments, false positives and ethical issues. In this workshop, patients and professionals will discuss the utility, care and follow up, the costs and the limits of these tools, as well as the consequences for families & patients.

*Benefits and risks of Newborn screening from medical, societal and ethical points of view*

Helena Kaariainen, EUCERD Vice Chair, Finland

*Practical aspects of implementing neonatal screening programs*

Martina Cornel, VLirne, The Netherlands

**Workshop 4** Room: Boreas

**Rare Disease Patient Registries**

Moderator: Twetta Schyns, European Network For Research on Alternating Hemiplegia

Rare Disease Patient Registries constitute key instruments for increasing knowledge on rare diseases: the results of the EPIRARE surveys set the ground for the development of the EUCERD recommendation on the subject and the foundation of the European Rare Diseases Registration Platforms.

*Presentation of the outcomes of the EPIRARE professional’s survey*

Luciano Vittozzi, Istituto Superiore di Sanità, Italy, EPIRARE coordinator

*Presentation of the outcomes of the EPIRARE patient’s survey*

Monica Ensini, EURORDIS, EPIRARE WP leader

**EUCERD Draft recommendations on registries and data collection for Rare Diseases**

Ségolène Aymé, EUCERD Chair, France

**Panel Discussion**

Friday 31 May

14:30 - 17:00

**15:45 - 16:15** Coffee break

Ingrid Jageneau, RaDiOrg Belgium

Lily Cannon, Cyprus Alliance for Rare Disorders

Panel discussion: What are the 10 key points to address when developing a National Plan?

Thinking out of the box: National Plans without dedicated funding

Yann Le Cam, EURORDIS Chief Executive Officer & EUCERD Vice-Chair

Alternative sources of funding

Dorica Dan, RONARD, EURORDIS, Romania

A patient view on ethics and governance in screening and advanced genetic testing

Cor Oosterwijk, VSOP, Netherlands

Comparative experience on lysosomal diseases across Europe

Anne Sophie Lapointe, Vaincre Les Maladies Lysosomales, France

**18:30 - 19:30** Welcome Reception at the Agora Bar - Hotel Rixos Libertas
Workshop 5 Room: Mistral A
European Reference Networks & Centres of Expertise
Moderator: Bianca Pizzera, Italy
IPOPI & EUCERD
The concepts of Centres of Expertise and European Reference Networks have been analysed and developed since 2005 and are now key elements of the EU strategy on rare diseases and in National Plans for Rare Diseases.
EUCERD Recommendations on Quality Criteria for Centres of Expertise for Rare Diseases in Member States
Ségolène Aymé, EUCERD Chair, France
Case study: Alstrom Clinics
Kay Parkinson, Alström Syndrome UK
Policy recommendations for Rare Disease Centres of Expertise
Ahmed Syed, NHS Commissioning Board for rare diseases / specialised services, Consultant for the Polka project, UK
11:00 - 11:30 Coffee break
EUCERD Draft Recommendation on European Reference Networks
Stephen Lynn, University of Newcastle, UK
Case study: ECORN CF
Daniela d’Alquen, Kinderklinik Würzburg, Germany

Workshop 6 Room: Mistral B
Specialised Social Services: need, policy, case studies
Moderator: Vlasta Zmazek, Croatian Alliance for Rare Diseases
Specialised Social Services are essential to improve the well-being and the health of people living with rare diseases. However, these services are still scarce and often face sustainability difficulties. How can we advocate for the creation and sustainability of these services? This workshop addresses these issues and presents a set of good practices that are crucial for the start-up / management of Specialised Social Services.
EUCERD Joint-Action Work Package on Specialised Social Services and Integration of RDs into Social Policies: state of the art
Raquel Castro, EURORDIS
Reinforcing the need for Specialised Social Services
Donica Dan, EUCERD. EURORDIS & RONARD, Romania
Guiding Principles for Specialised Social Services
Raquel Castro, EURORDIS
Introducing social services into National Plans: French example
Christel Nourissier, EUCERD & EURORDIS, France
11:00 - 11:30 Coffee break
Frambu Resource Centre
Lisen Mohr, Norway
Serious Fun Camps
Terry Dignan, European Network of Therapeutic Recreation Programmes, Ireland
Prader Willi Group Homes, Germany
Norbert Hildebeuck-Stuntebeck, Diakonische Stiftung Wittekindshof, Germany

Workshop 7 Room: Mistral C
Off-label use in Rare Diseases
Moderator: François Houÿez, EURORDIS
EURORDIS is conducting a survey on the off-label use of medicines in rare diseases. medicine prescribed for a use different from what is authorised on the label). Results of the pilot survey will be presented and the workshop will discuss launching the survey on a broader scale.
Pilot Survey on Off-label use: Results
Rob Camp, EURORDIS
Next Steps: large scale collection of Off-label use data
Richard West, Behcet's Society UK and EURORDIS DITA Task Force, UK
How French authorities are now regulating off-label use
Claudie Baleydidier, Friedrich Ataxia, France
11:00 - 11:30 Coffee break
Medicines, side effects & patient safety
Since 1995 the regulation of medicines has introduced more measures to improve the safety of medicines, including the Pharmacovigilance legislation. New major changes are of interest to patients and to their organisations in particular. Learn what role you can play.
EU Directive on Pharmacovigilance and the role of patients’ organisations
François Houÿez, EURORDIS
MPS Society UK role in Fabrazyme®/Cerezyme® shortages
Christine Lavery, MPS Society UK
13:00 - 14:00 Lunch - Mozaik Restaurant, Rixos Libertas
Workshop 8 Room: Boreas
European Rare Diseases Registration Platform
Moderator: Domenica Tarascio, ISS, Italy
One strategic objective of the European Commission is the creation of a European Rare Diseases Registration Platform providing common services and tools for the existing (and future) rare diseases registries in the European Union.
Data and cues for a European Rare Diseases Registration Platform
Fabrizio Bianchi, IFC-CNR Pisa, Italy, EPIRARE WP leader
The new Data Protection Regulation and its implications for registries
David Townsend, Maastricht University, The Netherlands, EPIRARE WP leader
Policy scenarios on data collection of Rare Disease patients across Europe
Antoni Montserrat, European Commission, DG SANCO
European Platform for Rare Diseases registration
Lauria Gribaldo, Joint Research Centre, Institute for Health & Consumer Protection, Ispra, Italy, European Commission
Open discussion
**Workshop 9** Room: Mistral A

DITA Task force meeting

Moderator: Rob Camp, EURORDIS/ATAC, USA

Members of EURORDIS Drug Information, Transparency & Access Task Force* meet with other EURORDIS members: How do we contribute to evaluating the impact of medicines in patients’ life?

**Patient-focused endpoints in the nitisinone study**

Oliver Timmis, Alkaptonuria Society, UK

**How do we help patients with treatment access issues?**

Research on Natural, Alternative, Complementary or Traditional treatments (NATC) for rare diseases

Rainald von Gizycki, Pro Retina, Germany

**How can a patient organisation support individual care plans for patients?**

The example of the complex case managers in the AFM

Béatrice de Montleau, AFM/EURORDIS, France

---

**Workshop 10** Room: Mistral B

Social & medical services initiated by patient organisations

Moderator: John Datt, Debra Int., EURORDIS

Social & medical services are frequently run by national/regional authorities, private foundations or NGOs. Nonetheless, patient organisations have an important role to play in the provision of Social & Medical services for people living with rare diseases. This workshop is dedicated to showcasing some European role model services idealised, created and sustained by patient organisations with the goal of attending the needs manifested by patients, families and carers in their daily lives.

**How can a patient organisation support individual care plans for patients?**

The example of the complex case managers in the AFM

Béatrice de Montleau, AFM/EURORDIS, France

---

**Workshop 11** Room: Mistral C

Improving Access to Orphan Medicinal Products (OMP)

Moderator: Birthe Holm, Rare Disorders Denmark

Some States experience or fear reduced access to OMP due to the economic crisis. As more, often high cost, OMP get approved, the impact on national budgets is under greater scrutiny for health authorities and payers. Improving access to OMP is a priority in the Council Recommendation of 2009 and should also be one in National Plans. This session presents concepts and proposals promoted by EURORDIS and their translation into official recommendations, particularly around the Clinical Added Value of OMP; the dialogue between EMA and national HTA; the new mechanism for coordinated access using the European Transparent Value Framework, and possible future policies, such as coordinated price negotiations, market entry agreement, and differential pricing. This workshop will help patient advocates to identify & discuss key measures to embed in National Plans to improve patient access.

**Overview of actions to improve access to OMP in Europe**

Yann Le Cam, EURORDIS Chief Executive Officer & EUCERD Vice Chair

**EUCERD recommendation for a CAVOMP Information Flow,**

Wills Hughes-Wilson, SOBI, Sweden

**The Common European Transparent Value Framework for Coordinated Access to OMP: new European collaborations, challenges & solutions**

Flaminia Macchia, EURORDIS

---

**Workshop 12** Room: Boreas

Standards of Diagnosis & Care, Therapeutic Education

Moderator: Geske Wehr, ENI, Germany

This workshop aims at demonstrating the importance of standards of care and diagnosis, for a better treatment of patients. It will also showcase the importance of the patient’s involvement, via Therapeutic Education.

**The Importance of Therapeutic Education in the context of Rare Disease Care**

Aymeric Audiau, Alliance Maladies Rares, France

**Patient Involvement and National Standards of care – The example of Tuberous Sclerosis**

Liselotte Wesley Andersen, Danish TS Society, Denmark

**European standards of care – The example of Duchenne Muscular Dystrophy**

Stephen Lynn, Treat NMD, UK
Sunday 2 June 10:00 - 11:30

Visit of Dubrovnik

“Dubrovnik Walks”
Discover the old town - general introductory tour
(Physically easy to walk - wheelchair accessible)

Meeting point: Pile gate (in front of Night Club Fuego)
Language: English; Free of charge for all registered participants.
The walking tour will run regardless of the weather conditions.
EURORDIS Membership Meeting 2013 - DUBROVNIK

30 May - 1 June, Hotel Rixos Libertas