



EURORDIS
Rare Diseases Europe



EURORDIS
Membership Meeting

Brussels, May 23, 2012



9:00 - 11:00

Annual General Assembly

11:00 - 11:30
Coffee break

11:30 - 13:00

Forums

Learning from each other

13:00 - 14:00

Lunch

14:00 - 17:30

Workshops

Patient Advocates Capacity Building

15:30 - 16:00

Coffee break

17:30 - 18:30

Reception

Patient Groups Welcome Reception
MCE Conference Centre, Foyer, Ground Floor



Forums (11:30 - 13:00)

Learning from each other - 6 parallel sessions

Forums are meant to offer patient advocates an opportunity to meet, discuss and learn from each other. In each session, patient representatives have been invited to share their experience and to make a 10 min presentation followed by a 10 min Q & A.

ROOMS
C+D

1 Fundraising for rare disease patient groups: *series of short presentations & discussion*

The question of how to raise funds for a rare disease patient organisation is a recurrent issue amongst the EURORDIS member organisations. In this forum, we present three very different experiences that might inspire you.

Moderator: Jill Bonjean, EURORDIS

“My experience” presentations by:

- Fighting Blindness, *Avril Daly, Ireland*
- AKU, *Nick Sireau, United Kingdom*
- Debra Croatia & Croatian Society for Rare Diseases, *Vlasta Zmazek, Croatia*

ROOM
1

2 Advocacy actions for access to treatment & medicines: *series of short presentations and discussions*

Many patient organisations have faced a problem concerning access to treatment and reimbursement for a medical product in their country, caused by the economic situation or the HTA procedures. In this Forum patient advocates share their advocacy strategy.

Moderator: Jonathan Ventura, RaDiOrg, Belgium

- Access to Kuvan – the experience in Sweden, *Anders Lundberg, Swedish PKU Association*
- Myeloma Patients fight for Revlimid, *Greetje Goossens, Belgium*
- Reimbursement of Enzyme Replacement Therapies, *Hanka Meutgeert, VKS Netherlands*

ROOM
G

3 Creating a disease specific European Federation - Dos & don'ts: *series of short presentations and discussions*

The idea of federating patient organisations across Europe for a specific disease is very appealing. But how? Hear the stories of some patient organisations that have done it.

Moderator: Rosa Sanchez, Aniridia Europe / FEDER / EURORDIS

- Osteogenesis Imperfecta Federation Europe, *Ute Wallentin*
- European Congenital Heart Disease Organisation, *Peter Van den Broeck, Belgium*
- Presentation of the *RareTogether!* project & website, *Rob Camp, EURORDIS*



ROOM
2

4 How can patient organisations address new issues of ageing in people with rare diseases?

Series of short presentations and discussions

Better care for rare disease patients has thankfully allowed a longer life for many, which has raised new questions that will be discussed in forum 4, for the first time at a EURORDIS Membership Meeting.

Moderator: Terkel Andersen, EURORDIS

- Survey “A long life with Haemophilia” *Lene Jensen, Danish Haemophilia Society*
- Myasthenia Gravis Romania, *Nadia Radulescu*
- Les Maisons de Lyliane: A home where rare disease patients with intellectual disabilities can grow old, *Janine Cayet, France*

ROOM
3

5 EURORDIS activities at the EMA & Training programmes

Learn more about EURORDIS’ training programmes in the areas of clinical trials, drug development and EU regulatory affairs. Learn from EURORDIS staff and volunteers who play a role in the drug development and regulatory process in Committees and Working Groups of the European Medicines Agency.

Moderator: Lesley Greene, COMP

- *Michele Lipucci*, Patient representative in the Committee for Advanced Therapies
- *Tsveta Schyngs*, Patient representative in the Paediatric Committee
- *Pauline Evers*, Patient representative in the Committee for Orphan Medicinal Products
- Presentation of EURORDIS Summer School and e-learning tool, *Maria Mavris, EURORDIS*

ROOM
4

6 EURORDIS’ activities on EU and National Policies

EURORDIS volunteers and members of the EUCERD (*European Union Committee of Experts on Rare Diseases*), will share their experience at the heart of EU rare disease policy-making, including National Plans, centres of expertise and registries.

Moderator: Christel Nourissier, EURORDIS / EUCERD

- The patient representative’s role, *Gabor Pogany, HUFERDIs, Hungary & Patient representative in the EUCERD*
- EUCERD recommendations on Centres of Expertise, *Bianca Pizzera, IPOPI, Italy & Patient representative in the EUCERD*
- Rare Cancer perspective in the Committee, *Jan Geissler, Patient representative in the EUCERD*

Workshops (14:00 - 17:30)

Patient Advocates Capacity Building - 6 parallel sessions

Capacity building workshops are three hours long. They include power point presentation followed by Q & A and debates. The objective is to give patient advocates the knowledge base and tools to actively participate in working groups at a national level, helping them to work to establish and monitor National Plans and Strategies for Rare Diseases.

ROOM
G

1 Online Communities

Moderators: Denis Costello, EURORDIS & Rob Pleticha, EURORDIS

- Defining Online Communities
 - What tools are rare disease patient groups using? How are we coping with these new tools?
Ongoing group discussion during presentation of survey results: What has your disease community achieved through online communities?
- Challenges of Creating an Online Community: Allocating Limited Resources
 - Challenges include: moderation, translation, sustainability, privacy
 - For each challenge discuss how RareConnect has faced it
 - Creating a social media strategy for your rare disease group
- Benefits of Online Communities: What an Empowered, United Group Can Accomplish
 - Case studies from patient advocates highlighting their use of social media
 - HAEi - International Patient Organization for C1 Inhibitor Deficiencies, *Henrik Boysen, Denmark*
 - Myopathies.info, *Alexandre Mejat, France*
 - Community Manager, DysNet, *Tania Tirraoro, UK*
- Social media connecting patients and researchers, *Merche Serrano, Guiametabolica.org, Spain*
 - How platforms allowed for interaction between researchers, patients, and families on medical and nutritional questions? What structures were put in place? What resources or time was needed from your staff? What was the benefit to families or patients?

ROOM
1

2 Information on medicines, Market Authorisation, Pharmacovigilance: How to work with your National Competent Authority?

Moderators: Maria Mavris, EURORDIS & Lut de Baere, RaDiOrg, Belgium

- "State of affairs" - What is happening at the EMA, how involved are patients, why should they be involved at a national level, *Lise Murphy, Swedish Marfan Association, DITA Task Force, PCWP*
- EPF toolkit, *Susanna Palkonen, EPF Belgium*
- Patient organisation's collaboration with the French authority Agence nationale de sécurité du médicament et des produits de santé (ANSM), *Claudie Baleyrier, Association Française de l'Ataxie de Friedreich, France*

Panel discussion with *Dr André Lhoir, Federaal Agentschap voor Geneesmiddelen en Gezondheidsproducten*
- *Agence Fédérale des Médicaments et des Produits de Santé, Belgium*

ROOM
2

3 Directive on cross-border care: Why and how can you advocate for the implementation of the EU directive on Cross-Border Health Care at a national level to support patient mobility?

Moderators: Flaminia Macchia, EURORDIS & Yann le Cam, EURORDIS

- Overview of the Directive, *Nathalie Chaze, European Commission, DG Health*
- National perspective, *Anne Calteux, Permanent Representation of Luxembourg to the EU*
- National perspective of a patient organisation, *Ingrid Jageneau, Debra Belgium*

ROOMS
C+D

4 Centres of Expertise: Quality criteria and How can patients participate in the evaluation of Centres of Expertise?

Moderators: Lene Jensen, Rare Disorders Denmark & Christel Nourissier, EURORDIS

- Why face-to-face meetings are so valuable for all parties, *Lene Jensen, Rare Disorders Denmark*
- Presentation of the EUCERD recommendations on Centres of Expertise, *Kate Bushby, EUCERD / Treat NMD & Care NMD, UK*
- Presentation of the Evaluation of Centres of Expertise in Denmark, England, and France, Delphi process and future use of the method and outcomes, *Rob Camp, EURORDIS*

ROOM
i

5 Registries, Biobanks, Research: patient contribution to operational models of national and international initiatives.

Moderators: Beatrice de Montleau, AFM-Téléthon & John Dart, Debra International

- Overview of on-going projects at international level and presentation of the EUCERD & Europlan Recommendations on registries, *Monica Ensini, EURORDIS*
- Patient and private foundations: a partnership for registry and biobank management, the German von Hippel-Lindau patient organization initiative, *Gerhard Alsmeyer, VHL Germany*
- Patient organisation direct access to biobanks, *Dr. Marco Crimi, Telethon Italia*
- Patients involvement at international level: the IRDiRC initiative, *Béatrice de Montleau, AFM / EURORDIS*

ROOM
3

6 Compassionate use programmes for orphan drugs

Moderator: Michele Lipucci, member of the Committee of Advanced Therapies, EMA, and Italian association for Thalassemia

- How do we define such programmes and why are they needed? *François Houijez, EURORDIS*
- Compassionate use programmes in France: the A.T.U legislation and its impact on orphan drugs. *Chantal Belorgey, Agence nationale de sécurité du médicament et des produits de santé (ANSM)*
- Recent attempt to create a compassionate use in Romania, *Etelka Czondi, Romanian Prader Willi Association*
- Survey on recent compassionate use programmes for orphan drugs. *François Houijez, EURORDIS*
- ERTC workshop conclusions on compassionate use (21/11/2011) and proposals to move forward. *Arielle North, European Medicines Agency*

Workshop exercises:

3 to 4 groups, each group receives a short text proposing to organise compassionate use for a product for a given indication, considering all the aspects of the implementation.

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With the support of :



And :



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