



Rare Disorders Denmark

Programme

**Eurordis Membership Meeting 2008 Copenhagen
16 - 17 May 2008**

**Quality Hotel Hoje Taastrup
Carl Gustavs Gade 1
DK-2630 Taastrup**

Hosted by Rare Disorders Denmark

“Acting together for patient-centred care for rare diseases”

*A European workshop empowering patients, families and their organisations
Towards multidisciplinary medical and social care
in Centres of Expertise and European Reference Networks for Rare Diseases*

Looking back on our progress, over the last two years, in promoting policies on Centres of Expertise for Rare Diseases

The EURORDIS Membership Meeting (Berlin 2006) was dedicated to “Centres of Reference for Rare Diseases: How can we make it happen?”. This was a starting point for EURORDIS members to develop a common approach to providing healthcare services for rare disease patients. Based on debates, we adopted a “Reflection Paper on Centres of Reference”.

Since this time, EURORDIS, national alliances and their members have worked together extensively on promoting the need for specialised healthcare services for rare disease patients and in facilitating the development of common concepts, a common language and common strategies. The core of these efforts was embodied in the Rare Disease Patient Solidarity project – RAPSODY – that commenced mid-2006 and will conclude mid-2008, and is supported by the European Commission and 10 partners.

Eleven national workshops, organised in spring 2007, with the same agenda and background information, gathered over 270 patient representatives, healthcare professionals and policy makers to develop recommendations. The two-day European Workshop in July 2007 in Prague, with 90 participants, synthesised the outcomes of these workshops and brought together leading experts from the first European Reference Networks and representatives of the EU High Level Group on Health Services and Medical Care, DG SanCo and the Rare Disease Task Force.

In parallel, thanks to the participation of 137 patient organisations and 6 000 respondents (representing 16 rare diseases and 22 countries across Europe), the EurordisCare 3 survey has collected solid data on the experiences and expectations of rare disease patients and their families.

Recently the European Conference on Rare Diseases in Lisbon (November 2007) provided an occasion to report to and debate with a larger audience on the positions of policy makers in the EU High Level Group on i) Health Services and Medical Care, ii) the outcomes of the RAPSODY national & European workshops and iii) the preliminary results of the EurordisCare 3 survey.

The state of play in 2008: a pivotal year for ‘grasping the nettle’

The rationale for addressing this theme via a European Workshop within the 2008 Membership Meeting is to conclude the internal efforts to conceptualise our needs and expectations and the external efforts to transform them into political advancements. The next cycle is all about grass-root work, sharing experiences and best practices, development of common tools and evaluation of progress.

Today in 2008, national Centres of Expertise and European Reference Networks for Rare Diseases have become a priority on the EU health political agenda. The future Directive on Health Services provides a basis for the establishment of European Reference Networks and specifically addresses the need to do so for rare diseases. It will also provide a new legal framework for the mobility of patients within the EU. The future Commission Communication on Rare Diseases – to be adopted later this year - clearly proposes the establishment of European Reference Networks for Rare Diseases in the coming years. It also encourages Member States to identify, select and support Centres of Expertise for Rare Diseases within the framework of National Plans for Rare Diseases.

EURORDIS and its members actively contribute to further promoting this policy at national and European levels. It is an essential tool for improving the quality of diagnosis and care for all rare diseases in all European countries. This has been illustrated by specific contributions from patient groups and in the EURORDIS Position Paper “Centres of Expertise and European Reference Networks for Rare Diseases” in the context of the Public Consultation.

In addition, EURORDIS and its national alliances are promoting the adoption of Recommendations on rare diseases by the Council in order to strengthen European Commission and Member State policies with more binding commitments. Important elements of these strategies include: i) the adoption of National Plans for Rare Diseases in a coordinated EU approach, ii) the establishment of Centres of Expertise and European Reference Networks, iii) the promotion of research, iv) the

gathering of expertise for the development of common guidelines, v) the improvement of access to orphan drugs and innovative therapies and vi) supporting empowerment of patient groups.

EURORDIS, national alliances, European federations and all disease-specific patient groups concerned play a key role in shaping and further advocating for national and European policies on Centres of Expertise and European reference Networks for Rare Diseases.

Beyond this, we have a key role to play as equal actors, taking responsibilities at the local, national and European levels to implement these policies through quality collaboration with experts leading the Centres of Expertise and European Reference Networks as well as through our pro-active participation in the decision making processes to identify, select and evaluate them.

We also share common concerns on i) the evaluation of patient outcomes, ii) a real multi-disciplinary approach, iii) good information sharing and networking between professionals to provide patient-centred services, iv) integrated medical and social approach to providing comprehensive care, v) the long term professional and financial sustainability of the infrastructures and essential tools - such as patient registries, biological resources repositories, patient electronic files - and vi) the involvement of these networks in translational research to deliver eagerly awaited therapeutic advancements for our patients.

Looking forward to the empowerment of patient advocates and support groups

The objectives and expected outcomes of this European Workshop are to:

- Consolidate our common approaches and share policy updates, concrete experience gained through case studies, concerns and priorities
- Debate and first adoption of two new important tools:
 - o A “Declaration on Centres of Expertise and European Reference Networks for Rare Diseases”.
 - o A “Charter of Best Practice for Collaboration between Centres of Expertise and Patient Groups for Rare Diseases”.

A common agenda across European countries and rare diseases would dramatically increase the effectiveness of our advocacy efforts.

A Declaration officially adopted by our movement will provide a common political reference, a flag and sword to each rare disease patient group, each national alliance and each European federation.

A Charter officially adopted by our movement will provide a common framework to regulate the relationship between rare disease patient groups and their related Centres of Expertise and a toolbox enabling patient groups to promote good practices and progressively build on them.

To achieve these objectives, the European Workshop will provide a policy update and will present a draft Declaration and Charter to be debated in small workshops. Throughout the meeting the results of the EurordisCare 3 survey and case studies will illustrate the reality of patient and families and the concrete practices between patient organisations and Centres of Expertise.

Draft Agenda

Thursday 15th of May:

Satellite workshops:

- 10.00-17.00 **6th Workshop of the Council of National Alliances:**
- Rare Disease Day 2008 & 2009
 - National Plans for Rare Diseases, Commission Communication and Recommendations to the Council
 - National Conferences on Rare Diseases
- 13.30-17.00 **European Help lines for Rare Diseases: New IT tools**

Disease specific network meetings:

- 16.00-18.00 **Myasthenia Gravis** network meeting
Organised by the Myasthenia Gravis Society of Croatia (domgh@zg.t-com.hr)
- 12.00-17.00 **European Network on Ichthyosis** Meeting,
Organised by Iktyosisforeningen i Danmark (iktyosis@hotmail.com)
Location: Greve Borgerhus, Greveager 9,2670 Greve

Friday 16th of May

Conference: "Common actions to change day to day care"

- 09.30-10.00 Welcome messages
Terkel Andersen, President of Eurordis
Torben Gronnebaek, President of Rare Disorders Denmark
- 10.00-10.20 Update on the Commission Communication on Rare Diseases
Toni Montserrat, DG SANCO
- 10.20-10.35 Question & Answer
- 10.35-10.55 Key findings from the final analysis of the Eurordiscare surveys
Yann Le Cam, Chief Executive Officer, Eurordis
- 10.55-11.10 Question & Answer
- 11.10-11.40 Coffee break*
- 11.40-12.00 Presentation of a draft **Declaration of common principles**, illustrated by results of the Eurordiscare surveys
Rosa Sanchez de Vega, President of FEDER, Spain
- 12.00-12.20 Presentation of a draft **Charter** on the relationship between patient organisations and Centres of Expertise, illustrated by results from the EurordisCare surveys
Christel Nourissier, Prader Willi France & Eurordis
- 12.20-12.45 Debate
- 12.45- 14.00 Lunch break*

14.00-15.30 Parallel Workshops:

Workshop 1: European reference networks for rare diseases	Workshop 2: Innovative partnerships between patient organisations and National centres of expertise
<p><u>Moderator:</u> <i>Soren Lildal, Danish Apert Syndrome Association</i></p> <p>European Centres of Reference Networks for Cystic Fibrosis (ECORN-CF)</p> <ul style="list-style-type: none"> ▪ The patients perspective <i>Karleen de Rijcke, Cystic Fibrosis Europe</i> <p>GENESKIN</p> <ul style="list-style-type: none"> ▪ The professional's perspective: <i>Dr. Marina D'Alessio, Istituto Dermopatico dell'Immacolata, Italy</i> ▪ The patients perspective <i>John Dart, DEBRA Europe</i> 	<p><u>Moderator:</u> <i>Dr.Allan Meldgaard Lund, Copenhagen University Hospital, Denmark</i></p> <p>Multidisciplinary clinics for Alström Syndrome initiated by a patient organisation,</p> <ul style="list-style-type: none"> ▪ <i>Dr Richard Paisey, Alstrom clinics at Torbay Hospital, United Kingdom</i> ▪ <i>Johanne Foster, Astrom Syndrome UK</i> <p>Marfan Sweden centre of expertise, initiated by a patient organisation</p> <ul style="list-style-type: none"> ▪ <i>Lise Murphy, Svenska Marfanföreningen, Sweden</i>
<p>After each presentation, time for Q&A will be allowed. The objective of these workshops is to share best practices in partnerships between patient organisation and European reference networks or national centres of expertise and to learn from the case studies presented.</p>	

15.30-16.00 *Coffee break*

16.00-17.30 Parallel Workshops:

Workshop 3: Debate on the draft Charter on relationships between patient organisations and centres of expertise	Workshop 4: Debate on the patient's Declaration on common principles	Workshop 5: Get the most out of the Eurordiscare survey
<p><u>Moderator:</u> <i>Birthe Holm, Danish Osteogenesis Imperfecta Society</i></p> <p><u>Rapporteur:</u> <i>Flaminia Macchia, Eurordis</i></p>	<p><u>Moderators:</u> <i>Lene Jensen, Rare Disorders Denmark</i></p> <p><u>Rapporteur:</u> <i>Dorica Dan, Romanian National Alliance for Rare Diseases</i></p>	<p><u>Moderator:</u> <i>Maria Mavris, Eurordis</i></p> <p>How to use, adapt and present the Eurordiscare 3 results to your members and other stakeholders and use it for advocacy purposes. <i>François Faurisson</i></p>

17.30-19.00 *Screening of short films related to rare diseases (optional)*

19.00 ***Welcome Dinner at the Conference venue***

Saturday 17th of May

09.00-11.00 Parallel Workshops:

Workshop 6: Challenges & expectations for future Networks of Reference for rare diseases	Workshop 7: Innovative social approach to cope with Rare Diseases
<p><u>Moderator:</u> Toni Montserrat, DG SANCO</p> <p>Key lessons learned from the first networks <i>Laura Fregonese, Leiden University, Netherlands</i></p> <p>European Network of Reference for Rare Paediatric Neurological Diseases launched by a patient organisation <i>Tsveta Schyns, ENRAH, Austria</i></p> <p>Our vision for a European Dysmelia Network <i>Alan Summerside, Thalidomide Trust, UK</i></p>	<p><u>Moderator:</u> Flavio Minelli, Unione Italiana Ittiosi & Eurordis</p> <ul style="list-style-type: none">- Social Guidelines for Rare Diseases in Denmark, <i>Torben Grønnebak, Rare Disorders Denmark</i>- How to support adult people living with Prader Willi syndrome? <i>Karin Juul-Pedersen, Prader Willi residence and activity center, Denmark</i>- The Danish Spielmeier-Vogt team (Batten disease), <i>Anne Hamann Coordinator, Merete Staureby, Danish Batten Disease association</i>

11.00-11.30 Coffee break

11.30-12.30 Plenary discussion on the Declaration and Charter

12.30-14.00 Lunch

14.00-17.00 Eurordis General Assembly (members only)
Business Agenda & Election of Directors

17.00-18.00 Farewell cocktail at the conference venue

Sunday 18th 11.00 to 12.00 Copenhagen Boat Cruise with DFDS Canal Tours

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