

European Conference on Rares Diseases



Lisbon 2007

27 - 28 November 2007 - Marriott Hotel \ Portugal \ Lisbon



Lisbon 2007

European Conference on Rare Diseases

organised by eurordis

Hosted by the Government of Portugal under
the European Union Presidency, with the
support of the European Commission



Public Health
Programme of
the European
Commission



Ministério da Saúde
Direcção-Geral da Saúde



EURORDIS
Rare Diseases Europe

Patients at the Heart of Rare Disease Policy Development

2007 is a pivotal year for rare diseases. A European policy integrating both national and Community initiatives is taking shape. In Member States, patients, health care professionals and policy makers are developing comprehensive measures and concrete services to improve the living conditions of people suffering from rare diseases. At the same time, the European Institutions are preparing patient-centred recommendations to ensure that the various initiatives being taken are of benefit to the largest possible number of our constituents.

Ten years after the creation of Eurordis, the European Organisation for Rare Diseases, we have the pleasure to invite you to attend the 4th edition of the European Conference on Rare Diseases in Lisbon. The previous European Conferences (Copenhagen 2001, Paris 2003, Luxembourg 2005) were a huge success. Lisbon 2007 is the second conference to be held under the E.U Presidency and to be financially supported by the European Commission – DG Public Health.

Together, patients and health care professionals shall review policies, strategies and examples of successful action; we shall voice our needs, promote patient-centred policies at national and European levels; and confirm the vitality of the rare disease community in Europe!

2007 : the pivotal year for rare diseases!

Terkel ANDERSEN
Co-chair of the programme
committee \ President of Eurordis

Prof Josep TORRENT I FARNELL
Co-chair of the programme
committee \ Fundacio Dr. Robert
COMP Member

ORGANISERS

A conference organised by EURORDIS and its partners: AFM-Téléthon (France), Barretstown (Ireland), Federacion Espanola de Enfermedades Raras (FEDER Spain), Fundacio Dr. Robert (Spain), Frambu (Norway), National Information Centre for Metabolic diseases (Climb, United kingdom), Rare Disorders Denmark, ORPHANET (France), State Institute for Drug Control (SUKL Czech Republic).



→ LANGUAGES

English, French, German, Portuguese, Spanish



Lisbon

→ PRELIMINARY PROGRAMME AND REGISTRATION

www.rare-diseases.eu

→ PROGRAMME COMMITTEE

- Terkel ANDERSEN, Denmark, Danish Haemophilia Organisation and president of Eurordis, co-chair of the programme committee
- Prof Josep TORRENT I FARNELL, Spain, Director of the Dr Robert Foundation, Autonomous University Barcelona, co-chair of the programme committee
- Dr Ségolène AYMÉ, France, Orphanet and leader of the DG SanCo Task Force on Rare Diseases
- Dr Jill CLAYTON-SMITH, United Kingdom, Consultant Clinical Geneticist
- Paula COSTA, Portugal, Rarissimas
- Michele LIPUCCI DI PAOLA, Italy, thalassemia organisation
- Prof Milan MACEK, Czech Republic, Medical Genetics, Charles University
- Prof Luis NUNES, Portugal, Service of Genetics, Dona Estefania Hospital
- Alicja ROSTOCKA, Poland, Cystic Fibrosis organisation
- Françoise SALAMA, France, AFM-Téléthon
- Prof Dr Jörg SCHMIDTKE, Germany, Human Genetics, Hannover Medical School
- Tsveta SCHYNS, Austria, EU Research Network for Alternating Hemiplegia
- Rik SERPENTIER, Belgium, Belgian Association for the Parents of Children with Metabolic Disorders
- Dr Sonja VAN WEELY, The Netherlands, Dutch Steering Committee for Orphan Drugs



Terkel ANDERSEN



Prof Josep
TORRENT I FARNELL

→ PROJECT MANAGEMENT

- Yann LE CAM, Chief Executive Officer, Eurordis
- François HOUÏEZ, Health Policy Officer, Eurordis
- Kasia PEALA, project assistant, Eurordis

Monday, November 26th 2007 :

→ **PRE-CONFERENCE WORKSHOPS**

Some European networks are taking the opportunity of the European Conference on Rare Diseases to meet the day prior to the conference. These are closed meetings; please contact ECRD organisers if you wish to receive more information.

- Orphanet network
- Thalassaemia International Federation and Pan-European Blood Safety Alliance
- Help lines for rare diseases
- Council of National Alliances for rare diseases
- Drug Information, Transparency and Access Task Force (Eurordis)

Tuesday, November 27th 2007 :

→ **RARE DISEASES, A RENDEZ-VOUS WITH EUROPE**

**9.30 AM \ Opening Ceremony \ The President of Eurordis \
The European Commission \ The Ministry of Health of Portugal**

\ Building on successful European policies and moving forward

2007 is a pivotal year for rare diseases. Member states and European Institutions are preparing the rendez-vous of national and European efforts in order to coordinate actions and propose recommendations to improve the live of people living with rare diseases. In this session, patients and their representatives, health care professionals and other stakeholders will have a great opportunity to debate with representatives of the Council, the European Parliament, the European Commission, and thus make their presence felt in this event.

- **New action taken by the European Commission action in the field of Rare Diseases : European strategies and achievements**
- **Recommendations on public health action on Rare Diseases : new perspectives for European and national policies**
- **Round table :** \ Views of the Council \ Views of the EU Parliament \ Views of DG Research \ Views of EMEA / DG Enterprise
- **Debate**

\ Enhancing Member States rare diseases policies

Member states' policies and actions in the field of rare diseases are rapidly evolving. They share common features, but diverge in some areas. Session 3 will propose an overview of policies in three different domains: general policies, genetic testing and neo-natal screening, and research. Speakers from the DG SanCo Rare Disease Task Force, EuroGentest and E-Rare will present them and open the debates.

- **Overview of Member States public health policies for rare diseases: a 2007 update**
- **Overview of Member States policies on genetic testing and neo-natal screening**
- **Overview of national research policies and collaboration between Member States**
- **Debate**

- Lunch break
- Press conference

\ Promoting national centres of expertise and European Reference Networks

Centres of expertise are at the core of the European Union thinking and experimentation on the future organisation of health services and medical care at the European level.

The Rare Disease Patient Solidarity (Rapsody) project conducted by Eurordis and funded by the European Commission and a consortium of partners has created an intense dialogue on their usefulness and on possible recommendations for the identification of centres of expertise, their support and their evaluation, both at the national and at the European levels.

The EU High Level Group of Health Services and Medical Care made of representatives of member states will present its analysis and its vision.

The patient perspective will be based on a large patient survey –EurordisCare 3- to analyse patient needs and expectations for 15 rare diseases from 18 countries and on workshops that took place in 2007 in 11 member states.

- **Building a European policy addressing citizen's needs / the vision of the EU High Level Group on Health Services and Medical Care**
- **Patients' needs and expectations concerning access to health services - The EurordisCare3 study**
- **Paving the road for integrated policies / Facilitating the integration of policies – The Rapsody workshops outcomes**
- **Debate**

- Poster session

\ Mobility in Europe: Framing healthcare pathways to patients' needs

Rare diseases have no frontiers; they affect people from all parts of Europe and beyond. The construction of Europe and the establishment of a single market for goods and services, the abolition of internal frontiers and the exchange of information have created a new kind of traveller: the patient in search for quality care. In this session, patients will explain why they can be tempted to move to a different state when affected by a rare disease, health care professionals will explain how they organise health services for foreigners, and the impact of population displacement. Patients' mobility needs are now fully part of the evolution of our health care systems.

- **A Patient's Testimony**
- **A Health Professional's Testimony (The search for devices tailored to patient needs)**
- **Patient centred provision of healthcare for rare diseases**
- **The new rare disease challenges of patient migration and EU enlargements (testing issues, incidence and consanguinity higher/lower European prevalence link to enlargement ...)**
- **Facing patient mobility needs in Europe**
- **Debate**

→ Wednesday, November 28th 2007:
SOWING THE SEEDS OF SUCCESS

\ Recent advancements in quality assessment relevant to rare diseases

“First, do not harm. Possibly, do good”. But how to do better? To best use medicinal products, quality information is essential. The same applies to specialised centres for rare diseases: evaluation is needed to provide the best possible care. Information on genetic testing is also key to their usefulness. In this session, speakers will demonstrate the importance of quality assessment relevant to rare diseases.

- **Assessing the quality of outcomes from centres of expertise**
- **Assessing quality of centres of expertise outcomes**
- **Assessing quality of information on genetic testing**
- **Debate**

\ Addressing all patient needs, beyond medical care (1)

For chronic and severe diseases, care is not restricted to medical and paramedical care. Patients and carers require other types of care throughout their entire lives: information and support via help lines, tools to break their isolation, leisure activities, respite care services, programmes to help their school curriculum, and organisation of the transition of the provision of medical services and treatment from childhood to adulthood.

- **Therapeutic recreation programmes**
- **Rare Disease Help lines**
- **Breaking isolation for very rare disease patients (through help lines and mailing lists)**
- **The role of online communities for people living with a rare disease**
- **Debate**

\ New initiatives in Member States for rare diseases

Europe is rich in its diversity. Any new initiative that arises in a member state can rapidly give birth to similar actions in all Member States. This session will highlight three examples of national action that can be multiplied by 27: the Portuguese plan for rare diseases, the Italian actions for rare disease research, the French emergency card for rare diseases.

- **Portugal - a national plan for rare diseases centred on patient needs**
- **New Italian actions**
- **The French Emergency Card experience**
- **Debate**

\ Addressing all patient needs, beyond medical care, (2)

- **Respite care services**
- **Rare diseases at school**
- **Patients suffering from rare diseases from childhood to adulthood – Learning from experience**
- **Debate**

- **Lunch break**

\ Making the most of the EU Research policy

In this session devoted to research on rare diseases, speakers will address the need for coordination between European and national research policies, explain the broad spectrum of research in biomedicine, public health, social science, and ethics, and they will also call for a favourable environment to transfer discoveries from academia to industry.

Three important workshops took place in 2007 on this theme, with the participation of European Commission DG Research, patients, and industry.

- **Achievements and perspectives – DG Research workshop (June) and Conference (September) outcomes**
- **Expectations and Contributions from patients – The European Workshop outcomes (May, Capoira project)**
- **Expectations and Contributions from pharma & biotech industry**
- **Debate**

\ Shaping future policies for orphan medicines and advanced therapies

The European policy for Orphan drugs is one of the most successful policies of the European Union. This session will present a brief status report as of 2007 and challenges identified by the Committee for Orphan Medicinal Products (COMP) for its 3rd mandate 2006-2009. One hurdle is unequal access to orphan drugs and the diverging national policies for health technology assessment. The outcomes of two important European workshops organised by the French National Health Agency and by EPPOSI will be presented. The voice of the rare disease community will also be heard in the shaping of the future EU policy on advanced therapies –gene therapy and cell therapy.

- **Seven years of orphan drugs policy: what's next?**
- **Preparing the European scenario for advanced therapies (opportunities for gene therapy, challenges, cell therapy, future EU Regulation)**
- **Timely and equitable access to orphan medicines across Member States – The European HAS workshop (November 2006) and the Epposi Workshop outcomes (October 2007)**
- **Debate**

4.15 PM

- **Conference closes : take home messages for action now**

→ **REGISTRATION**
 (Register before July 31st to benefit from preferential rates!)

Registration online (only) at www.rare-diseases.eu

→ **ACCOMODATION** (hotel booking before 30 September 2007)



Hotel rooms are pre-reserved at various rates ranging from 55 € to 120 €, in the conference hotel and in hotels nearby. We strongly encourage you to book early to benefit from the most convenient conditions. In any case, the deadline for hotel booking is: 30 September 2007.

Hotel rooms will be reserved and confirmed upon the receipt of your deposit payment. Mundiconvenius will send you a written confirmation of reservation. Please keep this confirmation letter (voucher) and present it when checking in. The hotel's final invoice will be based on the number of nights you booked in advance. Final hotel expenses less the pre-paid deposit must be paid directly to the hotel upon check out. The hotel will issue an invoice / receipt.

→ **HOTEL CHANGES AND CANCELLATIONS**

Please make changes or cancellations in writing directly to Mundiconvenius and not to the hotel. If you cancel your hotel reservation before September 1, 2007, you will receive a refund of the deposit after the end of the conference. However, an administration fee of EUR 20 per room will be retained. Unfortunately, no refund of the hotel deposit can be made on cancellations received after September 1, 2007 or for no-shows. In the case of a no-show the hotel reservation will be released after the first night.

→ **ATTENDANCE FEES**

	Before July 31 st , 2007	After July 31 st , 2007
Eurordis members	50€	100€
Patients' Organisation (non Eurordis member)	100€	150€
Health care professionals	100€	150€
Pharmaceutical industry	600€	850€

ECRD 2007 registration fees include: attendance at all conference sessions on 27 and 28 November, morning & afternoon tea/coffee breaks, lunch each day, delegate bag, one copy of the conference proceedings (to be published March-April 2008).

→ **IMPORTANT**

We strongly suggest that you register before July 31st to ensure your confirmation and to benefit from a preferential rate, as registration will close when the capacity of the venue is reached (450 participants).

→ MEDIA REGISTRATION

There is no registration fee for accredited media participants. In order to register as a media participant for the conference, the conference secretariat needs to receive proof of accreditation.

→ PROFESSIONAL CONGRESS ORGANISER (hotel booking and registration)

Please contact the congress organiser for your registration or hotel reservation if you have any difficulty using the on-line service:

Mundiconvenius
Sociedade de Congressos e Serviços, Lda.
Avenida 5 de Outubro, 53
1050-048 Lisboa
Tel: + 351 21 315 51 35
Fax: + 351 21 355 80 02
Email: sofia_silva@mundiconvenius.pt
www.mundiconvenius.pt

→ CONFERENCE SECRETARIAT (conference programme)

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→ A CONFERENCE SUPPORTED BY

- European Commission, DG Health and Consumers' Protection
- Government of Portugal, Ministry of Health, Direcção Geral de Saude
- Eurordis

→ LOCAL ORGANISING COMMITTEE

The organisers wish to thank the following organisations for their generous and volunteer participation to the local organising committee that is playing such an important role in the preparation of the European Conference on Rare Diseases 2007 in Lisbon :

- Associação Nacional de Pais e Amigos Rett
- Associação de Retinopatia de Portugal
- Associação Portuguesa de Doentes Neuromusculares
- Associação Portuguesa de Pais et Doentes com Hemoglobopatias
- Associação Portuguesa das Doenças do Lisosoma
- Associação "Spina Bífida e Hidrocefalia" de Portugal
- Associação Raríssimas
- Associação Portuguesa dos Hemofílicos
- Associação PXE Portugal
- Associação de Pais e Amigos de Portadores do Síndrome de Rubinstein-Taybi

If you have special requirements or needs to attend this conference, please contact :
Ana Rita Dagnino, Associação Portuguesa dos Hemofílicos at the following number :
Tel : +351 218 598 491 / +351 966 581 556
Email : rita.dagnino@aphemofilicos.org.pt

→ Call for abstracts

You are invited to submit an abstract for the European Conference on Rare Diseases to be presented during the poster session.

Posters will be selected by the programme committee.

We are calling for abstracts on the following cross-cutting issues :

- European, Regional, State and Local Policy
- European Research networks
- Centres of expertise and European reference networks for rare diseases
- Patient-driven innovative projects for rare diseases, services to patients, families and professionals
- New orphan drug development

The basic text of your abstracts should not exceed 300 words.

Use the following website : www.rare-diseases.eu

Deadline for Abstract Submission : 15 June 2007, 24.00 hrs UTC+1

Please also note that your abstract will be included in the scientific programme, if you register before 30 September 2007.