

European Conference on Rare Diseases 2005 (ECRD)

Chamber of Commerce of Luxembourg
21 & 22 June 2005

**FROM DIFFICULTIES TO SOLUTIONS FOR THE RARE
DISEASE COMMUNITY**



ORGANISERS

A conference organised
by EURORDIS and its partners:

AGRENSKA (Sweden),
ALAN (Luxembourg),
ALLIANCE MALADIES
RARES (France),
CZECH DRUG CONTROL
AGENCY (Czech Republic),
EUROCAT (UK),
FEDER (Spain),
KMS (Denmark),
and ORPHANET (France).

Dear Colleagues and friends,

IT IS WITH GREAT PLEASURE that we invite you to the European Conference on Rare Diseases to be held in the beautiful city of Luxembourg on the 21 & 22 June 2005.

This conference will provide a programme which will highlight important actions and information in the public health policy and research area, as we progress towards our goal of better care for people living with rare diseases.

The organising committee is sure that you will never forget your stay in Luxembourg and look forward to welcoming you to this European conference.

Christel Nourissier
Chairperson,
Organising
Committee

Ségolène Aymé
Chairperson,
Programme
Committee

HOSTED BY THE GOVERNEMENT OF LUXEMBOURG UNDER THE EUROPEAN PRESIDENCY,

WITH THE PARTICIPATION OF THE EUROPEAN COMMISSION,

An important event for the rare diseases community: the European Conference on Rare Diseases organised by the European Organisation for Rare Diseases (Eurordis) and its partners, with the support of the European Commission, the Government of Luxembourg, and the French neuro-muscular diseases organisation AFM.

The conference will bring together patients, researchers, health professionals, health policy experts, and industry representatives. The programme will address current problems and possible solutions to improve the situation of people living with rare diseases, as well as actions supported by the European Commission.

PRELIMINARY PROGRAMME

(visit www.rare-luxembourg2005.org for the final programme and speakers)

Programme Committee

Violetta ANASTASIADOU, Hospital Makarios III, [Cyprus](#)

Terkel ANDERSEN, KMS, Eurordis, [Denmark](#)

Ségolène AYMÉ, Orphanet, [France](#)

Stéphane BURON, Alliance Maladies Rares, [France](#)

Elisabeth DEQUEKER, Departement of Human Genetics, [Belgium](#)

Helen DOLK, Faculty of Life and Health Sciences, [United Kingdom](#)

Liz GONDOIN, ALAN, [Luxembourg](#)

Katarina KUBACKOVA, University Hospital of Motol, [Czech Republic](#)

Yann LE CAM, Eurordis, [France](#)

Christel NOURISSIER, Alliance Maladies Rares, [France](#)

Anders OLAUSON, Agrenska, [Sweden](#)

Hans-Hilgers ROPERS, Max Planck Institute for molecular genetics, [Germany](#)

Rosa SANCHEZ DE VEGA, FEDER, Eurordis, [Spain](#)

Hélène TACK-LAMBERT, French neuro-muscular diseases organisation, [France](#)

Domenica TARUSCIO, Centro Nazionale Malattie Rare, [Italy](#)

Josep TORRENT-FARNELL, Committee for Orphan Medicinal Products,

EMA, [European Union](#)

LANGUAGES

The conference will be in English. Simultaneous interpretation will be available in:

- French
- German
- Polish
- Spanish

LOCATION

The Chamber of Commerce is located on the plateau Kirchberg, easily accessible by car and public transport from the city centre (4 km from main train station), and from the airport (2.7 km).

FROM DIFFICULTIES TO SOLUTIONS FOR THE RARE DISEASE COMMUNITY



TUESDAY JUNE 21ST 2005

9.30 a.m.
Session 1

OPENING CEREMONY

Under the patronage of Her Royal Highness

The Grand Duchess Maria Teresa of Luxembourg

- Mr Terkel Andersen, Eurordis
- Mr Markos Kyprianou, EU Commissioner for Health and Consumer Protection
- Mr Mars di Bartolomeo, Minister of Health of Luxembourg

“Living with a rare disease”: a documentary by Josée Blanc Lapierre

10.45 a.m.

• **Coffee break**

11.00 a.m.
Session 2

DELAYS IN DIAGNOSIS, DISCRIMINATION AND INSUFFICIENT COMPENSATION

- Diagnosis and public health : diagnostic delays
- A patient testimony (or an association)
- A health professional testimony
- Disability : are financial compensations adapted?

Discussion

12.30 a.m.

- **Lunch break**
- Press conference
- Poster session (displayed until June 22nd, 5.00 pm)

2.30 p.m.
Session 3

BENCHMARKING INITIATIVES TO IMPROVE CARE

- Summary comparison of national plans and practices

Reference Centres
the Danish model
the Belgian model
the French model

Discussion

Trans-border access to care

Discussion

4.00 p.m.

• **Coffee break**

4.15 p.m.
Session 4

**BUILDING COOPERATION BETWEEN STAKEHOLDERS TO IMPROVE RESEARCH
TARGETING RESEARCH TO IMPROVE QUALITY OF LIFE**

Building cooperation between stakeholders to improve research

- A multi-disciplinary approach
- Transfer from academic research to industrial development
- Strengthening co-operation between academia and industry

Targeting research to improve quality of life

- Therapeutics and their limits
- Treatment strategy and academic trials

Discussion



WEDNESDAY JUNE 22nd 2005

9.00 a.m.
Parallel
session

Session 5

**ACCESSING APPROPRIATE CARE
ORGANISATION OF CARE
BEST PRACTICES GUIDELINES FOR CARE
MANAGEMENT**

- Testimony of a patient
- Results from surveys illustrating difficulties in access to care
- Clinical networks as a response to scarcity of databases and best practices guidelines

Discussion

Session 6

**FIGHTING THE FRAGMENTATION
OF RESEARCH**

Sharing data

- Collecting and sharing tissue and DNA
- Patient organisation registries: a platform of exchange between professionals
- Collecting and sharing registry data

Discussion

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|--|---|--|
| <p>9.00 a.m. (continued)</p> | <ul style="list-style-type: none"> • Specific difficulties in access to : <ul style="list-style-type: none"> - Biological tests, trans-border testing - Medical devices and equipment - Home care, paramedical care, care at school <p>Discussion</p> | <p>Building technology platforms and strategies</p> <ul style="list-style-type: none"> • Building a technology platform • Building strategies : prevention strategies based on the assesment of epidemiological evidence |
| <p>10.30 a.m.</p> | <p>• Coffee break</p> | |
| <p>11.00 a.m. Parallel session</p> | <p>Session 7</p> <p>BENCHMARKING INITIATIVES TO IMPROVE CARE : BEST PRACTICES GUIDELINES FOR CARE MANAGEMENT (continued)</p> <p>Information and training</p> <ul style="list-style-type: none"> • Help lines and written information • Internet resources • Training families and carers <p>Discussion</p> <p>Data collection and management</p> <ul style="list-style-type: none"> • Diseases with no code: the perspective of patients. • Why and how to code and classify rare diseases <p>Discussion</p> | <p>Session 8</p> <p>FIGHTING THE FRAGMENTATION OF RESEARCH (continued)</p> <p>Building a community of patients and professionals</p> <ul style="list-style-type: none"> • Patient representatives : examples of patients' organisations that successfully brought together researchers' networks • Establishing larger networks : <p>Co-ordinating funding initiatives</p> <ul style="list-style-type: none"> • Rare funding initiatives for rare diseases • DG Research : lessons learned from FP5 and FP6, plans for FP7 <p>Discussion</p> |
| <p>12.30 a.m.</p> | <p>• Lunch break</p> | |

WEDNESDAY JUNE 22nd 2005

14.00 a.m.
Session 9

TREATING WITH ORPHAN DRUGS

JOINT MEETING OF ALL INTERESTED PARTIES : THE EUROPEAN ENVIRONMENT FOR ORPHAN MEDICINAL PRODUCTS

- Status report and health benefits after five years of Orphan Drug legislation
- Experience gained by stakeholders
- Access to drugs and responsibilities of Member States

16.30
Session 10

MOVING FORWARD IN EUROPE

- Proposals for moving forward
- Proposals from the Task Force on Rare Diseases
- Next steps after the conference

17.00 **Conference closes**

IMPORTANT DATES

| | |
|---|-----------------|
| Call for abstracts: | 10 February |
| Deadline for abstract submission: | 15 April |
| Notification of selected posters emailed: | 30 April |
| Deadline for registration: | 31 May |
| Deadline for hotel booking: | 31 May |
| Opening Ceremony: | 21 June 9.30 am |
| Artistic event: | 21 June 8.30 pm |
| Closing ceremony: | 22 June 5.00 pm |
| Web-cast: | 24 June |
| Proceedings online: | October |

REGISTRATION

Registration online
(only) at www.rare-luxembourg2005.org

IMPORTANT

We strongly suggest that you register early to ensure your place, as registration will close when the capacity of the venue is reached (300 participants)

MEDIA REGISTRATION

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

Fees

| | |
|------------------------------------|-----------|
| Members States | 50 € |
| European Commission | 50 € |
| Project Leaders | 50 € |
| Patient Organisations | 50 € |
| Health care professionals | 110 € |
| Pharmaceutical industry | 850 € |
| Additional copy of the proceedings | 20 € each |

ECRD 2005 registration fees include: attendance at all conference sessions on 21 and 22 June, morning & afternoon tea/coffee breaks, lunch each day, artistic event on 21 June, delegates' bag, and a copy of the conference proceedings (to be published October 2005).



PROFESSIONAL CONFERENCE ORGANISER

(hotel bookings and registration)
Please contact the conference organiser for your hotel reservations:
Meetings, Translations
& Incentives Services SA
3, rue des Carrefours
L-8015 Strassen
Tel: 00352 459945-200
Fax: 00352 459945-210
Email: meetings@meetings.lu

ACCOMODATION

Hotel rooms are pre-reserved at various rates ranging from 55 € to 180 €, in the airport area (2.7 km from conference centre), near the conference centre or near the main train station. We strongly encourage you to book early to benefit from the most convenient conditions. In any case, the deadline for hotel booking is: 31 May 2005.

CONFERENCE SECRETARIAT

(conference programme)
EURORDIS
Plateforme Maladies Rares
102 rue Didot
F-75014 Paris
Tel: 00 33 1 56 53 52 63
Fax: 00 33 1 56 53 52 15
Email: william.gibon@eurordis.org

Call for posters

YOU ARE INVITED TO SUBMIT AN ABSTRACT FOR THE EUROPEAN CONFERENCE ON RARE DISEASES TO BE PRESENTED DURING THE POSTER SESSION.

We encourage project leaders or co-leaders who have conducted public health projects under the DG Health Programme of the European Commission, or research projects on rare diseases (Biomed programme, 5th or 6th framework programme of DG Research), to submit an abstract to the 2005 ECRD. We are also soliciting abstracts on cross-cutting issues such as:

LIVING WITH A RARE DISEASE :

- Delays in diagnosis
- Discrimination
- Compensation of disability
- Access to care: Access to biological tests, trans-border testing, access to medical devices and equipment, access to home care, care at school
- Best practice guidelines for care management

POLICY AND IMPLEMENTATION :

- Access & Advocacy
- European, Regional, State and Local Policy
- Information on rare diseases
- Internet resources and training, online tutorials

RESEARCH ISSUES :

- Data Collection and Use
- Research networks
- Funding of research
- Tissue and DNA collection
- Partnership between public and private sector

SYSTEMS AND ORGANISATIONAL ISSUES :

- Access & Advocacy
- Community Based Organisation and patients' groups as a factor for change
- Reference centres for rare diseases
- Trans-border access to care

The body text of your abstract must not exceed 300 words.

Use the following website:

www.rare-Luxembourg2005.org

Deadline for Abstract Submission :
Friday 15 April 2005, 24.00 hrs CET.

Please also note that your abstract will only be included in the scientific programme, if you register and pay the registration fee before 31 May 2005.

Artistic event

A dance spectacle with disabled performers : Psico Ballet from Madrid
Location : Grand Théâtre du Luxembourg
Date : 21 June 2005
Conditions for participants : free, but reservation mandatory

A conference supported by :

- European Commission, DG Health
- Government of Luxembourg
- Association Française contre les Myopathies AFM-Téléthon
- Prous Science

"This contribution represents the views of the author. These views have not been adopted or in any way approved by the Commission and should not be relied upon as a statement of the Commission's or DG Health & Consumer Protection's views. The European Commission does not guarantee the accuracy of the data included in this contribution, nor does it accept responsibility for any use made thereof."