



**EURORDIS**

# **The role of patient associations for improving the quality of life of people with rare diseases**

**1st Eastern European Conference on Rare Diseases and Orphan  
Drugs, "Promotion of Healthcare and Research on Rare Diseases and  
Orphan Drugs in Eastern European Countries"**

**27 May 2005**

**Christel Nourissier**

**EURORDIS**

---



EURORDIS

# What can be done ?

- Bring together people living with rare diseases and their families
    - from different social, professional backgrounds: anyone can be stricken by a rare disease
    - from different parts of each country
    - from all member states at European level
  - network with professionals
  - create a community
-



EURORDIS

# Our challenge

- People living with rare diseases do not exist
    - poor diagnosis, lack of epidemiological studies, absence of WHO codes
    - physical and social stigma : disabling and disfiguring diseases, 80% of genetic origin
    - schooling and employment problems
  - The burden affects the whole family:
    - parents, grand parents, siblings
  - Very high social and human costs
-



EURORDIS

**Regulatory bodies**

experts in scientific evaluation

law and regulations

**Patients and families**

**living with a disease**

experts in their own disease

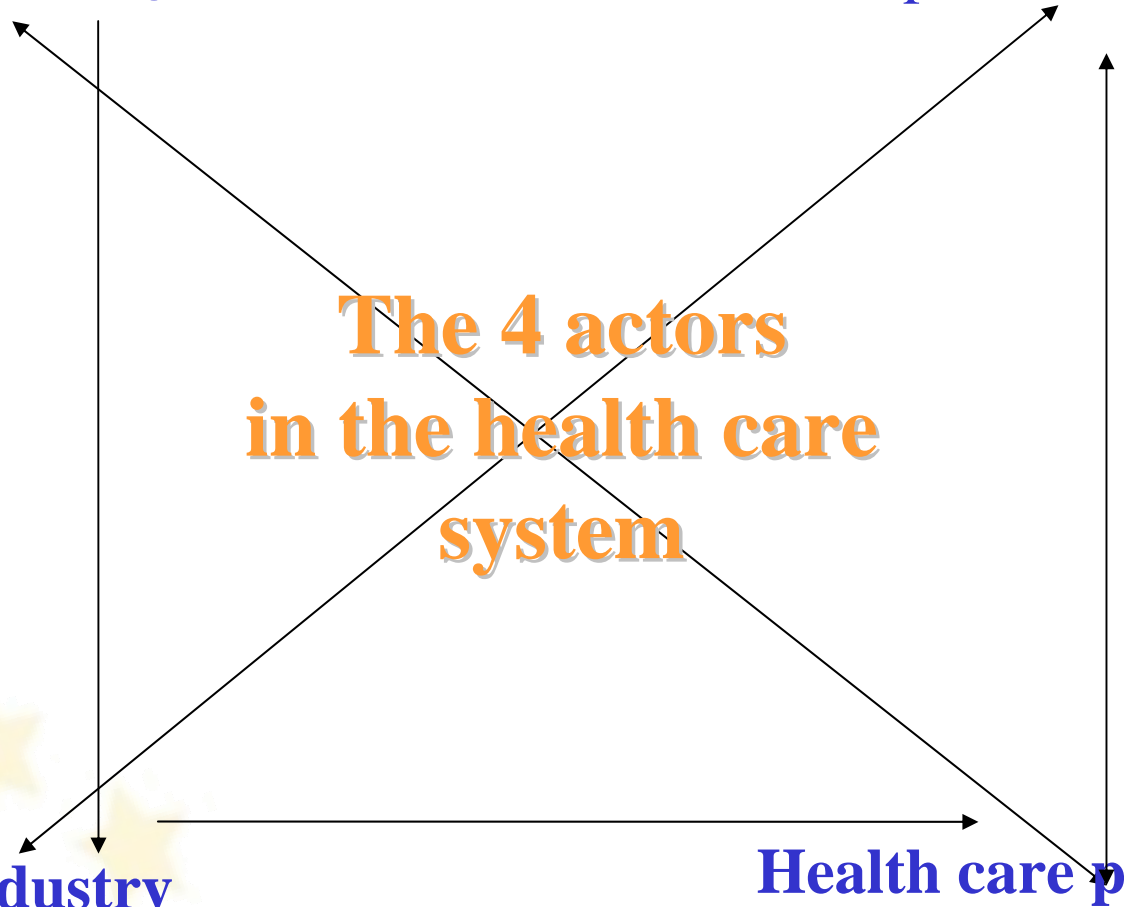
**The 4 actors  
in the health care  
system**

**Industry**

experts in developing  
and marketing drugs

**Health care professionals**

experts for  
diagnosis, care and treatment





EURORDIS

# What is Eurordis?

## European organisation for rare diseases

- Established in 1997 by 4 patient associations, modelled on the National Organisation for Rare Disorders – NORD, USA
  - non governmental, non for profit, independent
  - A membership based organisation:
    - 225 members in 23 countries, 10 National Alliances
    - covers over 1000 rare diseases
  - A pan-European active network:
    - Board: 12 patients or parents. Over 60 volunteers from different patient groups involved in task forces, committees...
    - over 400 patient groups participated to Eurordis activities in 2003-2004: projects, surveys, conferences, training, as experts
  - Represents 35 million people living with rare diseases
-



EURORDIS

# How do PA improve access to diagnosis?

- by creating web-sites, web-forums, sending disease-specific information to health professionals
  - by raising awareness of MDs, carers and general public about rare diseases: information campaigns, media contacts
  - by helping people suffering with very rare diseases to get together
  - *Eurordis Eurobiobank project*: a network of DNA and tissue banks
-



EURORDIS

# How do we encourage research?

- By creating PA data bases: useful epidemiological data, and a way to learn about the natural history of the diseases
  - by bringing together scientists, discussing research protocols, informing our members about research studies,
  - by finding funds: successful funding stories: Telethon in France and Italy
  - by disseminating outcomes
-



EURORDIS

# How do we improve access to care?

- by raising awareness about the necessity of early intervention and prevention
  - “there is always something to do”
  - by disseminating research outcomes and best practices
  - by providing clear, concise, information
  - by benchmarking best practices in EU
  - by supporting the creation of centres of excellence
-





EURORDIS

# Patient Associations advocating at the EU level

- Why?
  - To promote rare diseases as a public health issue
  - to make policy makers more aware of rare diseases
  - to bring forward concrete proposals
- How?
  - interactions with policy makers at national and European level
  - position papers, letters approved by members on EU public health, drugs and research policies
  - participation to the European Health Policy forum,  

---

the European Patients forum, the Rare Diseases task force



EURORDIS

# Advocacy: some successful outcomes

- 1999: European regulation for orphan drugs, 3 representatives of PA in COMP
  - creation of COMP WGIP, and EMEA WGPO
  - Project of regulation for paediatric drugs
  - 1999-2005: priorities for rare diseases in the European Framework Programmes for Research and Health
  - 2006-2013: EC proposals for the Research and health FP: budgets doubled
-



EURORDIS

# Improving access to drugs

- 2001: clinical trials directive: role of PA in the ethics committees
  - Eurordis charter for sponsors and patient organisations, PO training for understanding clinical trials protocols
  - Eurordis survey on real access to drugs after marketing authorisation
  - pharmacovigilance: improved reports, self-reporting in the future
-



EURORDIS

# How to support patients and families

- Reaching out isolated people: volunteer and professional help-lines, particularly
    - after diagnosis
    - in crisis situations
    - when participating in clinical trials
    - at the end of life
  - regional, national and European meetings
  - networking with professionals
-



EURORDIS

# The European Union disability strategy

- Towards a society open and accessible to all:
  - 2000: EU anti discrimination directive
  - needs and rights of people with disabilities integrated in all policies and programmes
  - people with disabilities involved in their planning, monitoring and evaluation (EDF)
  - 1999: Joint Declaration on Employment
  - High Level Group of Member States: implementation at national level
-



EURORDIS

# Better compensation and respite care

- PO explain their rights to people living with rare diseases and their families
  - advocate for financial, technical and human compensation policies at national level
  - support children integration in schools
  - and adults throughout their life (at home, in group homes, in the working environment)
  - organise holidays for children and adults with special needs, family week ends with siblings...
-



EURORDIS

# European Conference on Rare Diseases



European Conference on Rare Diseases  
2005

- 3rd European Rare Disease Conference, after 2001  
Copenhagen, 2003 Paris, 21-22 June 2005 in Luxemburg
- organised by Eurordis and its partners, supported by EU DG Sanco/EU Presidency, AFM
- **web-cast 24 June**  
**[www.rare-luxembourg2005.org](http://www.rare-luxembourg2005.org)**
- from difficulties to solutions for the rare disease community

*Bringing together patients, researchers, health policy experts, professionals...*



EURORDIS

# Key points to start successful actions

- Most rare diseases patient groups are run by volunteers, on very small budgets:
  - disseminate patient-friendly information
  - use the internet to fight isolation
  - organise the training of patient representatives
  - establish links with other groups at a national and European level
  - share best practices
  - *We can achieve a lot by getting together...*
-