

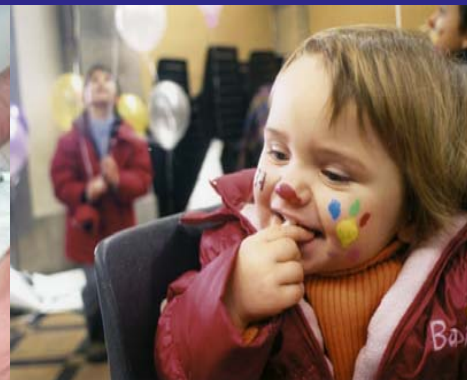
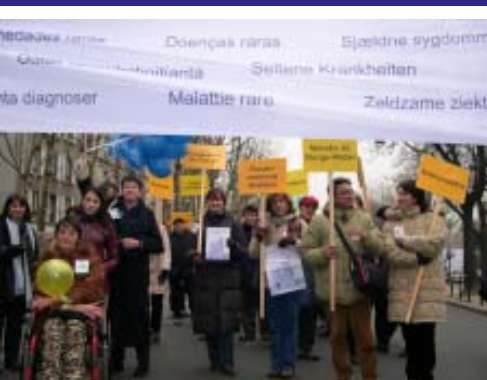


VHPB/ELPA MEETING

Prevention and Control of Viral Hepatitis

“The role and impact of patients and advocacy groups in and outside Europe”

Lucca (Italy) March 13-14, 2008





"European Patients Groups for Rare Diseases"

Dr. Michele Lipucci di Paola
Thalassemia AVLT / EURORDIS



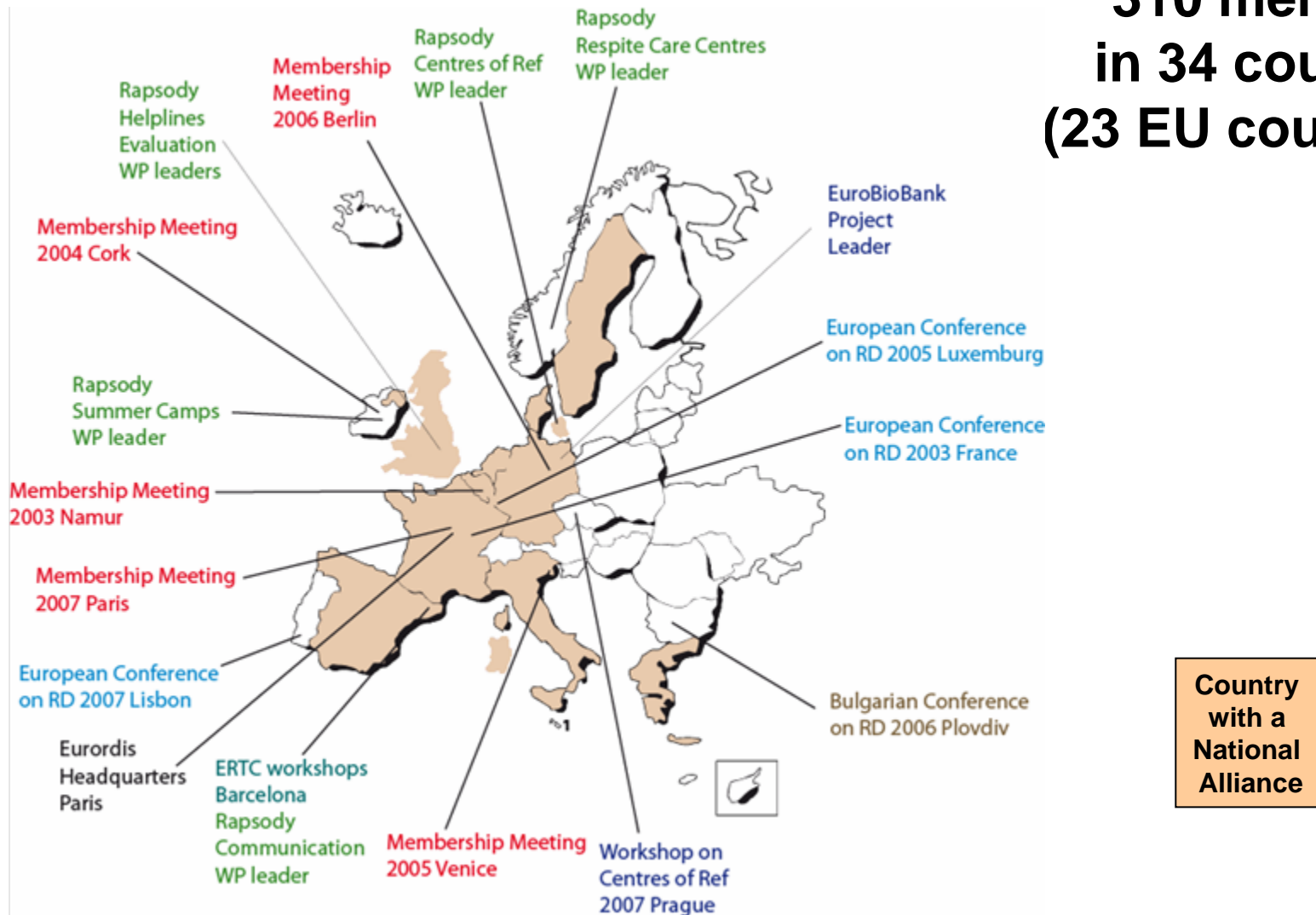
EURORDIS IN BRIEF

- **Founded in 1997**
- **Patient-driven**
- **Non governmental - Not for profit**
- **> 310 member organisations in 34 countries**
- **> 1.000 rare diseases covered**
- **18 staff in Paris and Brussels**
- **120 volunteers involved in task forces, steering committees, working groups and the Board of Directors**
- **700 patient groups participate in Eurordis' activities in 2007**
- **Partner in the Rare Disease Platform**



EURODIS' PRESENCE IN EUROPE

**310 members
in 34 countries
(23 EU countries)**



WHAT IS A RARE DISEASE?

A rare disease in Europe is a disease affecting less than 1 in 2,000 citizens

- **< 230,000 EU citizens per rare disease**
- **6,000 to 8,000 rare diseases**
- **30 million people affected in the EU.**
- **6 to 8 % of the EU population**



CHARACTERISTICS OF RARE DISEASES

- **80% genetic diseases**
- **Patients are few and geographically spread**
- **Relevant information is scarce**
- **Experts are few**
- **Specialised care centres for each disease cannot exist in every country**
- **Resources are limited**
- **Research is fragmented**



PRIORITY 1: NETWORKING & INFORMATION SHARING

- More than 310 members in 34 countries
- Council of 13 National Alliances & Network of > 300 Allied members
- Organisation of annual membership meetings (2002 Barcelona, 2003 Namur, 2004 Cork, 2005 Venice, 2006 Berlin, 2007 Paris)
- Organisation of the European Conference on Rare Diseases (2001 Copenhagen, 2003 Paris, 2005 Luxemburg, 2007 Lisbon) every two years
- Website in 6 languages: English – French – Spanish – Italian – German – Portuguese (www.eurordis.org)
- Monthly electronic newsletter in 6 languages

PRIORITY 2: ADVOCACY & POLICY DEVELOPMENT

- **Contribution to the adoption of the EU Regulation on Orphan Medicinal Products in 1999**
- **Contribution to the adoption for the EU Regulation on Medicinal Products for Paediatric Use in 2006**
- **Contribution to the promotion and maintenance of rare diseases as a priority in the EU Research Framework Programmes**
- **Contribution to the adoption of EU Regulation on Advanced Therapies April 2007**
- **Contribution to the promotion and maintenance of rare diseases as a EU public health policy priority: EU Commission Communication on European Actions in Rare Diseases 2007**
- **First European Rare Diseases Day, 29 February 2008: 18 countries**

EURORDIS IS PRESENT IN MANY EUROPEAN INSTITUTIONS AND PLATFORMS



European Institutions:

- **Committee for Orphan Medicinal Products at the EMEA (COMP)**
- **Committee for Pediatric Use Medicinal Product at the EMEA**
- **EMEA/CPMP Working Group with Patients' Organisations**
- **Rare Disease Task Force at DG Health and Consumer Protection**
- **EU Health Policy Forum at DG Health and Consumer Protection**

European Platforms:

- **European Patients' Forum (EPF)**
- **European Platform for Patients' Organisations, Science, and Industry (EPPOSI)**
- **European Forum for Good Clinical Practice (EFGCP)**
- **International Alliance of Patients' Organizations (IAPO)**
- **Pan-European Blood Safety (PBSA)**

PRIORITY 3: ACCESS TO INFORMATION, DIAGNOSIS, TREATMENT & CARE

- **Guidelines (9 languages) to support creation and management of rare disease patient groups and information services**
- **Mapping of rare disease patient organisations' profiles and activities in Europe**
- **Scientific surveys to increase our advocacy power**
- **Training sessions for patient representatives**

PRIORITY 4: THERAPEUTIC DEVELOPMENT & RESEARCH

- **Contribution to transparent and quality information on medicines for patients, improvement of pharmacovigilance: participation in the European Medicines Agency Working Group with Patients' Organisations**
- **Creation of a European network of rare disease Biological Resource Centres (EuroBioBank) for DNA, cells and tissue**
- **Partnering in many European research projects: EuroClinGene, ECRIN, Orphan Platform, TREAT-NMD etc**
- **Contribution to obtaining EU funding for rare disease research projects**
- **34 pharmaceutical companies are members of the Eurordis Round Table of Companies**

European network of biobanks of DNA, cells and tissue for rare disease research

- **Budget: 1,219,321 €** (funded by the European Commission within the 6th Framework Programme) and by major patient groups
- **16 partners in 8 countries** (Belgium, France, Germany, Hungary, Italy, Malta, Slovenia and Spain)

- **30 standard operating procedures**
- **A web-based catalogue** displaying 971 sample collections
- **7 languages** (English, French, German, Hungarian, Italian, Slovene, Spanish)
- **170,000 samples** in the network at the end of 2005
- **64,000** samples collected during the project
- **6,047 samples** exchanged in the network in 2005
- **41 scientific publications** have mentioned EuroBioBank



TRAINING SESSIONS FOR PATIENTS

- **‘Providing information on rare diseases’**
- **‘Searching for validated biomedical information on the Internet’**
- **‘Understanding clinical trial protocols’**
- **‘Getting involved into the EU drug regulatory decision making process’**
- **‘Patient databases, registries and cohorts’**
- **‘Gaining access to rare disease research resources’**



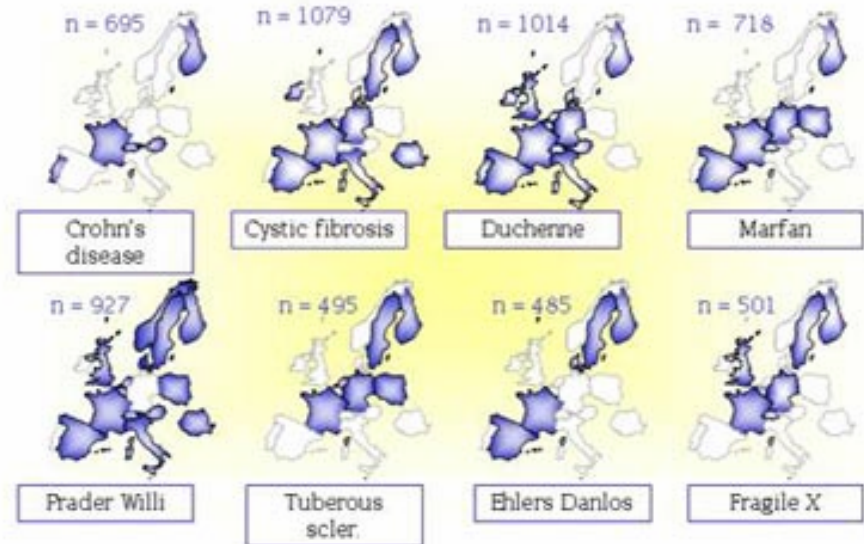
THE 'EURODISCARE' PROGRAMME

A series of surveys to compare access to care for rare disease patients across Europe

- Scientific methodology to collect new and solid data from patient groups, to increase their advocacy power
- Collaboration with scientific research teams (Inserm, London School of Economics)



Diseases and Countries



THE 'EURODISCARE' PROGRAMME

- **2004: Compare needs and access to care**
 - 6 rare diseases, 17 countries
 - 50 patient groups, English language

- **2005: Compare delays of access to diagnosis and the causes**
 - 8 rare diseases and 17 countries
 - 70 patient groups, 12 languages, 6 000 questionnaires filled
 - The objective of the study was to show the delays to diagnosis and to identify the main causes.

THE 'EURODISCARE' PROGRAMME

- **2006:**

Compare experience and expectations of patient and families on health care and specialised services in Europe

→ 12 rare diseases, 20 countries, 8,500 patients

→ Access to and organisation of health and social care and medical services. This survey will provide patient and family experience on centres of reference for rare diseases and their expectations.



ONLINE PATIENT COMMUNITIES

‘Mailing Lists for Patients with Rare Diseases’: a pilot

- **4 lists as of July 2006**
 - Ichthyosis
 - Fragile X
 - Prader-Willi
 - Sanfilipo

- **6 more lists at the end of 2007**
 - Guenther’s porfiria
 - Behcet
 - Anorectal malformations
 - Sarcoidosis
 - Dancing eyes
 - 11q

- **Good practice guidelines**

- **<http://eurordis.medicalistes.org/>**



THE RARE DISEASE PATIENT SOLIDARITY PROJECT

- **Creation / development of services & networks for people living with rare diseases**
 - Help lines
 - Integration at school
 - Summer camps
 - Respite care centres

- **Reflection process on the organisation of care for rare diseases, including centres of reference in Europe**
 - Patient survey (EurordisCare)
 - National workshops on centres of reference will be held in 10 European countries 2006-2007
 - European workshop on centres of reference to be held in Prague on 12-13 July 2007



EURORDIS PATIENT REPRESENTATIVES IN ODs

- **Patient groups play a key role in policy development:**
 - EU Regulation on Orphan Medicinal Products in 1999
 - EU Pharmaceutical legislation Review in 2003 (including conditional approval, EU centralised procedure for all orphan drugs)
 - EU Regulation on Paediatric Use of Medicines in 2006 (including extension of orphan drug market exclusivity to 12 years)
 - EU Regulation on Advanced Therapies in 2007
- **Patient representatives are in the decision making process**
 - 3 members in the COMP + 1 Observer: policy, guidelines, designation opinion, significant benefit opinion)
 - Patient experts in Protocol Assistance procedure
 - Patient experts in Risk Management Programme
- **Patient groups validate information to public**
 - Public Summaries of COMP Opinions
 - European Public Assessment Reports

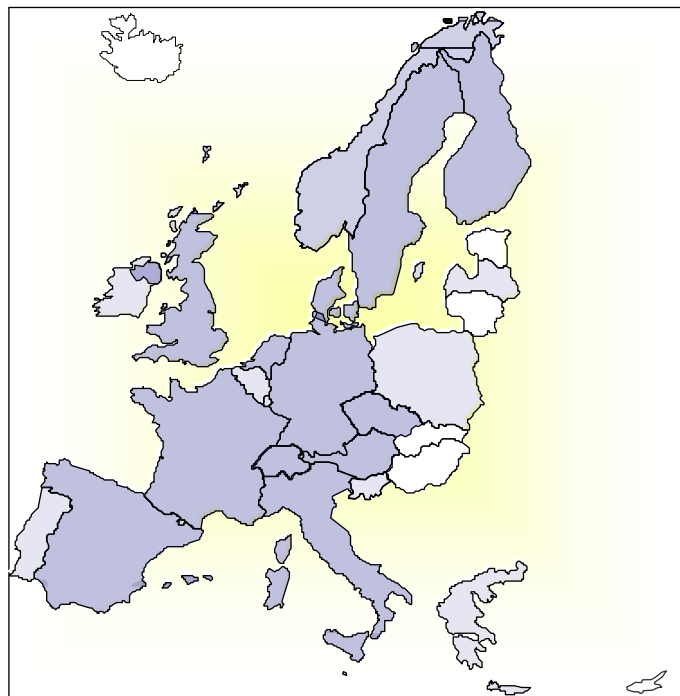
STATUS OF ORPHAN APPLICATIONS

- **783** Applications for orphan medicinal products
- **521** Designations of orphan medicinal products
- **45** Orphan orphan medicinal product MA

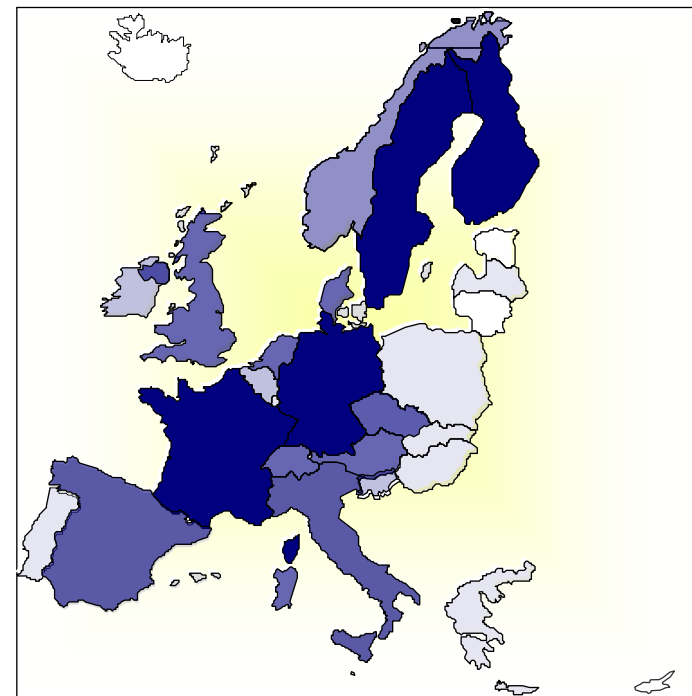
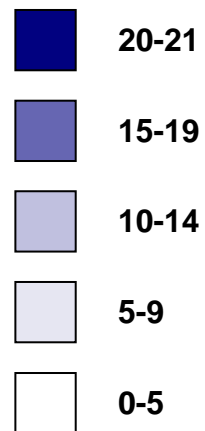
As of February 2008

Availability of OMPs by country in 2007

Availability for OMPs approved before 2004 (12 OMPs) & 2006 (22 OMPs)

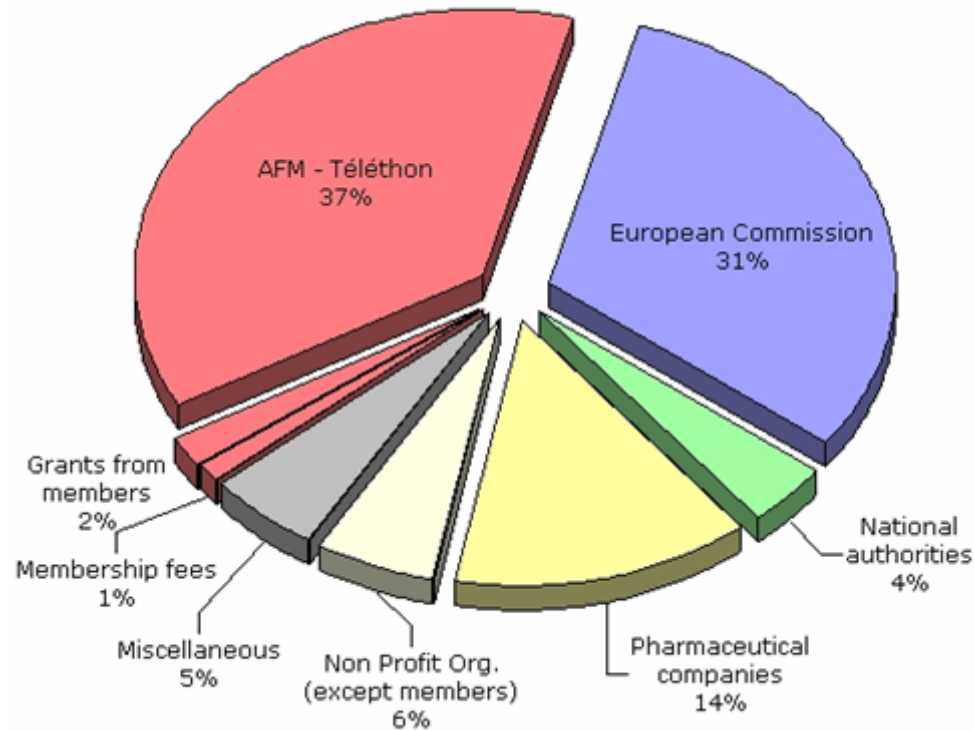


OMP authorised before 01/01/04



OMP authorised before 01/01/06

EURORDIS' FUNDING SOURCES



Total Revenue =
1,698,00 euros

Revenue % by source of funding in 2006

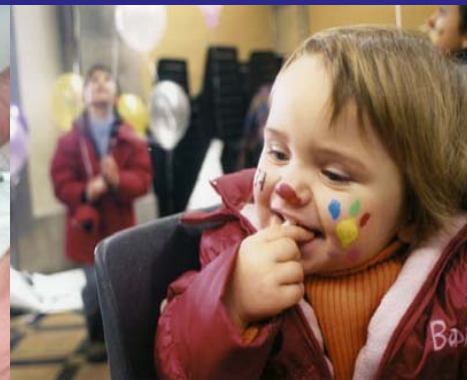
CONCLUSION

- **Empowerment of Patients:** *“ the process of increasing capacity of individuals or groups to make choices and to transform those choices into desired actions and outcomes”*
- **Information Sharing**
- **Training**
- **Networking**
- **High and Firm Commitment**
- **Policy Development: Member States, EU**



***Acting Together
For the Challenge
To Build a EU Coordination
of Health Systems and Policies
For Equity Access across Europe***

Thank You



FOR MORE INFORMATION

- ☑ Michele Lipucci di Paola
Former Vice President of Eurordis
Member of the Working Group with Interested Parties of the
Committee on Orphan Products at the European Medicines Agency
Board Member Pan-European Blood Safety (PBSA)

- ☑ Yann Le Cam - Chief Executive Officer

- ☑ Francois Houyez – Health Policy Officer

- ☑ Fabrizia Bignami - Therapeutic Development Officer

www.eurordis.org