





# A European organisation of patients in action



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Director of Health Policy



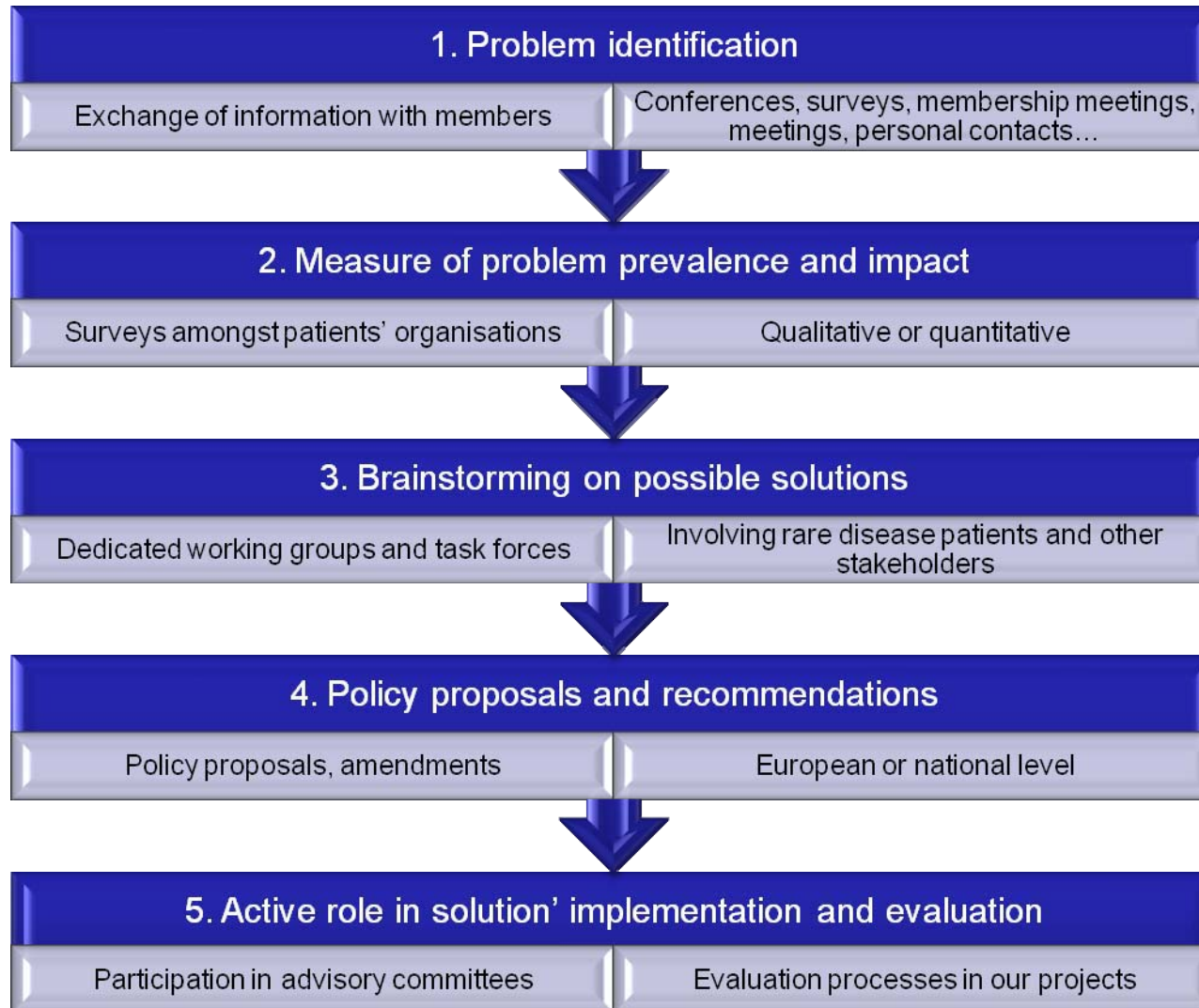
# EURORDIS

- **Created 1997**
- **Members are patients' organisations, not individuals**
- **480 members in 45 countries, of which 33 European**
- **Network of 1500+ rare disease patients groups at large**
- **General assembly elects its governing bodies**
- **Independent, structured around**
  - 26 National Alliances of Rare Diseases organisations: all rare diseases groups in one country
  - 33 European disease specific federations: all organisations, all countries, one disease
  - Individual organisations
- **Signatory of the Code of Practices for the Relations between Patients Organisations and the Health Industry**
- [www.eurordis.org](http://www.eurordis.org)
- **Operating grant DG Sanco**  

**EURORDIS role is to**

**FULFIL THE NEEDS OF PEOPLE  
LIVING WITH A RARE DISEASE**

# How do we work?



# Examples of our work in some key areas







Delays in diagnosis

Access to treatments

Research

Development (Orphan  
therapies)

# Delays in diagnosis

- 1) Many patients reported difficulties in obtaining an “exact” diagnosis (PARD I 2000-2001)  
- 2) EURORDISCARE 2 survey on Delays in Diagnosis (2003-2006, 8 rare diseases, 16 countries, 70 patients’ organisations involved, 12 languages, 5980 replies)
- 3) RAPSODY project + Rare Disease Task Force (2006-2008): Centres of Expertise & Reference Network  
- 4) Commission Communication and Council Recommendations: large participation of EURORDIS members (public consultation phase)
- 5) EUCERD + POLKA project (2008-2011) Evaluation of Centres of Expertise, Directive Cross-border Care  

# Measuring the problem



Disease	Median delay to diagnose 50% of patients	Median delay to diagnose 75% of patients
Cystic Fibrosis	1.5 months	15 months
Tuberous Sclerosis	4 months	3 years
Duchenne Muscular Dystrophy	12 months	3 years
Crohn's disease	12 months	5.8 years
Prader Willi syndrome	18 months	6.1 years
Marfan syndrome	18 months	11.1 years
Fragile X	2.8 years	5.3 years
Ehlers Danlos syndrome	14 years	28 years

**Table1: median time elapsed between the first symptoms and a correct diagnosis**

# Brain storming



**European workshop, Prague, 12-13 July 2007, 80 participants**  
**Synthesis of 11 national workshops, 272 participants total**

**RAPSODY project**    
→ **Centres of Expertise**

# Proposing policies



*Ms Androulla Vassiliou,*

*Member of the European Commission,*

*pleasure of inviting you to the launch of the book:*

*'The Voice of 12,000 Patients – Rare disease from the patients' perspective'*

*on 3 March 2009, from 11.00 to 13.00,*

*Galérie des Présidents, Berlaymont,*

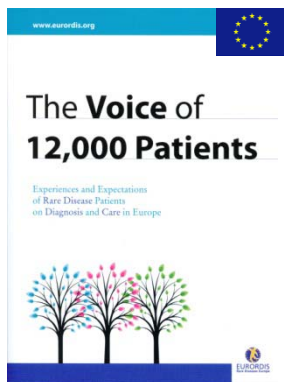
*Brussels*

*to mark the*

*European Rare Diseases Day*

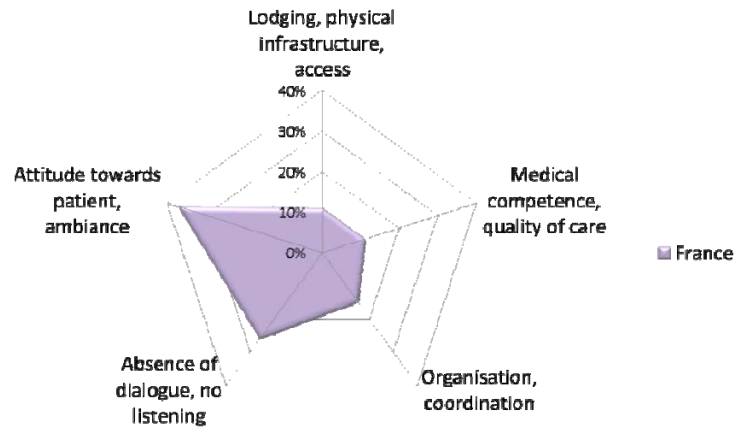


**Senator Deirdre**

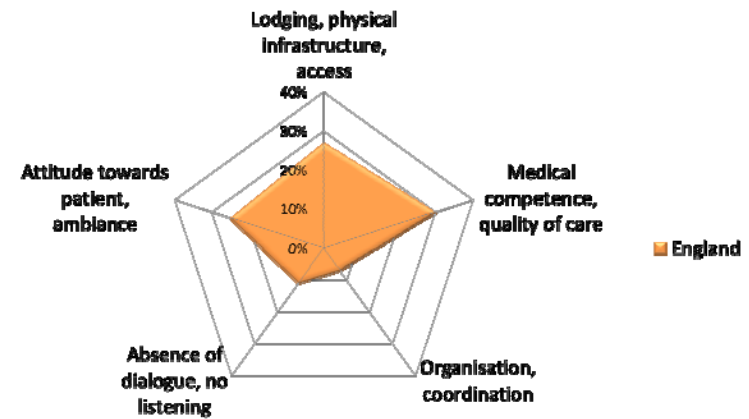


# Deployment -evaluation

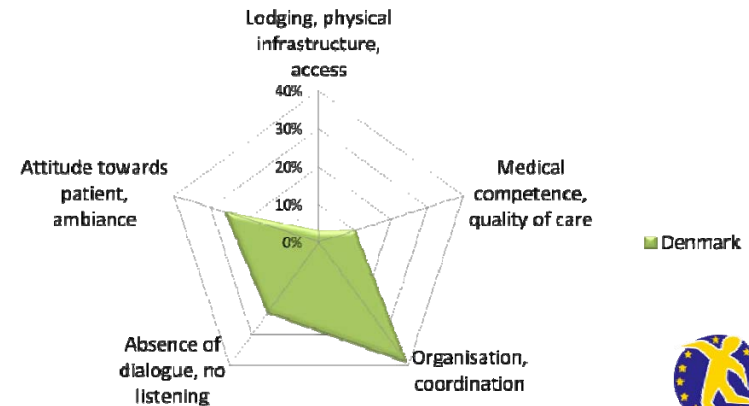
Factors that make patients feel unwelcome at the centre (108 comments, France)



Factors that make patients feel unwelcome at the centre (91 comments, England)



Factors that make patients feel unwelcome at the centre (31 comments, Denmark)



Delphi method (survey-meetings-validation)

207 participants

Patients and healthcare professionals

Centres of Expertise in

Denmark, England, France

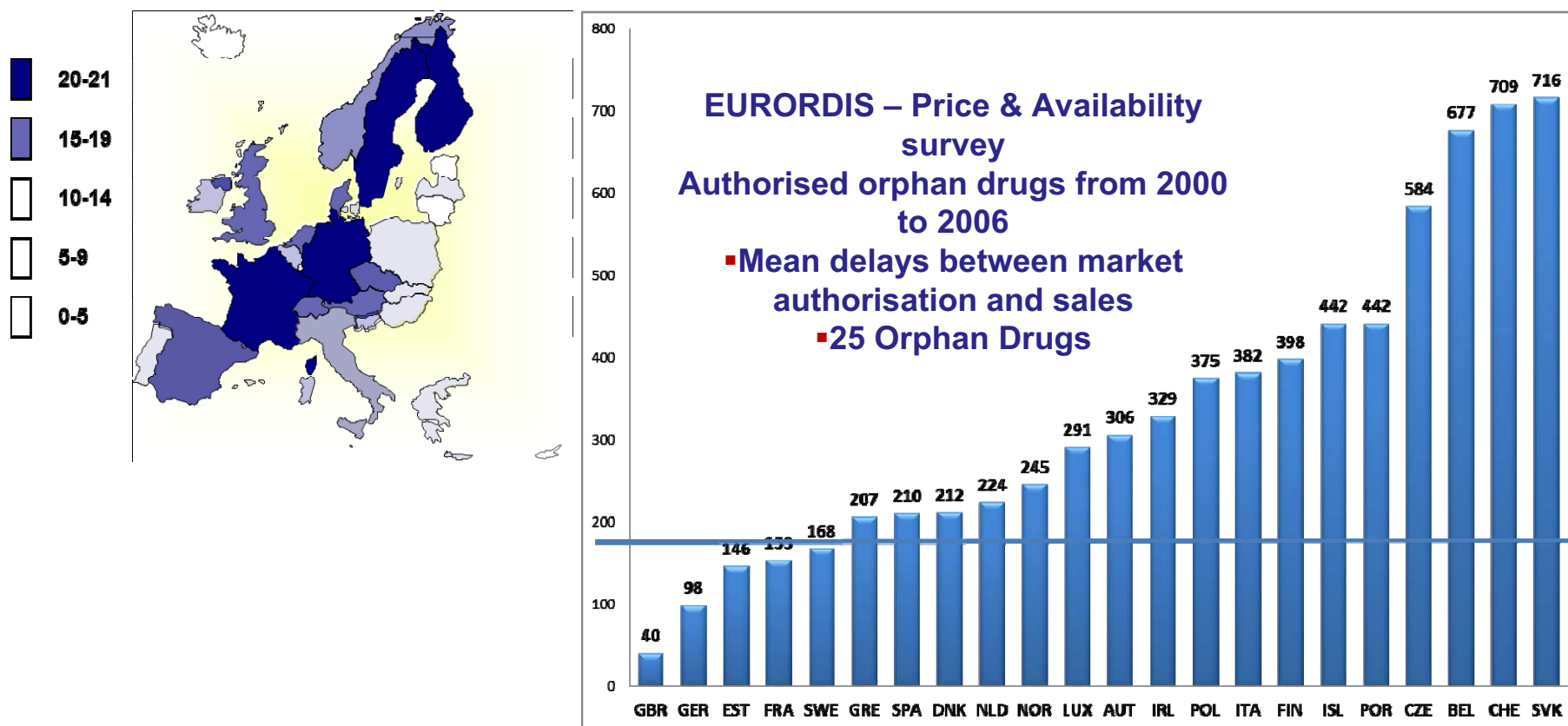
April-July 2011

POLKA project  

→ feedback to EUCERD, contribution to Quality Criteria for Centres of Expertise

# Access to treatments

- Measuring the problem: Price and Availability Surveys



# Brainstorming for solutions











- **At the COMP, where we have members**
  - **With industry (regulatory, drug development, level of evidence, clinical added value, new pricing schemes, compassionate use) – ERTC**
  - **Discussions with the European Commission / Pharmaceutical Forum / Pilot Orphan Drugs**
  - **Training patients on clinical trials & regulatory affairs & health technology assessment**
  - **DECIDE kit on the cost of orphan drug**  
- CAVOD, National Plans for RD



# Other domains where EURORDIS is the voice of the patients

- **Research**
  - Contributes to Research Framework Programmes priority definitions (FP6, FP7)
  - Represents patients in research networks (e.g. Clinigene, TREAT-NMD, ECRIN, ...)
- **Development (orphan therapies)**
  - Contributes to policy shaping and deployment
  - Designation of Orphan Drugs (COMP), + advanced therapies (CAT), + paediatric drugs (PDCO) + involvement of patients at EMA (PCWP) + CHMP consultations

# Major Achievements (recent)

- **European Conference on Rare Diseases ECRD**  
  - Next one 23-25 May 2012, Brussels
  - Largest multi-stakeholders conference on all rare diseases in Europe
- **International Rare Disease Day – last day February**  
  - 56 countries participated in 2011 – Next: 29 February 2012
- **120 patient representatives trained on regulatory issues and health technology assessment since 2008 (Summer School)**  
- **1 704 people expressed their opinion on key policies (neonatal screening, pre-implantation genetic diagnosis, stem cell research, cost of orphan drugs, cross-border care, diagnosis and counselling)**  
- **1 285 people participated in our activities in 2011 (+ more)**
- **8000+ subscribers to EURORDIS newsletter**  

## To conclude

- **EURORDIS is the link between rare disease patients and policy makers and stakeholders**
- **EURORDIS contributes to more democratic decision making process with the opinion of the patients taken into account (bottom-up)**
- **500 members, an active network, 4000 rare diseases, 30 000 000 patients → one voice**
- **Patients' organisations are a guiding light, they make rare diseases visible**