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# Potential to support research and increase awareness on rare diseases research by Eurordis

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**replacing and speaking on behalf of:**

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# A patient-driven European alliance of patient organisations

- Founded in **1997** by 4 patient groups:
- Non Governmental Organisation, Not for Profit, Independent
- A membership based organisation:
  - **225 members in 23 countries**
  - Covers over 1000 rare diseases
  - Represents millions of **People Living With Rare Diseases**
- A pan-European active network:
  - Board 12 members. 8 countries. 8 patients or parents. All representatives of patients groups.
  - Over 60 volunteers from various patient groups are involved in Eurordis' activities through task forces, steering committees, etc
  - Over 400 patient groups have participated to Eurordis' activities in 2003-2004: projects, surveys, conference, trainings, experts...
- [www.eurordis.org](http://www.eurordis.org)





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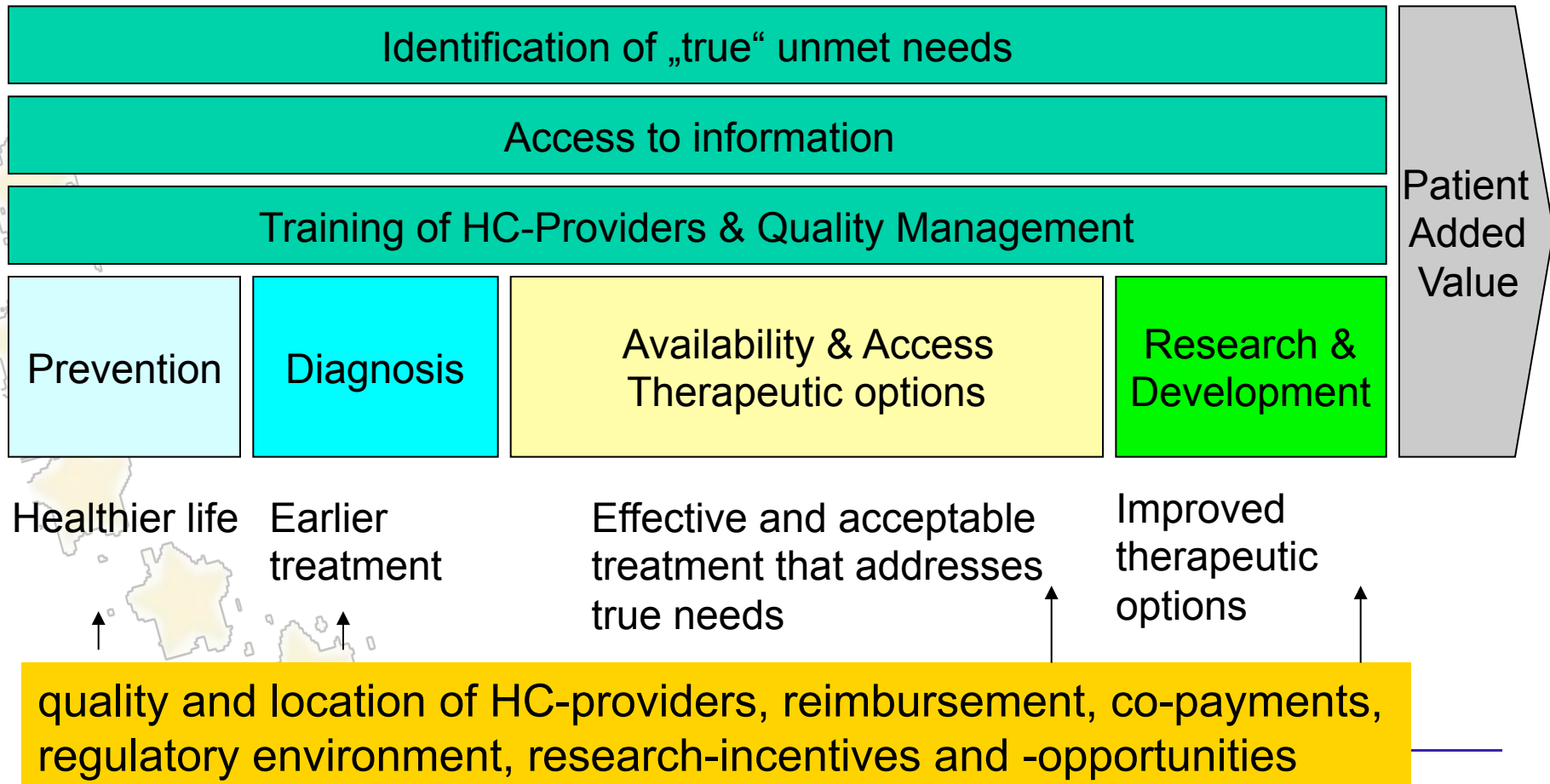
# Mission

Eurordis' mission is  
to build a strong pan-European **community**  
of patient organisations and people living  
with rare diseases, to be their **voice** at  
the European level, and – directly or  
indirectly –  
**to fight** against the impact of rare diseases  
on their lives



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# The patient-centred Health Care-Value Chain





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# Take home message 1

- It is the value to the individual patient, that counts when
  - providing health care services
  - doing research
  - developing new therapeutic options
- Moving from a product-centred to a patient-centred view!





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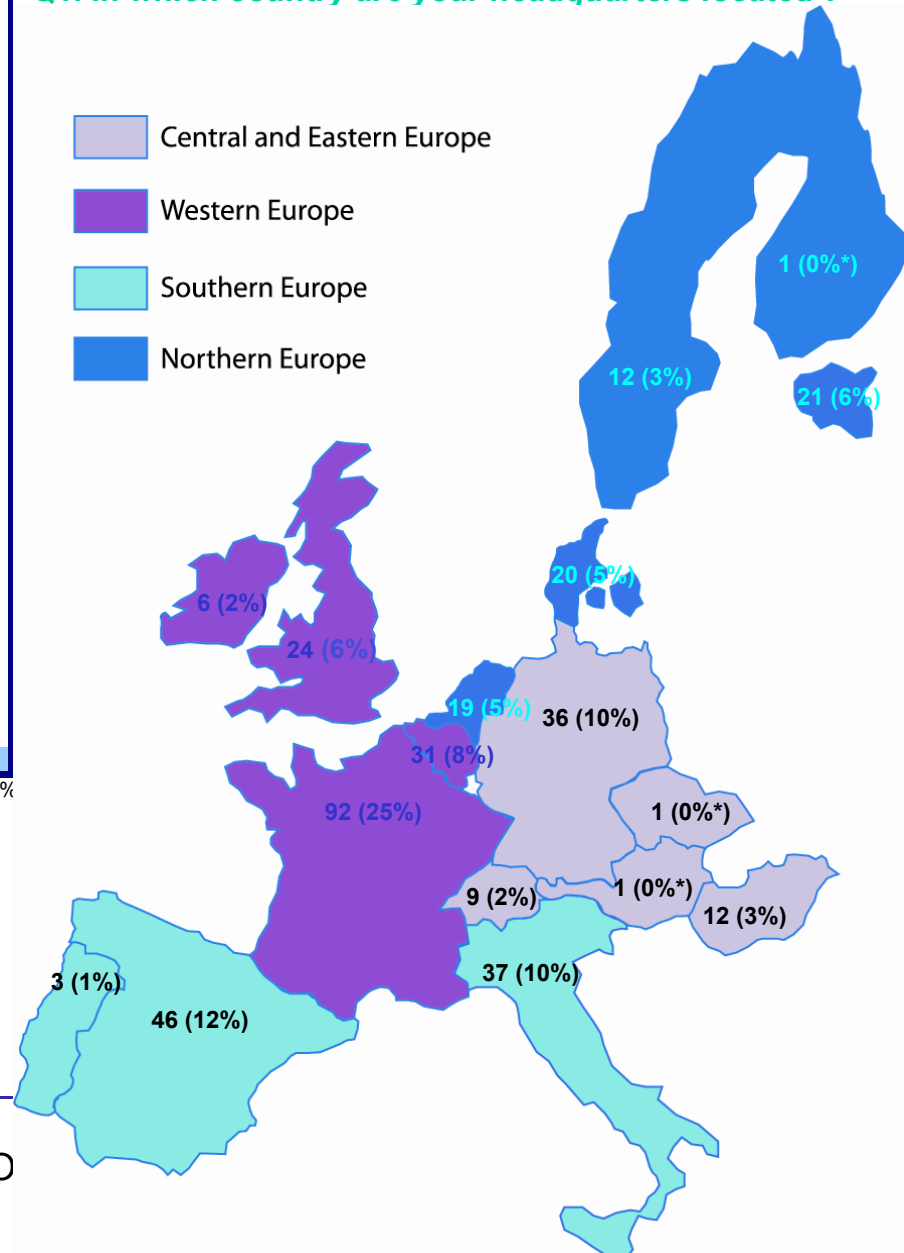
## What patient organisations can do to foster research and awareness for rare diseases ?

An Overview of Actions by Patient Organisations based on a survey conducted by Eurordis between September-December 2003



# 18 countries involved

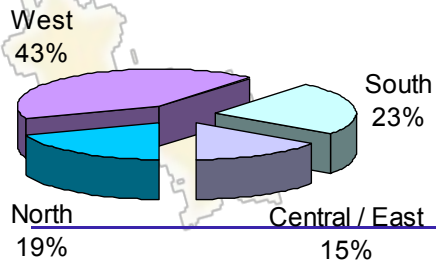
Q1. In which country are your headquarters located ?



Regions	Number of questionnaires analysed	% of total
<b>Northern Europe</b>		
Estonia	21	6
Denmark	20	5
Finland	1	0*
Netherlands	19	5
Sweden	12	3
<b>Total Northern Europe</b>	<b>73</b>	<b>19</b>
<b>Western Europe</b>		
Belgium	31	8
France	92	25
Great Britain	24	6
Ireland	6	2
<b>Total Western Europe</b>	<b>153</b>	<b>41</b>
<b>Southern Europe</b>		
Italy	37	10
Portugal	3	1
Spain	46	12
<b>Total Southern Europe</b>	<b>86</b>	<b>23</b>
<b>Central / Eastern Europe</b>		
Austria	1	0*
Czech Republic	1	0*
Germany	36	10
Hungary	12	3
Switzerland	9	2
<b>Total Central / Eastern Europe</b>	<b>59</b>	<b>15</b>
USA	1	1
<b>Total</b>	<b>372</b>	<b>100</b>

\* Less than 0.5%

## Origin of analysed questionnaires



ICO

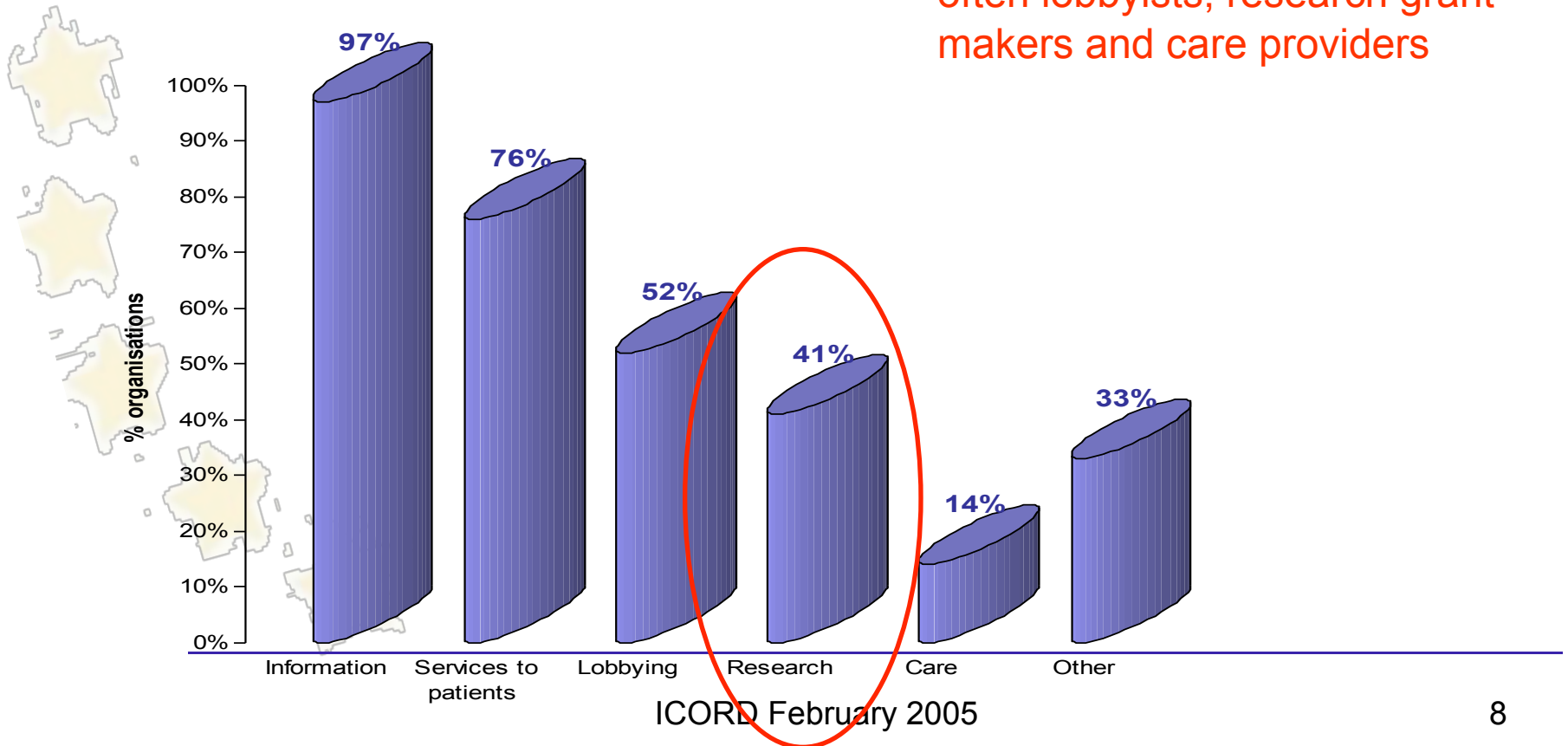


# Patient Org' Emphasis on research: room for improvement

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## Specific activities

However: older groups are more often lobbyists, research grant makers and care providers



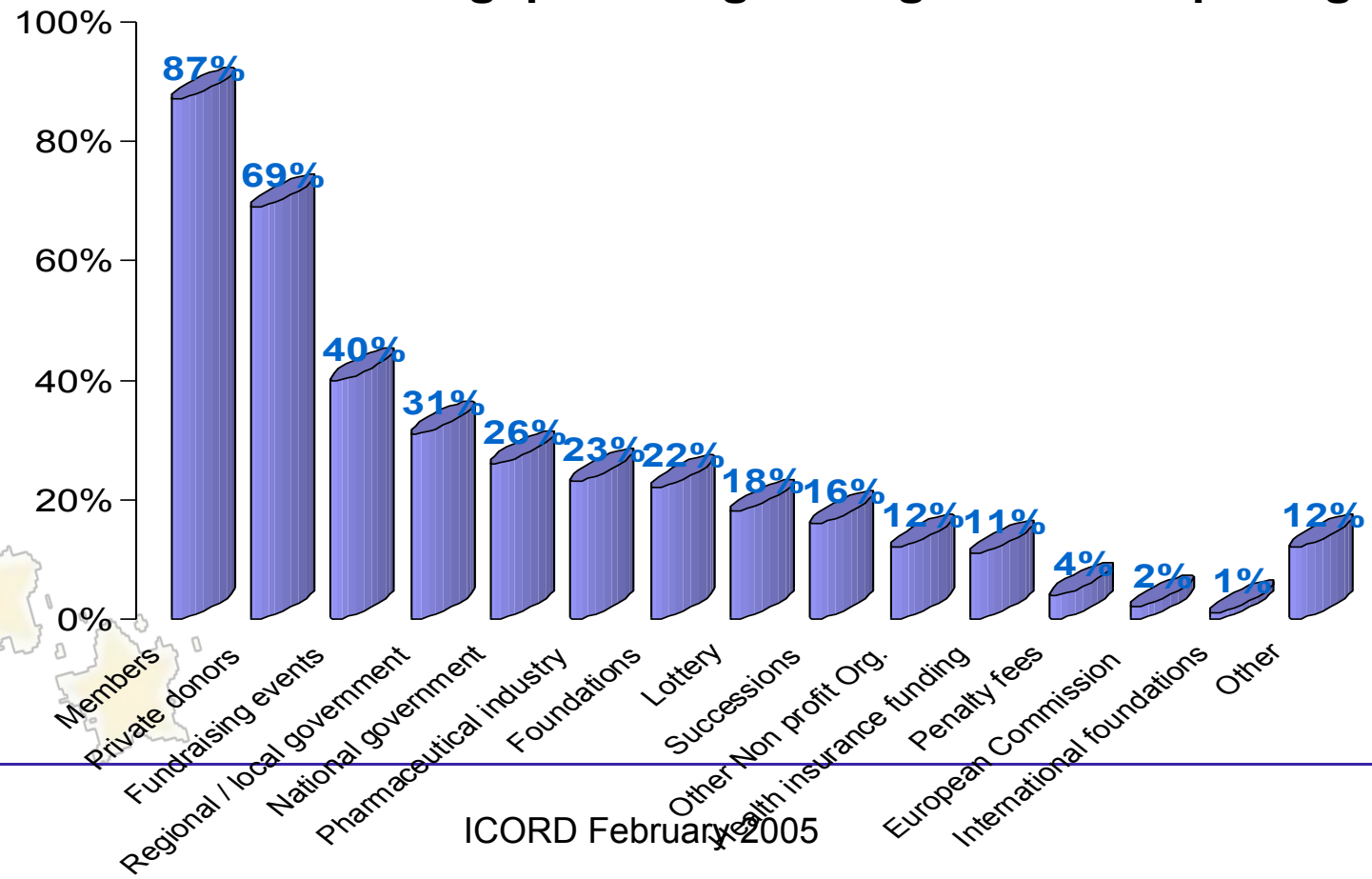




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# Funding: members & private donors

Sources of funding: percentage of organisations quoting source





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# Strong patient organisations are...

- **Empowering** rare disease patient groups,
- **Advocating** rare diseases as a public health issue,
- **Raising awareness** for rare diseases
- **Improving access** to information, treatment and care
- **Encouraging good practices**
- **Fostering rare disease research**
- **Supporting development** of treatments/orphan drugs,
- **Improving quality of life** through patient support, social, welfare and educational services



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# Take home message 2

- Direct support or funding of research is important but not the top priority for patient organisations yet.
- Funding comes from members and private donors mostly.
- Strong patient organisations are of essence





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# Fostering awareness and research for rare diseases

*"To achieve the quickest access to as many safe, efficient, and affordable, new treatments for all rare disease patients in the EU"*

- Participating actively in the regulatory process
- Advocating in the interest of the RD-community
- Partnering with academia and industry
- Driving research projects
- Undertaking public-health research through patient-organisation networks
- Empowering patient organisations



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# An evolving partnership with regulatory authorities

- Active Role at EMEA
  - COMP (2+1), COMP WG Interested Parties (3)
  - CHMP WG Patient Organisations (3)
- Providing patient & medical experts
  - Protocol Assistance: regular participation since 2004
  - Risk Benefit Assessment reports: first cases
  - Risk Management Programmes e.g. Thalidomide, ongoing
  - Over 50 rare disease patient representatives have taken part into EMEA activities in the last 5 years thanks to Eurordis and with EMEA support



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# Advocacy

- Why?
  - To promote rare disease as a public health issue,
  - To raise rare diseases awareness of policy makers
  - To bring forward concrete proposals
- How?
  - Regular interaction with policy makers and other partners
  - Position Papers, Official Comments, Letters
  - Internal European Public Affairs Committee in place
- What?
  - EU public health policy & RD: EU Health Policy Forum, DG Health Rare Disease Task Force, European Patient Forum
  - EU drug policy: orphan drugs regulation, future paediatric drugs regulation, new pharmaceutical legislation
  - EU research policy & RD: FP6, FP7



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# An ongoing example of research advocacy action

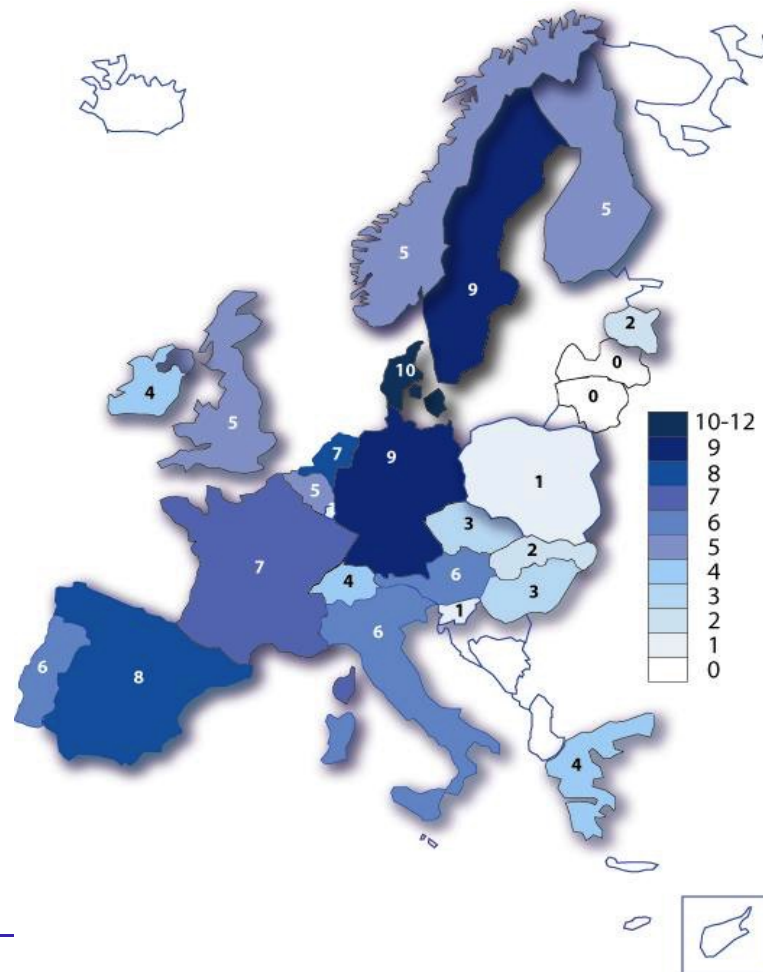
- **7th Framework Programme**: raising awareness on the need for more basic and clinical rare disease research, more EU public funding, more coordination, more competitive research, on key priorities
- Regular proposal and comments sent in 2004
- A recent Eurordis **Position Paper** based on collaborative work with the working group on research for the French Rare Disease Health Plan and further internal consultation with our European Public Affairs Committee
- A **dissemination** to key people and a broad dissemination through Eurordis' electronic newsletter in 5 languages
- A **Discussion Forum** on Eurordis Website, open to members and all interested parties



# A common market = equal access to treatment ?

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As for the Eurordis survey 2003, EU Member states do not provide access to all orphan products authorised prior to January 1st 2004.





# Partnering with industry, academia and regulators

- **COMP Working Group of Interested Parties**
  - 3 representatives from EMEA, COMP, Eurordis, EBE, academia
- **Eurordis Round Table of Companies**
  - created End 2004, with initial membership of 15 companies and with two workshops each year on targeted topics and regular information,
- **European Platform for Patient Organisations, Science and Industry (EPPOSI)**



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# Research project leader/partner

- **EuroBioBank (FP 5)**
  - Eurordis coordinates a European network of DNA, cells and tissue banks for RD with 16 partners including 12 Biological Resources Centres in 8 countries
  - Objective: making biological samples available to research
- **Orphan Platform 2004-2005 (EU FP6 Funding, leader: Segolène Aymé)**
  - Eurordis is a full partner
  - Objective: making information on ongoing rare disease research programmes available and strengthening co-operation with industry (OrphanXChange)
- **Further Projects in planning**



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# Public health research through rare disease patient organisations

- **EurordisCare 1:**

- survey on **access to care**
- 17 MS, 6 rare diseases, 50 associations

- **EurordisCare 2:**

- Survey on **access to diagnosis**
- 21 MS, 9 rare diseases, 70 associations
- 12 languages
- 6000+ questionnaires filled by patients & families, being analysed



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# Empowering patient organisations

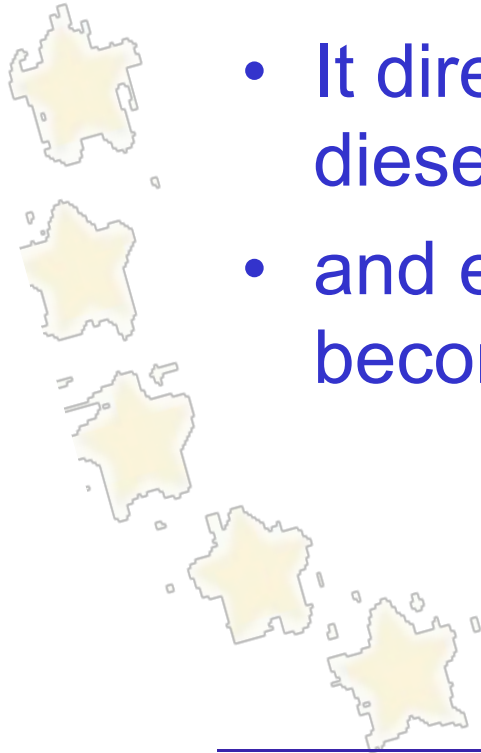
- “Understanding Clinical Trial Protocols” in 2004: 10 sessions for a total of 98 trainees, in partnership with Inserm
- “Understanding Clinical Trial Protocols” in 2005:
  - 4 additional sessions in France with Inserm;
  - Transfer to Spain in Partnership with Fondation Doctor Robert/ Autonomous University of Barcelona + Carlos III + Feder;
  - Ongoing discussion in Italy
- “Understanding Clinical Trial Protocols” in 2005: 2 pilot sessions for in depth 2 days training based on case study
- “How research works?” In 2005: 1 or 2 pilot seminars with Inserm. If successful will be transferred to other countries in following years.



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# Take home message 3

- EURORDIS is an accepted partner of EMEA, academia and industry
- It directly fosters research focused on rare diseases
- and empowers patient-organisations to become drivers of progress



# Added value to the community

- Rare diseases affect about 30 mio citizens of the EU
- Addressing their health needs has an impact on public-health
- RD: the case for EU-policy
- RD can act as „models“ for common diseases
- „Fall-out“ for more common diseases likely
- Developing RD-products makes Europe more competitive



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# Conclusion

- Patients with RD know their needs
- Re-focusing research around these needs
- Patient organisations play a fundamental role in driving patient-centred research for their (own) research
- Win-Win: the entire community benefits



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# European Rare Disease Conference



European Conference on Rare Diseases  
2005

- 3rd European Rare Disease Conference (Copenhagen 2001, Paris 2003)
- 21-22 June 2005 in Luxemburg
- Commission DG Sanco/EU Presidency/Eurordis
- 300 participants
- 5 languages
- A tool to promote interaction between scientists, clinicians, policy makers, patient groups, media and to develop a common agenda for rare diseases





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# For more information

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