THE ROLE OF PATIENT REPRESENTATIVES

„Start by doing what's necessary then do what's possible and suddenly you are doing the impossible" *Saint Francis of Assisi*

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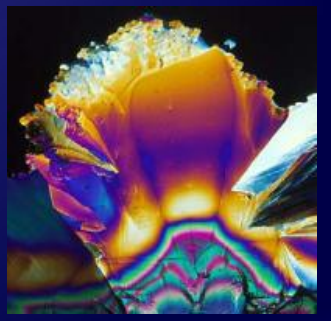
European Union Committee of Experts on Rare Diseases

EURORDIS Annual Membership Meeting, Brussels, 23-25 May 2012

General role

- Articulate the perspective of patients
- Provide insight on issues, problems, and/or questions appropriate to the viewpoint of patients and family members
- Ensure the exchange of information between patients and other stakeholders
- Protect patient's rights and help them learn how to obtain information and services
- Provide tools and information to help patients become empowered



It is not simple

You should have:

- Personal experience with and/or be knowledgeable about the specific illness or condition
- The ability to disseminate information about your experience to the patient community
- Experience as a patient advocate
- The ability to understand and actively participate in discussions with other stakeholders
- Formal affiliation with a patient advocacy organization
- The ability to identify issues that are important to patients

EURORDIS has broad activities on EU and National Policies

1, Policy issues:

- Road Map of DG Sanco for the implementation of EU policy on Rare Diseases
- EUCERD Joint Action, including our WP on social services and EUROPLAN 2
- The subject of the development of National Plans / Strategies in connection with the EUROPLAN 2 project
- Update on FP7 and the 8th EU Research Framework Programme (2014-2020) in the field of research on rare diseases
- The 3rd Public Health programme

EURORDIS has broad activities on EU and National Policies

2, More technical issues:

- Centres of expertise and European Reference Networks on Rare Diseases (eg. POLKA project)
- EU Directive on Cross-Border Healthcare
- Orphanet nomenclature and revision of the International Classification of Diseases (ICD) to include rare diseases
- Report on practices on neonatal screening in Europe
- Achievements of the EU regulation on orphan medicinal products, EUnet HTA and Clinical Added Value of Orphan Drugs (CAVOD)
- Rare cancers at the frontiers between Rare Diseases National Plans or Cancer Plans when they exist

EURORDIS has broad activities on EU and National Policies

3, Awareness raising actions:

- EURORDIS and EUCERD website, Orphanews Europe and EUCERD annual report: State of the Art …
- Global Rare Disease Day
- European Conference on Rare Diseases & Orphan Products (ECRD)
- EC conference on European actions to improve the life of patients with RD, 2011

Rare Disease Day

Brussels 23-25 May 2012

European Conference on Rare Diseases & Orphan Products

The patient representatives in the EUCERD

- 8 patient representatives
- Nominated for their expertise in advocacy issues related to rare diseases
- Covering a wide range of rare diseases including rare cancers
- Cover all parts of Europe

Our underlying working principles

- *Patients and families* are not only „end users" of national strategies and action plans = they *are careers, health and social actors, managing complex day to day care all along their life span*
- *Patient support & advocacy groups are the best allies* to take an active role in shaping research, healthcare and social national and regional policies for rare diseases
- Ensure that patients and *patient representatives are involved at each step of the policy and decision-making processes* in the field of rare diseases
- *Support patient groups and rare diseases national alliances*: awareness-raising, capacity building & training, exchange of information, networking and outreach

Key success factors

- Focus on essential building blocks
- Keep in mind the integrated, comprehensive and long term strategy
- Define clear objectives and indicators
- Build-in the sustainability

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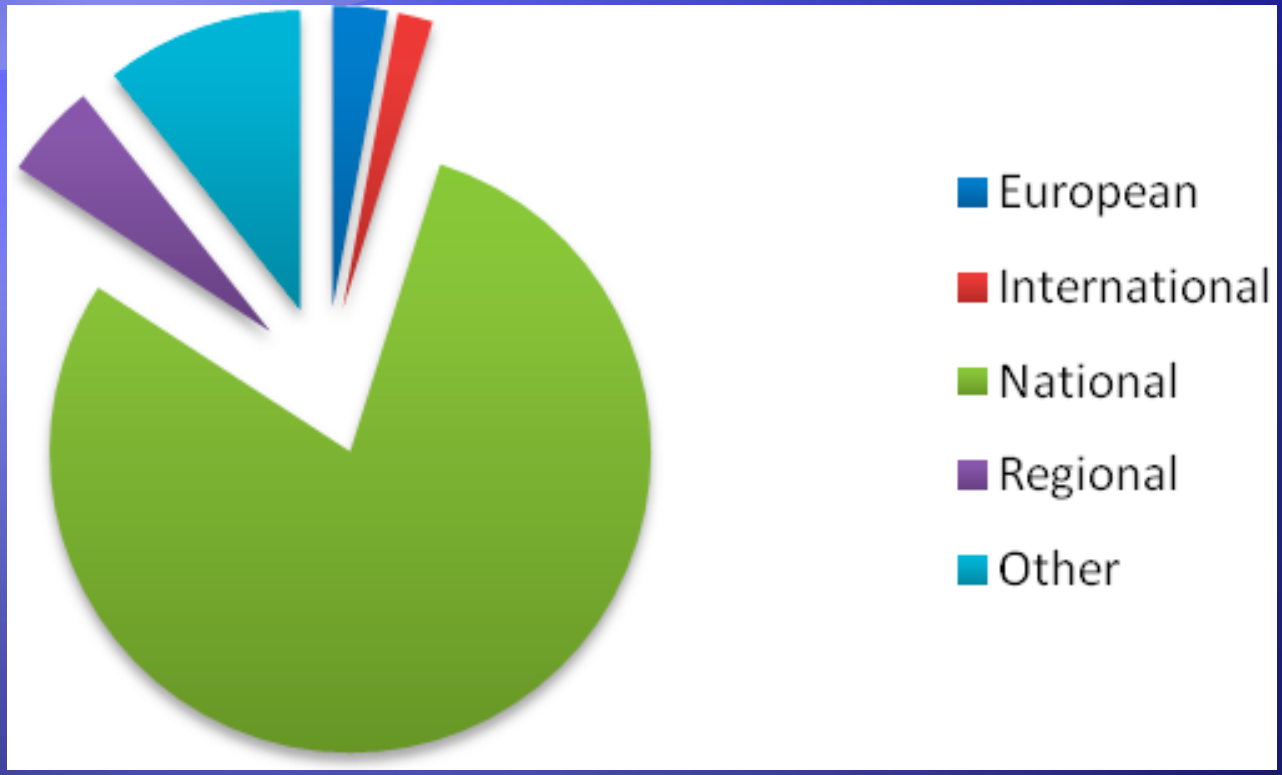
5 or 10 years long strategies and action plans will address only part of the needs of 30 million patients affected by all rare diseases for which there are no cure today!

Some results of the cooperative work

- **The Orphan Medicinal Product Regulation**
  - ***Adopted on16 December 1999***
- **The Commission Communication on Rare Diseases: Europe's challenge**
  http://ec.europa.eu/health/ph_threats/non_com/docs/rare_com_en.pdf
  - ***Adopted on 11 November 2008***
- **The Council Recommendation on an action in the field of rare diseases**
  http://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:C:2009:151:0007:0010:EN:PDF
  - ***Adopted on 8 June 2009***
- **Creating the European Union Committee of Experts on Rare Diseases (EUCERD)**
  http://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:L:2009:315:0018:0021:EN:PDF
  - ***30 November 2009***
- **Determination of EUROPLAN indicators**
- **Elaboration of EUROPLAN Recommendation**
  - ***Discussed in 15 National conferences, adopted on May 2011, Rome***
- **Directive on cross-border healthcare**
  - ***Adopted on 9 March 2011***

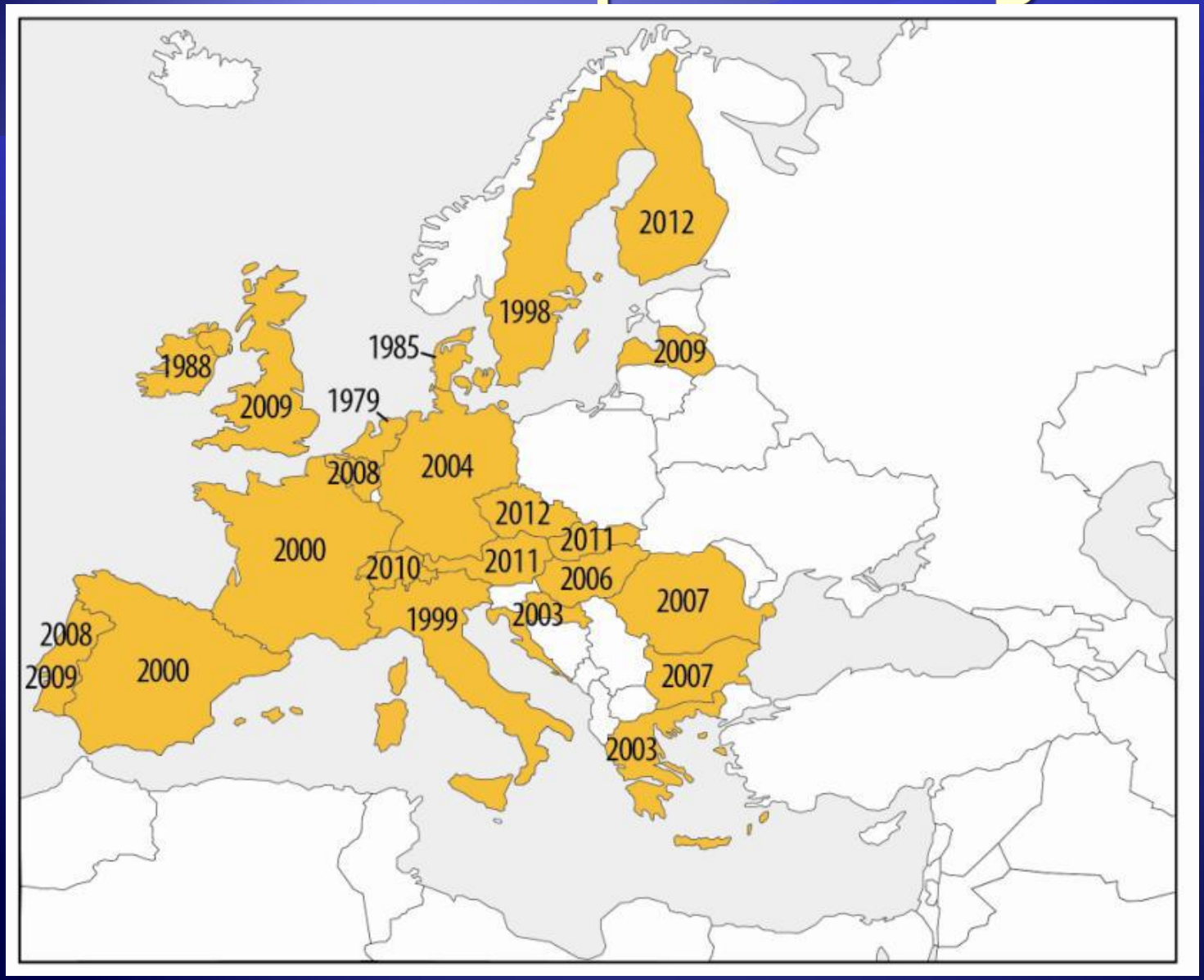
These were hard work! ☺ …

Patient organisations and their representatives had critical role



Altogether 2376 disease-specific patient organisations (Orphanet July 2011)

Countries with a national alliance for rare disease patient organisations



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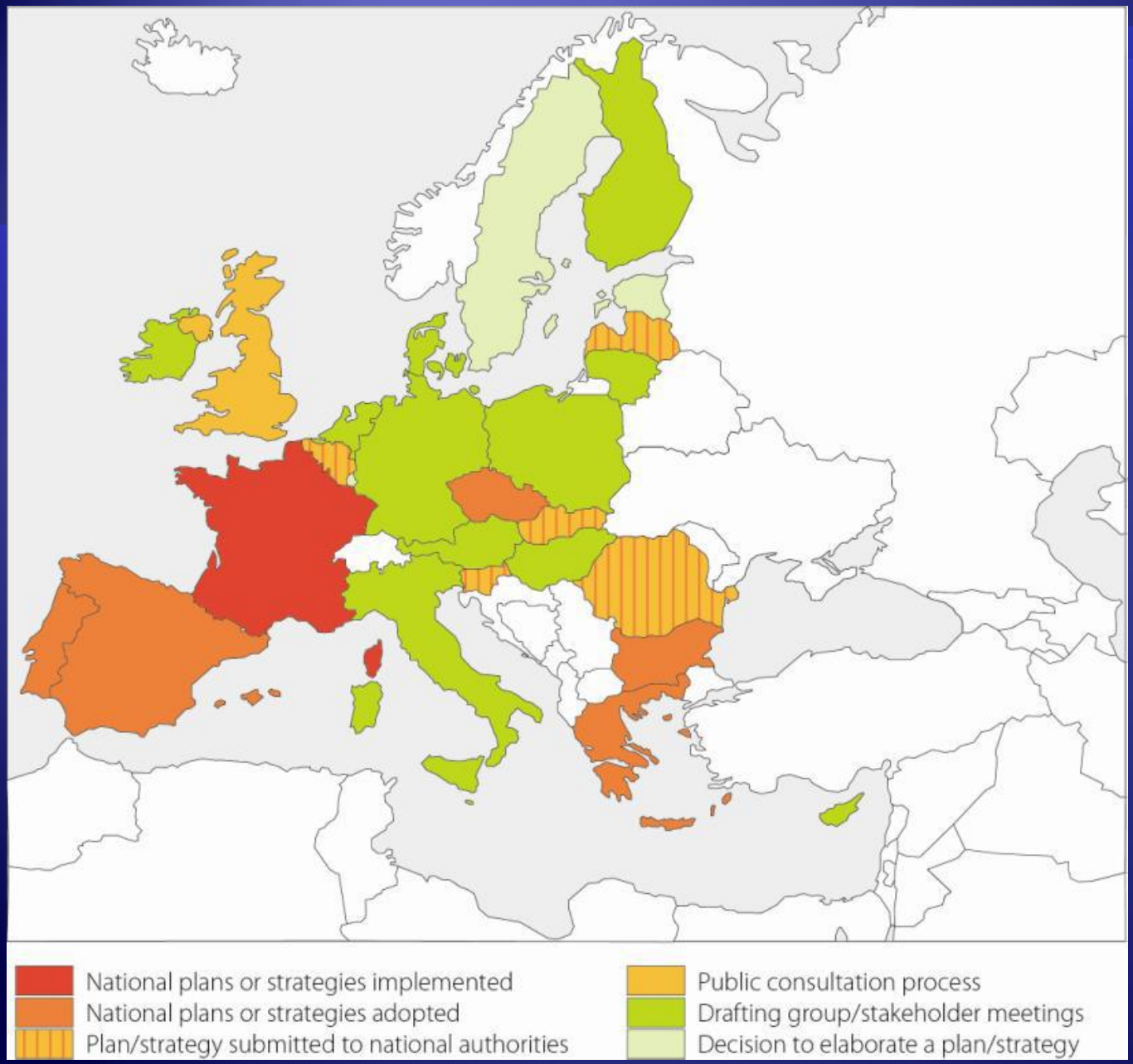
2012

Stages of development of national plans



(in December 2011)

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Thank you for your attention!

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