

# Overview of Member States Public Health Policies for Rare Diseases: 2007 Update

Ségolène Aymé  
Rare Diseases Task Force  
Orphanet / Paris / France  
[ayme@orpha.net](mailto:ayme@orpha.net)



## Source: Inventory by DG Enterprise

- Article 9.3 of Regulation (EC) N° 141/2000 of 16 December 1999
- EC must publish a regular update of the inventory of all incentives
  - To support research into orphan drugs
  - To support their development and availability
- Last inventory: 2006
- Accessible on the EC portal

# Source: OrphaNews Europe

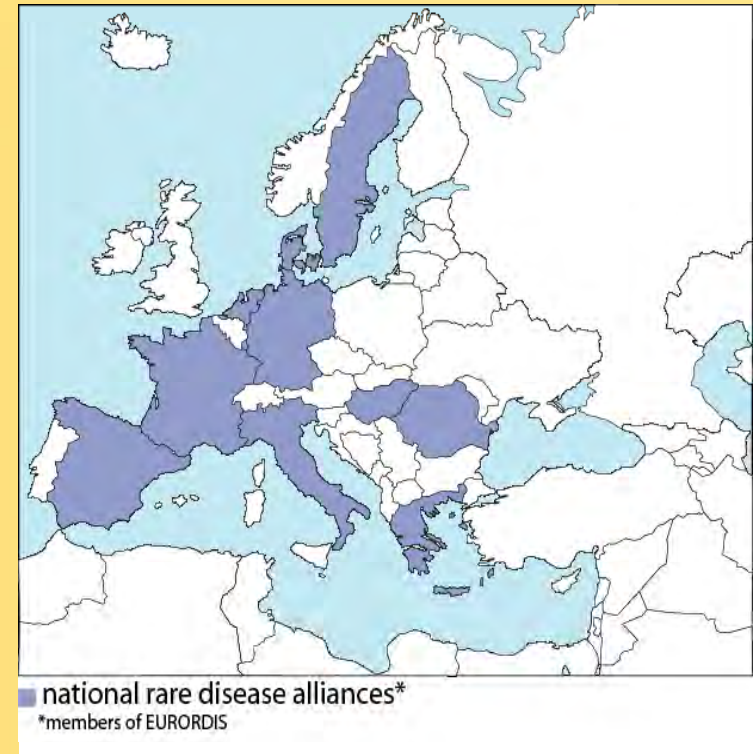
- Online newsletter of the Rare Diseases Task Force
- Over 8,000 registered readers
- Political News
- Scientific News
- Events
- Publications
- Register on Orphanet website



The screenshot shows a Mozilla Firefox browser window displaying the OrphaNews Europe newsletter. The browser's address bar shows the URL <http://www.orpha.net/actor/EuropaNews/2005/050615.html>. The page features a header with the logo "orphaNews Europe" and the subtitle "Newsletter of the Rare Diseases Task Force". The date "15 June 2005" and a "print" icon are visible in the top right. A left sidebar contains a table of contents with categories such as "Editorial", "Task Force Update", "Feedback", "Spotlight on...", "EU Policy", "National Policy News", "Research in Action", "Prevalence/Surveillance", "Partner Search", "Funding", "Orphan Drugs", "Ethical, Legal & Social", "Patients Associations", "Courses & Education", and "What's on?". Below the sidebar, there are links for "Subscribe / Unsubscribe" and "Archives". The main content area is titled "Editorial" and contains an introductory message from the EC's Rare Diseases Task Force, followed by two paragraphs of text discussing the EU's public health objectives and the Commission's support for rare diseases research. The text concludes with the name and title of John F. Ryan, Head of Unit for Health Information at the European Commission.

# Empowerment of patients organisations

- National organisations:  
Feder, Uniamo, Achse, Alliance Maladie Rares, Gard, Vsop....
- Registered with Eurordis
- Diversity of initiatives
  - Political lobbying
  - Help lines / services
  - Fund raising for research
  - Communication



# Research funding: targeted calls

- Belgium
  - New: Foundation Roi Baudoin fund
- France:
  - Basic and clinical research + networks / databases
  - 22 Million Euros for 4 years
- Germany:
  - 10 Networks with 30 Million for 5 years
- Italy
  - Basic and clinical research + with 10 Million for 3 years
  - academic clinical trials with 5 Million per year



calls for proposals for research

# Research funding: targeted calls

- Netherlands
  - Budget of 1.4 Million Euro per year
- Spain
  - 12 Networks with 20 Million Euros over 3 years
  - National network: CIBERER
- Sweden
  - Swedish Research Council: 1.1 Million euros in 2005
- Turkey
  - Budget of 0.5 Million per year
- Transnational funding (E-Rare)
  - France, Germany, Italy, Israel, Spain, Turkey



calls for proposals for research



# Types of Research Projects in the field

Genetic research  
Gene mutation research  
Gene expression  
Genotype/phenotype correlation  
Physiopathology and model *in vitro*  
Physiopathology and model *in vivo*  
Natural history  
Gene therapy  
Cell therapy  
Biomarkers  
Biological collections / resources  
Preclinical development of a new drug  
Development of a new diagnostic instrument

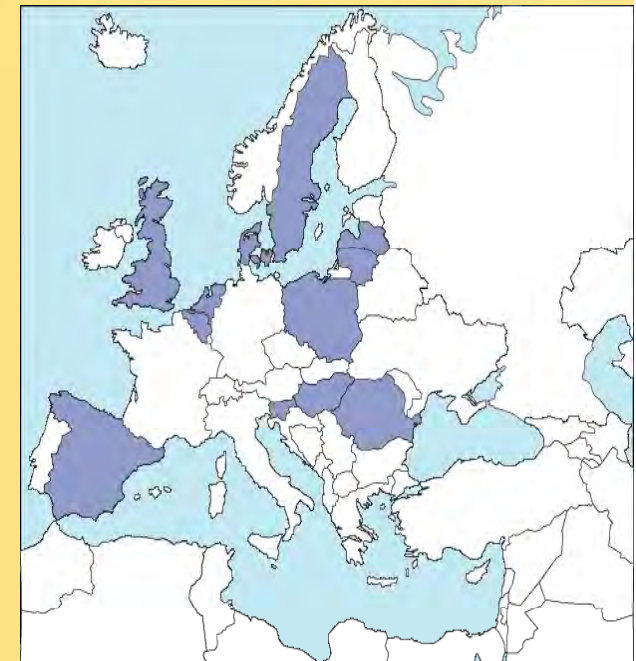
Development of a new diagnostic protocol  
Development of a new medical device  
Development of a new vaccine  
Clinical trial of a new drug  
Clinical trial of a new diagnostic instrument  
Clinical trial of a new protocol  
Clinical trial of a vaccine  
Epidemiological studies  
Registries/ Observatories/ Cohorts  
Epidemiological studies  
Research in health sociology  
Research in health economics  
Public Health

## Key figures

In Europe : **4 625** projects of which **13%** are clinical trials

# Measures for Orphan Drugs

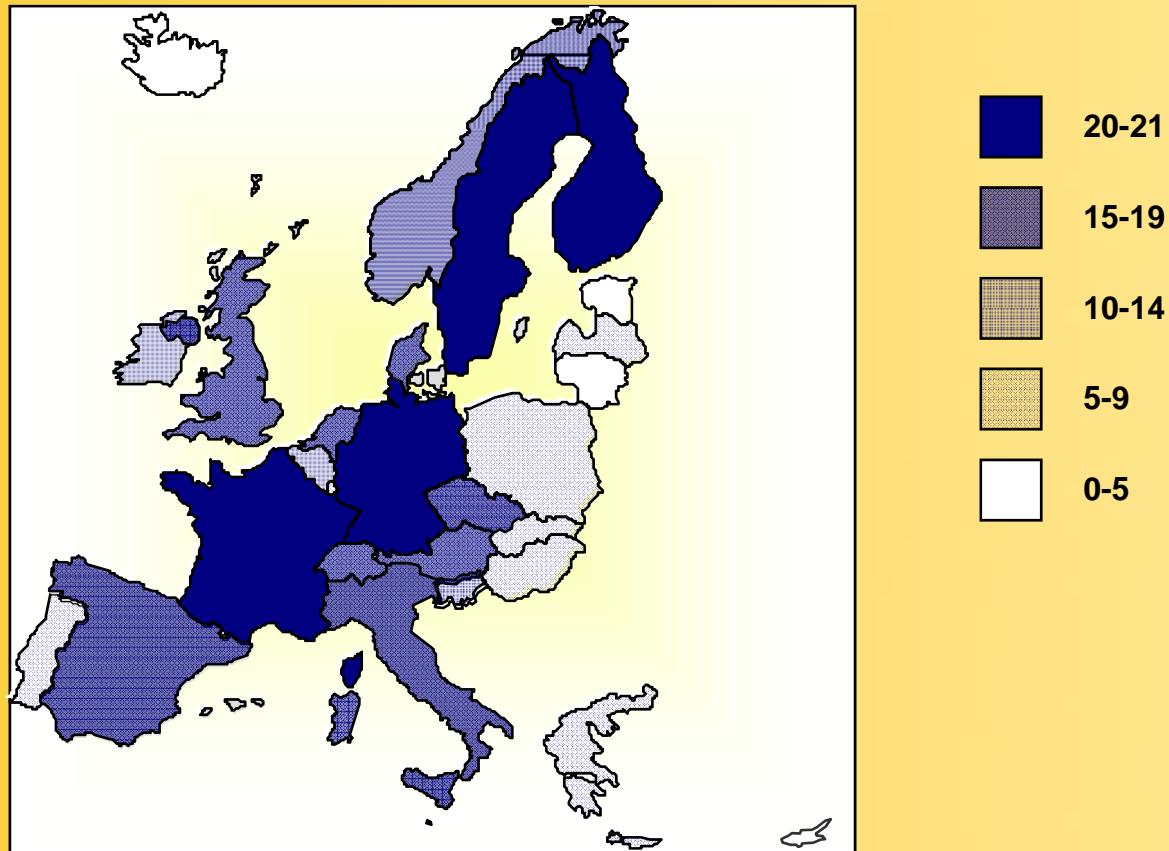
- Belgium:
  - New Committee just established
- France:
  - ATU system (temporary use authorisation + national funding + Committee with stakeholders)
- Hungary:
  - Committee on the treatment of rare conditions
- Ireland
  - Irish Platform of Patients, academics and Industry
- Netherlands:
  - Dutch Steering Committee on Orphan Drugs (2001)
- Poland:
  - National Forum on the treatment of Orphan Diseases



■ orphan drug accessibility measures

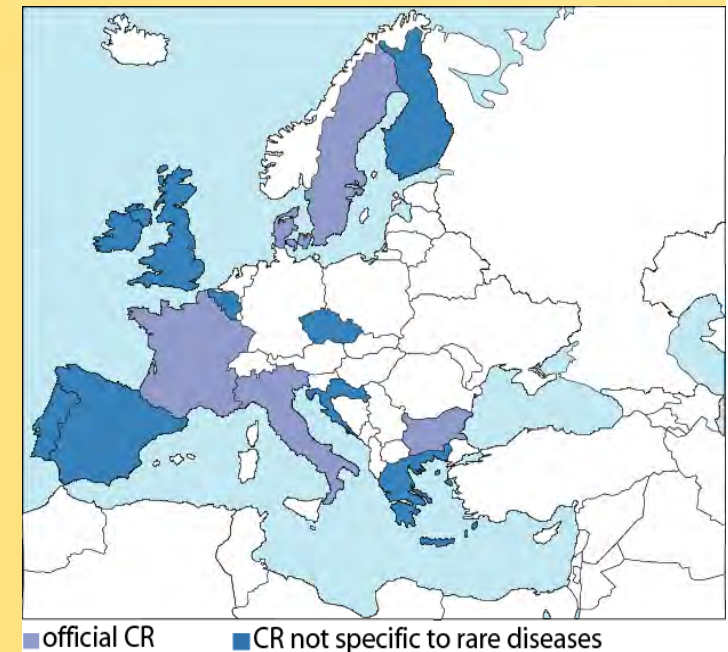


# Availability at national level in 2007 of the 22 first OMPs authorised at EU level before 1 January 2006 (source: Eurordis Survey)



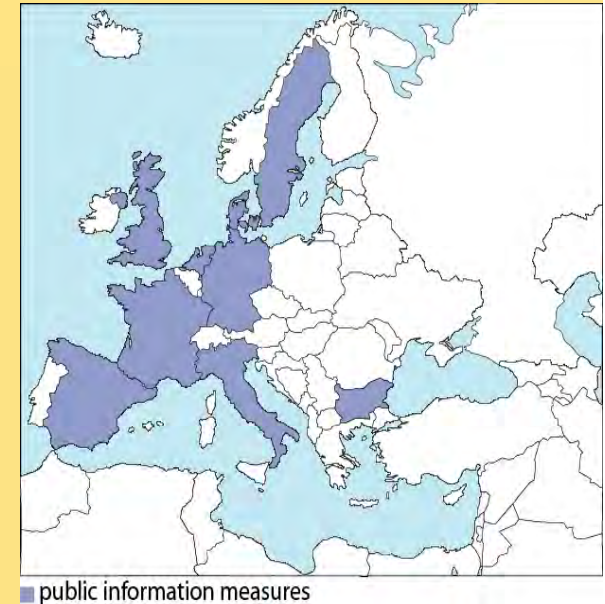
# Centres of Reference

- Official CR for Rare Diseases:
  - Denmark, France, Italy, Sweden
  - In preparation: Spain....
- Official networks of CR:
  - France, Germany, Spain
- Official CR but not for Rare Diseases specifically:
  - Austria, Belgium, Finland, Greece, Ireland, Netherlands, Spain, UK
- Non-official CR / established by reputation: all countries

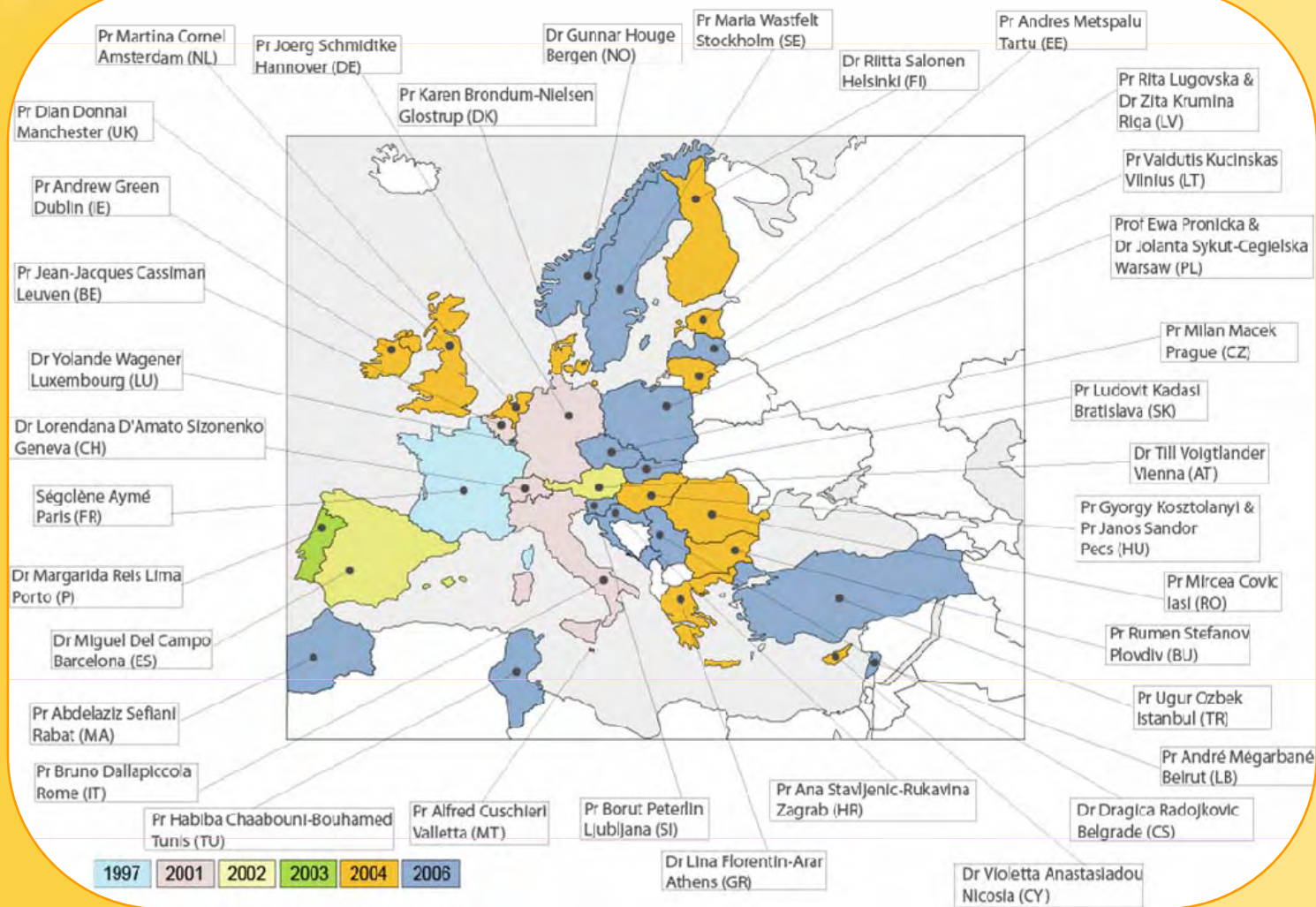


# Public Information Measures

- National Information Centres
- National website/helpline by governmental agencies
  - Belgium, Bulgaria, Denmark, France, Netherlands, Norway, Sweden
  - In preparation: Greece, Hungary, Italy
- National website/helpline by patient/professional organisation
  - Italy, Netherland, Spain, UK



# Orphanet in European countries

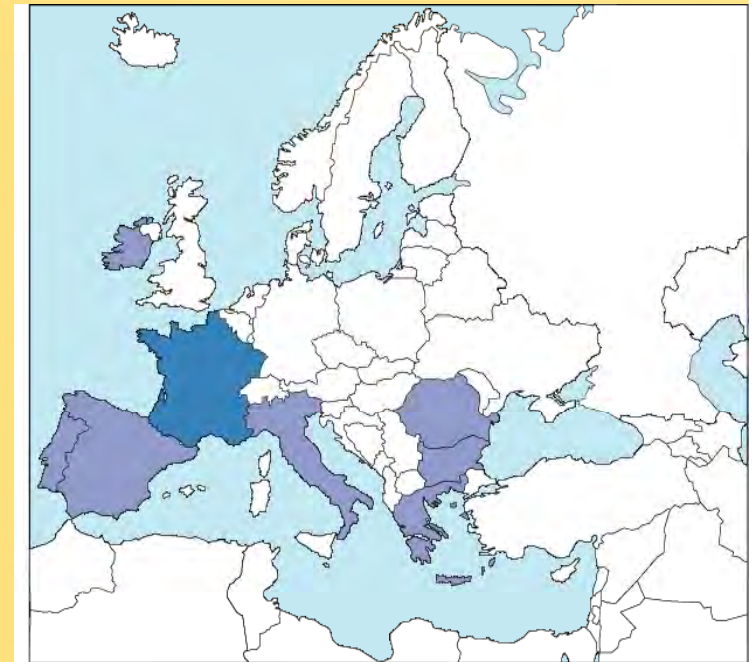


# Orphanet directory of 2,500 rare diseases published at national level



# National plans in preparation

- Effective National Plan
  - France (2005-2008)
- In preparation (first semester 2008):
  - Greece, Italy, Portugal, Romania, Spain
- In discussion:
  - Bulgaria, Ireland, Netherlands, Sweden, Slovenia



■ national plans in preparation ■ national plan for rare diseases

# CONCLUSION

- Many national / regional initiatives and incentives in the last few months
- To be made widely known
- To be monitored to learn about their efficacy and cost/effectiveness
- Indicators to be defined
- Encourage round table of stakeholders
- National plans all over Europe
- Coordination at EU level