

# The Council Recommendation on Rare Diseases: The Five Pillars for National Strategies

Based on a presentation by EURORDIS  
At the European Symposium  
On National Strategies & Plans for Rare Diseases  
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## Introduction

**Building a unique  
EU integrated, comprehensive  
and long term strategy  
to address patients' needs  
everywhere in Europe**

## Research

- Identify ongoing research projects and existing research resources + Identify **needs and priorities** for **basic, clinical, translational and social research**, link with **centres of expertise**
- Create **new additional financial resources** for research
- Targeted at transversal infrastructure across rare diseases and at disease specific projects
- Cover biomedical, **public health and social research**, Foster participation of researchers in EU funded project
- Promote **public-private partnership**
- Appropriate **funding mechanisms for the long term sustainability** of research infrastructures such as biobanks, databases, registries, clinical research infrastructure... because of the nature of rarity

# Centres of Expertise and European Reference Networks for Rare Diseases

- Identify Centres of Expertise at national level
- **Multidisciplinary approach + comprehensive medical & social + coordination hospital & community & home care**
- Integrate Centres of Expertise into European Reference Networks
- Provide **long-term public funding**, for continuity of care and knowledge development
- Support patient mobility for cross-border care
- Promote good practice relationship between patients support groups and centres of expertise: involve patients in the decision making processes, in the establishment and management of centres and patients in the evaluation of centres of expertise

# Information and Patient Services

- Use the same coding and classification approach
- Contribute to the inventory of rare diseases
- Support national and regional specific disease **information networks, registries and databases using common approach across EU**
- Raise public awareness: **Rare Disease Day**
- Support **national rare disease information helplines with a free number** –toward a unique number in EU
- Support web-based information tools such as local **Orphanet** services and patient groups web services
- Develop **respite care services and therapeutic recreative programmes** for patients and families

## Gathering of expert opinions

- EU common protocols and recommendations such as reference opinions on diagnosis, medical care, education and social care
- EU guidelines: population screening and diagnostic tests
- EU Assessment Reports on the Therapeutic Value of Orphan Drugs

= Toward more equal access to high quality care and to social rights

## Empowerment of patient organisations

- Patients and families are not only « end users » of national strategies and action plans = they are carers, health and social actors, managing complex day to day care all along their life span + patient support & advocacy groups are your best allies to take an active role in shaping research, healthcare and social national and regional policies for rare diseases
- Ensure that patients and patients' 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

« κάθε έμροδίο ιά κάλλο » !

« KATHE EMPRODIO YIA KALLO » !

...In every obstacle there is something good....