



**Centres of Expertise for Rare Diseases
and European Reference Networks
State of the art and
recommendations from the RDTF so far**

Rare Disease Task Force
European Commission
DG Public Health



Centres of expertise have always existed in practice...

- Hierarchy of knowledgeable experts
 - Teachers / Authors of articles
 - Leaders of opinion
- Technical platforms
 - Rich institutions with developed services
 - Research institution with innovative services

BUT

The information was restricted to a few professionals

No objective criteria



Reasons to move to a more formal approach.....

- **Budget constraint in teaching hospitals**
 - Annual budget to serve the area population
 - Strict budget for research activities
 - Analytical budget
- **Regulation of clinical research**
 - More administrative constraints
 - Higher costs
- **Accessibility of the information through Internet**
 - Second opinion requests by phone, fax, e-mail
 - Mobility of patients / free choice



Centres of expertise for RD are needed....

- Patients are rare but experts are rare too....
 - Need to identify them
- Expertise may be found only at International level
 - Impossible for most countries to offer appropriate services to all patients
- Clinical research is badly needed
 - Requires cohorts large enough
 - Systematic collection of data
 - Production of clinical recommendations

But patients should not have to travel....



- Financial constraints
 - Cost of travel + accommodation
- Communication constraints
 - 20 languages in Europe + cultural differences
- Logistical constraints
 - Expert departments have a limited number of beds and of staff



The Rare Diseases Task Force (RDTF)
was requested to provide an overview

Report to the High Level Group for
Health Services and Medical Care

Rare Diseases Task Force activities

Sept 2005: Issue of a first report on CR in Europe

- ▶ Ad hoc expert group
- ▶ 2 workshops (June and September 05)

Dec 2005: RDTF meeting

- ▶ Decision to update the 2005 report

March 2006: report to HLG on plans

Sept 2006: Workshop of the expert group

Dec 2006: Publication of the second report

Statements consensus reached



- European added-value of CE
- Need for disseminating the information on CE
- Definition of what is a CE
 - High level of expertise / high volume of activity / capacity to provide expert advice / multidisciplinary approach / links with other centres and support groups
- Priority for networks of centres
- Rejection of hierarchy between European centres and national/regional centres

Topics of disagreement (1)



- **Balance benefice / malefice**

- **Positive**

- Improving access for EU citizens
 - Maximising cost /effectiveness
 - Helping to share knowledge / training

- **Negative**

- Distant referral / long waiting list
 - Psychological burden: language + isolation
 - Loss of trust in local services
 - Loss of interest by professionals outside the CE

Topics of disagreement (2)



- **Scope of activity**
 - Disease centred
 - By disease
 - By medical specialty
 - Technology centred
 - Surgery / investigation / highly specialised treatment
 - Social support centred
 - Polyhandicap / familial dimension /

Topics of disagreement (3)



- Geographical reach of centres
 - European centres
 - National centres
 - Regional centres

VS

- Level of designation / funding
 - EU
 - Member states
 - Regions

Topics of disagreement (4)



- Centres of expertise
 - National centres
 - Regional centres
 - where patients may be referred

VS

- European networks of centres
 - Characterised by communication
 - Production of guidelines
 - Shared expertise for patients

Topics of disagreement (5)



- From the concepts to the reality
 - Diffusion of the concepts
 - Publication of experiences
 - Follow-up of national initiatives
 - Definition of responsibilities
 - European networks:
 - European Commission
 - bilateral contracts between countries
 - CR: member states or regions



**Main conclusions
of the RDTF expert group report**

The definition of what is a CE differs from one country to another...



- ▶ Definition of rare disease differs
- ▶ Size of the population to serve differs
- ▶ Definition of the task differs
- ▶ Number of diseases to be covered differs
- ▶ Process to identify CE differs
- ▶ Financial support differs
- ▶ Purpose for establishing CE differs

Recommendations to Member States

To explore all possible forms of cooperation between MS in the field of health services and medical care for patients with RD

For MS having official CE:

- ▶ To agree on the definition / mission / evaluation (done by HLG)
- ▶ To share experience and outcome measures (to be done)

For MS not having official CE:

- ▶ Establish CE or find other appropriate way to meet the needs of patients (by contracting with CE)
- ▶ Develop electronic communication with “CE”

Recommendations to Member States (2)

Contribute to the identification of their expert centres and support them as much as possible

Organise health care pathways for their patients

- ▶ through the establishment of cooperation with all necessary expert centres from the country
- ▶ and from abroad when necessary

To recognise and fund the activity of expert opinion

Recommendations to European Commission

- ◆ To avoid using the wording CR when referring to expert centres collaborating within a network - prefer “centre of expertise” OK
- ◆ To play an important role in supporting the identification of centres of expertise and in the diffusion of information about them (OK info)
- ◆ To identify and fund the collaboration between centres of expertise through “European networks of centres of expertise” (pilot projects)

Recommendations to European Commission

- ◆ To continue its financial support to networks of centres of expertise in the field of RD until an evaluation of the output of the networking process
- ◆ To open its call for proposals to the definition of a methodology to assess the benefit from such networks from the perspective of the different stakeholders
- ◆ To encourage the development of electronic tools necessary for the development of telemedicine in the field of RD



Plans



- Issue of a new report in Fall 08
- Workshop to be held
- Topic: Methods to assess the added-value of
 - Centres of expertise
 - Reference networks of centres of expertise