EUCERD Recommendations
Quality Criteria for Centres of Expertise for Rare Diseases in Member States

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Version: November 2012
CENTRES OF EXPERTISE FOR RARE DISEASES IN EUROPE: THE CONTEXT
The need for centres of expertise (CEs) for rare diseases

- There are over 6’000 rare diseases
- The challenge of rarity:
  - Patients are rare
  - Experts are rare
- Centres of expertise area means of:
  - Revealing where expertise lies
  - Gathering experience to improve knowledge and care
Specificities of CEs

- To provide multi-disciplinary healthcare services (high quality and cost-effective care) to patients with conditions requiring a particular concentration of resources or expertise.

- To act as focal points for medical training, research and information dissemination.
European context

- Development of CE in the field of rare RD encouraged explicitly in:
  - Council Recommendation on an Action in the Field of RD (2009/C 151/02) (8 June 2009)
  - Directive on the application of patients’ rights in cross-border healthcare (2011/24/EU) (9 March 2011)
Centres of expertise in the Council Recommendation

Council Recommendation on an Action in the Field of RD (2009/C 151/02) (8 June 2009) encourages MS to:

• Identify CE throughout their national territory by the end of 2013, and consider supporting their creation
• Foster participation of CE in European reference networks
• Organise healthcare pathways for patients suffering from rare diseases
• Support the use of tele-medicine to ensure distant access to healthcare
• Include, in their plans or strategies, the necessary conditions for the diffusion and mobility of expertise and knowledge in order to facilitate the treatment of patients in their proximity.
• Encourage CEs to be based on a multidisciplinary approach
Directive on the application of patients’ rights in cross-border healthcare (2011/24/EU) (9 March 2011)

• The Commission shall support Member States in the development of European reference networks between healthcare providers and centres of expertise in the Member States, in particular in the area of rare diseases

• Member States are encouraged to facilitate the development of the European reference networks:
  
  (a) by connecting appropriate healthcare providers and centres of expertise throughout their national territory and ensuring the dissemination of information towards appropriate healthcare providers and centres of expertise throughout their national territory;

  (b) by fostering the participation of healthcare providers and centres of expertise in the European reference networks.
CEs in national plans/strategies for RD

- EU Member States (MS) are currently elaborating national plans/strategies for rare diseases in line with the Council Recommendation by 2013
- CEs are key components of these future RD plans/strategies
- CE will be the building blocks of future European Reference Networks foreseen in the Cross-Border Healthcare Directive
CEs in national plans/strategies for RD

- MS are looking for guidance, in particular concerning how best to structure care for RDs, including the designation of CEs.

- Indeed, very few countries in Europe have experience of designating CEs for RDs.

- This experience should be shared and lessons learned.
Countries with designated CE

A few countries have designated centres either at national or regional level:

- 1990 Norway (16 centres)
- 1990 UK (50-60 centres)
- 2001 Denmark (2 centres)
- 2001 Italy (215 regional centres)
- 2006 France (131 centres)
- 2006 Spain (62 centres)
Heterogenous designation processes

- Processes for designation in these countries differ greatly from one country to another:
  - **In form**
    - Reflecting heterogeneity of national health care systems
    - Depending on budget allocation
  - **In focus**
    - Specialised centres vs general centres
    - Clinical only vs clinical research
    - Focus on technology / expert intervention
  - **In designation process**
    - Specific policy regarding RD or not
    - National vs regional approach
Designation of CEs: questions faced by Member States

• **How to organise** the designation process:
  – Model 1: call for proposals (bottom/up)
  – Model 2: public health plan (top/down)

• **How to define the scope** to ensure all needs are met
  - General centres for rare diseases (perhaps appropriate for small countries)
  - Possible groupings of diseases
Designation of centres of expertise: The dynamic in European countries

- **Officially designated in scope of national RD plan**
  - France
- **Officially designated outside of scope of RD plan**
  - Denmark
  - France
  - Norway
  - Spain
  - Italy (regional)
  - United Kingdom
- **Officially recognised to varying degrees/non-designated**
  - Austria, Belgium, Croatia, Czech Republic, Cyprus, Germany, Greece, Hungary, Ireland, Israel, the Netherlands, Portugal, Romania, Sweden, Switzerland
- **By reputation only/self-declared**
  - Bulgaria, Estonia, Finland, Latvia, Lithuania, Poland, Slovak Republic, Slovenia
- **Plans to designate centres in future/current RD plan**
  - Austria, Belgium, Bulgaria, Czech Republic, Cyprus, Finland, Germany, Hungary, the Netherlands, Portugal, Romania, Slovak Republic, Slovenia, Turkey.
How best to provide guidance based on experience

• MS are planning to establish CE in their plan
• MS are looking for guidance concerning:
  • The mission and scope of CE
  • How to organise the designation process
  • The European dimension of CE in view of the future ERNs

➢ The experience of the EUCERD members makes it an ideal forum to reflect on these issues and make recommendations to guide MS
Elaboration of the Recommendations

- Concepts defined by the High Level Group on Health Care & Medical Services (HLG) and by the Rare Disease Task Force (RDTF)
- 1st draft presented at EUCERD workshop on National Centres of Expertise (CE) and European Reference Networks (ERN) for rare diseases (RD) on 21-22 March 2011
- Further elaborated by the EUCERD Scientific Secretariat & all EUCERD members for comments
- Workshop on 8 September 2011 with members of the EUCERD & MS representatives
- Revised draft submitted to EUCERD membership ahead of the 3rd EUCERD meeting on 24-25 October 2011.
- Recommendations were unanimously adopted by the Committee in October 2011.
Aim of EUCERD recommendations:

- To help MS in their reflections/policy developments concerning national plans and strategies for RD
  - Specifically in addressing the organisation of healthcare pathways at national and European level
  - Potentially helpful to the Cross Border Health Care Directive Committee in the context of the development of ERNs
Scope of the Recommendations

45 recommendations
4 main areas:

- Mission and scope of CEs
- Criteria for designation
- Process for designation
- European dimension
Recommendations: Mission and scope

• Definition
• Coverage
• Patient focus
• Core competencies
• Role in spreading information and education
• Role in research
Recommendations: Definition of what is a CE

• CEs tackle diseases or conditions requiring specific care due to difficulty in establishing diagnosis, to prevent complications and/ or to set up treatments

• CEs are expert structures for the management and care of RD patients in a defined catchment area, preferably national and at international level if necessary
Recommendations: Coverage of CEs

• The combined scope of all CEs within a MS covers all RD patients’ needs even if they cannot provide a full range of services with the same level of expertise for each RD

• The scope of diseases covered by each CE, or by a CE at a national level, will vary depending on the size of the country and the structure of the health care system

• CEs liaise with other CEs at National or European level as appropriate

• A national directory of formally designated CEs is compiled and made publically available, including on Orphanet
Recommendations: Patient focus of CEs

- CEs collaborate with patient organisations to bring in the patients’ perspective
- CEs respond to the needs of patients from different cultures and ethnic groups (ie have cultural sensitivity)
- According to national/ international ethical and legal frameworks, centres of expertise should ensure respect of non-discrimination and non-stigmatisation of RD patients across Europe, within their sphere of competencies
Recommendations: Competencies of CEs

- CEs bring together or co-ordinate within the specialised healthcare sector multidisciplinary competencies/ skills including paramedical skills and social services in order to serve the specific medical, rehabilitation and palliative needs of RD patients.
- CEs contribute to building healthcare pathways from primary care.
- CEs have links with specialised laboratories and other facilities.
Recommendations: CEs’ role in spreading information

- CEs contribute to the elaboration of good practice guidelines and to their dissemination
- CEs provide education and training to healthcare professionals from all disciplines, including paramedical specialists and of non-healthcare professionals (such as school teachers, personal/homecare facilitators) whenever possible
- CEs contribute to and provide accessible information adapted to the specific needs of the patients and their families in collaboration with the patient organisations and with Orphanet
Recommendations: CEs role in research

• CEs contribute to research, to improve the understanding of disease and to optimise diagnosis, care and treatment, including the clinical evaluation of long term effects of new treatments
Recommendations:
Criteria for designation of CEs

• Leadership and credibility
• Multidisciplinarity and inclusiveness
• Capacity
• Links and collaborations
• Mechanisms for measuring performance/evaluation
Recommendations: Leadership and credibility

• High level of expertise and experience documented for example by the annual volume of referrals and second opinions, and through peer reviewed publications, grants, positions, teaching and training activities.

• Contribution to state of the art research.
Recommendations: Multidisciplinarity and inclusiveness

• Demonstration of a multidisciplinary approach when appropriate, integrating medical, paramedical, psychological and social needs (e.g. RD board)

• Appropriate capacity to manage RD patients and provide appropriate advice

• Organisation of collaborations to ensure continuity of care
  • Between childhood, adolescence and adulthood
  • Between all stages of the disease
Recommendations: Capacity of CEs

• Appropriate arrangements to improve the delivery of care and especially to shorten the time taken to reach a diagnosis

• Capacity to produce and adhere to good practice guidelines for diagnosis and care

• Capacity to propose quality of care indicators in their area and implement outcome measures including patient satisfaction
Recommendations: Capacity of CEs

• Capacity to participate in data collection for clinical research and public health purposes
• Capacity to participate in clinical trials, if applicable
• Quality management in place to assure quality of care, including national and European legal provisions, and participation in internal and external quality schemes when applicable
• Consideration of E-Health solutions
Recommendations: Links and collaborations

• Links and collaborations with patient organisations where they exist
• Links and collaborations with other CE at national, European and international level
• Appropriate arrangements for referrals within individual MS from/to other EU countries where applicable
Recommendations

• Mechanisms will need to be in place to capture measures of:
  – Leadership and credibility
  – Capacity and quality assurance
  – Appropriate links and collaborations
Recommendations: Process for designation of CEs covers

- Core principles of designation
- Designation criteria
- Duration of designation
Recommendations: Core principles

• MS take action concerning the establishment and designation and evaluation of CEs and facilitate access to these centres
• MS establish a procedure to define and approve designation criteria and a transparent designation and evaluation process
• The designation process at MS level ensures that the designated CEs have the capacity and the resources to fulfill the obligations of designation
Recommendations: Designation criteria

• The designation criteria defined by MS are adapted to the characteristics of the diseases or groups of diseases covered by the CE

• CEs may not fulfill some of the designation criteria defined by the MS so long as the absence of fulfillment of these criteria does not impact on the quality of care and as long as CEs have a strategy in place to attain designation criteria in a defined time period
Recommendations: Duration of designation

• The designation of a CE is valid for a defined period of time

• CE are re-evaluated on a regular basis through a process incorporated into the designation process at MS level

• The designating authority at MS level may decide to withdraw the designation of a CE if one or more of the conditions that formed the basis for designation is no longer satisfied or if there is no longer a need to maintain the national service
Process for Designation of CEs

• MS responsible for designation process based on recommended criteria but adapted according to the MS
• Designation is for a defined duration and subject to quality-based review
The European dimension of CEs
Recommendations:
Sharing experience and indicators

• MS with established CEs share their experience and quality indicators with other MS and co-ordinate their efforts to identify CEs for all RD patients at EU level

• This is an important principle but requires the co-operation of national accrediting and quality assurance bodies
Recommendations: Cross-border considerations

• MS should provide adequate information to professionals, citizens and POs concerning the possibilities and conditions of access to healthcare at national and international level in the field of RD

• Cross-border healthcare is organised where appropriate with designated CEs in neighbouring or other countries where patients or biological samples can be referred
Recommendations: Networking

• Networking of CEs is a key element of their contribution to patient diagnosis and care, to ensure that expertise travels rather than patients where appropriate; exchange of data, biological samples, radiological images, other diagnostic materials and e-tools for tele expertise are promoted

• Designated CEs at MS level are the key elements of the future ERNs
Thank you for your attention!

The text of the recommendation can be found on the EUCERD’s website

www.eucerd.eu