Cross-border cooperation in hospital care:

Experts centers on rare diseases: European reference networks

Directive 2011/24/EU on the application of patients' rights in cross-border healthcare.

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European Commission

Health and Consumers

Aim & content

European Commission

Legal basis for the developments of the EU Public Health Policy

Based on new Article 168 (former 152) of the EU Treaty

- **A Community action programme on Rare Diseases, including genetic diseases, was adopted for the period of 1 January 1999 to 31 December 2003 with the aim of ensuring a high level of health protection in relation to RD. As the first EU effort in this area, specific attention was given to improving knowledge and facilitating access to information about these diseases.**

- **Orphan Medicinal Product Regulation (Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products, was proposed to set up the criteria for orphan designation in the EU and describes the incentives (e.g. 10-year market exclusivity, protocol assistance, access to the Centralised Procedure for Marketing Authorisation) to encourage the research, development and marketing of medicines to treat, prevent or diagnose rare diseases.**

- **As a consequence Rare diseases are now one of the priorities in the Second EU Health Programme 2008-2013. According to the DG SANCO Work Plans for the implementation of the Public Health Programme, main lines of action and priorities are choosed very year. Work Plan for 2013 adopted.**

Aim & content

European Commission

Legal basis for the developments of the EU Public Health Policy

- **In the current Framework Programme, the FP7, the Health Theme of the "Cooperation" Specific Programme, is designed to support multinational collaborative research in different forms. The main focus of the Health theme in the rare diseases area are Europe-wide studies of natural history, pathophysiology, and the development of preventive, diagnostic and therapeutic interventions.**
- **Commission Communication COM (2008) 679/2 to the European Parliament, the Council, the Economic and Social Committee and the Committee of the Regions on Rare diseases: Europe's challenges creating an integrated approach for the EU action in the field of rare diseases. Adopted 11th November 2008.**
- **Council Recommendation on a European action in the field of rare diseases recommending actions at national level to implement the EU action (e.g. National Plans for Rare Diseases). Adopted 8th June 2009.**
- **Decision of the Commission creating a European Union Committee of Experts on Rare Diseases during 2009. To be composed by 51 members representing Member States, patient's organisations, industry, FP Projects, Health Programme projects, etc. Adopted 30th November 2009.**
- **Directive of the European Parliament and of the Council on the application of patients' rights in cross-border healthcare (COM(2008)414) provides for the development of European reference networks (ERNs) to be facilitated by the Member States. The ERN for Rare Diseases will have a strategic role in the improvement of quality treatment for all patients throughout the European Union as called by the patients' organisations. Adopted 9th March 2011.**

Aim & content

European Commission

The Commission Communication and the Council Recommendation on rare diseases

The European Union approach

There is probably no other area in public health in which 27 national approaches could be considered to be so inefficient and ineffective as with rare diseases. The reduced number of patients for these diseases and the need to mobilise resources could be only efficient if done in a coordinated European way.

Aim & content

The Commission Communication and the Council Recommendation on rare diseases

The European Union approach

1. **Plans and strategies in the field of rare diseases**
2. **Adequate definition, codification and inventorying of rare diseases**
3. **Research on rare diseases**
4. **Centres of expertise and European reference networks for rare diseases**
5. **Gathering the expertise on rare diseases at European level**
6. **Empowerment of patient organisations**
7. **Sustainability**

International Rare Diseases Research Consortium

IRDiRC
INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

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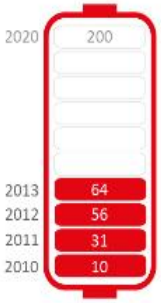
MORE ARTICLES

IRDiRC is now a part of Global Alliance

The cost of genome sequencing has fallen a million fold, and it is becoming more and more affordable and accessible for the average person. Even though the advances in genome sequencing have provided a wealth of knowledge, the data collected and studied are mostly disorganized

NEW THERAPIES



Objective 2020: 200 new therapies

Disclaimer: the numbers do not reflect IRDiRC initiatives only

SPOTLIGHT

Updates on IRDiRC policy and on the first IRDiRC conference

IRDiRC policies and guidelines refer to the principles that the IRDiRC members agree to follow as well as the recommendations from the Scientific Committees.

Accreditation

European Commission

Accreditation in the EU

Regulation 765/2008, which sets out requirements for accreditation and market surveillance relating to the marketing of products, establishes the legal framework for accreditation in Europe.

The Regulation promotes a uniformly rigorous approach to accreditation across Member States – so that ultimately one accreditation certificate will be enough to demonstrate the technical capacity of a conformity assessment body.

The main principles of accreditation in the Regulation (which complement the relevant international standard for accreditation bodies) are:

- **One accreditation body per Member State (but it is possible to have recourse to another Member State's national accreditation body, should a Member State decide not to set up its own).**
- **Accreditation is a public sector activity.**
- **There is no competition between national accreditation bodies.**
- **Accreditation is a not-for-profit activity.**
- **Stakeholder representation is ensured.**
- **Accreditation is the preferred means of demonstrating technical capacity in the regulated area - in the appointment of notified bodies.**
- **The European accreditation infrastructure**

Furthermore, Regulation 765/2008 recognises a body known as the European co-operation for accreditation, the EA, of which national accreditation bodies are members and which cooperates with the European Commission.

It is EA's task to set up and manage a sound peer evaluation

Certification

European Commission

European standards (certification)

Standardisation is the voluntary process of developing technical specifications based on consensus among all interested parties (industry including Small and Medium-sized Enterprises (SMEs), consumers, trade unions, environmental Non Governmental Organisations (NGO), public authorities, etc). It is carried out by independent standards bodies, acting at national, European and international level.

While the use of standards remains voluntary, the European Union has, since the mid-1980s, made an increasing use of standards in support of its policies and legislation.

More Standardisation has contributed significantly to the completion of the Internal Market in the context of ' New Approach' legislation, which refers to European standards developed by the European standards organisations.

Furthermore, European standardisation supports European policies in the areas of competitiveness, Information and Communication Technologies (ICT), innovation, interoperability, environment, transport, energy, consumer protection, etc.

Standardisation is an excellent tool to facilitate international trade, competition and the acceptance of innovations by markets. A key challenge for European standardisation is to strengthen its contribution to the competitiveness of Small and Medium-sized Enterprises (SMEs).

Designation

European Commission

EUCERD Recommended criteria for designation of Centres of Expertise at national level

- Capacity to produce and adhere to good practice guidelines for diagnosis and care.
- 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.
- Capacity to propose quality of care indicators in their area and implement outcome measures including patient satisfaction.
- High level of expertise and experience documented, for instance, by the annual volume of referrals and second opinions, and through peer-reviewed publications, grants, positions, teaching and training activities.
- Appropriate capacity to manage RD patients and provide expert advice.
- Contribution to state-of-the-art research.
- Capacity to participate in data collection for clinical research and public health purposes.
- Capacity to participate in clinical trials, if applicable.
- Demonstration of a multi-disciplinary approach, when appropriate, integrating medical, paramedical, psychological and social needs (e.g. RD board).
- Organisation of collaborations to assure the continuity of care between childhood, adolescence and adulthood, if relevant.
- Organisation of collaborations to assure the continuity of care between all stages of the disease.
- Links and collaboration with other CE at national, European and international level.
- Links and collaboration with patient organisations where they exist.
- Appropriate arrangements for referrals within individual Member States and from/to other EU countries if applicable.
- Appropriate arrangements to improve the delivery of care and especially to shorten the time taken to reach a diagnosis.
- Consideration of E-Health solutions (e.g. shared case management systems, expert systems for tele-expertise and shared repository of cases).

Designation

European Commission

EUCERD Recommended criteria for designation of Centres of Expertise at national level: Process for designating and evaluating CEs for RD in MS

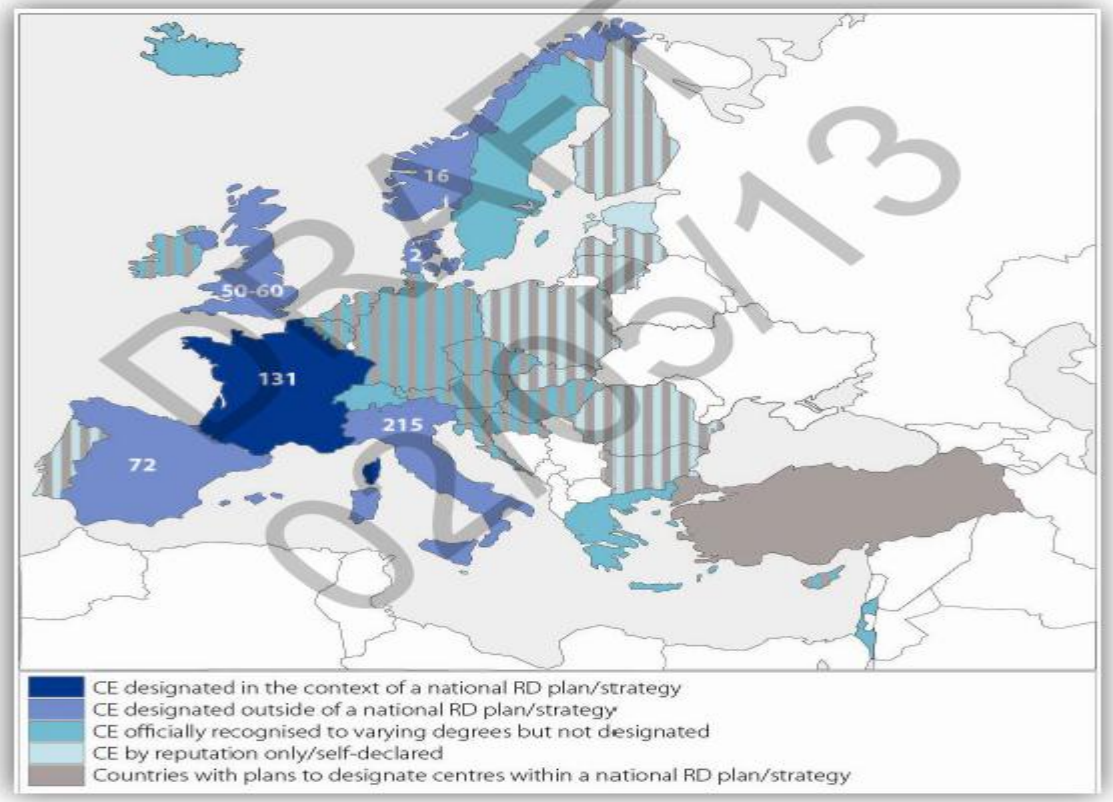
- MS take action concerning the establishment and designation and evaluation of CEs and facilitate access to these centres.
- MS establish a procedure to define and approve designation criteria and a transparent designation and evaluation process.
- The designation criteria defined by MS are adapted to the characteristics of the disease or group of diseases covered by the CE.
- CEs may not fulfill some of the designation criteria defined by the MS as long as the absence of fulfillment of those 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.
- The designation process at MS level ensures that the designated CEs have the capacity, and the resources to fulfill the obligations 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 centre of expertise 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.

Designation

European Commission

New priorities after Commission Communication and Council Recommendation

IV. Designated Centres of expertise and European reference networks for rare diseases



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Challenges

- Providing clear rules and reliable information to patients regarding access and reimbursement for healthcare received in another EU country. The new "national contact points" will do this.
- Meeting patients' expectations of the highest quality healthcare, which are even higher when they seek treatment away from home. The information given by national contact points on healthcare quality and patient safety will help them make informed choices before going abroad for healthcare.
- Ensuring EU countries work closer together in the interest of patients.
- Clearing up years of legal uncertainty. The new rules also strike the right balance between maintaining the sustainability of health systems while protecting patients' right to seek treatment outside their home country.

EU legislation

Directive 2011/24/EU on patients' rights in cross-border healthcare clarifies the rules on access to healthcare in another EU country, including reimbursement.

*EU countries have until **25 October 2013** to pass their own laws implementing the Directive.*

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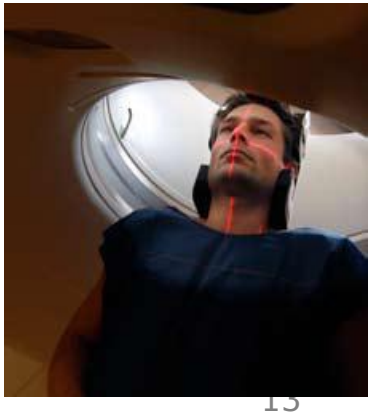
The 3 Aims of this Directive

1. Help patients to exercise **their rights to reimbursement** for healthcare received in another EU country

2. *Provide assurance about **quality and safety** of cross-border healthcare*

3. ***Establish formal cooperation between health systems***



Context & main Goal

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Art. 12: ***enhanced cooperation of Member States*** *in the area of European reference networks (ERN).*

Main goal: ***facilitate improvements in the diagnosis and treatment*** *across the EU:*

- **By delivery of high-quality, accessible and cost-effective healthcare**
- **For Medical conditions requiring:**
  - **Concentration** of expertise or resources**,**
  - In medical domains **where expertise is rare.**

Health and Consumers

European Commission

Model of ERN should guarantee:

- **Appropriated scope: relevant conditions and diseases**
- **Prerequisites and criteria for Healthcare providers acting as centres of expertise**
- **That EU co-operation is cost-effective**
- **The development/disemination of best practices to the advantages of Member States.**

Goals & benefits

Improvement diagnosis & delivery of high-quality, Accessible and cost-effective healthcare

cost-effective use
& concentration of resources

help Member States:
insufficient patients
& lacking technology or expertise

mobility of expertise, virtually or physically
spread information ,knowledge and best practice

research, training,
epidemiological surveillance

quality and safety benchmarks,

knowledge sickness prevention

exploiting innovations
medical science & health technologies

Tasks and responsibilities

European Commission

- ***Commission***

  - ✓ **Support MS in the development of ERN**
  - ✓ **Adopt Delegated & Implementing Acts**

- ***Member States***

  - ✓ **Connecting providers & Centres of Expertise at national level**
  - ✓ **Fostering participation in the ERN.**

- ***Voluntary*** *participation of providers**:***

  - ✓ **Fulfilling all required conditions and criteria**

Milestones and timeline for the implementation (ERN)

European Commission

2011 - 2015

Delegated Acts
(Art. 17)

Adoption of a list of criteria and conditions for the CR & ERN to fulfil
Art. 12.5

Implementing acts
(Art. 16)
Committee

Exchange of information and expertise for ERN
Art. 12.4(c)

criteria for establishing and evaluating ERN
Art. 12.4(b)

legislative process

Deployment Process Establishment of ERNs

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Work on progress

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Looking at best practices: MS and Centres visits

Public Consultation

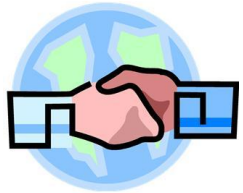
Technical Brainstorming & workshops

Meeting with Stakeholders

Cross-border Healthcare Expert Group

Reports and technical papers

Advise



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Vote of Committee

Adoption of Implementing Act

Adoption of Delegated Act

Step-by-step approach (rigorous and best practice-based)

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Delegated Act

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Criteria for ERNs and Centres:

ERNs' Criteria

- ✓ **Scope**
- ✓ **Functions**
- ✓ **Structure and elements (model)**
- ✓ **Governance and coordination**

Centres' Criteria

- ✓ **General**
- ✓ **Specific**

European Commission

3 Sets of Criteria

1. *Scope of ERN (selection of conditions – diseases)*

2. *General criteria centres in ERN*

3. *Disease(s)/condition(s) specific criteria*

1.- Scope of the ERNs (targeted conditions – diseases)

Highly specialised healthcare

- **Complexity of the diagnosis and treatment**
- **High cost of treatment and resources**
- **Need of advanced/highly specialised medical equipment or infrastructures**

Need of a particular concentration of expertise and resources

- **Rare Expertise/need of concentration of cases**
- **Low prevalence/incidence/number of cases**
- **Lesson learned and experiences of Member States**

Based on high-quality, accessible and cost-effective healthcare

- **Evidence of the safety and potential positive outcome (clinical)**
- **Feasibility and evidence of value of the treatment**
- **Cost-effectiveness**

Diseases or domains not under the scope

Chronic and common diseases *(Alzheimer, diabetes, COPD, asthma, maternal health…) Only if at some stage a specific and high complex intervention is needed-*

High/Medium prevalence/incidence/number of cases *or procedures per year (should be dealt at country level)*

Networks focusing mainly on research, training or guidelines production *(should be prerequisites, not the main goal)*

No strong evidence on effectiveness *and appropriateness of the treatment or diagnostic technique (HTA, recognized guidelines etc..)*

Diseases or techniques ***not prioritised*** *by MS*

Examples

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Pilot networks of cooperation under Directive 2011/24/EU (Public Health WP 2013)

- ✓ ***Network of Paediatric Oncology Centres dealing with low prevalence and rare solid tumours:***
  - **Retinoblastoma (04/100.000), Wilms tumour (0,8/100.000), soft tissues (1/100.000) , bone sarcoma (06/100.000) and other rare cancers**
- ✓ ***Highly specialised neurological diseases and procedures:***
  - **refractory epilepsy (6/100.000) ,brachialis plexus injuries (0,6/100.000), hereditary ataxia (3/100.000) refractory neuropathic pain, severe craniofacial conditions , and other rare conditions.**
  - **complex neurosurgery, movement disorders surgery, and brain neuro-modulation Examples**

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2.- General criteria of the Centres of Expertise

1. ***Patient*** *empowerment and centred care*
2. *Overall* ***organisation and management*** *of the Centre*
3. *Framework for* ***research and training*** *capacity*
4. ***Business continuity****, contingency planning and response capacity*
5. ***Information*** *system, technologies and* ***e-health tools*** *and applications*
6. ***Quality, patient safety*** *and* ***evaluation*** *framework and policies*
7. *Specific* ***commitment of the management/direction*** *of the centre/hospital to ensure a* ***full and active participation*** *in the ERN*

European Commission

3.- Specific criteria of the Centres of Expertise (Disease (s) specific domains)

- *Competence, experience and good outcomes & care*
- *Specific **human resources** and organisation*
- *Specific **structural** and **equipment** resources*
- ***Research, training**, HTA and clinical tools development*
- ***Information system** and surveillance*
- *External coordination, **care management and follow-up of patients***

Structure of the ERNs

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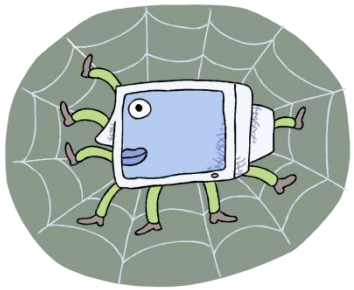
Main elements



- ✓ *Thematic ERNs*
- ✓ *Centres of Expertise (EU designation)*
- ✓ *National Reference/excellence Centers (MS designation)*
  - *Associated national Reference Centers and national networks*
  - *National colaborative/competence centers*

Typology of Centres

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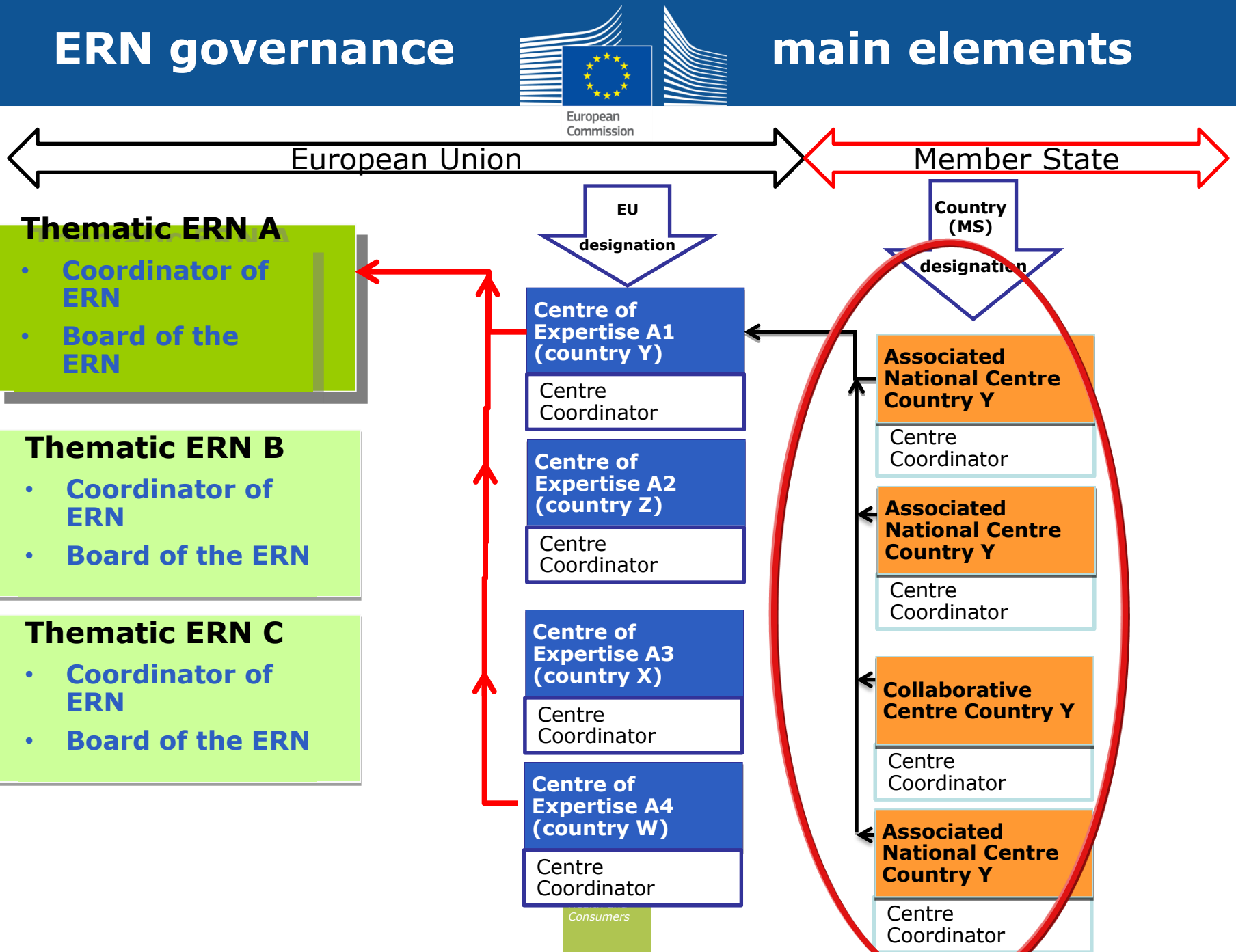
EU Centres of Expertise

- ✓ *Principal constituents of the Thematic networks*
- ✓ *Fulfilment of the criteria provided in the delegated act.*
- ✓ *Designated at EU level*
- ✓ *Full responsibilities, tasks and participation in all the technical and governance bodies of the ERN*
- ✓ *Acting as a hub (interconnector) between the national healthcare providers and the ERN*

ERN governance main elements



Challenges & oportunities (1)

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- ✓ ***Identifying the right targets***
  - *Based in **needs, priorities, economy of scale and added value** (current experience on referral and reception of patients) at regional level and at EU level (figures, type of diseases/procedure/ system of referral-reception)*
  - Applying clear and **robust criteria** to select the diseases
- ✓ ***Moving knowledge and expertise rather than patients***
  - ✓ *By increasing **capacity and expertise at national/regional level** (not losing patients nor expertise)*
  - ✓ *By **new models and partnerships of networks** between healthcare providers at national/regional level By adequate criteria , commitments and relation of all the players (formal and informal)*
  - ✓ *By using new means for **transfer of knowledge** and by **training**.*

Challenges & oportunities (2)

- **Implementing the networking tools and elements**
  - *(communication, exchange of clinical information, expertise, samples etc. (telemedicine etc…)*
- ***Identifying the services and the outputs*** *and the* ***roll*** *of the different providers at regional / EU level.*
- ***Aligning efforts, understanding and cooperation*** *between patients, professionals and healthcare authorities*

EUCERD contribution

European Commission

24 October 2011

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

31 January 2013

EUCERD Recommendations on European Reference Networks for Rare Diseases

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Next steps:

- ✓ Public consultation on ERN (finished)
- ✓ Non-paper on the functions, structure and governace of the ERNs
- ✓ Draft Delegated act
- ✓ Preparation of implementing act (stablishement and evaluation of the ERNs)

European Commission

Thank you for your attention!

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