

CENTRE OF EXPERTISE FOR RARE DISEASES

HOW DOES IT WORK?

WHAT DOES IT ADD TO THE PATIENTS, HEALTHCARE PROFESSIONALS & PAYERS?

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Who are we?

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of nearly **800 rare disease patient organisations from more than 69 countries** that work together to improve the lives of the 30 million people living with a rare disease in Europe.

Our vision is to **enable better lives and cures** for people living with a rare disease.

Our mission is to **work across borders and diseases to improve the lives** of people living with a rare disease

By connecting patients, families and patient groups, as well as by bringing together all stakeholders and mobilising the rare disease community, **EURORDIS strengthens the patient voice and shapes research, policies and patient services.**

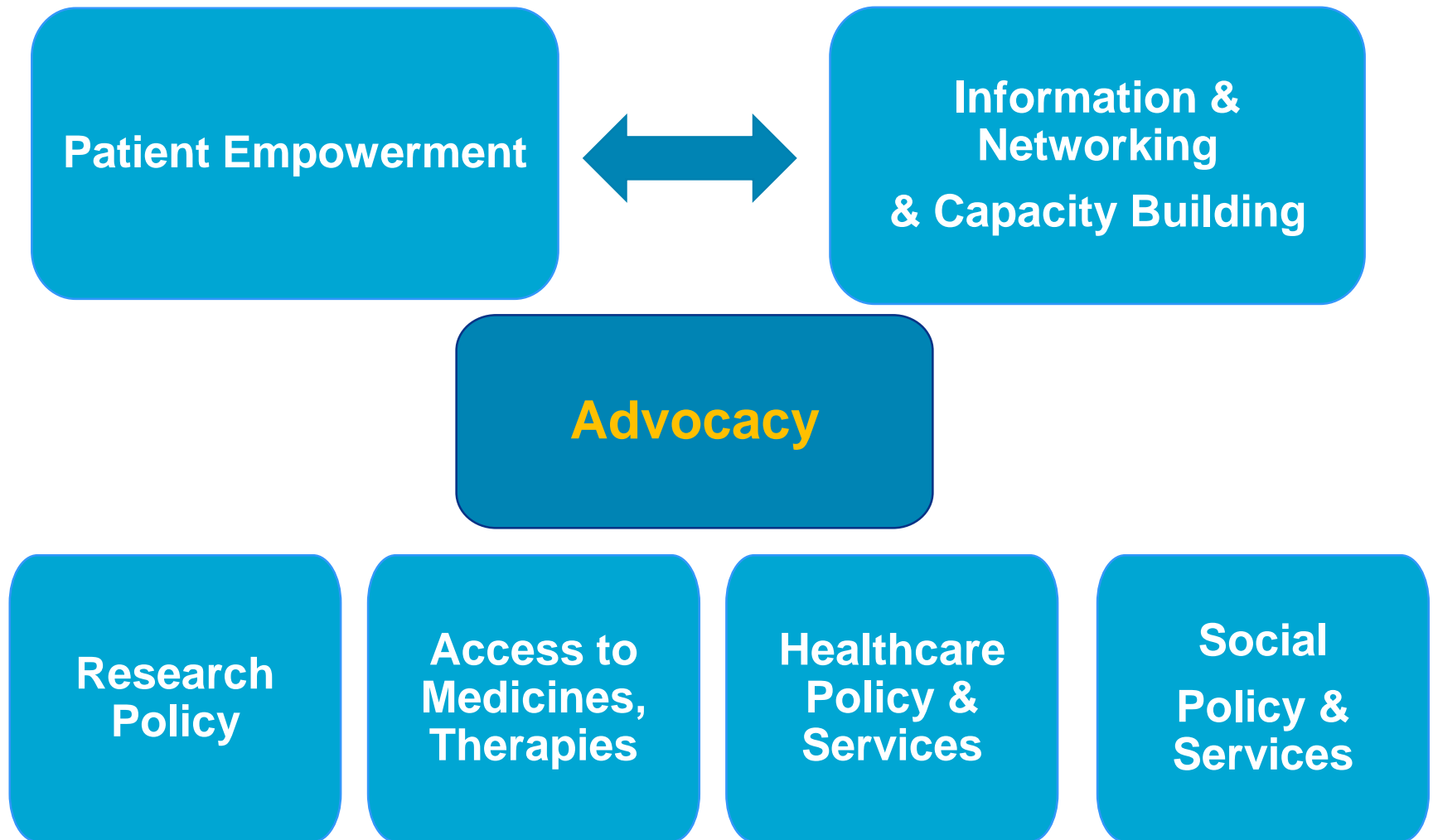


EURORDIS in brief

- Founded in 1997
- **798** member patient organisations
- **69 countries** (28 EU countries)
- **41 National Alliances** of RD Patient Organisations
- **60 European Federations** of specific rare diseases
- Outreach to over **1800 patient groups**
- **40+ staff**, offices in Paris, Brussels, Barcelona
- **Over 320 Volunteers**: +80 patient advocates and +250 moderators on online RD patient communities
- 6 Million € Budget



What we do



EURORDIS Summer School



- Capacity building programme started in 2008
- Multi-stakeholder Programme Committee (EMA, LUMC, Catalan RD platform)
- Therapeutic Development & beyond – Emphasis on Patient engagement all along the life cycle of product development
- On-line pre-training modules
 - 5-day annual on-site training on:
 - Clinical Research – Methodology, Ethics, etc
 - EU Regulatory Processes – EMA & patient engagement
 - HTA & Reimbursement – national opportunities
 - Networking

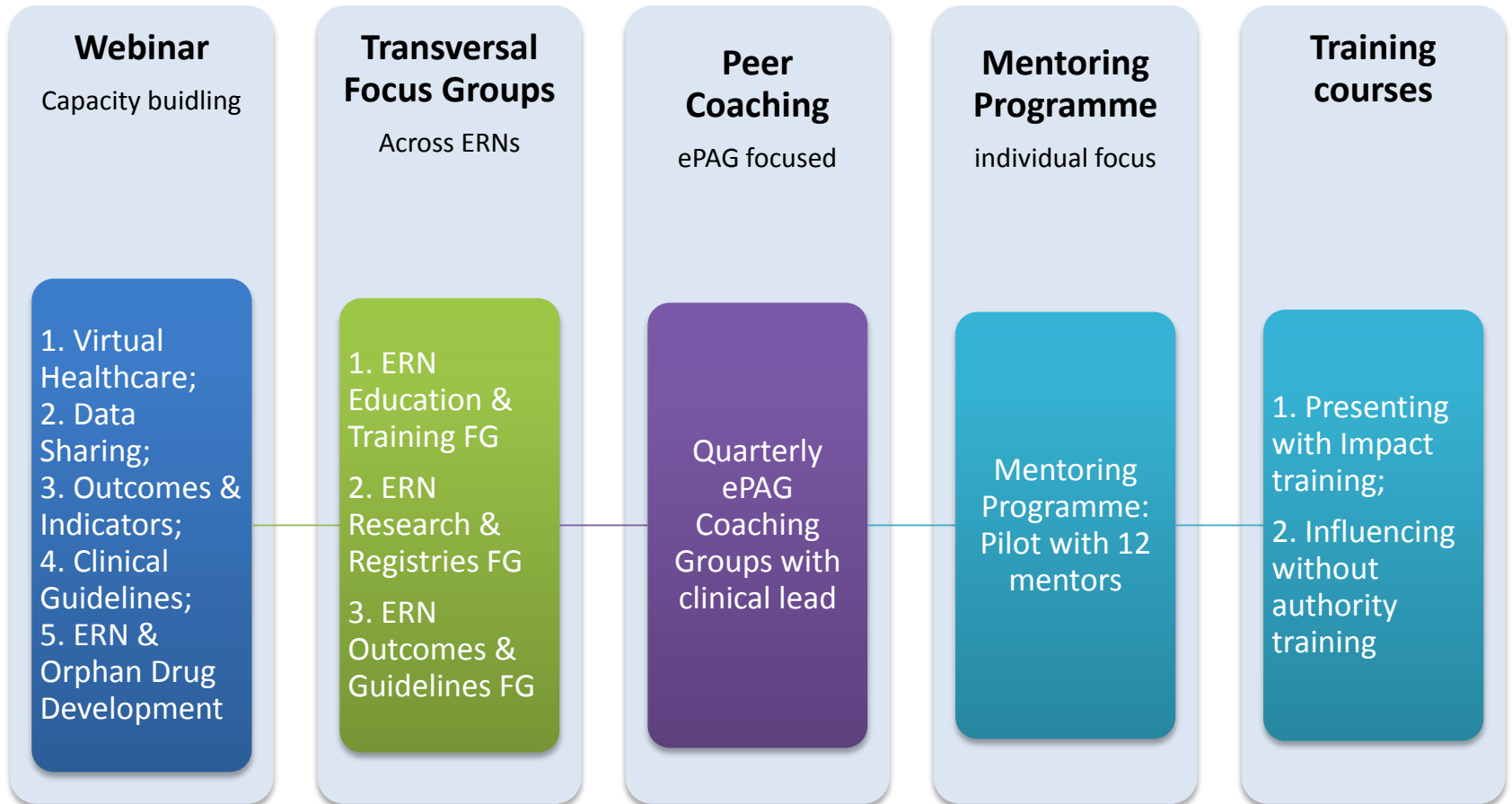
EURORDIS Winter School (2018)



- Training Programme on Scientific Innovation & Translational Research in partnership with others (New project Solve-RD)
- 5-day annual on-site training (supported by e-learning) on:
- Overview of the Research landscape/environment and opportunities
- Diagnosis (history, state-of-the art, interpretation, ethics)
- Data (data collection, interoperability, data sharing and protection)
- Therapies (animal models & alternatives, data mining/repurposing, success in gene/cell T)
- Care (ERNs)
- Visits to sequencing lab, bioinformatics institute and animal facilities

ePAG Leadership Programme

A capacity-building programme for ePAG representatives to provide them with skills and knowledge to effectively engage in ERN governance



Advocacy for EU regulations, directives and founding texts for rare diseases



- EU Regulation on Orphan Medicinal Products, 1999
- EU Regulation on Medicinal Products for Paediatric Use, 2006
- EU Regulation on Advanced Therapy Medicinal Products, 2007
- EU Directive on Patients' Rights in Cross-border Healthcare, 2011  provides legal framework for ERNs
- Communication from the European Commission on "Rare Diseases: Europe's Challenges", 11 November 2008
- Recommendation from the Council of the EU on "Action in the field of Rare Diseases," 8 June 2009

Broad cooperation



Where to treat patients living with a rare disease?

The solution supported by the RD patient community and other stakeholders :

National Centres of Expertise

Concentrating knowledge and resources, specialised in one single RD or group of RDs

Centres of Expertise (CEs)

- Mapped out at regional/national level
- Officially designated at national level

- Bring together multidisciplinary competences
- Concentrate highly specialised medical expertise

- Provide RD patients with high standards of care
- Timely diagnosis
- Appropriate treatments and follow up care

CEs for RDs are promoted in the following policy texts

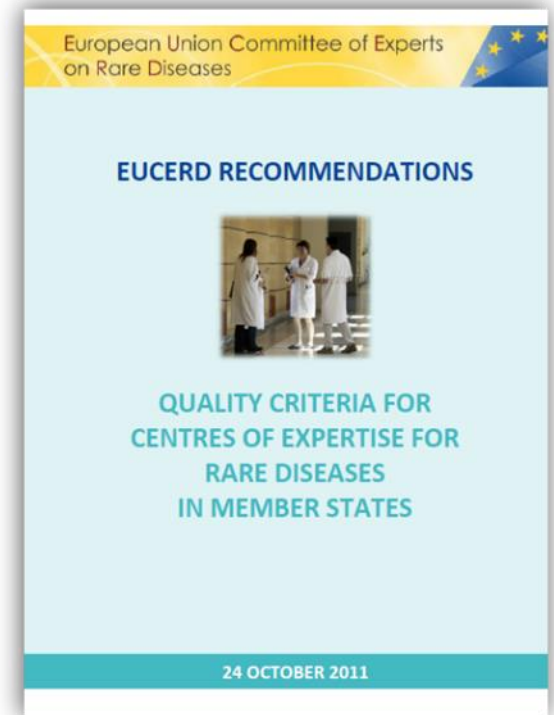
- **Communication from the European Commission** to the European Parliament, the Council, the European Economic and Social Committee, and the Committee of the Regions on: **“Rare Diseases: Europe’s challenges”**, 11 November 2008
- Recommendation from the **Council of the EU** on **“Action in the field of Rare Diseases”**, 8 June 2009

The EUCERD Recommendations

The EU Committee of Experts on Rare Diseases - adopted the **Recommendations on quality criteria** for Centres of Expertise for Rare Diseases in Member States, 24 October 2011

➔ **Founding principles of RD CEs**

➔ **Correspond to RD Patients' and their families' expectations**



The added value of CEs for RDs

Two key principles

Patient centeredness

Multidisciplinary approach to care



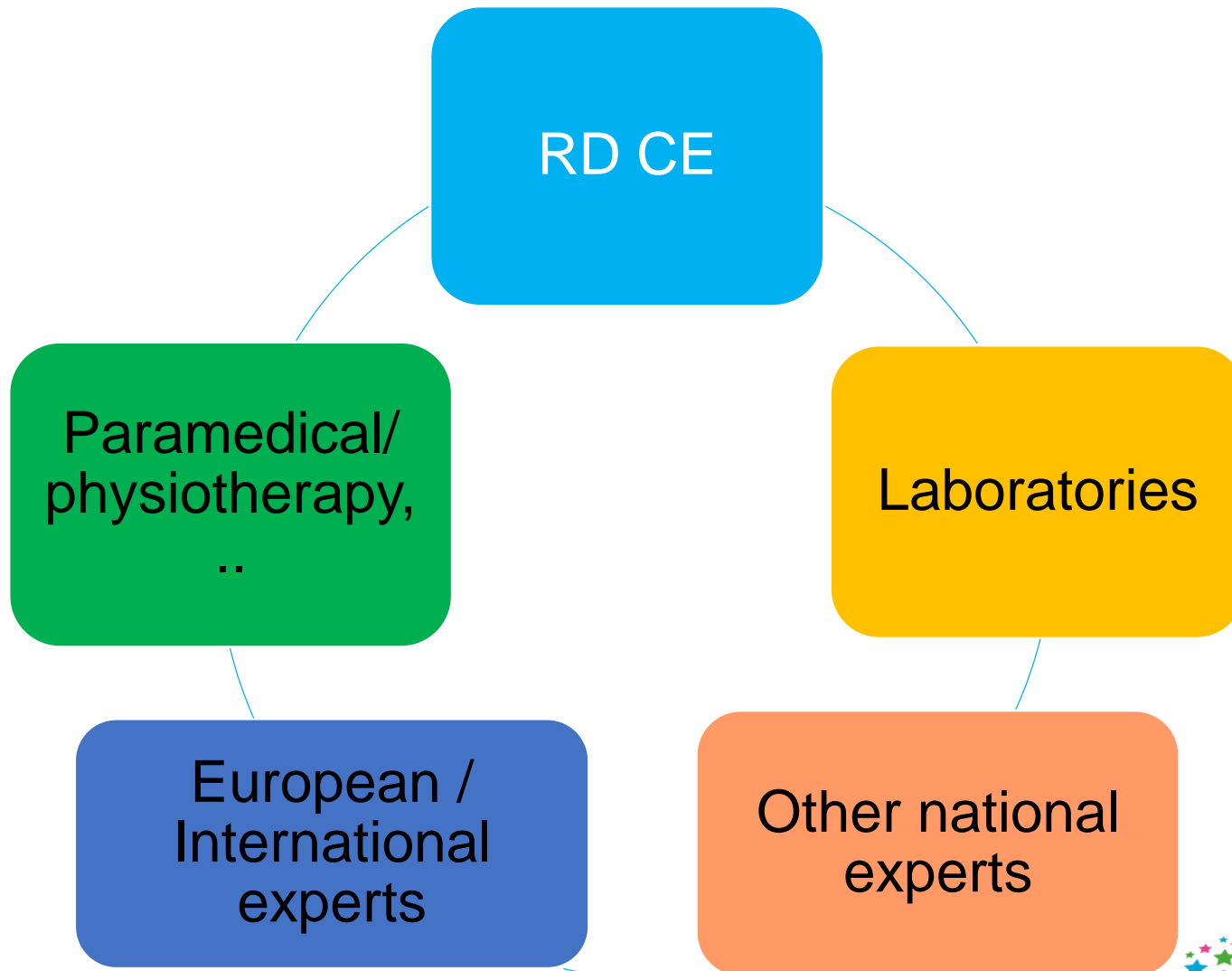
The added value of CEs for RDs

- **Concentration of expertise on RD/ group of RDs in one location**
- **Relevant partnerships with laboratories to perform genetic tests for instance**
- **Relevant partnerships with other medical experts at national, European and international level**
- **Multidisciplinary includes both medical and paramedical expertise**

 **Improve access to accurate diagnosis, adequate treatments and follow up care**



The added value of CEs for RDs



The added value of CEs for RDs

- Provide a **safe environment** for patients living with a rare disease and their families
- They can feel welcome and understood by specialists of their diseases
- CEs for RDs should ensure the respect of **non-discrimination and non-stigmatisation of RD patients** according to national/international ethical and legal frameworks (EUCERD Recommendations)

The added value of CEs for RDs

Build healthcare pathways from and to primary care, bring the expertise to the place of living of the patient



use of information and communication technologies such as telemedicine

The added value of CEs for RDs

EUCERD Recommendations:

- 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

The added value of CEs for RDs

In addition to providing medical expertise/care, the mission of the CE includes:

- Updating / elaborating / disseminating **clinical practice guidelines**
- Participating in **research studies**, in **clinical trials** where applicable
- Designing/ promoting **training programmes** for both healthcare and non-healthcare professionals (carers, school teachers...).

The added value of CEs for RDs

From an economic point of view:

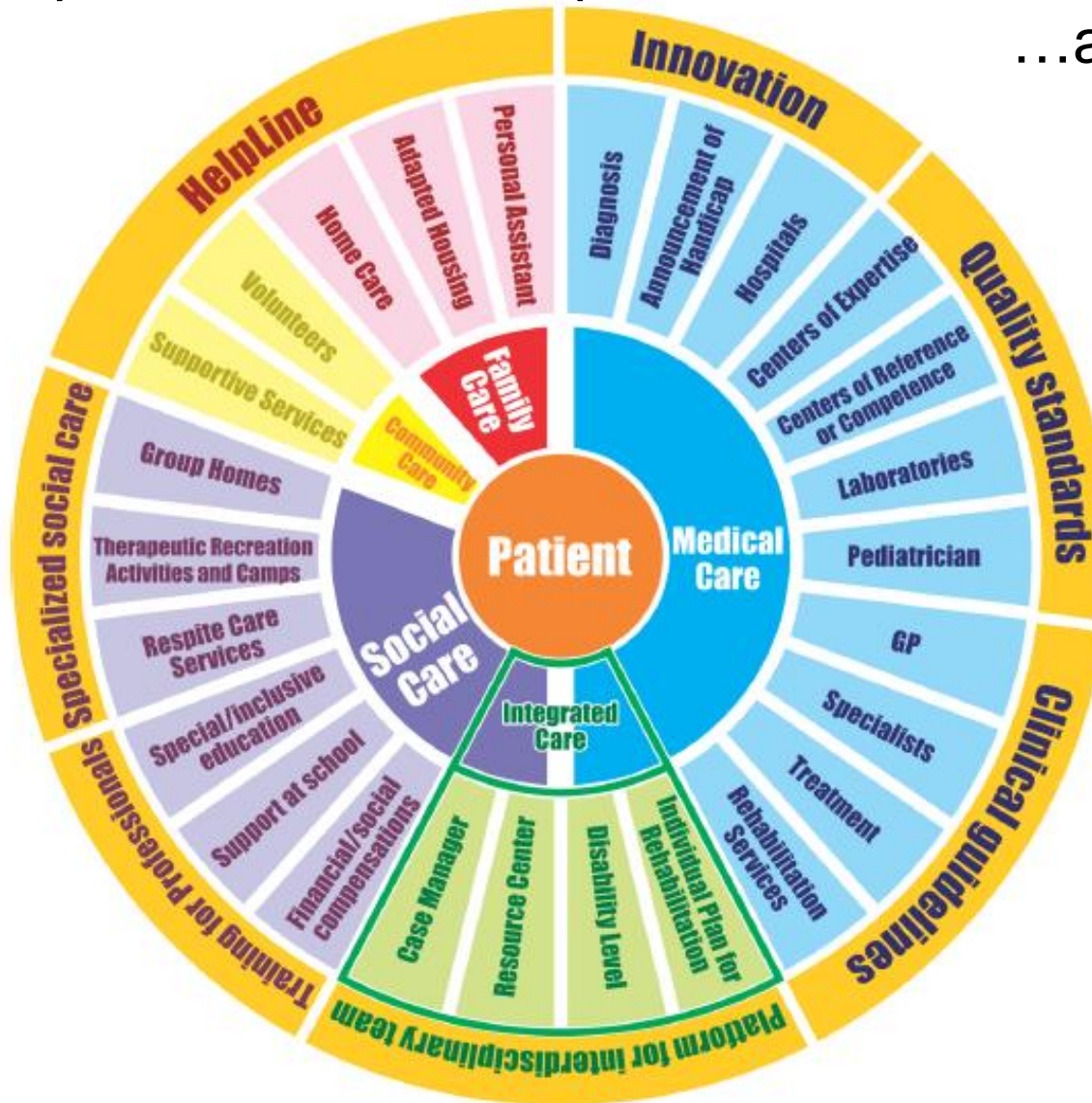
- **CEs for RDs help reduce costs in healthcare systems by contributing to:**
 - **Shorter delays in diagnosis**
 - **Avoid unnecessary treatments (including surgery) which may even lead to adverse consequences for the patient, thereby further increasing health expenses**

Even more partnership is necessary

The patients' life is complex

...a Russian roulette??

...a wheel of fortune??



Commission Expert Group Recommendations to Support Integration of RD into Social Policy

- **Centres of Expertise have a key role in facilitating integrated care** provision in line with the EUCERD recommendations on Quality Criteria for Centres of Expertise on Rare Diseases:
 - Centres of Expertise (CEs) bring together, or coordinate, within the specialised healthcare sector multidisciplinary competences/skills, including paramedical skills **and social services**;
 - CEs provide education and training to (...) **non-healthcare professionals** (such as school teachers, personal/homecare facilitators);
 - CEs contribute to and provide accessible information adapted to the specific needs of patients and their families, of **health and social professionals**.

The added value of CEs for RDs

CEs and Patient Organisations for RDs

team up at all level

- **The Patients' and carers' expertise on the daily management of their rare disease is very beneficial to improve:**
 - **Clinical Practice Guidelines**
 - **Clinical study design**
 - **Communication/Information to both healthcare professionals and non-healthcare professionals**

Designation of CEs for RDs

- **The responsibility of the designation process lies with the Member State**, based on the recommended EUCERD criteria but adapted according to the MS's healthcare system and specificities such as size and population
- **The designation of a centre is always for a defined duration** and subject to quality-based review against defined indicators
- **The CEs are a core element of a National Plan / Strategy for Rare Diseases**

Promotion of CEs for RDs

- **Specific budget needs** to be allocated by the Member State to help the CEs achieve their mission
- The Member State should ensure that CEs for RDs are **clearly identified and widely known** at national level though:
 - **Communication towards regional/ local hospitals**
 - **Communication to GPs**
 - **Communication to the public**
 - **Orphanet**



Why the CHBC was created

The Regulation 883/2004 was contradictory to other EU laws, therefore the EU wants to:

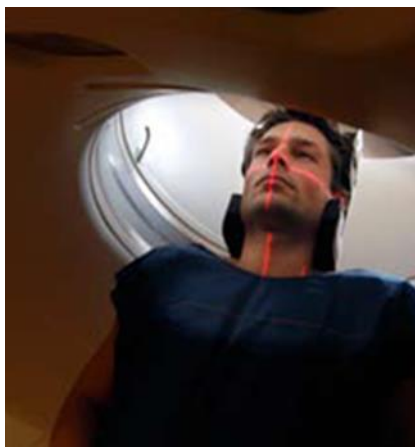
- help patients across the EU to get the care they need and to improve the effectiveness and the efficiency of healthcare systems overall.
- address the lack of information and uncertainties regarding patient rights and quality of treatment abroad. It can prevent patients from seeking out these opportunities

Directive 2011/24 was adopted in 2011 to codify the rights to healthcare abroad, which derived directly from the free movement provisions of the European Treaty, and which exists alongside the rights created by the Regulation.



The 3 aims of the Directive

- Help patients to exercise their **rights to reimbursement** for healthcare received in another EU country
- Provide assurance about **safety and quality** of cross-border healthcare
- Establish **formal cooperation** between health systems



Cooperation between health systems

- **Recognition of prescriptions**

A prescription issued in another country will be more effectively recognised

- **European Reference Networks**

They will bring together specialised centres across Europe helping health experts to disseminate information and expertise

- **Health Technology Assessment**

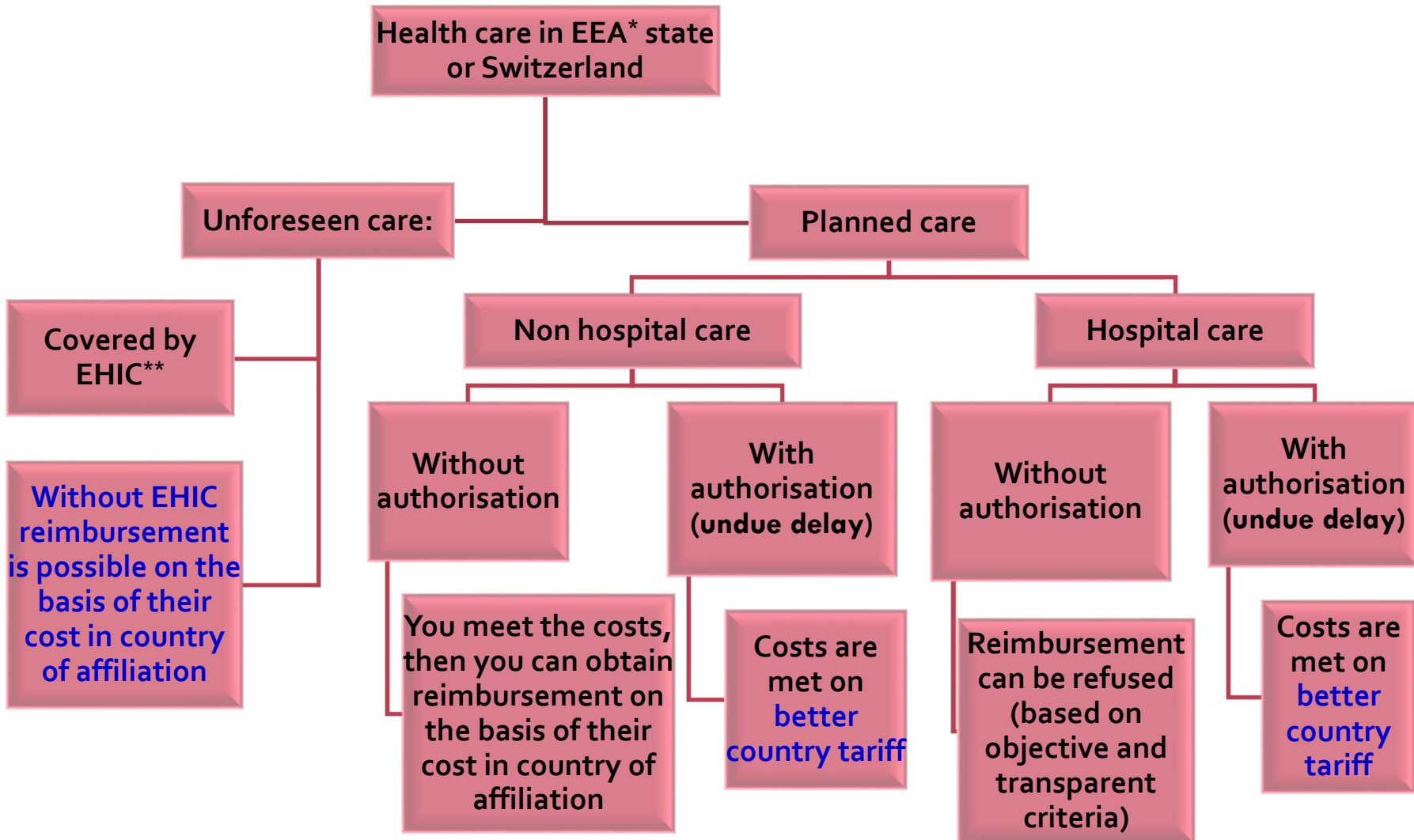
A permanent EU structure of cooperation to help decision-makers to make the right decisions on health investment and spending

- **E-Health**

A first step towards „interoperability” of ICT for health at EU level for safety and quality of care, continuity of care, and health research



New reimbursement scheme



Revolution in the European health care - ERNs

The main idea behind ERN is to improve equal access to diagnosis and care to the patients, wherever they live in the EU

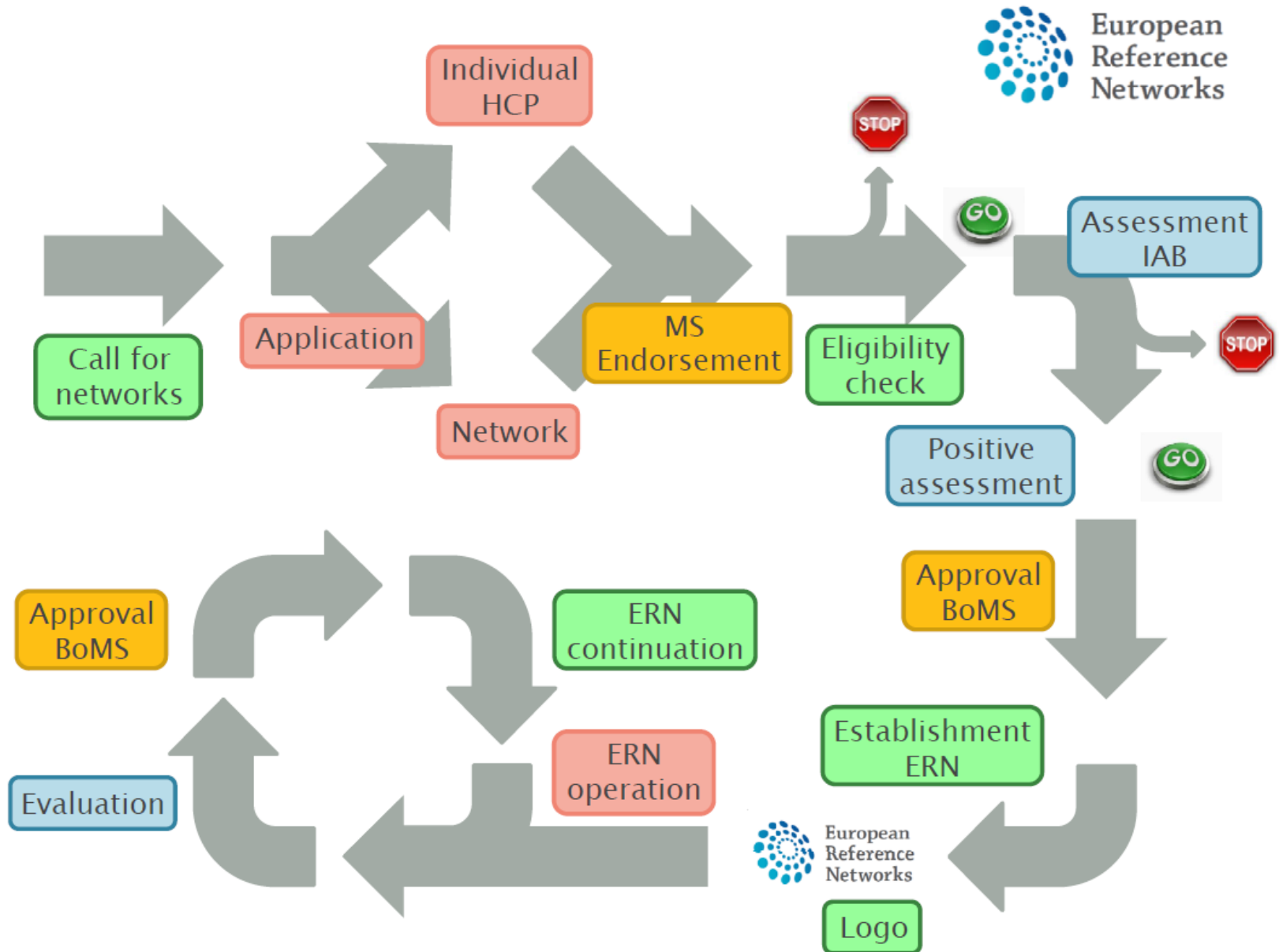
First step to harmonize and finance care which is the competence of Member States!



Share. Care. Cure.

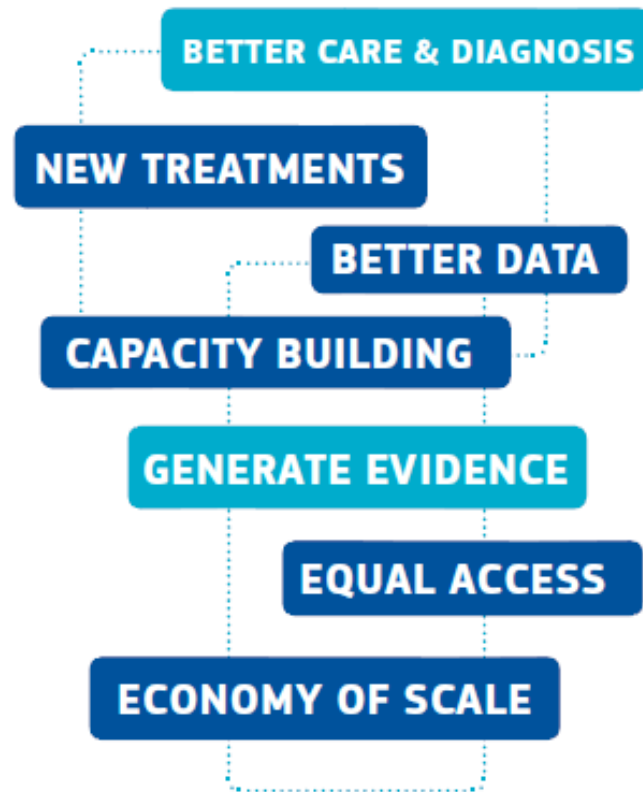


Scheme of creation



Cross-border healthcare and ERNs

One of the biggest achievements of the EC
& RD Community:
Launch of 24 ERNs on 1st March 2017 in Vilnius



https://ec.europa.eu/health/ern_en

Balanced Network Coverage needed

ERNs aim to be:

- Inclusive and foster a collaborative spirit
- Balanced and equal representation from its 'members' in all Member States
- ERN Members to act as 'national gateway or hub' connecting the ERN to the national healthcare system, linking our local and regional hospitals and the ERN.



ERN members presently

Member State	No. ERNs / 24	Number HCP	Member State	No. ERNs / 24	Number HCP
Italy	23	189	Bulgaria	6	8
France	All	129	Romania	6	8
Germany	19	117	Slovenia	7	7
UK	22	105	Estonia	4	5
Netherlands	All	92	Croatia	3	5
Belgium	All	71	Austria	3	4
Spain	15	35	Norway	3	4
Czech Republic	19	29	Ireland	4	4
Sweden	21	26	Latvia	2	2
Portugal	15	24	Luxembourg	2	2
Poland	16	21	Malta	1	1
Denmark	12	17	Cyprus	0	0
Finland	13	15	Greece	0	0
Hungary	10	14	Slovak Republic	0	0
Lithuania	11	11	Total	-	945

Commission Expert Group Recommendations to Support Integration of RD into Social Policy

- European Reference Networks **have a key role in facilitating integrated care** provision in line with the EUCERD recommendations on European Reference Networks for Rare Diseases (10)23 and the Directive on patients' rights in cross-border healthcare:
 - European Reference Networks **need to collaborate** with each other, as well as **with patient groups, health and social care providers**;
 - ERNs **follow a multi-disciplinary approach**;
 - ERNs could function as **a platform to share experiences** and promote cooperation between MS, to develop precise descriptions of the services required and elaborate common guidelines.

European Patient Advocacy Groups

- ERNs are created on founding principles of patient-centred care and patient engagement
- EURORDIS has established 24 **European Patient Advocacy Groups (ePAGs)** corresponding to the 24 ERNs:
 - **foster the collaboration between patients' representatives and specialised healthcare professionals across borders and contribute to the development of these ERNs.**



Key messages

- ERNs are only now being established and will need to be anchored into national health systems where they will have their foundation
- Unmissable opportunity to:
 - Build collaborative spirit between governments, clinicians and patients
 - Foster inclusiveness and support national endorsement process
 - Foster transparency of expertise to drive better quality of care
 - Clarify national recognition of centres with clear referral pathways
- Advocacy actions at national level are still needed to:
 - Monitor implementation of plans of successful HCPs and potentially support more HCPs wishing to join ERNs
 - Identify national hubs, affiliated & collaborative centres and create national networks for better organisation of care, improved care & diagnosis
 - Promote the adoption of ERN evidence base practice, guidelines etc. in local treating hospitals and drive quality outcomes at local level
 - Improve understanding of RDs, mapping resources, optimise spending
 - Achieve common codification and quality of data collected and shared for research, therapy development and faster diagnosis

Thank you for your attention



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