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SYNTHESIS of NATIONAL WORKSHOPS

Denmark - Germany - France
- Italy - Spain - Sweden - UK
- Netherlands- Portugal -
Belgium

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SYNTHESIS

- Global overview of results :
 - ↳ Split Europe/National
 - ↳ Split total « voice »
- Analysis by themes
- Questions / discussion



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Analysis of European Recommendations

Rank	Proposals	Total	DK	FR	GE	IT	SP	SW	UK
1	Make an inventory of orphan medicinal products & make them available to patients in all member states - Develop European Data base	6		X	X	X	X	X	X
2	Provide information about new drugs, new methods of treatment, clinical trials equally in all countries	4	X	X	X		X		
3	List of molecules abandoned by Pharmaceutical industries before development and possible withdraw of products developed for common diseases but efficient for RD,	3		X			X	X	
3	Develop epidemiological studies on RD, data base on RD	3		X	X				X
3	Develop specific clinical trials methodology and "Fast-track" process	3		X	X	X			
4	Join orphan drugs task force regrouping associations, industry, Researcher, GP	2		X	X				
4	Adoption of normative, economic and insurance tools to get quick access of new orphan drugs in all member states	2				X	X		
4	Create a European day on RD	2		X		X			
4	Inform patients on safety of clinical trials and need to create a patient registry	2		X			X		
5	Create an orphan Drug economic committee managed by EMEA	1		X					
5	Improve procedures of recruitment of patients	1				X			
5	Establish "Care Standards and Guidelines" for therapy in collaboration with patients	1			X				
5	Set up a european network of DNA and tissues banks	1		X					
5	Develop a European policy on Rare Diseases together with EMEA	1		X					
5	Pharmacovigilance -side effects of Medicinal products	1			X				
5	Identify barriers of new cooperation between Researchers and producers	1	X						



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Analysis of National Recommendations

Rank	Proposals	Total	DK	FR	GE	IT	SP	SW	UK
1	Identify specific incentives and national funds for research on RD, to implement the Regulation on Orphan Medicinal Products and promote research	5	X		X	X	X		X
2	Centers of competence aiming at care and treatment - Standard & guidelines therapy Strengthen cooperation between health professionals and patients organizations	4			X	X	X	X	
3	Facilitate a more timely diagnosis and new methods of diagnosis	3	X		X				X
3	National Action Plan research on Rare Diseases	3		X		X	X		
4	Develop compassionate Programme	2			X		X		
5	Disease Management for RD (mediators need between stakeholders)	1			X				
5	Support development of Regional Alliances in a country	1				X			
5	Creation of one national organization representing all regions	1				X			
5	Creation of Permanent Regional Commissions of study on RD in each region	1				X			
5	Request public health system to finance OMPs as for chronic diseases	1					X		
5	Improving nationally the distribution of adequate treatment in hospitals, pharmacies	1					X		
5	Create a specialty in genetics within the schools of medicine	1					X		
5	New born screening for all rare disorders for which a treatment exists - nationwide	1			X				
5	Reform reimbursement of costs for the care of patients with RD	1			X				
5	Develop Rare Disorders Awareness, Health care system, insurances....	1			X				
5	Educate patients organizations to become active and strong actors	1			X				
5	Involve patients when writing the patients information for prescribed drugs	1			X				
5	Need to improve patients/GP/stakeholders relationships	1			X				
5	Address the concern of Rare Diseases at Health Ministry level	1							X



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SYNTHESIS

- Themes > 3 X
- « Orphan » themes
- Discussion



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SYNTHESIS

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NATIONAL

➤ **10X**

- **KM : OMP database**
 - Existing treatment
 - Clinical trials

- **LEGAL & RESEARCH :**
 - Implement OMP regulation
 - National Funds on OMP

➤ **5 X**

➤ **3X**

- **RESEARCH :**
list of molecules abandoned by industry

- **CARE :**
Centers of Competence
 - Diagnostic
 - Prevention
 - Disease Management

➤ **4X**

➤ **3X**

- **RESEARCH :**
Epidemiological studies

- **CARE :**
Compassionate Programmes

➤ **2X**

➤ **3X**

- **RESEARCH :**
Dedicated methodology for clinical trials & Fast track



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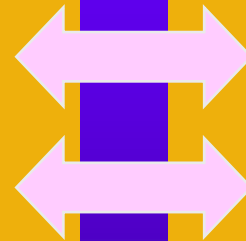
ANALYSIS

European Research Networks on RD

- ◆ Epidemiological studies
- ◆ Clinical trials
- ◆ Abandoned products
- ◆ DNA network

European Care Network on RD

- ◆ Cooperation between shareholders
- ◆ Standards & Guidelines therapy



Improve procedures of patient/investigators recruitment

EMEA

EC

Patients

Ind
Pharma

Gvt
(price)

Insurances

Physicians

Ind
Pharma

Patients



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ANALYSIS BY THEMES

- Themes > 3 X
- « Orphan » themes
- Discussion



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“ORPHAN IDEAS”

**European Economic
and Insurance Tool**

**Identify barriers between
research & producers**

**Create an OMP
Economic Committee
managed by EMEA**

**Improve patient / MD /
stakeholder relationship**



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ANALYSIS BY THEMES

- Themes > 3 X
- « Orphan » themes
- Discussion



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➤ **Your feedback**

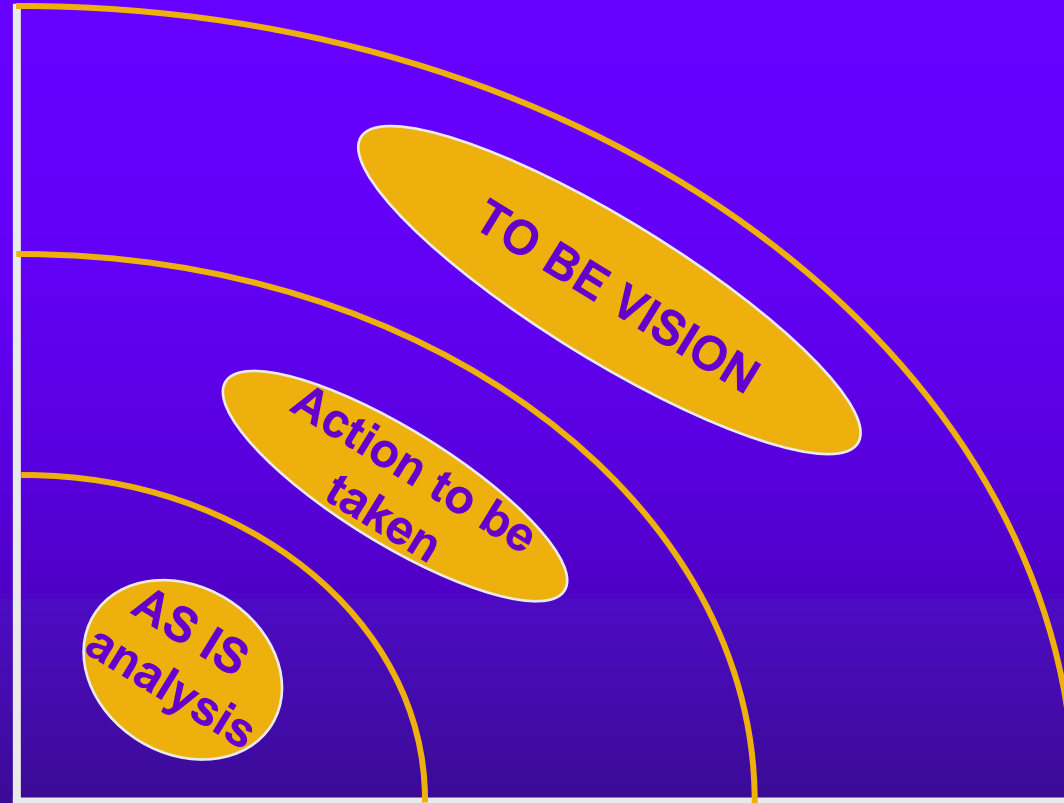


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Example : “Review Clinical trials methodology”

European Research Center on RD

Targets



Time

- Phase I : avoid testing molecules on healthy people

- Skip phase III- Fast track approval

- Create a European Task Force to reassess clinical trials

- Phase II : small group

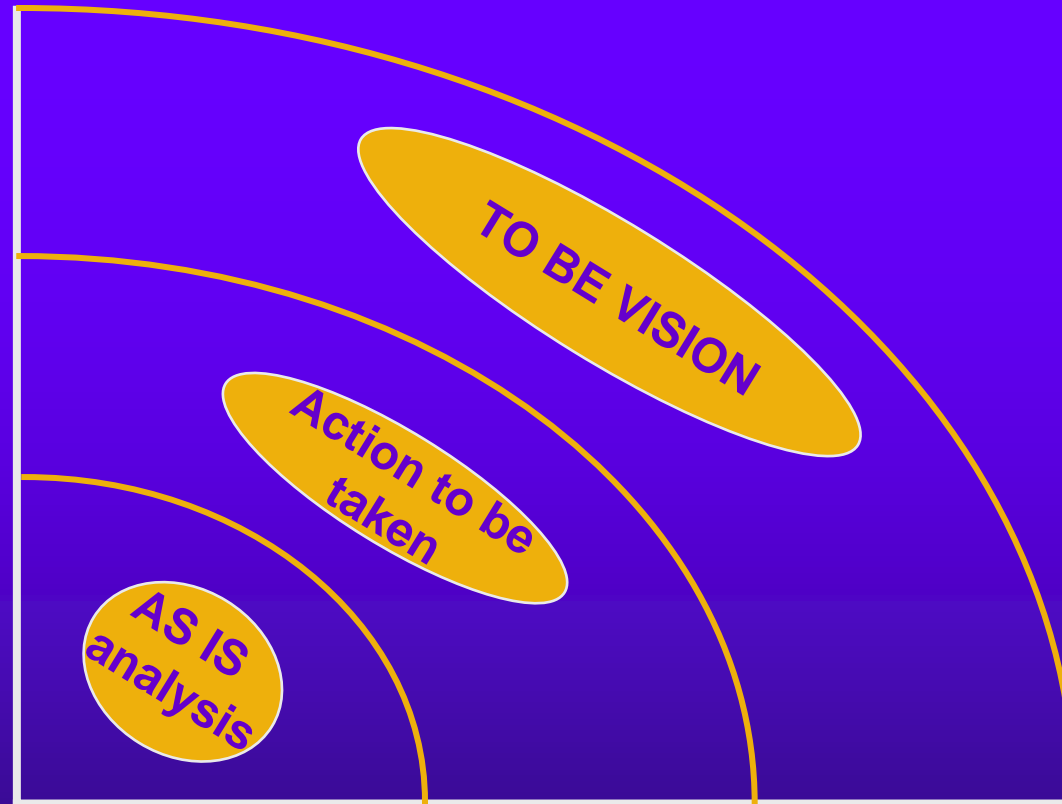
Compassionate program



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Example : "Towards supranational OMPs"

Targets



Time

- **Incentives** : harmonization

- **Pricing** : indicative price range

- **Retailing** : blue box

- **Convey a European Harmonization Task Force to suppress the blue box**

- **Create an OMP Economic Committee to be managed by EMEA**



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➤ **Back up**

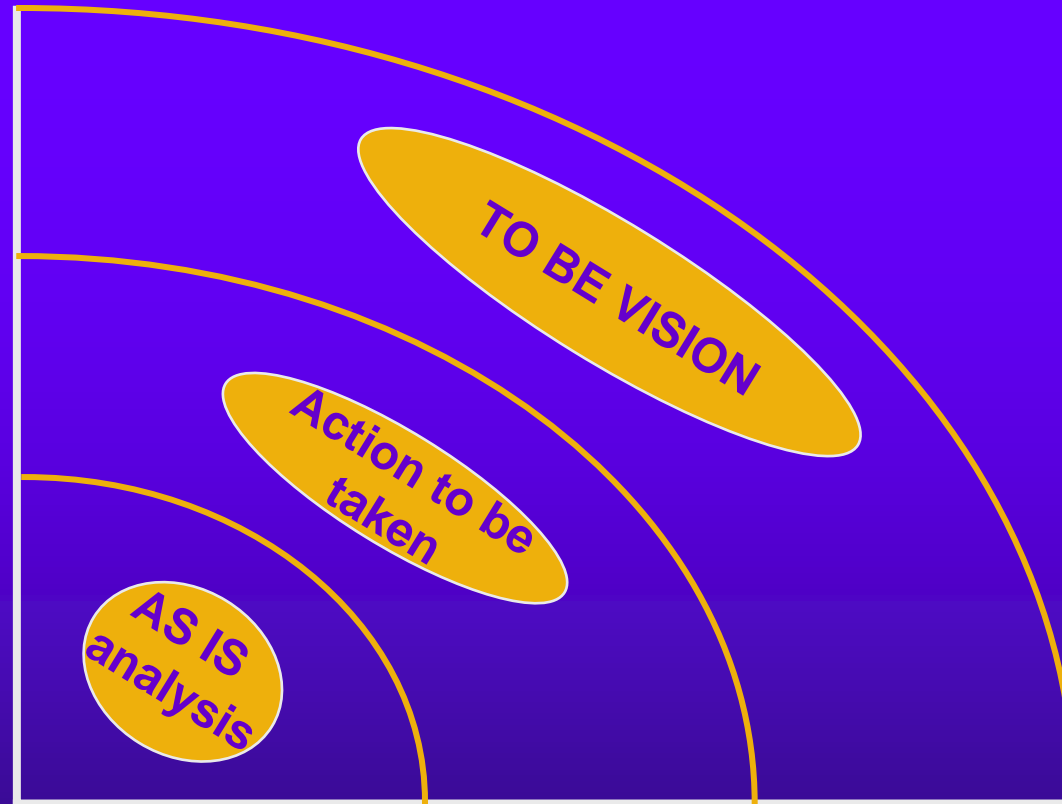


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Example : “Epidemiological studies”

European Research Network on RD

Targets



Time

- | | | |
|----------------------------------------------------------------------------------------|---------------------------------------------------------------------------|----------------------------------------------------------------------------|
| <ul style="list-style-type: none">• Explore organization methods - tools | <ul style="list-style-type: none">• Call for ad hoc tenders | <ul style="list-style-type: none">• European research center |
| <ul style="list-style-type: none">• Collect existing data | <ul style="list-style-type: none">• Co-ordinate studies | |



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ORGANIZATION



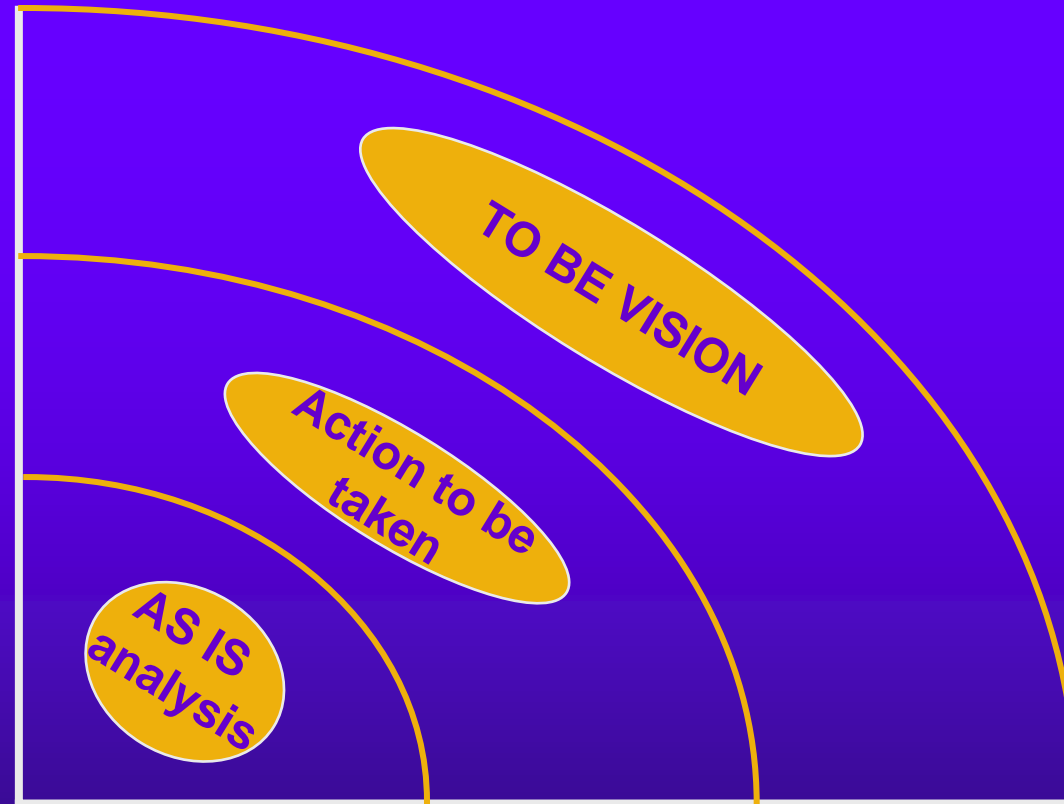


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Example : "Center of Excellence"

European Care Network on RD

Targets



Time

- Explore organization methods - tools

- Collect existing data

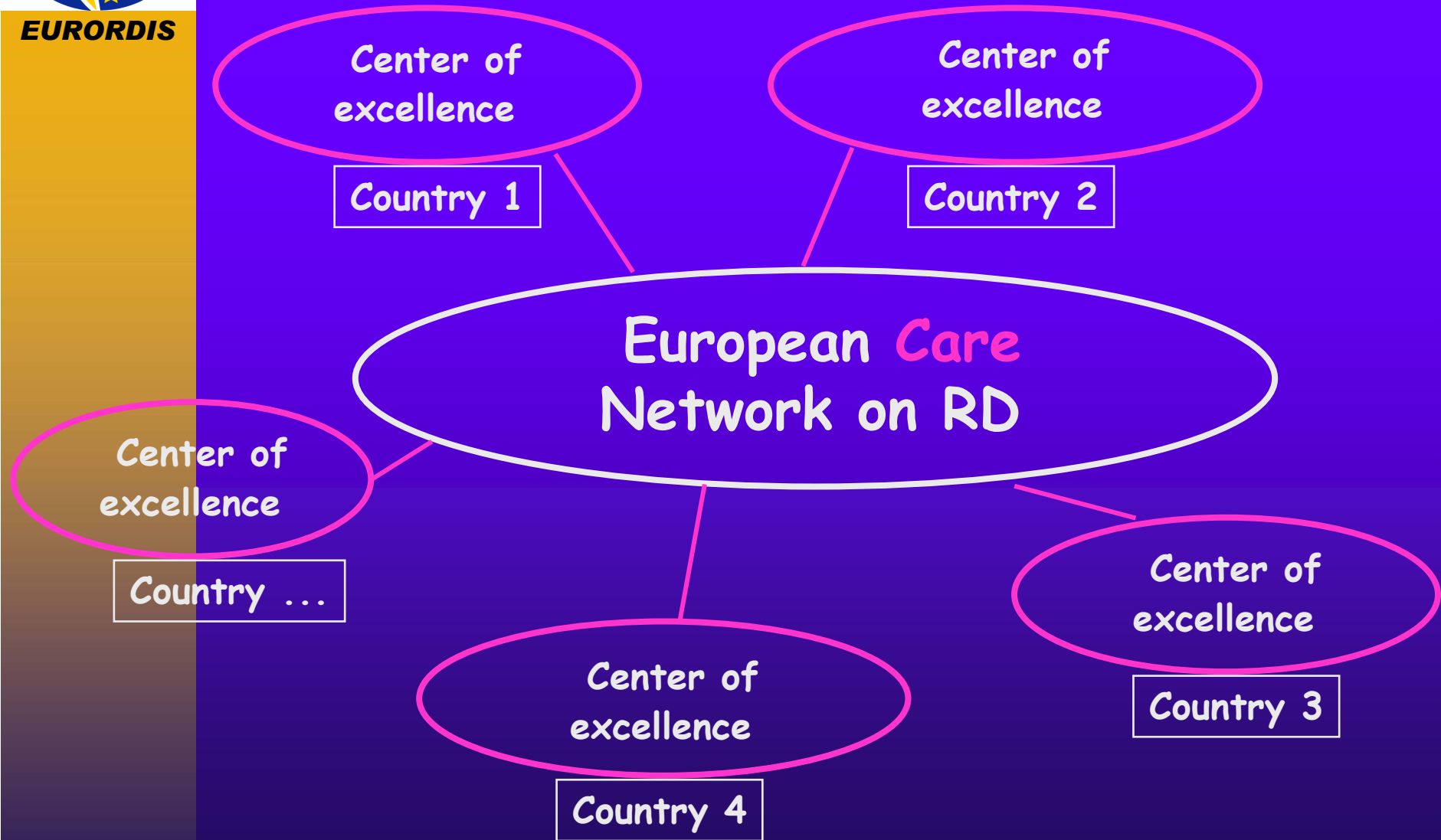
Cooperation at a European level

- All group of diagnoses belong to a center



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ORGANIZATION



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