EUCERD
Joint Action

EUROPLAN
European Project for Rare Diseases National Plans Development
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Italian National Institute of Health
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USE OF CORE INDICATORS IN MEMBER STATES

Study Results

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BACKGROUND AND METHOD

BACKGROUND

- 59 Indicators (EUROPLAN 2008-2011)
- EUROPLAN 2012-15: 21 selected core indicators adopted by EUCERD Recommendations (June 2013)
- Follow-up study for fine-tuning &improving potential to orient RD policies

METHOD

- Survey on the use of indicators in selected MS: Bulgaria, Croatia, Italy, Romania and Spain
- Descriptive exploratory collection of lessons to take into account

RESULTS: *Background Indicators*

	BULGARIA	CROATIA	ITALY	ROMANIA	SPAIN
Existence of Regulations/Laws, or equivalent official national decisions **supporting** the establishment and development of a **Rare Diseases (RD) plan**	In progress /in development	In progress /in development	Yes, existing, partly embedded	Yes, existing, partly embedded	Yes, existing, fully embedded in a regulation/law/official national decision
Existence of a RD **advisory committee**	YES, exists but partly functioning and includes all relevant stakeholders	YES, exists and meets regularly and includes all relevant stakeholders	No	YES, exists, meets regularly and includes all relevant stakeholders	YES, exists, meets regularly and includes all relevant stakeholders
Permanent and official patients'representation in plan development, monitoring and assessment	Yes, at all stages	Yes, at all stages	No	Yes, at all stages	Yes, at all stages
Adoption of the **EU RD definition**	YES, the NP/NS measures are applied using the EU definition	YES, the NP/NS measures are applied using EU definition	YES, but the NP/NS measures are applied using a different definition	YES, the NP/NS measures are applied using the EU definition	YES, the NP/NS measures are applied using the EU definition
If NO, specify the definition used in the NP/NS	-	less than 1 in 2000	The list of RDs includes life-threatening or chronically debilitating diseases, with a high level of complexity and requiring high costs treatments	Romania accepts the definition of rare disease, as stated in the European Regulation on Orphan Medicinal Products, as a disease with a prevalence of no more than 5 in 10,000 individuals. This definition has been adopted by the National Plan for Rare Diseases.	

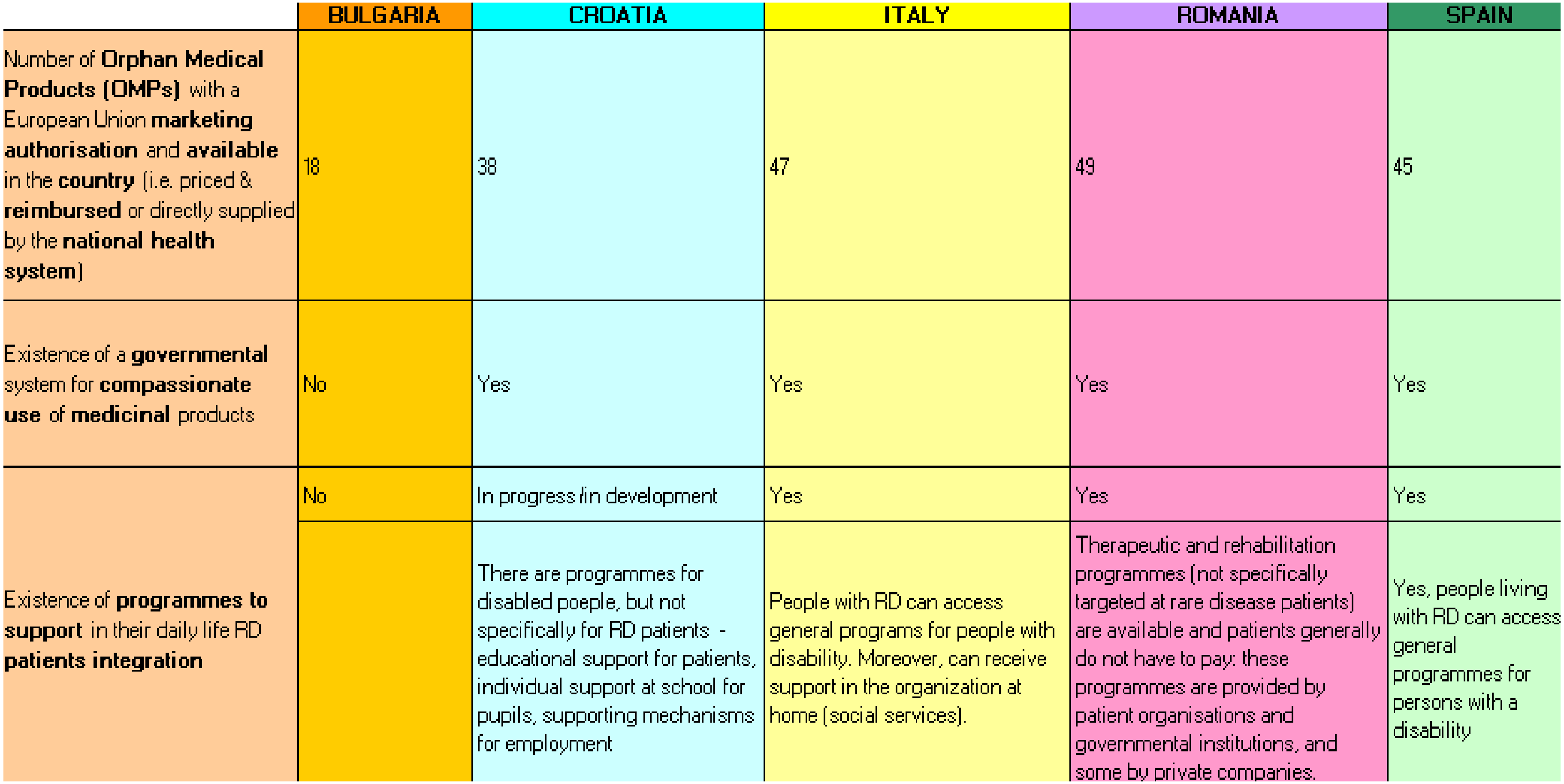
Content Indicators: CoE & Information

	BULGARIA	CROATIA	ITALY	ROMANIA	SPAIN
Existence of a **national policy for** establishing **Centres of Expertise on RD**	In progress/in development	Yes, , existing, fully implemented	In progress/in development	In progress/in development	Yes, existing, fully implemented
Number of national and regional **Centres of Expertise** adhering to the national policy	0	6	All the CEs identified by Regions		78
	0	1 per 750000 (but these centers are not for all RD)			
	0	probably 3			78
	n/a			Romania is still working on quality criteria for CEs using EUCERD recommendations in order to appoint CEs throughout national territory.	
Participation of national or regional centres of expertise **in European Reference Networks**	n/a	I am not aware that ERNs are officially establihsed	ERNs are still in progress	Romania is still working on quality criteria for CEs using EUCERD recommendations in order to appoint CEs throughout national territory.	We don't know at this moment
NP/NS support to the development of / participation in an **information system on RD**	Yes	Yes	Yes	Yes	Yes
	Participation in the Orphanet Joint Action		Participation in the Orphanet Joint Action		
	Yes, national	Yes, national	Yes, national	Yes, national	Yes, national
	Yes, participates in Orphanet JA but does not produce information in national language(s)	Yes, participates in Orphanet JA and produces information in national language(s)	Yes, participates in Orphanet JA and produces information in national language(s)	No	Yes, participates in Orphanet JA and produces information in national language(s)
Existence of **Help lines** for RD	YES, supported by private funding	YES, supported by public funding and by private funding	YES, supported by public funding and by private funding	YES, supported by public funding and by private funding	YES, supported by public funding and by private funding

Content Indicators: Kowledge, Classification/Coding, Registries, Research

	BULGARIA	CROATIA	ITALY	ROMANIA	SPAIN
Existence of a **national policy for** developing, adapting and implementing **clinical practice guidelines**	No	No	Yes, a policy exists for developing CPGs	Yes, a policy exists for developing CPGs	Yes, a policy exists for developing CPGs
Type of **classification / coding** used by the health care system			ICD-9		ICD-9
	ICD-10	ICD-10		ICD-10	ICD-10
					SNOMED
					ICD-O
					ICD-O
Existence of a national policy on **registry** and data collection on RD	Yes, for national/centralised registry and data collection	No	Yes, both for national/centralised and regional registry and data collection	Yes, Do Not Know	Yes, for national/centralised registry and data collection
Existence of a **RD research programmes** / projects in the Country	No	No	Yes, specific PROJECTS for RD within general research programme	No	Yes, specific PROJECTS for RD within general research programme
Participation in **European** & **international research** initiatives	Yes	No	Yes, E-RARE, IRDIRC	Yes, others (Romania contributes/contributed to the EUROPEAN LEUKEMIA NET European research project and the European Network for Study of Adrenal Tumours - ENS@T)	Yes, E-RARE

Content Indicators: Therapies & Social Services



	BULGARIA	CROATIA	ITALY	ROMANIA	SPAIN
Number of **Orphan Medical Products (OMPs)** with a European Union **marketing authorisation** and **available** in the **country** (i.e. priced & **reimbursed** or directly supplied by the **national health system**)	18	38	47	49	45
Existence of a **governmental** system for **compassionate use** of **medicinal** products	No	Yes	Yes	Yes	Yes
Existence of **programmes to support** in their daily life RD **patients integration**	No	In progress /in development	Yes	Yes	Yes
		There are programmes for disabled poeple, but not specifically for RD patients - educational support for patients, individual support at school for pupils, supporting mechanisms for employment	People with RD can access general programs for people with disability. Moreover, can receive support in the organization at home (social services).	Therapeutic and rehabilitation programmes (not specifically targeted at rare disease patients) are available and patients generally do not have to pay: these programmes are provided by patient organisations and governmental institutions, and some by private companies.	Yes, people living with RD can access general programmes for persons with a disability

Financial Support Indicators

	BULGARIA	CROATIA	ITALY	ROMANIA	SPAIN
Existence of a **policy** / decision **to ensure long-term funding** and / or sustainability of the measures in **RD plan** / strategy	No	In progress /in development	Yes, a policy/decision to ensure long-term sustainability	No	Yes a budget exists for the plan
Amount of public funds allocated **to RD plan** / strategy		0	0	0	0
Specific public funds allocated **for RD research**	No	In progress /in development	No	No	Yes
Public funds specifically allocated for RD research actions/projects **per year since the plan started**	0	the plan is not adopted yet	N/A	It is incorporated in the general research funds.	16.100.551

Positive aspects while using indicators

- Collection of important information about NP on RD and easiness to answer
- Excellent opportunity to share knowledge and comparability among countries
- Political usefulness
- Chance to adapt national RD policies to best examples available and recommended at EU level
- Tool to timely follow up and report on national RD activities
- Possibility to follow-up the progress
- Focus on relevant issues regarding development and implementation of NP which are common to the 28 EU MSs;
- Harmonisation of monitoring procedures & criteria and assessment of common RD policies in the 28 EU MSs;
- Usefulness to capture and describe the situation as far as the general measures adopted by countries are concerned

Difficulties faced while using indicators

- Quantitative indicators may not reflect qualitative improvements
- Comparing MS different rules and regulation to make clear the different organization of the countries through short answers can be very difficult
- Indicator on participation in ERNs is difficult to use (ENR for RD are not yet established)
- Low visibility of what is done for RD patients in programmes to support in their daily life integration (in place in all Countries)
- Difficulties in collecting information from the regional level (Spain)

Opportunity of integrating indicators

> *“The core indicators are selected from EUCERD indicators. To integrate them with EUCERD indicators means to go back to the starting line“*

However:

- possible future integration with EUCERD criteria on centres of expertise for RD and RD reference networks
- include information on newborn screening policies and on the genetic diagnosis and genetic counselling policies.

THANK YOU
FOR YOUR ATTENTION!