

EUROPLAN

European Project for Rare Diseases National Plans Development

Coordinated by the Italian National Centre for Rare Diseases

Italian National Institute of Health

2012-2015



Co-funded by the



Selecting indicators to evaluate the achievements of RD initiatives

Manuel Posada, María José Carroquino and Manuel Hens
on behalf the Working Group of Indicators

Institute of Rare Diseases Research, Instituto de Salud Carlos III
Madrid, Spain

INCEPTION WORKSHOP ON NATIONAL PLANNING FOR RARE DISEASES

10-11 September 2012, Rome, Italy





COMMISSION OF THE EUROPEAN COMMUNITIES

Brussels, 11.11.2008
COM(2008) 679 final

**COMMUNICATION FROM THE 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

{SEC(2008)2713}

{SEC(2008)2712}



COUNCIL RECOMMENDATION**of 8 June 2009****on an action in the field of rare diseases****(2009/C 151/02)**

THE COUNCIL OF THE EUROPEAN UNION,

Having regard to the Treaty establishing the European Community, and in particular the second subparagraph of Article 152(4) thereof,

Having regard to the proposal from the Commission,

- (4) Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products⁽⁵⁾ provides that a medicinal product shall be designated as an 'orphan medicinal product' when intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than 5 in 10 000 persons in the Community when the application is made.





ISTITUTO SUPERIORE DI SANITÀ (ISS)
NATIONAL CENTRE FOR RARE DISEASES
(CNMR)



THE NETHERLANDS ORGANISATION FOR
HEALTH RESEARCH AND DEVELOPMENT
(ZONMw)



INSTITUTO DE SALUD CARLOS III (ISCIII)



KAROLINSKA INSTITUTE (KI)



LONDON STRATEGIC HEALTH AUTHORITY

UNIVERSITY OF TARTU (UT)



TARTU ÜLIKOOOL
 UNIVERSITY OF TARTU



EURORDIS
 Rare Diseases Europe

EUROPEAN ORGANISATION FOR RARE
DISEASES (EURORDIS)



BULGARIAN ASSOCIATION FOR PROMOTION
OF EDUCATION AND SCIENCE (BAPES)



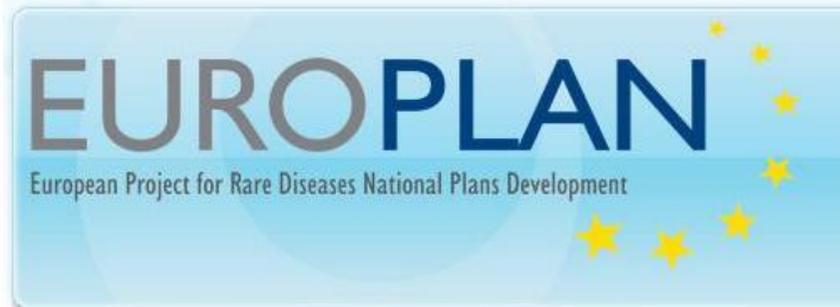
FUNCIS
 Fundación Canaria de Investigación y Salud



FUNDACION CANARIA DE INVESTIGACION
Y SALUD (FUNCIS)



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THE PROJECT

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STRUCTURE OF EUROPLAN 2008 - 2011

WP1-WP3 deal with the management of the project and the dissemination and evaluation of the results.

WP4-WP8 are the core WPs.

WP4 collects information on the initiatives undertaken by EU MSs on rare diseases. A dedicated survey is aimed at gathering examples of different MSs experiences and lessons learned allowing the sharing of information among Member States.

WP5 develops indicators for monitoring the implementation and for evaluating the impact of National Plans or Strategies for rare diseases.

WP6 analyses case studies to identify relevant experiences.

WP7 develops the content of the EUROPLAN recommendations including a methodological guidance to design comprehensive and integrated strategies for guiding and structuring all relevant actions in the field of rare diseases.

WP8 deals with the organisation of National Conferences where national stakeholders are convened to discuss the EUROPLAN Recommendations Document within the specific national context.

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STRUCTURE OF EUROPLAN 2008 - 2011

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WP4-WP8 are the core WPs.

WP5: Selecting indicators to evaluate the achievements of RD initiatives

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WP5 Scope and purpose

To develop a list of indicators for evaluating RD activities following those areas of action previously identified



WP5 - Selecting indicators to evaluate the achievements of RD initiatives

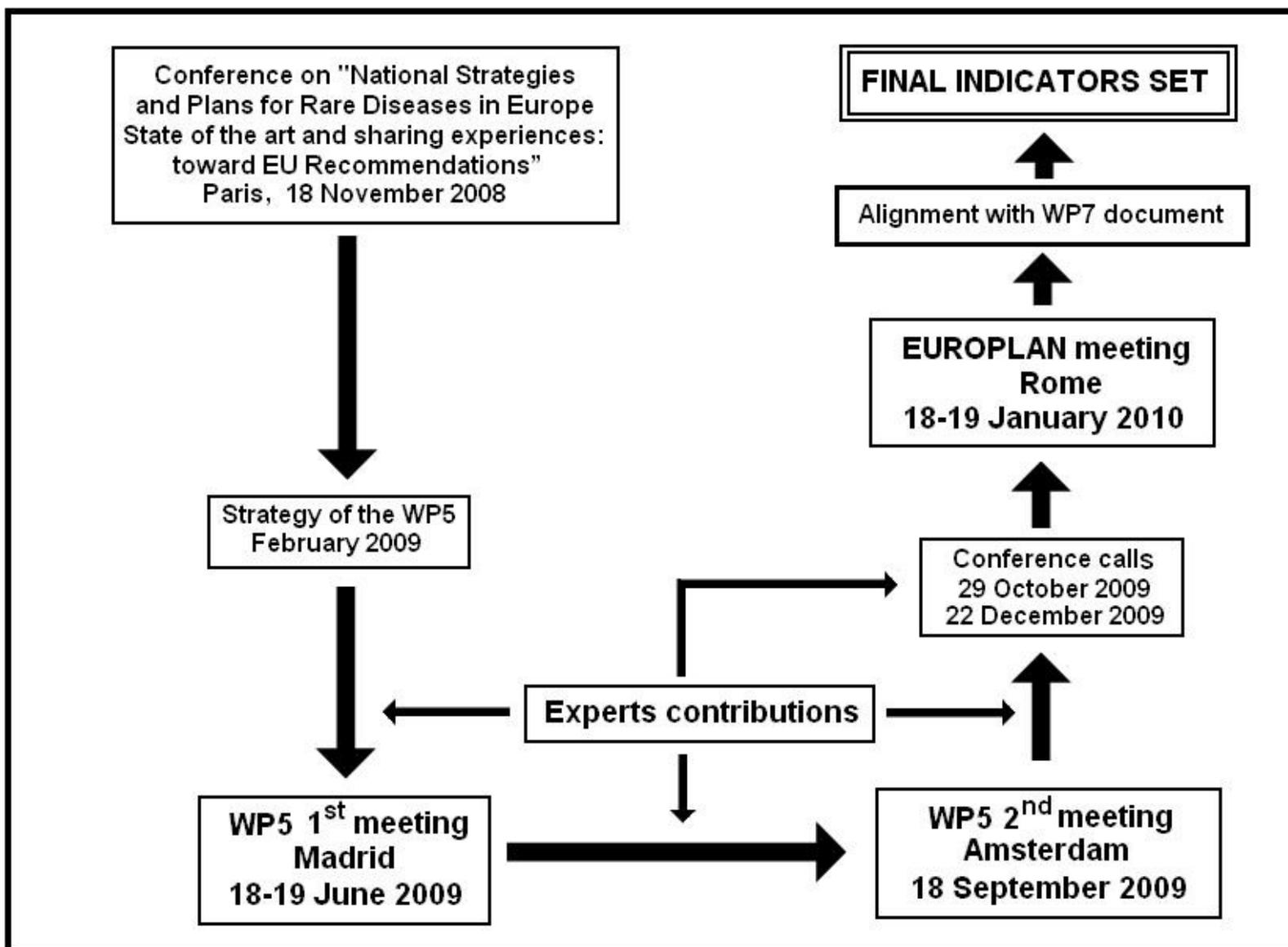
- WP5 will elaborate indicators for monitoring the implementation and evaluating the impact of national plans for rare diseases.
- The activities required to identify indicators will include:
 - comprehensive literature review on rare diseases indicators,
 - discussion with experts including the engagement of the RDTF
 - a workshop will be held with internationally renowned experts. Partners will participate to the workshop and will contribute providing the national perspective on the feasibility of the data collection.



METHODS

- Panel of experts: 27 members (Associated & Collaborating partners)
- Consensus-seeking strategy
- Elaboration of an indicators set, its definition and calculation
- Refining method during several rounds
- Indicators properties: defined and categorized
- Qualitative analysis of such properties





RESULTS



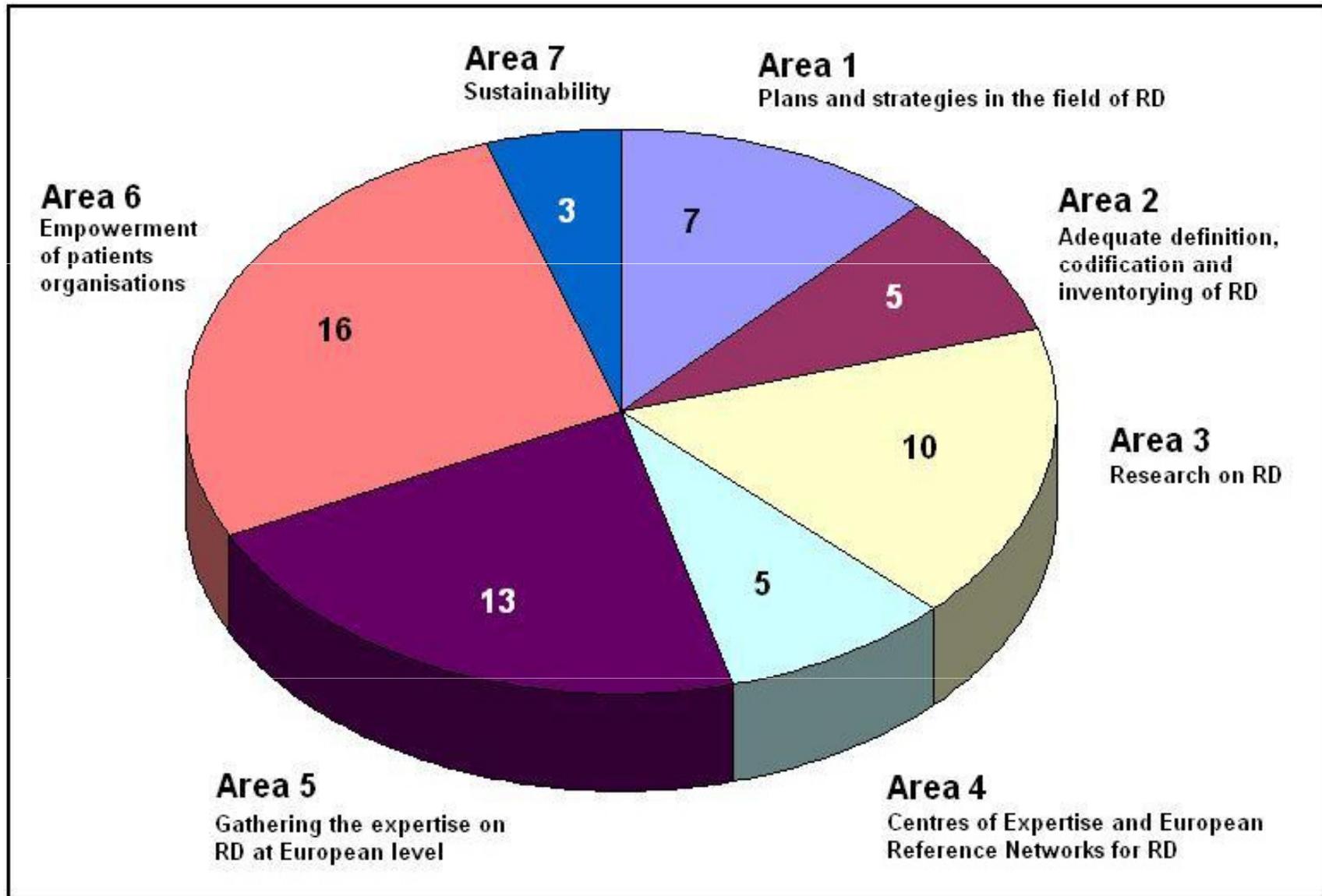
- 59 indicators' set
- 7 areas (Council Recommendation)
- Related with:
 - 14 aims
 - 27 actions

EUROPLAN Recommendations

reflect the EU Council Recommendation structure:

- AREA 1. plans and strategies in the field of rare diseases**
- AREA 2. adequate definition, codification and inventorying**
- AREA 3. research on rare diseases**
- AREA 4. centres of expertise and European reference networks for rare diseases**
- AREA 5. gathering the expertise on rare diseases at European level**
- AREA 6. empowerment of patient organizations**
- AREA 7. sustainability**





Area 1.- Plans and strategies in the field of Rare Diseases

Area to be explored	Aims	Actions	Indicators	Type of indicator	Answers	
Plans and strategies in the field of Rare Diseases	To establish National/Regional plans and/or strategies on RD	Development of Regulations/Laws	1.1.	Existence of regulations/laws that support the creation and development of a RD plan	Process	<ul style="list-style-type: none"> • <i>Not existing, not clearly stated</i> • <i>Existing, clearly stated, partly implemented and enforced</i> • <i>Existing, clearly stated and substantially implemented and enforced</i>
			1.2.	National/regional (percentage of regions)	Process	<i>Index based on the number of regions with a plan divided by total number of regions. A national plan will account for this index equal 100%</i>
		Establishment of Coordination mechanisms	1.3.	Existence of a coordination mechanism	Process	<ul style="list-style-type: none"> • <i>Not existing, not clearly stated</i> • <i>Existing, clearly stated, partly implemented and enforced</i> • <i>Existing, clearly stated and substantially implemented and enforced</i>
			1.4.	Existence of an expert advisory committee	Process	<ul style="list-style-type: none"> • <i>Existing and meets regularly</i> • <i>Exists but partly functioning</i> • <i>Does not exist</i>
		Establishment of an external evaluation of the plan/strategy procedure	1.5.	Existence of an external evaluation body/procedure	Process	<ul style="list-style-type: none"> • <i>Number of meetings held by year</i>
		Degree of comprehensiveness	1.6.	Number of priority areas included in the plan	Process	<ul style="list-style-type: none"> • <i>Number ranging from 0 to 10</i>
		Establishing of a budget for developing the plan/strategy	1.7.	Budget of plan/strategy	Process	<ul style="list-style-type: none"> • <i>Overall budget allocated</i>



Indicators categorization

According to 3 criteria:

- Intrinsic properties
- Resources demand
- Decision making



Qualitative evaluation

16 properties

Understandability

Reliability

Validity

Consistency

Sensitivity

Specificity

Feasibility

Availability

Sustainability

Implementability

Workload demand

Timeliness

Applicability

Coherence

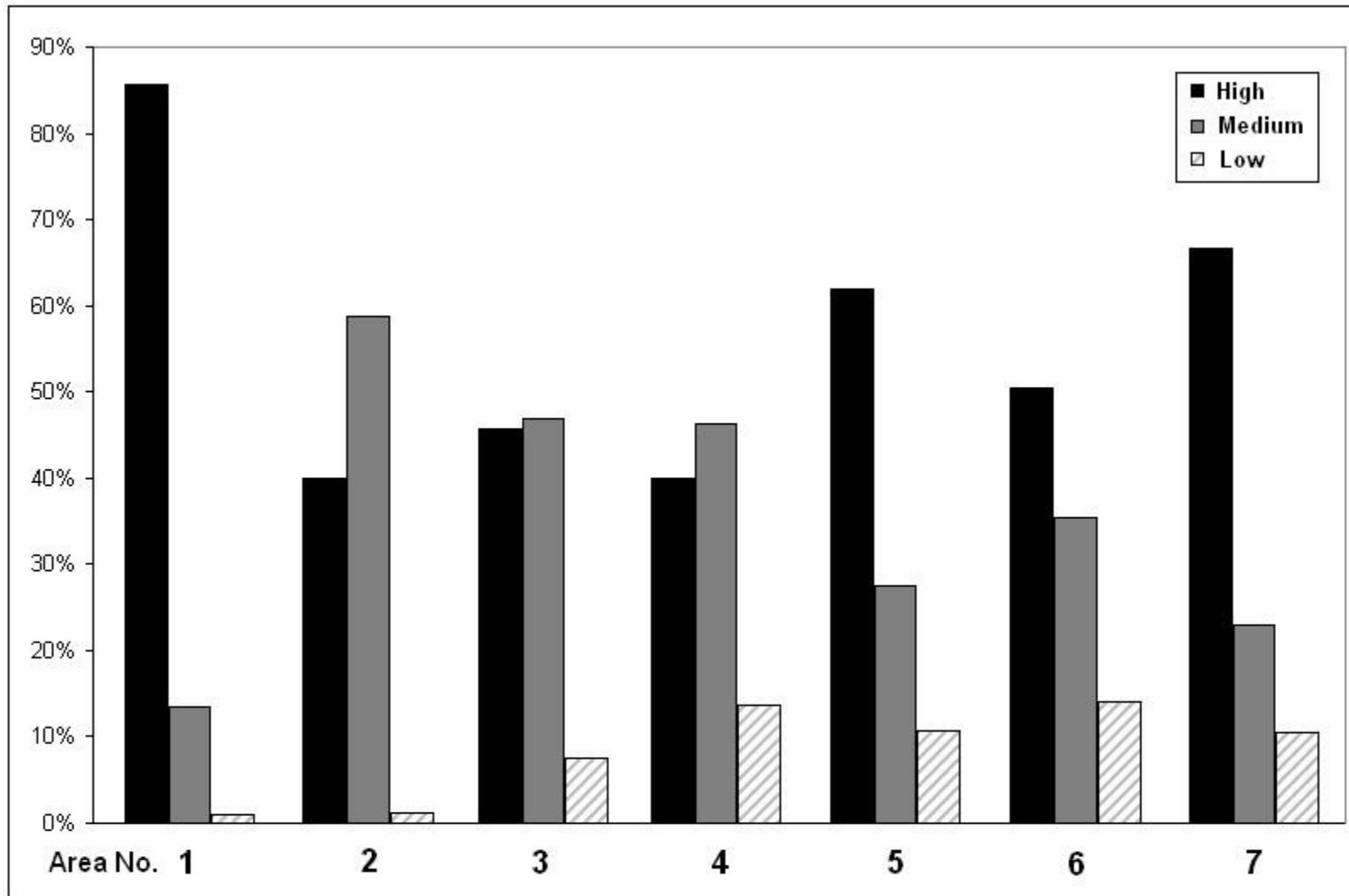
Comprehensiveness

Relevance.

High ■ Medium ■ Low ■ non-applicable

List of Indicators		Intrinsic properties						Resource demand					Decision making				
		Understandability	Reliability	Validity	Consistency	Sensitivity	Specificity	Feasibility	Availability	Sustainability	Implementability	Workload demand	Timeliness	Applicability	Coherence	Comprehensiveness	Relevance
4.1.	Existence of a policy for establishing centers of expertise at the national/regional level																
4.2.	Number of centres of expertise adhering to the policy defined in the country																
4.3.	Groups of rare diseases followed up in centres of expertise																
4.4.	Centres of expertise adhering to the standards defined by the Council Recommendations - paragraph d) of preamble																
4.5.	Participation of national or regional centres of expertise into European reference networks																





Evaluation score of indicators



CONCLUSIONS

This is the first experience in building an indicators' set for evaluating National and Regional Plans and Strategies on Rare Diseases.

EUROPLAN indicators' set could be an useful tool for monitoring National and Regional actions on Rare Diseases.



EUROPLAN Indicators Working Group

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EUROPLAN (2008-2011, 2012-2015)

is project co-funded by the EU Commission (DG-SANCO) to promote and implement National Plans or Strategies to tackle rare diseases, to share relevant experiences within Countries, linking national efforts with a common strategy at European level. This "double-level" approach ensures that progress is globally coherent and follows common orientations throughout Europe. [\[read more\]](#)

EUROPLAN 2012-2015

a three year project, embedded in the EUCERD Joint Action as Work Package 4, it is coordinated by the Italian National Institute of Health - Italian National Centre for Rare Diseases. [\[read more\]](#)



[Download the Europlan flyer](#)



[Click on the flag to see the actions of the country to tackle rare diseases](#)

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Thank you
very much

