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

EUCERD
Joint Action

Co-funded by the

European Commission

Executive Agency for Health and Consumers

Core Indicators for RD National Plans: the Selection of EURORDIS EUROPLAN Advisors

Yann Le Cam, Valentina Bottarelli

EURORDIS

EUROPLAN Workshop –

25 March 2013, Rome

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

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu

Selection of Core Indicators in EJA WP4

1. Delphi Method to identify core indicators involving all stakeholders and EUCERD members – ISS

2. Approach to identify core indicators for National Conferences – EURORDIS & National Alliances with national MoH representatives

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

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu

The starting point

The REPORT ON EUROPLAN INDICATORS

developed within EUROPLAN (2008-2011)

www.europlanproject.eu

59 Indicators to monitor implementation & impact of NP/NS

→ a good and comprehensive basis for EU MS

EUROPLAN

Draft version. Selecting indicators to evaluate the achievements of RD initiatives

EUROPLAN

European Project for Rare Diseases National Plans Development

European Project for Rare Diseases National Plans Development (EUROPLAN)

Selecting indicators to evaluate the achievements of RD initiatives

By WP5: Manuel Posada, Manuel Hens, Maria José Carroquino and EUROPLAN Working Group on Indicators

Rare Diseases Research Institute
Instituto de Salud Carlos III

Madrid March, 29th, 2010

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

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu

Towards Core Common Indicators

– **CORE Indicators** - To narrow down the list of nearly 60 Indicators, too difficult to collect systematically in all countries

– **COMMON Indicators** - To have a good vision across 27 MSs and in the long term

➔ Necessary to agree on a common set and commit to its collection

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu

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

Selection of Core Indicators by EURORDIS Advisors

Why?

- To identify core indicators in view of EUROPLAN National Conferences on RD National Plans/Strategies

- To "shape" the discussion on indicators during National Conferences

- To cross over with the ISS Delphi method

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

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu

Selection of Core Indicators by EURORDIS Advisors

An initial approach to the establishment of a set of core common indicators for RD National Plans

BASED ON

first-hand experience

of EUROPLAN Advisors and their MoH contacts

in RD policy implementation

Selection of Core Indicators by EURORDIS Advisors

- Led by EURORDIS within EJA WP4 Part B

- **Working Group:**
  - EURORDIS-EUROPLAN Advisors, with:
    - their respective National Alliances and
    - MoH Rep. involved in National RD Plan / Strategy
  - EURORDIS Staff

EURORDIS – EUROPLAN Advisors

EURORDIS Advisors	Country	National Alliances of Rare Diseases
Avril Daly	Ireland	GRDO; Genetic and Rare Disorders Organisation
Christel Nourissier	France	French Alliance for Rare Diseases
Dorica Dan	Romania	RONARD; Romanian Alliance for Rare Diseases
Lene Jensen	Denmark	RDD; Rare Disorders Denmark
Lily Cannon	Cyprus	CARD; Cyprus Alliance for Rare Disorders
Maria Gardsäter	Sweden	Rare diseases Sweden, Sällsynta Diagnoser
Melissa Hillier	United Kingdom	Rare Disease UK
Oleg Kvlividze	Georgia	GeRaD; Georgian Foundation for Genetic and Rare Diseases
Simona Bellagambi	Italy	UNIAMO; Italian Federation for Rare Diseases
Vlasta Zmazek	Croatia	Croatian Alliance for Rare Diseases

The process

A) national level

- *Christel Nourissier* and *Alain Garcia* (France) tested the methodology.

- Later on all other Advisors & National Alliances:

1. Assessed the 59 EUROPLAN Indicators following the selected criteria and on the **basis of their direct experience with their NP/NS and RD policy**

2. Worked with their MoH contacts to perform the assessment

3. Submitted to EURORDIS the scores assigned to the 59 EUROPLAN indicators

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

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu

The process

In practice :

- 59 Indicators assessed

- 3 criteria used:

  - **Usefulness for Patients and Care Providers**

  - **Feasibility**

  - **Political Usefulness** (= usefulness for the policy makers to take decisions that should support the development of policies for rare diseases in the future)

- Each Indicator scored from 1 to 5 for each criterion

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu

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

The process
an example of national scores

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

List of Indicators		Feasibility					Usefulness for Patients and Care Providers					Political Usefulness					Overall score
		5	4	3	2	1	5	4	3	2	1	5	4	3	2	1	
1.1	Existence of Regulations /laws that support the creation and development of a RD plan		x				x					x					14
1.2	National / regional (percentage of regions)		x					x				x					13
1.3	Existence of a coordination mechanism		x					x				x					13
1.4	Existence of an expert advisory committee	x					x					x					15
1.5	Existence of an external evaluation body / procedure	x					x					x					15
1.6	Number of priority areas included in the plan	x							x					x			11
1.7	Budget of plan/strategy	x						x					x				13

The process

B) group level

The 10 EURORDIS-EUROPLAN Advisors:

4. Compiled the results with the highest scores highlighted

5. Held a **group discussion** to come up with a list of selected core Indicators: 12 February

6. Deadline for submission of further inputs: 15 February

7. Integration of last inputs

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

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu

The results
comparative table – all Advisors

List of Indicators			Advisors' Group Selection Feb 2013	EURORDIS	Alliance Maladies Rares	Rare Disease UK	RONARD	UNIAMO	CARD	Rare Disorders Denmark	GeRaD	RD Sweden
					FR	UK	RO	IT	CY	DK	GE	SE
Area 1 - Plans and strategies in the field of Rare Diseases												
1.1	Existence of Regulations/laws that support the creation and development of a RD plan		**X**		X	X	X	X	X	X	X	
1.2	National / regional (percentage of regions)			X		O	O					
1.3	Existence of a coordination mechanism						O		X			X
1.4	Existence of an expert advisory committee		**X**	X	X	X	X	X	O	X	X	X
1.5	Existence of an external evaluation body /procedure				X	X			X	X	X	X
1.6	Number of priority areas included in the plan								X			
1.7	Budget of plan/strategy		**X**	X	X		O	X	X	X	X	

X = score from 13,5 to 15 included   **O** = score from 12 to 13 included

The results

- The EURORDIS-EUROPLAN Advisors group selected a list of

    - **22 Core Indicators** ("must have") &

    - **6 Desirable Indicators** ("nice to have")

    on the basis of:

    - Highest scores (group results)

    - Group discussion

    - …keeping an eye at the preliminary results of the ISS Delphi method (EUCERD presentation)

- ➜ Additionally, it was proposed to **reword some Indicators**

Results Area 1

		Area 1 - Plans and strategies in the field of Rare Diseases	
1.1		Existence of Regulations/laws that support the creation and development of a RD plan	X
1.2		National / regional (percentage of regions)	
1.3		Existence of a coordination mechanism	
1.4		Existence of an expert advisory committee	X
1.5		Existence of an external evaluation body /procedure	
1.6		Number of priority areas included in the plan	
1.7		Budget of plan/strategy	X

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

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu

Results Area 2

		Area 2 - Adequate definition, codification and inventorying of rare diseases	
2.1		Adoption of the EC RD definition	
2.2		Type of classification used by the health care system	X
2.3		Developing policies for recognising RD by the care information systems	O
2.4		Registering activity	X
2.5		Number of diseases included	

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

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu

Results Area 3

		Area 3 – Research on Rare Diseases	
3.1		Existing of RD National/Regional research programmes	
3.2		RD research programme monitoring	**X**
3.3		Number of RD research projects approved by year (if possible yearly starting the year before plan commencement)	
3.4		Clinical trials funded by public bodies	
3.5		E-RARE joining	
3.6		Including public health and social research in the field of rare diseases	**X**

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

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu

Results Area 3

		Area 3 – Research on Rare Diseases (*part 2*)	
3.7		Research platforms and other infrastructures are also funded by the research programme	**X**
3.8		Number of young scientists recruited every year to work specifically on rare diseases	
3.9		There are specific public funds allocated for RD research	**X**
3.10		Funds specifically allocated for RD research actions /projects per year since the plan started	**X**

Results Area 4

		Area 4- Centres of Expertise and European Reference Networks for Rare Diseases	
4.1		Existence of a policy for establishing centres of expertise at the national/regional level	**X**
4.2		Number of centres of expertise adhering to the policy defined in the country	**X**
4.3		Groups of rare diseases followed up in centres of expertise	**O**
4.4		Centres of expertise adhering to the standards defined by the EUCERD Recommendations on Quality Criteria for Centres of Expertise	
4.5		Participation of national or regional centres of expertise into European reference networks	**X**

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

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu

Results Area 5

	Area 5 - Gathering the expertise on Rare Diseases at European level	
5.1	Existence of a comprehensive national and/or regional RD information system supported by the government	**X**
5.2	Help lines for professionals and patients	**X**
5.3	Clinical guidelines (number of -)	**X**
5.4	Number of such as activities promoted by the plan/strategy	
5.5	Number of diseases included in the neonatal screening programme	O
5.6	Number of diseases included in the neonatal screening programme properly assessed	O
5.7	Existence of a public directory/ies of both genetic and biochemical tests on Rare Diseases	
5.8	Proportion of laboratories having at least one diagnostic test validated by an external quality control	

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

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu

Results Area 5

	Area 5 - Gathering the expertise on Rare Diseases at European level (*part 2*)		
5.9	Number of ~~ODD~~ OMPs* which were granted a market authorisation by the EMA and are available ~~placed in the market~~ in the country (i.e. priced and reimbursed) - *orphan medicinal products*		**X**
5.10	~~Time from the placement in the market in the country to the positive decision for reimbursement by public funds~~	To merge 5.10 and 5.11 and reword: *"Time between the date of granting MA by the EMA and its actual date of the positive decision for reimbursement by public funds"*	
5.11	~~Time between the date of a ODD OMP* market authorisation by EMA and its actual date of placement in the market for the country~~		
5.12	~~* Number of ODD reimbursed 100% -~~	*To replace with reworded 5.9*	
5.13	Existence of a governmental programme for compassionate use for Rare Diseases		**X**
5.14	Existence of a government policy to monitor and support patients' access to off-label use of medicines for rare disease treatments	*Proposed new Indicator*	**O**

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

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu

Results Area 6

		Area 6 - Empowerment of patients	
6.1		Number of umbrella organisations specific on rare diseases	
6.2		Having a directory of RD Patients' organisations	
6.3		Number of patients' Associations	
6.4		Number of diseases covered by patients' associations	
6.5		Permanent and official patients' representatives in plan development, monitoring and assessment	X
6.6		Participation of patients' organisations in the development of RD research strategies	
6.7		Participation of patients organisations in the RD centres of expertise designation and evaluation	X
6.8		Number of umbrella organisations specific on rare diseases	

Results Area 6

	Area 6 – Empowerment of patients (*part 2*)		
6.9	~~Resource (funding) provided for supporting the activities performed by patient organisations~~ ~~Support to sustainable activities to empower patients, such as: Awareness raising, capacity building and training, exchange of information and best practices, networking, outreach to very isolated patients~~	** 6.8 and 6.9 should be merged and replaced with:* Resource (funding) provided for supporting the activities performed by patient organisations, such as: Awareness raising, capacity building and training, exchange of information and best practices, networking, outreach to very isolated patients	
6.10	Availability of Help line for RD		X
6.11	Existence of official programs supporting patients and families with disabilities		
6.12	Existence of an official directory of social resources for patients with disabilities		O

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

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu

Results Area 6

	Area 6 – Empowerment of patients		
6.13	~~Existence of national schemes promoting access of RD patients and their families to Respite Care services~~	*6.13, 6.14 & 6.16 should be merged into one:* Existence of national schemes supporting access of RD patients and families to Specialised Social Services for RD: Respite Care Services, Therapeutic Recreational Programmes, Resource Centres, Adapted Housing and other rehabilitation services	
6.14	~~Existence of public schemes supporting Therapeutic Recreational Programmes~~		
6.15	Existence of programmes to support integration of RD patients in their daily life		**X**
6.16	~~Existence of programmes to support rehabilitation of RD patients~~	*see above 6.13-6.14*	

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

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

www.europlanproject.eu

Results Area 7

		Area 7 - Sustainability of National plans	
7.1		Existing policy/decision to ensure long-term sustainability of the RD plan /strategy	
7.2		Amount of funds allocated for ensuring RD plan /strategy sustainability	X
7.3		Existing policy/decision to ensure the contribution to support RD European Infrastructures	

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

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu

Overall results - Core

1.1 - Existence of Regulations/Laws that support the creation and development of a RD plan

1.4 - Existence of an expert advisory committee

1.7 - Budget of plan/strategy

2.2 - Type of classification used by the health care system

2.4 – Registering activity

3.2 - RD research programme monitoring

3.6 - Including public health and social research in the field of rare diseases

3.7 - Research platforms and other infrastructures are also funded by the research programme

3.9 - There are specific public funds allocated for RD research

3.10 - Funds specifically allocated for RD research actions/projects per year since then plan started

4.1 - Existence of a policy for establishing centres of expertise at the national/regional level

4.2 - Number of centres of expertise adhering to the policy defined in the country

4.5 - Participation of national or regional centres of expertise into European reference networks

5.1 - Existence of a comprehensive national and/or regional RD information system supported by the govern.

5.2 - Help lines for professionals + 6.10 - Availability of Help line for RD

5.3 - Clinical guidelines

5.9 - Number of OMPs granted a market authorisation by EMA, available in the country (priced & reimbursed)

5.13 - Existence of a governmental program for compassionate use for Rare Diseases

6.5 - Permanent and official patients'representatives in plan development, monitoring and assessment

6.7 - Participation of patient organisations in RD centres of expertise designation and evaluation

6.15 - Existence of programmes to support integration of RD patients in their daily life

7.2 - Amount of funds allocated for ensuring RD plan /strategy sustainability

Overall results - Desirable

2.3 - Developing policies for recognising RD by the care information systems

4.3 - Groups of rare diseases followed up in centres of expertise

5.5 - Number of diseases included in the neonatal screening programme properly assessed

5.6 - Existence of a public directory/ies of both genetic and biochemical tests on Rare Diseases

5.14 (new) Existence of govern. policy to monitor & support patients access to off-label use of medicines for RD treatments

6.12 - Existence of an official directory of social resources for patients with disabilities

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

EUROPLAN II WORKSHOP "Key Indicators for National Plans"
25 March 2013, Rome, Italy

www.europlanproject.eu