EUCERD
Joint Action

EUROPLAN
European Project for Rare Diseases National Plans Development
Coordinated by the Italian National Centre for Rare Diseases
Italian National Institute of Health
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EURORDIS
Rare Diseases Europe

EUROPLAN NATIONAL CONFERENCES 2012-2015

CONTENT GUIDELINES FOR WORKSHOP 1 / THEME 1
METHODOLOGY, GOVERNANCE
AND MONITORING OF THE NATIONAL PLAN

Table of Contents

Annex (*separate document*) – Overall outcomes from Final Report & Synopsis of detailed outcomes of 2010 EUROPLAN National Conferences on Governance and Monitoring of a National Plan (NP)

A. How to read and use these Content Guidelines

The EUROPLAN Content Guidelines cover 6 main Themes. For each Theme, these Content Guidelines cover all the core topics to be addressed in the Workshop dedicated to that Theme. These Guidelines include:

1st column – RESOURCES	**2nd column - TOPICS FOR DISCUSSION**
This column includes the background documents and relevant material that should be referred to in preparation for the discussion. They mainly include: • Specific articles of the EU Council Recommendation on an action in the field of rare diseases; • Specific recommendations from the "EUROPLAN Recommendations"; • Specific EUCERD Core Indicators; • Specific EUROPLAN Indicators. **NB: Full documents of the sources referenced above can be found in Section C**	**The topics for discussion are questions formulated to stimulate the discussion within the Workshop**. The conference organisers, with the help of their Advisor, will **select those questions that are relevant for the discussion in their countries**. As such, **not all listed questions need to be addressed in a mandatory way**. They rather represent a "menu" from which to pick the questions that address the most relevant topics in the country, having considered the level of advancement of the national policy on rare diseases in the country.

B. Guidelines for discussion for Workshop 1 – Theme Methodology, Governance and Monitoring of the National Plan

RESOURCES	TOPICS for DISCUSSIONS
B.1 Mapping policies and resources	
EUROPLAN Recommendations R 1.4 A situation analysis is carried out including: - An inventory of existing healthcare resources, services, clinical and basic research activity and policies directly addressing rare diseases as well as those from which rare disease patients may benefit. - Unfulfilled needs of patients are assessed. - Available resources for improving health and social care of people affected by rare diseases at national level are evaluated. - European collaboration and the European documents in the field of rare diseases are taken into account in the development of the National Plan or Strategy. Examples from EUROPLAN Recommendations (page 18): • MEASURES TO IMPROVE THE HEALTH SITUATION OF PERSONS WITH RARE DISEASES IN GERMANY". Study by the German Federal Ministry of Health (August 2009)	• What is the level of **awareness on RD** in your country (epidemiologic figures, dimension of the problem, etc.)? More precisely, what level of public awareness, awareness among the medical profession, policy makers etc. does exist? • Is an inventory being made, or a report, of the existing resources and actions on RD (or of which RD patients can benefit) in the national health care and social system? • Are the **unmet needs** of RD patients being evaluated? • What EU relevant documents are taken into account in view of the development of the national plan (NP)?
B.2 Development of a National Plan /Strategy	
Council Recommendation on RD 1. Establish and implement plans or strategies for rare diseases at the appropriate level or explore appropriate measures for rare diseases in other public health strategies, in order to aim to ensure that patients with rare diseases have access to high-quality care, including diagnostics, treatments, habilitation	• In your country, is there a legal/policy framework in the form of a national plan or strategy created to address the health care and social needs of patients with RD with specific actions? • If <u>**YES**</u> or if it is in the process of being developed:

for those living with the disease and, if possible, effective orphan drugs, and in particular: (a) elaborate and adopt a plan or strategy as soon as possible, preferably by the end of 2013 at the latest, aimed at guiding and structuring relevant actions in the field of rare diseases within the framework of their health and social systems; b) take action to integrate current and future initiatives at local, regional and national levels into their plans or strategies for a comprehensive approach;	○ What are the next steps (monitoring, revision, peer review, etc)? ○ **What key actions would this workshop recommend to be undertaken before the NP comes to its end?** • If a National Plan or strategy has already been adopted, is an evaluation and discussion of its outcomes performed at regular intervals with patient organisations? Are the outcomes taken into consideration to influence future developments? • If <u>**NO** National Plan or Strategy has already been adopted</u>: ○ How is your country preparing to meet the 2013 deadline? What are the main obstacles to the development of a NP? ○ What are the steps being undertaken by relevant authorities towards the developing a NP? ○ **What (realistic) list of actions would this Workshop draft to take home to move forward the development of a NP or strategy in the field of RD?**
	NB The following sub-themes and questions are both relevant for countries where a NP exists or is being prepared, and for those where the NP is not yet being developed, as they may serve as checklist of topics that will need to be addressed in preparation of a NP.
B.3 Structure of a National Plan /Strategy	
Council Recommendation on RD 1. […] (c) define a limited number of priority actions within their plans or strategies, with objectives and follow-up mechanisms; **EUROPLAN Recommendations** R 1.5 The National Plan or Strategy is elaborated with well described objectives and actions. The general objectives of a National Plan or Strategy are based on the general overarching values of universality, access to good quality care, equity and solidarity. R 1.6 The policy decisions of the National Plan or Strategy are integrated i.e. structured maximizing synergies and avoiding duplications with existing functions and structures of the health care system of the country.	• Is the NP created in the form of a written document with a clear structure? • What general and specific objectives or **priority areas** have been / can be identified in your country? • Are the specific actions envisaged in the NP accompanied by **clear deliverables** and **measurable results**? • Is there a timeline for the achievement of priority actions with specific deliverables? • How does the NP guide and structure relevant actions in the field of RD within the existing health and social systems? Assess the degree of **integration** (whether and how it maximises synergies of functions and structures of the

R 1.7 The policy decisions of the National Plan or Strategy are comprehensive, addressing not only health care needs, but also social needs. Examples from the EUROPLAN Recommendations (page 20 to 21): • Structure and priority areas of National Plans or Strategies: case of France, Bulgaria, Greece, Portugal, Spain.	healthcare system) and **comprehensiveness** of the NP (i.e. if it addresses **health care as well as social needs**). • Does the NP integrate current and future initiatives at local, regional and national level? How does the NP respond to the regionalised healthcare systems (if relevant)?
B.4 Governance of a National Plan	
EUROPLAN Recommendations R 1.3 A mechanism (e.g. interdisciplinary panel, committee) including relevant stakeholders is established to assist the development and implementation of the National Plan or Strategy. Examples from the EUROPLAN Recommendations (page 16): • Examples of Steering Committees in Belgium, Netherlands, Portugal, Poland and Hungary	**STEERING COMMITTEE or other GOVERNANCE MECHANISMS** • Is there a Steering Committee (SC) (or otherwise named e.g. coordination committee, interdisciplinary panel, etc.) in charge of governing the implementation of the NP? • Do RD stakeholders participate in the governance of the NP – healthcare, research and social affairs authorities, patients, healthcare professionals, academics, health insurances, representatives of the industry, etc.? Do these stakeholders cover all areas of expertise relevant to the NP, such as pharmacology, regulatory, clinical, health and social services, epidemiology, administrative policies, etc.? • Is the participation of patients envisaged at all phases of the NP so to ensure that patients are actors in the decisions on measures directed to them? • Does the SC adopt “Statutes” or “Rules of Procedures” with respect to its role, the role of its members and its activities? • As the experience of the 1st French National Plan Evaluation suggests, is it recommended to have a minimum mandatory number of meetings per year of the members belonging to the SC. Does the SC meet regularly? • Are there working groups preparing the meetings of the Steering Committee? Do they cover all priority areas defined?

	TRANSPARENCY of the governance structure • Are appointments to the SC made in a transparent, fair and inclusive manner? • Are the targeted audience/ stakeholders (including the patients) well informed in advance of the establishment of a SC? Were open calls for expression of interests launched to appoint its members?
B.5 Dissemination and communication on the National Plan	
EUROPLAN Recommendations R 1.10 Information on the National Plan or Strategy is made accessible to the public and it is disseminated to patients' groups, health professionals' societies, general public and media, making the plan known also at European level.	The dissemination of information on the NP (both at development and adoption stage) in the country should also be part of the strategy to ensure an effective impact on the behaviour of patients with rare diseases and on the health system performance. • Is the process around formulating and implementing a National Plan open and transparent? Is it possible for the general public to follow the process? • Is the **NP publicised to the general public**? Is it made public in all its parts, including all specific actions, timelines and results of its evaluation when performed? • What awareness measures are planned or need to be planned? • What awareness measures targeted to specific audiences (healthcare professionals, patients, local and regional authorities, etc.) need to be developed? • What communication actions are envisaged or are needed in relation to the different phases of the life cycle of the NP (adoption/ implementation/ evaluation, etc.)?

B.6 Monitoring and evaluation of the National Plan	
EUROPLAN Recommendations R 1.12 The National Plan or Strategy has a duration of three to five years. An intermediate deadline is established, after which, an evaluation process is undertaken and corrective measures are adopted. For longer time scales or no defined time frame, a 2- to 3-year cyclic evaluation and adaptation process is adopted, if needed. R 1.13 The National Plan or Strategy is monitored and assessed at regular intervals using, as far as possible, EUROPLAN indicators. R 1.14 The implementation of the actions and their achievements are assessed. R 1.15 The most appropriate evaluation of a National Plan or Strategy is by an external body and takes into account also patients' and citizens' views. Patients' needs are assessed at the beginning and the end of the plan implementation using the same methodology. Evaluation Reports are made public. Examples from the EUROPLAN Recommendations (page 24 to 26): • MAIN OUTCOMES OF THE FRENCH NATIONAL PLAN 2004-2008 • EVALUATION OF PATIENTS' EXPERIENCE AND SATISFACTION **Other resources** • **EUROPLAN Indicators:** In the framework of the EUROPLAN project a list of indicators has been prepared, based on the main areas and on some possible actions in such areas. The indicators have been chosen to be able to monitor the actions recommended and to be adaptable to the different national situations. A specific EUROPLAN Document deals with the list of indicators: http://www.europlanproject.eu/_newsite_986987/_down/results/2008-2011_3.EUROPLANIndicators.pdf **Please note that within EUROPLAN II a selection of Indicators based on the EUROPLAN list will be made and distributed to conference organisers as soon as they are available (early 2013).**	Assess the **monitoring mechanisms** to evaluate the NP: • What type of Indicators is used to monitor the implementation of a NP? - The EUCERD 21 Core Indicators are recommended to be collected each year; - Moreover, the EUROPLAN Indicators can serve as a good basis to monitor the plan; - Some additional Indicators could also be developed to monitor specific national measures outlined in the NP. • Is the evaluation of the NP ensured by an external body, i.e. different from the SC? • Does the evaluation include also the collection of opinions and satisfaction surveys addressed to patients? And to healthcare professionals?

B.7 Sustainability of the National Plan	
Council Recommendation 20. Together with the Commission, aim to ensure, through appropriate funding and cooperation mechanisms, the long-term sustainability of infrastructures developed in the field of information, research and healthcare for rare diseases. **EUROPLAN Recommendations** R 1.9 Appropriate resources are allocated to ensure the feasibility of the actions in the planned time. […] R 1.11 Measures are taken to ensure the sustainability, transfer and integration of the actions foreseen by the national plan or strategy into the general health system of the country. **Other resources:** • **WHO Europe Report “How health systems can address health inequities through improved use of Structural Funds”:** http://www.euro.who.int/__data/assets/pdf_file/0007/129868/e94606.pdf	**BUDGET for a NP** • **Is there a specific budget attached to the NP? Assess whether there is a budget or the NP consists of ‘good wishes’ without a significant budgetary commitment.** • Are there cost estimates for each action? Is each action funded with sufficient/ adequate financial resources? Assess the allocation of the budget by action. • Altogether, is the overall budget allocation such as to ensure the long-term sustainability of the actions planned? **FUNDING SOURCES** • What are the main sources of funding for the NP? • Analyse and discuss alternative sources of funding, including in particular opportunities offered by **Structural Funds** for the period 2014-2020.

C. Background Documents

C1. Council Recommendation of 8 June 2009 on an action in the field of rare diseases (2009/C 151/02)

Whereas
[…]
9) In order to improve the coordination and coherence of national, regional and local initiatives addressing rare diseases and cooperation between research centres,
relevant national actions in the field of rare diseases could be integrated into plans or strategies for rare diseases.
[…]
22) The development of research and healthcare infrastructures in the field of rare diseases requires long-lasting projects and therefore an appropriate financial effort to ensure their sustainability in the long term.

(The Council of the EU) hereby recommends that Member States:

“I. PLANS AND STRATEGIES IN THE FIELD OF RARE DISEASES
1. Establish and implement plans or strategies for rare diseases at the appropriate level or explore appropriate measures for rare diseases in other public health strategies, in order to aim to ensure that patients with rare diseases have access to high-quality care, including diagnostics, treatments, habilitation for those living with the disease and, if possible, effective orphan drugs, and in particular:
(a) elaborate and adopt a plan or strategy as soon as possible, preferably by the end of 2013 at the latest, aimed at guiding and structuring relevant actions in the field of rare diseases within the framework of their health and social systems;
(b) take action to integrate current and future initiatives at local, regional and national levels into their plans or strategies for a comprehensive approach;
(c) define a limited number of priority actions within their plans or strategies, with objectives and follow-up mechanisms;
(d) take note of the development of guidelines and recommendations for the elaboration of national action for rare diseases by relevant authorities at national level in the framework of the ongoing European project for rare diseases national plans development (EUROPLAN) selected for funding over the period 2008-2011 in the first programme of Community action in the field of public health.”

http://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:C:2009:151:0007:0010:EN:PDF

C2. EUROPLAN Recommendations

R 1.1 Patients with rare diseases deserve dedicated public health policies to meet their specific needs.
R 1.2 Initiatives are taken to raise awareness about the dimension of the problem and to create joint responsibility.
R 1.3 A mechanism (e.g. interdisciplinary panel, committee) including relevant stakeholders is established to assist the development and implementation of the National Plan or Strategy.
R 1.4 A situation analysis is carried out including:

- An inventory of existing healthcare resources, services, clinical and basic research activity and policies directly addressing rare diseases as well as those from which rare disease patients may benefit.
- Unfulfilled needs of patients are assessed.
- Available resources for improving health and social care of people affected by rare diseases at national level are evaluated.
- European collaboration and the European documents in the field of rare diseases are taken into account in the development of the National Plan or Strategy.

R 1.5 The National Plan or Strategy is elaborated with well described objectives and actions. The general objectives of a National Plan or Strategy are based on the general overarching values of universality, access to good quality care, equity and solidarity.
R 1.6 The policy decisions of the National Plan or Strategy are integrated i.e. structured maximizing synergies and avoiding duplications with existing functions and structures of the health care system of the country.
R 1.7 The policy decisions of the National Plan or Strategy are comprehensive, addressing not only health care needs, but also social needs.
R 1.8 Specific areas for action are indicated, with priority given to those of the Council Recommendations, taking into account the major needs identified in the member state.
R 1.9 Appropriate resources are allocated to ensure the feasibility of the actions in the planned time.
R 1.10 Information on the National Plan or Strategy is made accessible to the public and it is disseminated to patients' groups, health professionals' societies, general public and media, making the plan known also at European level.
R 1.11 Measures are taken to ensure the sustainability, transfer and integration of the actions foreseen by the national plan or strategy into the general health system of the country.
R 1.12 The National Plan or Strategy has a duration of three to five years. An intermediate deadline is established, after which, an evaluation process is undertaken and corrective measures are adopted. For longer time scales or no defined time frame, a 2- to 3-year cyclic evaluation and adaptation process is adopted, if needed.
R 1.13 The National Plan or Strategy is monitored and assessed at regular intervals using, as far as possible, EUROPLAN indicators.
R 1.14 The implementation of the actions and their achievements are assessed.
R 1.15 The most appropriate evaluation of a National Plan or Strategy is by an external body and takes into account also patients' and citizens' views. Patients' needs are assessed at the beginning and the end of the plan implementation using the same methodology. Evaluation Reports are made public.

http://www.europlanproject.eu/_newsite_986987/_down/results/2008-2011_2.EUROPLANGuidance.pdf

C3. EUCERD Core Indicators

http://www.eucerd.eu/wp-content/uploads/2013/06/EUCERD_Recommendations_Indicators_adopted.pdf

NB: Out of the 21 EUCERD core indicators, please find below selected indicators for this specific theme.

1. Existence of regulations/laws, or equivalent official national decisions that support the establishment and development of a Rare Diseases (RD) plan
2. Existence of a RD advisory committee
3. Permanent and official patients’ representation in plan development, monitoring and assessment
18. Existence of a policy/decision to ensure long-term sustainability of the RD plan/strategy
19. Amount of public funds allocated to the RD plan/strategy

Core Indicators – Definitions and associated answers

INDICATOR	**AREA OF COUNCIL REC.** (2009/ C151/02)	**INDICATOR DESCRIPTION**	**TYPE OF INDICATOR**	***SHORT* ANSWER**	***DETAILED* ANSWER** *(multiple answers are possible, if needed)*
BACKGROUND INDICATORS *(PREPARATION OF THE PLAN/STRATEGY)*					
1. Existence of Regulations/Laws, or equivalent official national decisions that support the establishment and development of a Rare Diseases (RD) plan	1	This Indicator refers to the fact that National Plans/Strategies for Rare Diseases should be devised/regulated at national level in accordance with the Council Recommendation on RD, relevant Recommendations of the EUCERD e.g. those on Centres of Expertise and European Reference Networks, as well as relevant legislation (Regulation EC n° 141/2000 on Orphan Medicinal Products, Directive EU/2011/24 on Cross Border Healthcare, etc.). The National Plan or Strategy is adopted via binding legislative acts, the exact nature or level of which may vary (regulation, laws, or other types of decisions). They may be established at the appropriate level of governance (federal vs. federated state level) depending on the country’s system of government. It is therefore embedded in a legislative or operational framework	Process	YES	**YES**, existing, fully embedded in a regulation/law/official national decision
					YES, existing, partly embedded
				In progress /in development	
				NO	

2. Existence of a RD advisory committee	1	The Expert Advisory Committee refers to the existence of a coordination mechanism that oversees the development and implementation of the National Plan/Strategy for Rare Diseases. This body is composed of representatives of all relevant stakeholders, including patient representatives, national government, industry, treating physicians, payers, academia, etc.	Process	YES	**YES**, exists and meets regularly and includes all relevant stakeholders
					YES, exists but partly functioning and includes all relevant stakeholders
					YES, exists and meets regularly but does not include all relevant stakeholders
					YES, exists but partly functioning and does not include all relevant stakeholders
				NO	
3. Permanent and official patients' representation in plan development, monitoring and assessment	6	Patients are officially represented at all stages of plan development and governance, including its monitoring and evaluation.	Process	YES	**YES**, at all stages
					YES, but only as observers
					YES, but only consulted before the final document is approved
				NO	
FINANCIAL SUPPORT INDICATORS *(IMPLEMENTATION OF THE PLAN/STRATEGY)*					
18. Existence of a policy/decision to ensure long-term funding and/or sustainability of the measures in the RD plan/strategy	7	The indicator verifies whether the financial commitment for rare disease care and treatment is clearly defined in a budget decision that supports the implementation of the National Plan/Strategy actions.	Process	YES	**YES**, a policy/decision to ensure long-term sustainability
					YES a budget exists for the plan
				In progress /in development	
				NO	

19. Amount of public funds allocated to the RD plan/strategy	7	The indicator is the overall budget (in EUR) allocated per year to the National Plan/Strategy (excluding reimbursement of care and cost of standard care, excluding cost of orphan drugs). As with the previous indicator, this indicator aims to ensure that RD actions include appropriate provisions to ensure their sustainability over time. Efficient and effective actions for rare diseases depend on integrating scarce and scattered resources both nationally and within a common European effort.	Outcomes	*Number*	Value
					Value / million inhabitants
					Value available partially: only for funds allocated exclusively to National Plan (N/A for funds allocated in the general budget)
					N/A: it is incorporated in the general budget
					Value available partially: only for funds allocated exclusively to National Plan (N/A for funds allocated in the general budget)
					N/A: it is incorporated in the general research funds

C4. EUROPLAN Indicators

http://www.europlanproject.eu/_newsite_986989/Resources/docs/2008-2011_3.EuroplanIndicators.pdf

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	• *Not existing, not clearly stated* • *Existing, clearly stated, partly implemented and enforced* • *Existing, clearly stated and substantially implemented and enforced*
			1.2.	**National/regional (percentage of regions)**	Process	*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%*
		Establishment of Coordination mechanisms	**1.3.**	**Existence of a coordination mechanism**	Process	• *Not existing, not clearly stated* • *Existing, clearly stated, partly implemented and enforced* • *Existing, clearly stated and substantially implemented and enforced*
			1.4.	**Existence of an expert advisory committee**	Process	• *Exiting and meets regularly* • *Exists but partly functioning* • *Does not exist*
		Establishment of an external evaluation of the plan/strategy procedure	**1.5.**	**Existence of an external evaluation body/procedure**	Process	• *Number of meetings held by year*
		Degree of comprehensiveness	**1.6.**	**Number of priority areas included in the plan**	Process	• *Number ranging from 0 to 10*
		Establishing of a budget for developing the plan/strategy	**1.7.**	**Budget of plan/strategy**	Process	• *Overall budget allocated*