



EUROPLAN PROJECT

NATIONAL CONFERENCE ON RARE DISEASES

WORKSHOP

METHODOLOGY AND GOVERNANCE OF A NATIONAL PLAN



**RELEVANT EXTRACT FROM THE
EUROPEAN COUNCIL RECOMMENDATIONS
ON ACTIONS IN THE FIELD OF RARE DISEASES**

LUXEMBOURG, 9 June 2009

COUNCIL RECOMMENDATIONS

“HEREBY RECOMMENDS that Member States:

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:

COUNCIL RECOMMENDATIONS

- **(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;**

COUNCIL RECOMMENDATIONS

- **(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.”**



**RELEVANT EXTRACT FROM THE
SPECIFIC EUROPLAN RECOMMENDATIONS
FOR THE DEVELOPMENT OF NATIONAL PLANS
FOR RARE DISEASES**

EUROPLAN RECOMMENDATIONS

- R 1.1 Patients with rare diseases deserve dedicated public health policies to meet their specific needs.
- R1.2 Initiatives are taken to raise awareness about the dimension of the problem and create joint responsibility.
- R1.3 A mechanism (e.g. interdisciplinary panel, committee) including relevant stakeholders is established to assist the development and implementation of the National Plan or Strategy.

EUROPLAN RECOMMENDATIONS

- R1.4 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.

EUROPLAN RECOMMENDATIONS

- R1.5 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.
- R1.6 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.
- R1.7 The policy decisions of the National Plan or Strategy are comprehensive, addressing not only health care needs, but also social needs.
- R1.8 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.

EUROPLAN RECOMMENDATIONS

- R1.9 Appropriate resources are allocated to ensure the feasibility of the actions in the planned time.
- R1.10 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.
- R1.11 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.
- R1.12 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.

EUROPLAN RECOMMENDATIONS

- R1.13 The National Plan or Strategy is monitored and assessed at regular intervals using, as far as possible, EUROPLAN indicators.
- R1.14 The implementation of the actions and their achievements are assessed.
- R1.15 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.



**RELEVANT EXTRACT FROM THE
EUROPLAN INDICATORS
TO EVALUATE THE ACHIEVEMENTS OF RD INITIATIVES**

EUROPLAN INDICATORS

ACTIONS	INDICATORS	TYPE	ANSWERS
Development of Regulations/ Laws	Existence of Regulations /laws that support the creation and development of a RD plan	Process	<p>Not existing, not clearly stated</p> <p>Existing, clearly stated, partly implemented and enforced</p> <p>Existing, clearly stated and substantially implemented and enforced</p>
	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	Existence of a coordination mechanism	Process	<p>Not existing, not clearly stated</p> <p>Existing, clearly stated, partly implemented and enforced</p> <p>Existing, clearly stated and substantially implemented and enforced</p>
	Existence of an expert advisory committee	Process	<p>Existing and meets regularly</p> <p>Exists but partly functioning</p> <p>Does not exist</p>

EUROPLAN INDICATORS

ACTIONS	INDICATORS	TYPE	ANSWERS
Establishment of an external evaluation of the plan/strategy Procedure	Existence of an external evaluation body / procedure	Process	Number of meetings held by year
Degree of comprehensiveness	Number of priority areas included	Process	Number ranging from 0 to 10
Establishing of a budget for developing the plan/strategy	Budget of plan/strategy	Process	Overall budget allocated



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FURTHER GUIDELINES FOR DISCUSSION

MAPPING EXERCISE BEFORE DEVELOPING A NP

- Is there an awareness of the situation of RD in your country (epidemiologic figures, dimension of the problem)?
- 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?

GOVERNANCE OF A NATIONAL PLAN

- Is there a Steering Committee (SC), or coordination committee, governing panel... governing the implementation of the Plan?
- If there is no Steering Committee or Coordination Committee yet in place, how would you perceive the implementation of such Steering Committee /Coordination Committee?
- Do all stakeholders participate in the governance of the NP – healthcare authorities, patients, healthcare professionals, academics, 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 to all phases of the NP so to ensure that patients are actors in the decision on health care measures directed to them?

GOVERNANCE OF A NATIONAL PLAN

- Does the SC meet regularly?
- Does the SC write a status for their activities and the responsibilities of its members?
- Does the SC write a regular (yearly) report on the achievement of the objectives of the NP and deliverables?
- Is the NP made public in the general content and specific actions?

DEVELOPMENT AND STRUCTURE OF A NP

- Is there in your country 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? What are the steps to be taken?
- Is the NP integrated and comprehensive so to respond to all patients' needs?
- 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?

MONITORING THE NATIONAL PLAN

- Is there a monitoring system for the NP?
- What type of indicators is used to monitor its implementation? Are the EUROPLAN indicators used as a basis for monitoring and evaluating the actions of the NP?
- Is the evaluation of the Plan 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?

SUSTAINABILITY OF THE NATIONAL PLAN

- Is there a specific budget attached to the NP? Does it ensure the long term sustainability of its actions?
- What are the main sources of funding of the National Plan?
- Are there specific budget provisions accompanying specific actions in certain priority areas e.g. orphan drugs, CoE, diagnosis, research, etc.?



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PROPOSALS FROM THE AUDIENCE