



**GOVERNANCE,
MONITORING
OF A NATIONAL PLAN /
STRATEGY
FOR RARE DISEASES**




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- Relevant extracts from the **RECOMMENDATION of the EUCERD** - European Union Committee of Experts on Rare Diseases – on ‘**Core Indicators**’ for planning, implementing and monitoring national Rare Disease Plan or Strategy.

The EUCERD brought together the 28 EU Member States plus Norway, Iceland and Switzerland, and stakeholders from patients’ organisations, academia and industry.

- Relevant extracts from the **RECOMMENDATION of EUROPLAN** – EU co-funded project aimed at developing and implementing national Rare Disease Plan or Strategy.
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Relevant extracts from the
RECOMMENDATION
of the EU COUNCIL
on an action in the field of rare diseases
(2009/C 151/02)
8 June 2009

EU COUNCIL RECOMMENDATION

“HEREBY RECOMMENDS that Member States:

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;

EU COUNCIL RECOMMENDATION

- (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 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 extracts from the
EUCERD CORE INDICATORS for
RD National Plans / Strategies



EUCERD CORE INDICATORS

N°1. Existence of regulations/laws, or equivalent official national decisions that support the establishment and development of a Rare Diseases (RD) plan

Short Definition =
RD Plan/ Strategy adopted via binding legislative acts.
Embedded in a legislative or operational framework

N°2. Existence of a RD advisory committee

Multi-stakeholders body that oversees and monitors
the development of the plan / strategy

N°3. Permanent and official patients' representation in plan development, monitoring and assessment

Patients are represented at all stages of plan development
and governance, including its monitoring and assessment

EUCERD CORE INDICATORS

N°18. Existence of a policy/decision to ensure long term sustainability of the RD plan/strategy

Short Definition =
Financial commitment for RD care and treatment is clearly defined in the plan /strategy budget

N°19. Amount of public funds allocated to the RD plan/strategy

Overall budget (in EUR) allocated per year. Ensure RD actions include appropriate provisions for their sustainability over time.

Relevant extracts from the
EUROPLAN RECOMMENDATIONS
**for the development of RD National Plans/
Strategies**



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 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.

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.

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.
- 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.

EUROPLAN RECOMMENDATIONS

- 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.

EUROPLAN RECOMMENDATIONS

- 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.

Relevant extracts from the
EUROPLAN INDICATORS
**for the development of RD National Plans/
Strategies**



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	<input type="checkbox"/> Not existing, not clearly stated <input type="checkbox"/> Existing, clearly stated, partly implemented and enforced <input type="checkbox"/> Existing, clearly stated and substantially implemented and enforced
	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	<input type="checkbox"/> Not existing, not clearly stated <input type="checkbox"/> Existing, clearly stated, partly implemented and enforced <input type="checkbox"/> Existing, clearly stated and substantially implemented and enforced
	Existence of an expert advisory committee	Process	<input type="checkbox"/> Existing and meets regularly <input type="checkbox"/> Exists but partly functioning <input type="checkbox"/> Does not exist

EUROPLAN INDICATORS

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