

**EUCERD Joint Action / Work Package 4
EUROPLAN National Conference**

**Workshop Theme 1
METHODOLOGY, GOVERNANCE and
MONITORING of NATIONAL PLANS**

Relevant extracts from the

COUNCIL RECOMMENDATION

on an action in the field of rare diseases

(2009/C 151/02)

8 June 2009

COUNCIL RECOMMENDATION

“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:

- (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;

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 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.”

EUCERD RECOMMENDATIONS ON CORE INDICATORS FOR RD NATIONAL PLANS / STRATEGIES

Preparation and Implementation of the Plan / Strategy

EUCERD Core Indicators, full version:

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

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**

Core Indicators

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

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.

GUIDELINES FOR DISCUSSION

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1. Mapping Policies and Resources

- 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?

1. Mapping Policies and Resources

- 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)?

2. Development of a National Plan (NP) /Strategy

- Is there a legal/policy framework in the form of a NP or strategy created to address the health care and social needs of patients with RD with specific actions?

If **YES** or in the process of being developed:

- 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?**

2. Development of a National Plan (NP) /Strategy

- If a NP 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?

2. Development of a National Plan (NP) /Strategy

- If **NO** NP or strategy has already been adopted:
 - 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 recommend to move forward the development of a NP / strategy in the field of RD?**

3. Structure of a National Plan /Strategy

- 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?

3. Structure of a National Plan /Strategy

- How does the NP guide and structure relevant actions in the field of RD within the existing health care and social systems?
- Assess the degree of **integration** (how it maximises synergies of functions and structures of the healthcare system) and **comprehensiveness** of the NP (i.e. 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)?

4. Governance of a National Plan

STEERING COMMITTEE (SC) or other GOVERNANCE MECHANISMS

- Is there a SC 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.?

4. Governance of a National Plan

STEERING COMMITTEE (SC) or other GOVERNANCE MECHANISMS

- Is the participation of patients envisaged at all phases of the NP so that they can decide 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?
- Does the SC meet regularly? (as recommended in the evaluation of the 1st French NP)
- Are there working groups preparing the meetings of the Steering Committee? Do they cover all priority areas defined?

4. Governance of a National Plan

TRANSPARENCY of the governance structure

- Are appointments to the SC made in transparent, fair and inclusive manner?
- Are/were the targeted audience/ stakeholders (including the patients) well informed in advance of the establishment of a SC?
- Are there/were there open calls for expression of interests?

5. Dissemination and communication on the NP

- 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?

5. Dissemination and communication on the NP

- 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.)?

6. Monitoring and evaluation of the National Plan

- 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? To healthcare professionals?

7. Sustainability of the National Plan

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?

7. Sustainability of the National Plan

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.

PROPOSALS

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