

Recommendations for Rare Diseases national plans development

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Community action programme on RD 1999-2003

The aim of the programme was to contribute, in co-ordination with other Community measures, to ensure a high level of health protection in relation to RD improving knowledge and facilitating access to information about these diseases

- RD are now one of the priorities in the **EU Public Health Programme 2003-2008**
- RD will continue to be a priority for action in the new Public Health Programme (2008-2013).



COMMUNICATION FROM THE COMMISSION

Aim

General aim of the document is to sum up the necessary elements of an efficient policy addressing RD in Europe. The strategic objective of the EC intervention in this field is aimed at improving the chance for patients to get appropriate and timely diagnosis, information and care.



COMMUNICATION FROM THE COMMISSION

Specific objectives

- To improve identification and knowledge on RD
- To improve diagnosis and care of patients with RD
- To accelerate research and developments in the field of RD and OD
- To empower patients with RD at individual and collective level
- **To coordinate policies and initiatives at MS level and EU level**

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Adoption of National/regional Plans for RD

Only a limited number of MS have adopted or will adopt soon a National Plan or will launch initiatives:

- Only France has established a comprehensive action plan (2005-2008),
- Other MS have national policies in a limited number of areas (Italy, Sweden, Denmark, United Kingdom) or are in the process to establish policies (Bulgaria, Portugal, Spain, Romania, Luxembourg)
- Other MS have a targeted policy only in the area of research (Germany, The Netherlands).

In order to integrate all the necessary initiatives which have to be taken at national and/or regional level, MS are invited to establish national or regional action plans for RD.

[European guidelines for the elaboration of action plans for RD might be useful.](#)



European Project for Rare Diseases National Plans Development (EUROPLAN)

The project aims at providing information on the different steps to develop a strategic plan and at **identifying best practices** in order to share information, models and data on effective strategies to address RD.

The recommendations including a critical analysis of the functioning of the current activities in key areas of intervention for RD will serve as a guidance manual to support EU MS in the development of national plans for RD.

Specific objectives

1. Describe EU Member States initiatives on RD
2. Identify indicators to assess RD initiatives
3. Assess RD initiatives on the basis of the selected indicators to identify best practices
4. Develop recommendations for the development of RD strategic plan
5. Discuss the recommendations and present the Commission Communication on Rare Diseases

Methodology

- Identification of EU MS experiences:

A comprehensive review will be conducted to identify RD initiatives. On the basis of agreed on criteria several initiatives will be identified in partner countries to better understand their functioning and achievements.

- Identification on indicators for evaluating the achievements:

Indicators will be identified on the basis of a comprehensive review, discussion with experts and the collaboration and engagement of the RDTF.

- Data collection:

On the basis of the selected indicators, data will be collected in partners countries. The data collected will be evaluated with a peer-review system. Best practices will be identified in collaboration with key stakeholders in an open workshop



Methodology

- Development of the recommendations:
An editorial working group made by partners will be established to draft the recommendations. The draft will be submitted to a refereeing process including experts in the field and RDTF members.
- Assessment of the transferability of the recommendations:
National Conferences will be organised to discuss the transferability of the recommendations.

The final version of the recommendations will be launched by an European workshop and presented in relevant EU and national conferences.

Target groups

- Ministries
- Regional and local authorities
- Health care planners
- Programme managers
- Health care professionals
- Researchers and
- Patients

Partners

The project includes 19 out of the 27 EU MS and Eurordis (European Organisation for rare diseases). This will ensure a broad representation of different EU contexts and experiences and patients' point of view.

Bulgaria	Greece
Cyprus	Hungary
Croatia	Italy
Czech Republic	Malta
Denmark	Portugal
Estonia	Spain
Finland	Sweden
France	The Netherlands
Germany	Turkey
	UK

The collaboration of the Office for RD (NIH-USA) will ensure additional insights from a well experienced Country.



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Expected outcomes

- The project will develop recommendations on how to establish a strategic plan for RD ensuring an inclusive and wide engagement of stakeholders and a broad dissemination of the recommendation in all EU MS.
- The project will engage stakeholders since the first steps of the development of the recommendations and identification of best practices in order to:
 - 1) facilitate their understanding of RD and RD challenges
 - 2) make them part of the decision making process with regard to the best practices
 - 3) demonstrate the evidence based approach used for the development of the recommendations and
 - 4) create a sense of ownership of the recommendations.

Expected outcomes

- The engagement of stakeholders is considered essential to achieve the 2 major outcomes of the project:
 - 1) reach a consensus on evidence based strategies for RD and
 - 2) promote the development of RD strategic plan on the basis of best practices.
- Because of the inclusive and collaborative approach that will be used to reach consensus on the recommendations, the project will contribute to increase awareness on RD and the recommendations will also serve as an important advocacy instrument at policy level.

Important dates

- EUROPLAN officially started April 1^o
- Kick-off meeting – Rome July 7th
- European Workshop (Colloque Européen) "Rare Diseases: a challenge for research and public health strategy of EU Member states" **under the French Presidency** – Paris, 18 November 2008



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