

EUROPLAN

European Project for Rare Diseases National Plans Development



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The Council Recommendation and EUROPLAN

The Commission has proposed a “Council Recommendation on a European action in the field of rare diseases”

inviting Member States to define plans / strategies / actions

(at the appropriate level)

to put in place strategies organised around ideas and approach set out in the Communication.

The recommendations developed by EUROPLAN will provide practical guidance on how this can be done on successful actions

Efficient and effective action for rare diseases depends on a coherent overall strategy based on common approach and integrated into a common European effort

Goal

EUROPLAN is **a three-year project** of the Programme of Community action in the field of Public Health (2003 - 2008), which **began in April 2008.**

MAIN GOAL: to elaborate **recommendations on how to define a national plan / strategy / action** for rare diseases.

Focusing on the already available MS experiences on RD

EUROPLAN will contribute to share information, models and data on effective strategies to address RD.

Structure of the project

- **8 work packages**
 - WP1-WP3 deal with the management of the project and the dissemination of the results
 - WP4-WP8 are the core WPs
- **the coordinator:** Dr. Domenica Taruscio – Director of the National Centre for Rare Diseases (Istituto Superiore di Sanità, Italy)
- The Coordinator is supported by the **Steering Committee (SC)** and the **Advisory Committee (AC)**.

Specific objectives of EUROPLAN

1. **Collect information on EU Member States initiatives** on rare diseases
2. **Identify successful actions** (EUROPLAN will identify the lessons learned ensuring that relevant information will be shared and make available to Member States to facilitate the development of national strategies on RDs taking into account the already available experiences)
3. **Elaborate indicators for monitoring the implementation and evaluating the impact of national plans/strategies/actions** for rare diseases
4. **Elaborate the recommendations**
5. **Discuss the recommendations with local stakeholders**

Disseminate the EUROPLAN recommendations

EUROPLAN: an inclusive project

- **Associated partners**

Institution	Country
Istituto de Salud Carlos III	<u>Spain</u>
University of Tartu	Estonia
European Organisation for Rare Diseases (<u>Eurordis</u>)	
The Netherlands Organisation for Health Research and Development – Steering Committee on Orphan Drugs	The Netherlands
<u>Karolinska Institutet</u>	Sweden
Istituto di Ricerche <u>Farmacologiche</u> Mario Negri	<u>Italy</u>
Bulgarian Association for Promotion of Education and Science	Bulgaria
London Strategic Health Authority	UK
Fundacion Canaria de Investigacion y Salud	Cruz de Tenerife, Spain

- **Collaborating partners**

Institution	Country
Federal Ministry of Health	Germany
Ministry of Health - Health Care Organisation Department	France
<u>Italian Ministry of Health</u>	<u>Italy</u>
<u>German Federal Institute for Drugs and Medical Devices</u>	Germany
National Organisation for Medicines	Greece
<u>Läkemedelsverket</u>	Sweden
Ospedale S. Giovanni Bosco - <u>Clinical Research Center for RD</u>	<u>Italy</u>
Hospital Dona Estefania	Portugal
Children's University Hospital Zagreb	<u>Croatia</u>
Mater Dei Hospital	Malta
The Cyprus Institute of Neurology & Genetics	Cyprus
University Hospital of <u>Motol</u>	Czech Republic
Department of Medical Biology, <u>IGOM</u> - <u>Georgiev Institute of Genetics and Biotechnology</u>	Turkey
Faculty of Health Sciences, University <u>PECS</u>	Hungaria
La Sapienza University	<u>Italy</u>
EUROCAT	<u>Italy</u>
<u>Epidemiology Unit</u> - Istituto Nazionale Tumori	<u>Italy</u>
Fundació Doctor Robert	Spain
The Family Federation of Finland	Finland
Office for Rare Diseases, National Institute of Health	USA

National Authorities & health care planners

Health care professionals

Researchers

Patients

EUROPLAN Partners

EUROPLAN include:
30 Countries
and **Eurordis**



EURORDIS
Rare Diseases Europe

**EUROPLAN collaborates with
the Office for rare diseases
(NIH-USA)**



Increasing number of partners

26 out of the 27 EU MS

1. Austria (Additional Collaborating Country - ACC)
2. Belgium (Additional Collaborating Country - ACC)
3. Bulgaria
4. Canada (Additional Collaborating Country - ACC)
5. Croatia
6. Czech Republic
7. Cyprus
8. Denmark
9. Estonia
10. Finland
11. France
12. Germany
13. Greece
14. Hungary
15. Ireland (Additional Collaborating Country - ACC)
16. Italy
17. Latvia (Additional Collaborating Country - ACC)
18. Lithuania (Additional Collaborating Country - ACC)
19. Luxembourg (Additional Collaborating Country - ACC)
20. Malta
21. Netherlands
22. Poland (Additional Collaborating Country - ACC)
23. Portugal
24. Romania (Additional Collaborating Country - ACC)
25. Slovenia (Additional Collaborating Country - ACC)
26. Spain
27. Sweden
28. Turkey
29. United Kingdom
30. USA



On going activities

(1) Survey on activities / actions performed at MS level

The survey aims at listing the activities actually on going related to RD in EU Member States.

The survey will encompass the following information:

- National plans/strategies/actions for RDs
- Definition, inventorying and provision of information on RDs
- Research on RDs
- Empowerment of patients organisations
- Compassionate use programme of orphan drugs
- Funds dedicated to RDs
- Specialised social services

The results of the questionnaire will contribute to identify the Countries where successful experiences will be analysed and described (WP6)

(2) Develop indicators for monitoring the implementation and evaluating the impact of national plans for RD



European Project for Rare Diseases National Plans Development (EUROPLAN)

Selecting indicators to evaluate the achievements of RD initiatives

By WP5 leader: Manuel Posada & Maria José Carroquino

Rare Diseases Research Institute.
Instituto de Salud Carlos III

Madrid March, 17, 2009

The first draft of indicators has been elaborated and it is under revision by Europlan partners

Next step

- **Workshop on indicators** for monitoring the implementation and evaluating the impact of national plans for RD
 - **Madrid: 18-19 June, 2009**
 - Many experts will be invited, including those from the RDTF WG indicators members

Elaboration of recommendations

First draft under revision
(by EUROPLAN partners)

RD national plans recommendations

Draft 1, Jan 2009
Laura and Jolanda

INTRODUCTION

Rare diseases (RD), defined as those diseases with a prevalence up to than 5 in 10000, represent an important public health and social issue for several reasons:

- even though each disease is rare, around 8000 of them have been described, accounting for 25-30 million of affected people in the EU.
- RD are in most cases serious, chronic and debilitating, and often require heavy specialized treatments. They often result in handicaps, sometimes extremely severe, and account for a substantial proportion of mortality in infancy and childhood.
- Health professionals have insufficient knowledge of most rare diseases. The lack of knowledge causes diagnostic error and delayed care provision, which can sometimes be prejudicial.
- Early diagnosis and follow-up require multidisciplinary teams, with scientific expertise and medical competence. However, if diseases are rare, experts are rare as well.
- Due to the situation of invisibility to most health care systems, diagnosis delay, and inappropriate care, RD patients are object of isolation and social exclusion.

In 2004 the EU created a Task Force (Rare Diseases Task Force, RDTF) to specifically address this problem. Recently, a Commission Communication on RD, drafted by members of the RDTF and modified accordingly to the results of a public consultation has been published, together with the proposal for a Council Recommendation. The Communication advocates a common European approach to RD and underlines the fact that, for the geographic spread of the patients and the shortage of experts, that of RD is an area which can benefit by coordinated actions. To this aim, one important recommendation of the Communication is that of creating national plans (NP) to specifically address the problems of RD.

The project Europlan was funded by the EU to investigate the existing national initiatives and provide recommendations for the development of NP. This project, which involves at present representatives of the national health authorities of 21 out of the 27 EU MS, is an important instrument to create political awareness about RD at single national level and to provide guidelines for the establishment of national initiatives for RD, based on best practices.

RECOMMENDATION

RD should be recognized in every MS as an area with specific health care and social needs.

1) DEFINITION OF A NATIONAL PLAN FOR RARE DISEASES

A RD national plan can be defined as a comprehensive and integrated strategy for actions in the area of RD, developed and put into force at national (MS) /regional level. 'Comprehensive' means that the strategy should cover all areas that are crucial for an effective health policy for RD, and

Preliminary main chapters:

1. Introduction
2. Definition of National Plan/strategy/action
3. Budget
4. Methodology
5. Flexibility and duration
6. Areas of action
7. Dissemination

Next step

Workshop to discuss topics and content of the recommendations: The Hauge, 17 September 2009

Possible topics for discussion:

- Problems and solutions for small countries
- National versus regional plan?
- What should be done in your country before developing a national plan? How to create awareness and support?
- Feasibility and flexibility of a NP
- Position of pharmaceutical industry or insurance companies concerning access to orphan drugs in relation to national plan
- Lessons learned from evaluations

(4) Discuss the recommendations with local stakeholders

National Conferences will be organized by EURORDIS

- a) to present the EUROPLAN recommendations**
- b) to discuss the transferability of the recommendations with local stakeholders in different countries**
- c) to present the Commission Communication and Council Recommendations on rare diseases**

The selected Countries are 16:

Bulgaria, (Belgium), Croatia, Denmark, France, Germany,

Greece, Hungary, Ireland, Italy, Luxembourg, Netherlands,

Dissemination of EUROPLAN

National Strategies and Plans for Rare Diseases in Europe"

November 18, 2008

"State of the art and sharing experiences: toward EU Recommendations"

Under the auspices of:

- EUROPLAN
- French Presidency of the EU
- EURORDIS
- European Commission



Summary of Conference 'National Strategies

available on the EU web site

http://ec.europa.eu/health/ph_threats/non_

Dissemination

- **ICORD International Conference on “Rare diseases and orphan drugs”, Rome, 23-25 February 2009**
- **Parliament of the Czech Republic, Chamber of Deputies, International Conference, Prague, May 21, 2009**
- **International Congress on Rett syndrome, Milan 5-7, June 2009**
- **IV Eastern European Conference on rare diseases and orphan drugs, Bulgaria, 13-14 June**

Main conclusions

- Rare disease is an area where pool our resources is needed
- There is **need to share experiences, objectives**, both as regards strategy and resource activation.
- Keys to success:
 - to promote a global approach
 - to support synergies between all stakeholders
 - to identify a national steering for the development of the plans
 - to guarantee the evaluation
 - to cooperate at European level



- Project**
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The European Project for Rare Diseases National Plans Development (EUROPLAN) is a three-year project of the Programme of Community action in the field of Public Health (2003 - 2008), which began in April 2008.

The main goal is to develop recommendations on how to define a strategic plan for rare diseases. The recommendations will provide information on the different steps to develop a strategic plan and, more important, it will include priority areas and actions of intervention in the field of rare diseases.

The project will collect and disseminate information on EU MS national initiatives on rare diseases, on expectations on national plans for rare diseases and on best practices contributing to share experiences, data and effective strategies to address rare diseases.

The National Centre for Rare Diseases (Istituto Superiore di Sanità, Italy) is the leading organization; 30 countries and Eurordis (the European Organisation for rare diseases) participate in the project. This will ensure a broad representation of different EU context and experiences and patients' point of view. In addition, the project will ensure an inclusive and wide engagement of stakeholders - Ministries, regional and local authorities, health care planners, programme managers, health care professionals, researchers and patients

The expected outputs of EUROPLAN are:

- To stimulate a discussion and reach a consensus on the importance of national plans for structuring all relevant actions in the field of rare diseases
- To list priority areas and actions of intervention for addressing rare diseases
- To promote the development of national plans for rare diseases within

Contact us!



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