



European Project for Rare Diseases National Plans Development (EUROPLAN)

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 to define a National Plan or Strategy 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 **specific objectives** of the project follow:

1. describe EU Member States initiatives on rare diseases;
2. list priority areas and actions of intervention to include in national plans for rare diseases;
3. develop indicators for monitoring the implementation and evaluating the impact of National plans for rare diseases;
4. analyse relevant experiences;
5. develop recommendations for the development of strategic plans for rare diseases including methodological guidance;
6. discuss the recommendations and present the Commission Communication on Rare Diseases with local stakeholders.

STRUCTURE OF THE PROJECT

EUROPLAN is organised in 8 work packages. The coordinator is 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**).

The SC consists of one representative from each associated partner of the project and is responsible for all strategic planning.

The AC is composed by leading experts in the field of rare diseases, representatives of European and international organisations working on health service and medical care, Committees and Authorities active in the areas of interest of the project. The AC acts as liaisons to the European Organizations, scientific community and policy arena for optimising cooperation and synergistic activities.

AC main members:

- Jordi Llinares – EMA;
- Valentina Hafner - Country policies and systems - Division of country health systems - WHO Regional Office for Europe;

- Concha Colomer – Ministry of Health and Social Politics (Spain);
- Cecilia Berni - (Italy).

WORK PACKAGES (WP) OVERVIEW

- **-WP1-WP3** deal with the management of the project and the dissemination of the results.
- **-WP4-WP8** are the core WPs.

WP4 - Describing EU Member States initiatives on rare diseases - WP4 collects information on the initiatives undertaken by EU MSs on rare diseases. The information are collected focusing on the priority areas and actions of intervention to include in a National Plan or Strategy for rare diseases accordingly to the EU Communication on Rare Diseases and to the Proposal for a Council Recommendation on a European action in the field of rare diseases. Such areas and actions will include: .

Area 1. PLANS AND STRATEGIES IN THE FIELD OF RARE DISEASES

- 1.2 The creation of a national policy
- 1.3 Creation of a mechanism for the governance of the National Plan or Strategy
- 1.4 Awareness raising
- 1.5 Assessing patients' needs
- 1.6 Small population countries
- 1.7 Areas and actions of a National Plan or Strategy
- 1.8 Target population of the National Plan for rare diseases
- 1.9 Dissemination of the information on National Plans and Strategies
- 1.10 Sustainability and duration of the National Plans and Strategies
- 1.11 Monitoring
- 1.12 Evaluation and audit

Area 2. ADEQUATE DEFINITION, CODIFICATION AND INVENTORYING OF RARE DISEASES

- 2.1. Coding and Classification of rare diseases
- 2.2. Inventory
- 2.3. Epidemiology, registries and surveillance
 - 2.3.1.Registries

Area 3. RESEARCH ON RARE DISEASES

- 3.1. Fundamental research
- 3.2. Translational and clinical Research

Area 4. CENTRES OF EXPERTISE AND EUROPEAN REFERENCE NETWORKS FOR RARE DISEASES

- 4.1. Definition of centres of expertise
- 4.2. Identification and designation of centres of expertise
- 4.3. Healthcare Pathways
- 4.4. Networking

- 4.5. Diagnosis
- 4.6. Screening
- 4.7. Rehabilitation

Area 5. GATHERING THE EXPERTISE ON RARE DISEASES AT EUROPEAN LEVEL

- 5.1. Information, education and training of health professionals
- 5.2. Neonatal Screening
- 5.3. Clinical guidelines
- 5.4. Access to orphan drugs

Area 6. EMPOWERMENT OF PATIENT ORGANISATIONS

- 6.1. Information to the public and patients
- 6.2. Specialised social services

Area 7. SUSTAINABILITY

- 7.1. The governance of the cooperative initiatives foreseen in the National Plan or Strategy
- 7.2. Using European funding for National Plans or Strategies for Rare Diseases

The WP4 survey provides examples of different experiences and lesson learned allowing the sharing of information among Member States. Collecting the information on the initiatives in EU MSs, the WP4 will explore also the following:

- perspective on what is working well in countries;
- lessons learned and key steps to considered for promoting rare diseases initiatives;
- areas where collaboration among Member States is needed.

Accordingly to the information collected, an inventory of the different initiatives and of the related available documents will be created to develop a database containing the relevant documents collected. The database will be accessible from the project website.

WP4 deliverable: Report on rare diseases initiatives

WP5 - Selecting indicators to evaluate the achievements of rare diseases initiatives

WP5 develops indicators for monitoring the implementation and for evaluating the impact of National Plans or Strategies for rare diseases. The activities required to identify indicators include a comprehensive literature review on rare diseases indicators, discussion with experts including the engagement of the RDTF. In addition, a workshop is

envisaged with internationally renowned experts in the areas of epidemiology and health indicators.

WP5 deliverable: Report of the workshop including the list of key indicators.

WP6 - Evaluating selected activities and identifying relevant cases

WP6 will analyse case studies starting from the data collected by the WP4.

These cases will be translated into Europlan Recommendations and will be used as explicative examples.

WP6 deliverable: Report including the list of case studies identified

WP7 - Preparing recommendations for rare diseases national plan development

WP7 will develop the content of the EUROPLAN recommendations including:

- methodological guidance on how to develop a **National Plan or Strategy**;
- priority areas and actions of intervention in the field of rare diseases;
- relevant experiences previously identified by the WP6.

An editorial working group made by partners is established to draft the recommendations. The first draft will be submitted to a refereeing process including experts in the field and RDTF members. A second draft of the recommendations will be developed accordingly to the feedback of the refereeing process and will be approved by the Advisory Members before being submitted to European Member States National Authorities. A meeting with these authorities is envisaged in May 2010 in Krakow to approve the final draft to be circulated in National Conferences (WP8). The final version of the recommendations will be developed including the feedbacks received by the national consultations.

WP7 deliverable: Recommendations for rare diseases national plans development

WP8 - Promoting the recommendations on National Plan and Strategy for rare diseases and presenting the Commission Communications on rare diseases

WP8 will organise several national conferences in order to present the EUROPLAN recommendations, discuss the transferability of the recommendations with local stakeholders in different countries and present the Commission Communication and Council Recommendations on rare diseases.

The activities envisioned follow:

- launch of a call for 'candidate Conferences';
- pre-selection of the conferences;

- final selection;
- organisation of the conferences;
WP8 deliverable: Summary report of the national conferences

EXPECTED OUTPUTS

Expected outputs of the project 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 promote the development of national plans for rare diseases within EU MSs; and
- to provide an instrument to support Countries in elaborating national plans for rare diseases.

PARTNERS

EUROPLAN includes **31 Countries** and Eurordis (the European Organisation for rare diseases).

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

The project aims at ensuring a wide engagement of stakeholders - Ministries, regional and local authorities, health care planners, programme managers, health care professionals, researchers and patients.

The list of the Countries contributing to the project follow:

1. Austria
2. Belgium
3. Bulgaria
4. Canada
5. Croatia
6. Cyprus
7. Czech Republic
8. Denmark
9. Estonia
10. Finland
11. France
12. Germany
13. Greece
14. Hungary
15. Ireland
16. Italy
17. Latvia
18. Lithuania
19. Luxembourg
20. Malta
21. Netherlands
22. Poland
23. Portugal
24. Romania
25. Russia
26. Slovenia

27. Slovakia
28. Spain
29. Sweden
30. Turkey
31. UK
32. USA

Additional Cooperating Partners

Europlan aims at being an inclusive project since the sharing of experiences is very important for developing common agreed recommendations and for providing examples for addressing rare diseases.

In this context additional partners interested in the project are welcomed as Additional Cooperating Countries (ACC).

ACCs are formally not part of the grant agreement however they have the same rights and duties as Collaborating Partners thus they can be included in the project website, participate in WP meetings and open workshops but at their own expenses.

For further information please contact the EUROPLAN management team:

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