



## **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 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 **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 case studies to identify successful experiences
5. Develop recommendations for the development of strategic plan 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.

Five experts and institution have been identified as main members while the engagement of additional experts/institutions will be considered case by case.

AC main members:

1. High Level Group on Health Services and Medical Care (European reference network)
2. European network for Health Technology Assessment, EUnetHTA
3. The European Medicines Agency, EMEA
4. World Health Organisations (European Observatory on Health System);
5. Rare Diseases task Force, RDTF

## **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 will collect information on the initiatives undertaken by EU MS on rare diseases.

The information will be collected focusing on the priority areas and actions of intervention to include in national plan 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: epidemiological monitoring of rare diseases; dissemination of information on rare diseases to patients, health professionals and the general public; prevention and early diagnosis; treatment and care; access to social services including support measures; research on rare diseases. The WP4 survey will provide examples of different experiences and lesson learned allowing the sharing of information among Member States.

Collecting the information on the initiatives in EU MS, 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 will develop indicators for monitoring the implementation and evaluating the impact of national plans for rare diseases.

The activities required to identify indicators will include a comprehensive literature review on rare diseases indicators, discussion with experts including the engagement of the RDTF. In addition, a workshop will be held with internationally renowned experts in the areas of epidemiology and health indicators. Partners will participate to the workshop and will contribute providing the national perspective on the feasibility of the data collection.

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

### **WP6 - Evaluating selected activities and identifying best practices**

WP6 will analyse case studies to identify successful experiences. Case studies will be selected considering the data collected by the WP4.

The results of the data collection will be evaluated with a peer-review system by the partners of the project. In addition a workshop with partners and AC members will be organised to present the data collected and evaluated by the peer-review process, with the objective of identifying successful experience.

WP6 deliverable: Report including the list of success cases identified

## **WP7 - Preparing recommendations for rare diseases national plan development**

WP7 will develop the content of the EUROPLAN recommendations including:

1. methodological guidance on how to develop a national plan,
2. priority areas and actions of intervention in the field of rare diseases,
3. successful experiences previously identified by the WP6

An editorial working group made by partners will be 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. The final version of the recommendations will be developed including the feedbacks received by the national consultations (see WP8).

WP7 deliverable: Recommendations for rare diseases national plans development

## **WP8 - Promoting the recommendations for rare diseases national plan and presenting the Commission Communications on RD**

WP8 will organise several national conferences in order to present the EUROPLAN recommendations (second draft), 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**

- 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 MS
- To provide an instrument to support Countries in elaborating national plans for rare diseases

## **PARTNERS**

EUROPLAN includes **30 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. Czech Republic
7. Cyprus
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. Slovenia
26. Spain
27. Sweden
28. Turkey
29. United Kingdom
30. USA

#### Additional Cooperating Partners

Euoplan 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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