

EUROPLAN

European Project for Rare Diseases National Plans Development

Recommendations to support rare diseases research through National Plans and Strategies

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Council Recommendation

of 8 June 2009

on an action in the field of rare diseases (2009/C 151/02)

... RECOMMENDS THAT MEMBER STATES:

- 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 all relevant actions in the field of rare diseases within the framework of their health and social systems
- 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 strategy, **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)...**



EUROPLAN

It's a three year (2008-2011) project funded by the EU Commission

(Programme of Community action in the field of Public Health, 2003 - 2008)

Co-ordination: Italian National Center for Rare Diseases (Istituto Superiore di Sanità, Italy)

Partners: 30 Countries and EURORDIS

Main goals:

Develop recommendations for the elaboration of a national plan or strategy for rare diseases

Elaborate indicators for monitoring national plans/strategies

Discuss the recommendations with stakeholders

Disseminate the EUROPLAN recommendations

Focusing on the already available MS experiences on rare diseases, EUROPLAN will contribute to share information, models and data on effective strategies



EUROPLAN: an inclusive project

- **Associated partners**

Institution	Country
Istituto de Salud Carlos III	Spain
University of Tartu	Estonia
European Organisation for Rare Diseases (Eurordis)	
The Netherlands Organisation for Health Research and Development – Steering Committee on Orphan Drugs	The Netherlands
Karolinska Institutet	Sweden
Istituto di Ricerche Farmacologiche Mario Negri	Italy
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
Italian Ministry of Health	Italy
The National Centre of Medicines	Greece
National Organisation for Medicines	Greece
Läkemedelsverket	Sweden
Ospedale S. Giovanni Bosco - Clinical Research Center for RD	Italy
Hospital Domus Infancia	Portugal
Children's University Hospital Zagreb	Croatia
Mater Dei Hospital	Malta
The Cyprus Institute of Neurology & Genetics	Cyprus
University Hospital of Motol	Czech Republic
Department of Medical Genetics, Ege University	Turkey
Faculty of Health Sciences, University Pecs	Hungary
La Sapienza University	Italy
EUROCAT	Italy
Epidemiology Unit - Istituto Nazionale	Italy
Fundació Doctor Robert	Spain
The Family Federation of Finland	Finland
Office for Rare Diseases, National Institute of Health	USA

Health authorities & health care planners

Clinicians, health and social operators

EURORDIS, patients' groups

Scientists



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Partners

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



EUROPLAN

SPECIFIC OBJECTIVES

- ✓ **Collect information on EU Member States initiatives and experiences on rare diseases**
- ✓ **Develop the recommendations for the elaboration of a national plan or strategy for rare diseases (WP7)**
- ✓ **Develop indicators for monitoring the implementation and evaluating the impact of National Plans or strategies for RD (WP5)**

It is working capitalising the existing efforts and experiences, avoiding duplication of activities and strengthening possible synergies among RD community



European Project for Rare Diseases National Plans Development (EUROPLAN)

WP7: Recommendations for the development of National Plan or Strategies for rare diseases

WP7 Leader:

The Netherlands Organisation for Health Research and Development (ZonMw) – Steering Committee on Orphan Drugs, Netherlands

The document “Recommendations” has been prepared by WP7, in collaboration with the coordination team, several other EUROPLAN partners and experts

WP5: Indicators for monitoring national plans/strategies

WP5 Leader:



Rare Diseases Research Institute – Instituto de Salud Carlos III (Madrid, Spain)



EUROPLAN DEFINITION OF A NATIONAL PLAN OR STRATEGY

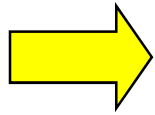
*a set of integrated and comprehensive health and social policy actions for rare diseases
to be developed and implemented at national level,
and characterized by identified objectives to be achieved within a
specified timeframe.*

The allocation of appropriate **resources (human, financial, infrastructural)** and its **monitoring and evaluation** are of special value to ensure the efficacy of the plan or strategy.

AREAS

- | | |
|----------------|--|
| AREA 1. | PLANS AND STRATEGIES IN THE FIELD OF RARE DISEASES |
| AREA 2. | ADEQUATE DEFINITION, CODIFICATION AND INVENTORYING |
| AREA 3. | <u>RESEARCH ON RARE DISEASES</u> |
| AREA 4. | CENTRES OF EXPERTISE AND EUROPEAN REFERENCE NETWORKS FOR RARE DISEASES |
| AREA 5. | GATHERING THE EXPERTISE ON RARE DISEASES AT EUROPEAN LEVEL |
| AREA 6. | EMPOWERMENT OF PATIENT ORGANISATIONS |
| AREA 7. | SUSTAINABILITY |

Council Recommendation (2009/C 151/02)



III. RESEARCH ON RARE DISEASES

... RECOMMENDS THAT MEMBER STATES:

6. **Identify ongoing research and research resources in the national and Community frameworks** in order to establish the state of the art, assess the research landscape in the area of rare diseases, and improve the coordination of Community, national and regional programmes for rare diseases research

7. **Identify needs and priorities for basic, clinical, translational and social research** in the field of rare diseases and modes of fostering them, and promote interdisciplinary cooperative approaches to be complementarily addressed through national and Community programmes


8. **Foster the participation of national researchers in research projects** on rare diseases funded at all appropriate levels, including the Community level

9. **Include in their plans or strategies provisions aimed at fostering research** in the field of rare diseases

10. **Facilitate, together with the Commission, the development of research cooperation** with third countries active in research on rare diseases and more generally with regard to the exchange of information and the sharing of expertise



EUROPLAN RECOMMENDATIONS (1)

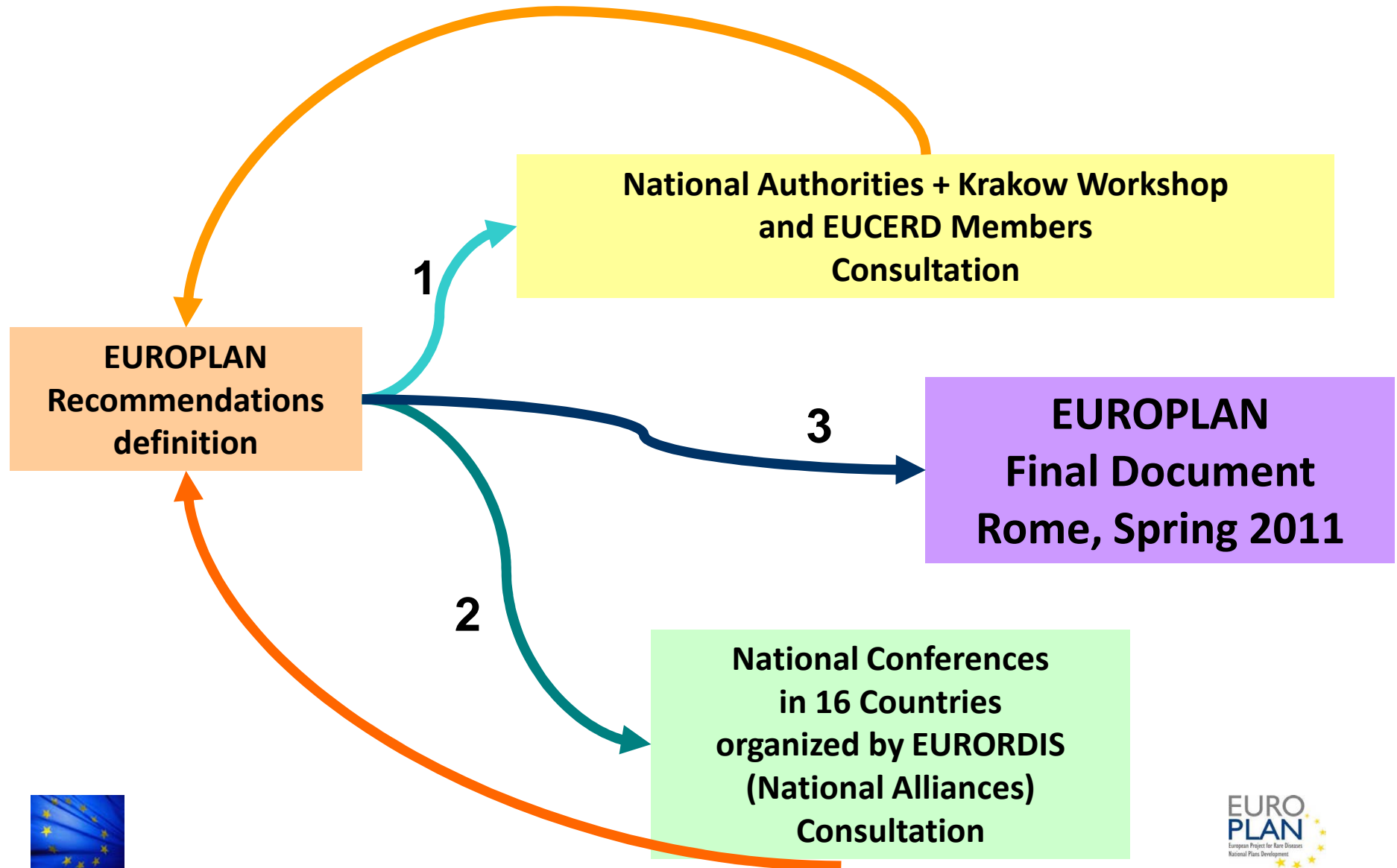
- **Specific national research programmes** for rare diseases are organized and supported with dedicated funds, preferably for a long period.
- **National networks are promoted** in order to foster research (including public and social research) on rare diseases .
Special attention is given to translational research in order to facilitate the application of new knowledge into rare disease treatment.
- **Proper initiatives are developed to foster participation in international research calls** on rare diseases, including the  **EU framework program** and **E-RARE**.

EUROPLAN RECOMMENDATIONS (2)

- **Specific technological platforms and infrastructures** for rare disease research are established and supported.
- **Multi-centre national and trans-national studies are promoted**, in order to reach a critical mass of patients for clinical trials and to exploit international expertise.
- **Instruments and measures (e.g. centres) to support clinical research and clinical trials** on rare diseases are set.
- **Specific programs are launched for funding and/or recruitment of young scientists** on rare disease research projects.



Overview of EUROPLAN consensus process



HERE WE ARE:

- ✓ We are going to send the recommendations to the National Health Authorities and to the EUCERD Members
- ✓ EUROPLAN Workshop in Krakow (May 13, 2010) with Health Authorities
- ✓ Discuss the recommendations with stakeholders throughout National Conferences organized by EURORDIS (National Alliances) in 16 countries
- ✓ Elaborate the final version of the Document containing the recommendations including all stakeholders' remarks
- ✓ Present the final version of EUROPLAN documents (recommendations and indicators) during the final international conference (Roma, Spring 2011)



EUROPLAN milestones

- **1 April 2008** – Project starts
- **18 November 2008** – Conference “National Strategies and Plans for Rare Diseases in Europe”, Paris, under the aegis of the EU Presidency (France), EURORDIS and EUROPLAN
-  **January- February 2010** – Indicators and EUROPLAN Recommendations have been finalised
- **13 May 2010** – Krakow Workshop with National Authorities to discuss the EUROPLAN Recommendations
- **May 2010 to December 2010** – National Conferences on Rare Diseases organized by EURORDIS- National Alliances in 16 Countries
- **February 2011** – Recommendations finalised
-  **April 2011** – Project ends

European Project for Rare Diseases National Plans Development (EUROPLAN)

EXPECTED OUTPUTS

- ✓ To stimulate a discussion and reach a consensus on the importance of national plans for structuring all relevant actions, including research, in the field of rare diseases,
- ✓ To promote the development of National Plans and Strategies for rare diseases within EU MS
- ✓ To provide instruments and tools (recommendations, indicators) to support Countries in elaborating national plans or strategies for rare diseases



<http://www.europlanproject.eu/>



The screenshot shows the homepage of the EUROPLAN website. At the top, there is a blue header with the text "EUROPLAN" in large, bold, blue letters, and "European Project for Rare Diseases National Plans Development" in smaller text below it. To the right of the text are several yellow stars. Below the header is a navigation bar with links for "Home", "News", "Events", "Search", and "Print".

On the left side, there is a "Project" menu with the following items:

- Overview of the Project
- Partners
- Deliverables and Meetings
- Database including MS official documents
- Contact us
- Links
- Access restricted to partners

The main content area contains three paragraphs of text:

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

