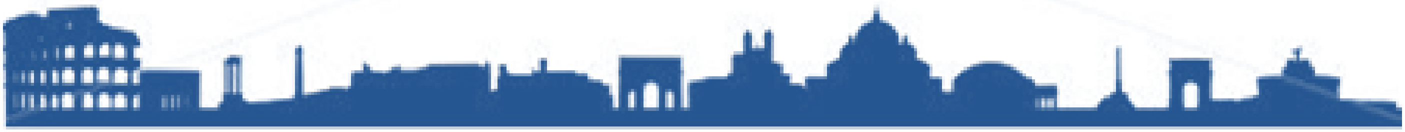
EUROPLAN

European Project for Rare Diseases National Plans Development
Coordinated by the Italian National Centre for Rare Diseases
Italian National Institute of Health
2012-2015

REPORT

INCEPTION WORKSHOP ON NATIONAL PLANNING FOR RARE DISEASES

10-11 September 2012
Rome, Italy



EUCERD Joint Action
Executive Agency for Health and Consumers
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www.europlanproject.eu

INTRODUCTION

The three-year project EUROPLAN 2012-2015, embedded in the EUCERD Joint Action as Work Package 4, has the main goal of establishing an international and interactive network of stakeholders (mainly policy makers) to speed up the elaboration and the implementation of Rare Diseases National Plans/Strategies, through scientific and technical assistance, Workshops and the active participation of patients Groups (EURORDIS and National Alliances).

Over the first year of the project, the “Inception Workshop on National Planning for Rare Diseases” (10-11 September 2012 - Rome, Italy) was organized with the purpose of sharing strengths and weaknesses and identifying opportunities for developing NP/NS, as well as identifying communication targets and stakeholders in this process.

Main objectives:

- To share among the participants strengths and weaknesses in developing National Plans and Strategies (NP/NS) on Rare Diseases (RD)
- To identify opportunities for developing NP/NS on RD
- To identify targets and stakeholders of communication for developing NP/NS on RD.

Format of the workshop

This workshop was structured in oral presentations, working groups by Cooperative learning method (Jigsaw technique) and plenary discussion.

Working groups method

Cooperative learning is a method of instruction that gets participants working together in groups, usually with the goal of completing a specific task. This method can help participants develop leadership skills and the ability to work with others as a team.
The research that originally gave credibility to the Jigsaw approach—creating heterogeneous groups of students, diving them into new groups to become expert on a topic, and then returning them to their home groups—touted its value as a means of creating positive interdependence in the classroom and improving students’ attitudes toward school and each other.
The structure it provides also lends itself naturally to differentiating instruction. Because learning experiences can be differentiated by content based on student readiness and interests, the Jigsaw technique allows students to learn from text that is matched to their interests and independent reading level while also learning from their peers, who have worked with text that is appropriate for them. *(Aronson, Elliot, and Shelley Patnoe. The Jigsaw Classroom: Building Cooperation in the Classroom. 2nd ed. New York: Longman, 1997).*
In the specific context of the Inception workshop the objectives of Group Activities were:

- to identify actions that would support the development of NP/NS on RD
- to identify targets and stakeholders of communication for NP/NS on RD

Expected Results:
A list of initiatives aimed at making the best use of the strengths identified for developing NP/NS.

WORKSHOP REPORT

In order to prepare a background discussion of this EUROPLAN Workshop, a web-based questionnaire (SurveyMonkey) on strengths and weaknesses in developing NP/NS had been elaborated and distributed to all participants.

The results of the survey (available on www.europlanproject.eu – survey section) enabled all stakeholders to discuss the strengths and weaknesses that have been identified, taking a step forward towards a more effective sharing of objectives.

DAY 1 - 10 SEPTEMBER 2012

WELCOME - *DOMENICA TARUSCIO* *(EUROPLAN Leader, Italian National Institute of Health, Rome)*

As EUROPLAN Leader, Domenica Taruscio extended a welcome to all participants and explained the expected the workshop program, underlined that the discussion will focus on the strengths and weaknesses of national plans and health strategies on rare diseases.

EUCERD JOINT ACTION: AN OVERVIEW - *STEPHEN LYNN* *(EUCERD Joint Action Manager, Newcastle University, Newcastle upon Tyne)*

The EUCERD Joint Action Manager has explained what the Joint Action and which the partners are. Specifically, this Joint Action addressed the following priority areas:

- Implementation of national plans and strategies (WP4)
- Standardisation of RD nomenclature (WP5)
- Mapping the provision of specialised social services (WP6)
- Mapping of national initiatives across continuum of care with emphasis on quality assurance (WP7)
- Integration of RD activities across thematic areas, MS and related activities (WP8).

The other WP are:

WP1 – Management: Reporting, communication with EC, etc
WP2 – Communication: EUCERD web site, newsletters, annual state of the art reports
WP3 – Evaluation: Input to project to coordinate process indicators, outputs and outcomes.

The expected outcome is an integrated strategy for the implementation of rare disease policies through the exchange of experience between Member State health authorities already involved in rare disease policy definition and implementation.

EUROPEAN PROJECT FOR RARE DISEASES PLANS DEVELOPMENT: FROM 2008 UP TO NOW - *DOMENICA TARUSCIO (EUROPLAN Leader, Italian National Institute of Health, Rome)*

The Project Leader explained that EUROPLAN was planned and implemented as an operational measure to fulfill the specific objective of the EU strategy for RD. The project started in 2008 and comprises two phases: Phase 1 (2008-2011) to build the consensus definition of operational tools (recommendations and indicators), and the ongoing Phase 2 (2012-2015) – contained in the EUCERD Joint Action: 'Working for Rare Diseases'- to promote capacity building with the active involvement of various stakeholders.

In particular, EUROPLAN (2012-2015) intends to provide tailored technical and scientific support, maximizing experiences, and strengthening international interactions and cooperation. The project to date involves 32 Associated and Collaborating Partners that include clinicians and scientists, health authorities, and EURORDIS as representative of patients' groups.

The objectives are:

- To establish an interactive network (mainly policy makers)
- To produce a complete, coherent and feasible operational proposal for NP/NS.
- To support EURORDIS National Conferences.

The deliverables will be:

- Info Pack for organisers of National Conferences
- Operational proposals for National Plan/Strategy
- Final reports of National Conferences
- Final report on Capacity Building

EURORDIS' ACTIVITIES FOR NATIONAL PLANS - A LONG-STANDING COMMITMENT - *VALENTINA BOTTARELLI (EURORDIS - European Organisation for Rare Diseases)*

EURORDIS has been a leading actor in the process aimed to design, adopt and implement national policies on RDs. The presentation addresses in detail three topics:

1. Paving the way for NPs
2. Common guidelines for NPs– EUROPLAN I
3. Towards tailor-made plans– EUROPLAN II

Since 2005 EURORDIS was present and active in the main processes of political strategy and health at national level (by participating in the drafting of the first NP in France) and European level. EURORDIS was involved in the drafting process of the Commission proposal for a Council Recommendation, also coordinating the advocacy to national Health Ministries via its National Alliances, in view of the Council adoption. EURORDIS was the only patient organisation partner in EUROPLAN I (2008–2011) and also responsible for a full Work Package - "WP8 - Promoting the recommendations for Rare Diseases National Plans and presenting the Commission Communication on Rare Diseases". It was involved in the development process of key deliverables - EUROPLAN Recommendations and EUROPLAN Indicators - and supported 15 National Alliances (NAs) to organise as many National

Conferences. Overall success of EUROPLAN I experience and method - many countries expressed their interest in having a 2nd national conference to further discuss the measures of National Plans, their assessment or implementation. EURORDIS is involved in the EUROPLAN II (2012-2015), supports and coordinates National Conferences on RDs to be organised by National Alliances/patient groups, in order to contribute to the adoption of a plan or strategy for rare diseases in all EU Member States by the end of 2013.

STATE OF ART OF NATIONAL PLANS FOR RARE DISEASES - *EDMUND JESSOP* *(National Commissioning Group – NHS, London)*

It was presented a snapshot of the European situation. All 27 Member States of the European Union are active in the preparation of national plans for rare disease, but at different stages of development. Also the scope of each plan is different. Some Countries already have adopted a NP and now are working for implementation; others are organizing drafting group or stakeholder meetings, still others have just submitted NP to National Authority or in Public consultation by National Authority.

The stages of development are:

- Decision to elaborate a plan
- Drafting group or stakeholder meetings being held
- Plan submitted to national authority
- National authority consulting public on plan
- National plan adopted
- National plan implemented

Early lessons include the need to update the plan, and attention to implementation.

STRENGTHS AND WEAKNESSES IN DEVELOPING NATIONAL PLANS: RESULTS OF EUROPLAN 2012-2015 SURVEY - *RITA MARIA FERRELLI* *(EUROPLAN 2012-2015 Coordinating Team, Italian National Institute of Health, Rome)*

The report briefly outlined the results of the survey on strengths and weaknesses in developing National Plans. The questionnaire was filled in by July 10, 2012 by the stakeholders for developing NP/NS on:

- 27 Member State policy-makers identified by the project and confirmed by EUCERD as contacts points for developing NP/NS;
- Norway and Croatia;
- Non EU collaborating partners: Armenia, Georgia, Russia.

The response rate was 97% : 31 out of 32 filled in the questionnaire.

The strengths highlighted three areas: stakeholders networking, knowledge and legal framework. In further detail:

Stakeholders networking

- Involvement of all stakeholders /wide range of inputs / cooperation among various actors (Gvt, clinicians, patients organisations, Parliament, industry …)
- Work team /working groups involving different representatives of institutions and organisations (pts, professionals, researchers ..)
- Empowerment of patients / public consultations
- Partnership between MoH and National Alliance for RD
- Steering group set by Ministry (professionals and patient organisations)
- Good relationships between patient organisation and State Administration
- National Action League for people with RD (NAMSE): set up by MoH, MoEduc, Ntl Alliance of Patient Groups for RD
- Identification of centres for RD

Knowledge

- National study on diagnostics, treatment and rehabilitation/overview of local health and social situation
- EUROPLAN recommendations/ international practice in planning process /building on recommendations from other MS
- Well educated members of the team and personal reputation
- Exchange good practice on organisational development
- Information centre for RD

Legal framework

- Recognition that RD are a priority for the MoH
- Potential for long-term financing
- Funding health care and research on RD
- Legal base for Orphan Drugs

The difficulties faced in NP were connected to local features (i.e. Budget/economic crisis/lack of funds; Large Country/small population) and legal framework and problems of knowledge and networking (bad communication among some stakeholders; Lack of multidisciplinary teams to cover all needs; Different interests of different institutions, Low level of research etc.).

In conclusion, the key factors for developing a National Plan are: knowledge of the local situation, technical knowledge on RD, Stakeholders involvement and networking, political willingness.

LESSONS LEARNT FROM SUCCESSES AND FAILURES IN DEVELOPING NATIONAL PLANS - *RITA MARIA FERRELLI* *(EUROPLAN 2012-2015 Coordinating Team, Italian National Institute of Health, Rome).*

In order to support the participants were presented the objectives, the expected results and method of the working group. It were notified the timing and actions for each work session. At last, it was supplied groups composition and were designated the roles of Chairperson and Rapporteur.

The group activities were conducted in 4 different phases:

phase 1 – Home group (30 min.) The participants discuss about three main topics: weaknesses, opportunities and communication.
phase 2 - Expert group (30 min.). Each topic is discussed in the respective Expert Group
phase 3 - Home group (30 min.) The participants of each Expert Group return to their own Home Group and present the results of the discussion held in the Expert Group. The Home Group discusses and agreees the answers to the 3 questions; then they choose a name. The rapporteur takes note of the group conclusions in order to present them in the plenary
phase 4 – Plenary (50 min.) Each rapporteur presents his/her group conclusions and all participants can comment

DAY 2 - 11 SEPTEMBER 2012

RECOMMENDATIONS FOR THE DEVELOPMENT OF NATIONAL PLANS OR STRATEGIES FOR RARE DISEASES - *DOMENICA TARUSCIO* *(EUROPLAN Leader, Italian National Institute of Health, Rome)*

The report provided the background to Europlan Document on Indicators. The Council of the European Union, in its Recommendation of 8 June 2009 on an action in the field of rare diseases, recommends that "Member States 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 relevant actions in the field of rare diseases within the framework of their health and social systems".

In order to sustain this process, the programme of Community action in the field of Public Health supported "EUROPLAN" (2008-2011) had elaborated a "Report on indicators for monitoring the implementation and evaluating the impact of National Plans or Strategy Strategies for rare diseases" .
The "EUROPLAN Indicators" are a set of 59 indicators for health and social planning monitoring, intended for policy makers and planners.

The indicators are divided into seven specific areas, reflecting the EU Council Recommendation structure:

1. Plans and Strategies in The Field of Rare Diseases
2. Adequate Definition, Codification and Inventorying
3. Research on Rare Diseases
4. Centres of Expertise and European Reference Networks for Rare Diseases
5. Gathering the Expertise on Rare Diseases at European Level

6. Empowerment of Patient Organisations
7. Sustainability

SELECTING INDICATORS TO EVALUATE THE ACHIEVEMENTS OF RD INITIATIVES - *MANUEL POSADA, MARÍA JOSÉ CARROQUINO AND MANUEL HENS* *(Institute of Rare Diseases Research, Instituto de Salud Carlos III Madrid, Spain)*

EUROPLAN 2008-2011 was organized in 8 Work Packages (WPs), each focused on specific objectives. WP5 develops indicators for monitoring the implementation and for evaluating the impact of National Plans or Strategies for rare diseases. The WP5 scope and purpose was to develop a list of indicators for evaluating RD activities following those areas of action previously identified.

Dr. Posada, WP5 Leader, described in detail the required activities to identify the indicators and the method used to get to the final selection. In particular, the work groups utilized 3 criteria:

- Intrinsic properties
- Resources demand
- Decision making

and 16 properties for qualitative evaluation (Understandability, Reliability, Validity, Consistency, Sensitivity, Specificity, Sustainability, Implementability, Workload demand, Timeliness, Applicability, Coherence, Feasibility, Availability, Comprehensiveness, Relevance).

THE DELPHI PROCESS FOR SELECTING INDICATORS - *RITA MARIA FERRELLI* *(EUROPLAN 2012-2015 Coordinating Team, Italian National Institute of Health, Rome).*

The development and use of indicators is an integral part of planning and designing health and social services, as they can be used as management tools for health care and social services and health systems, as well as to monitor the implementation of National Plans for Rare Diseases. The 59 EUROPLAN Indicators from an operational perspective, but they were quite numerous and difficult to handle during the first steps of the implementation process. For this reason, in the ongoing Phase 2, a specific stepwise elaboration on indicators has been carried out to select a smaller set of "Core Indicators". The National Centre for Rare Diseases (ISS) carried out the Delphi process, involving the Ministry of Health representatives of 27 MS, 10 EURORDIS Advisors and 4 experts.

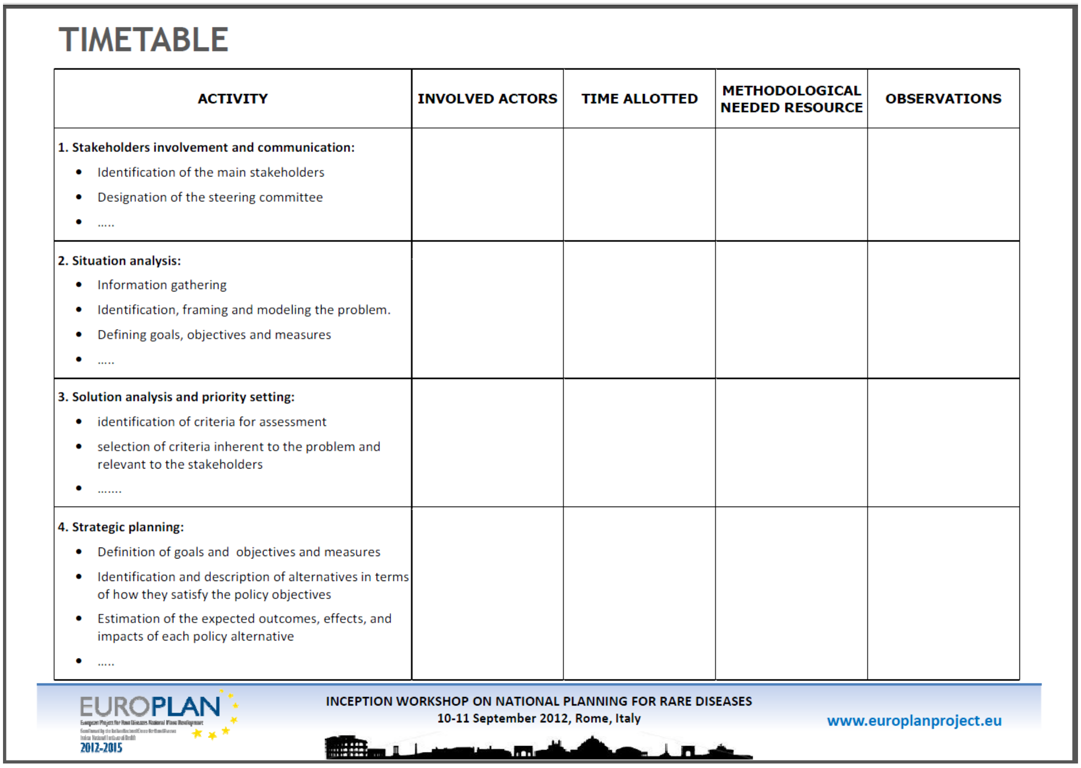
Delphi is a qualitative research technique for consensus building. The communication is organised in a group of experts in order to achieve their opinion in a systematic way and to group subjective judgments. Every expert can express his/her own opinion anonymously.

Delphi often uses a panel of experts who do not meet face to face, yet participate in an interactive group process. This is achieved by using a series of questionnaires, called "Delphi Rounds", each of which (after the first) include summary information about the entire panel's response to the previous round. The succeeding rounds are designed to bring the group to focus and consensus. This approach creates a structured, anonymous interaction that avoids such group effects as dominance by one or two participants, deference to more powerful participants, or bandwagon effects.

Usefulness and feasibility of data collection are the two main criteria that EUROPLAN II identified for selecting a limited number of indicators among those produced by EUROPLAN I.
The dedicated website was accessibile, using username and password, in the private area of the EUROPLAN website. A User Manual facilitated the process and ensured methodological homogeneity.

Definition of actions supporting National Plans for Rare Diseases *(Group activity – Day 2)*

The Objective of this second Group Activities was to define a action plan to support the development of NP/NS on RD, in order to define an action plan for each group.
The method utilized was group discussion. The results were described on specific timetable (picture 1)



TIMETABLE

ACTIVITY	INVOLVED ACTORS	TIME ALLOTTED	METHODOLOGICAL NEEDED RESOURCE	OBSERVATIONS
1. Stakeholders involvement and communication: • Identification of the main stakeholders • Designation of the steering committee •				
2. Situation analysis: • Information gathering • Identification, framing and modeling the problem. • Defining goals, objectives and measures •				
3. Solution analysis and priority setting: • identification of criteria for assessment • selection of criteria inherent to the problem and relevant to the stakeholders •				
4. Strategic planning: • Definition of goals and objectives and measures • Identification and description of alternatives in terms of how they satisfy the policy objectives • Estimation of the expected outcomes, effects, and impacts of each policy alternative •				

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Picture 1: Timetable scheme

HOW EU CAN SUPPORT NATIONAL PLANS ON RARE DISEASES - *ANTONI MONTSERRAT (Health and Consumers General-Directorate (SANCO), European Commission, Luxembourg)*

Antoni Montserrat illustrated the European Union action in the field of Rare Diseases and focused his report on funding opportunities.

ANNEXES

All annexes are available on www.europlanproject.eu (Event section):

- Program
- Participants
- Accommodations
- Presentations
- Secretariat
- How to get to the workshop site
- Photogallery