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

II WORKSHOP
KEY INDICATORS FOR NATIONAL PLANS

25 March 2013
Rome (Italy)

INTRODUCTION

This workshop is part of the articulated path to an EUCERD Recommendation on Indicators for Rare Diseases National Plans/Strategies.

Previous steps:

Step 1 - From DECEMBER 2012 to FEBRUARY 2013 two independent methodologies (Delphi process carried out by the ISS and EURORDIS approach carried out by EURORDIS, the European Organisation for Rare Diseases) were used for selecting a limited number of indicators ("Core indicators") starting from the initial set of 59 Indicators. This process was presented by Yann Le Cam and Domenica Taruscio during the 7th EUCERD meeting (31 January-1 February 2013) .

Step 2 - On 20 February 2013 the drafting group elaborated the first draft of the Recommendations, comparing and merging the results from the two methodologies (Delphi process and EURORDIS approach) to select the list of the highest scoring indicators, plus the suggestions of the drafting group members. This first draft circulated among Ministry of Health representatives of 27 MS, 10 EURORDIS Advisors and 4 experts for comments and amendments, that were included in the preparation of the second draft.

The second draft was discussed during the EUROPLAN Workshop "Key Indicators for National Plans" (ISS, Rome, 25th March 2013) and amended by the participants (Ministry of Health representatives of MS, EURORDIS Advisors and experts).

Main objectives:

- To share the results of both EUROPLAN "A" & "B" processes to select indicators with participants
- To discuss/finalize the Draft EUCERD Recommendation on Indicators for National Plans/Strategies for Rare Diseases
- To discuss/finalize the template for collecting data

Format of the workshop

This workshop was structured in oral presentations and discussion.

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WORKSHOP REPORT

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

The EUROPLAN Leader extended a welcome to all participants and explained the objectives of the II Workshop in Rome. She has briefly reviewed the story of EUROPLAN and the first steps of the process that led to discussion of the second draft of Core Indicators.

The 60 Indicators of EUROPLAN 1 are good to pick and choose from to develop the set of indicators adapted to the National Plan or Strategies, but difficult to collect systematically for each country and difficult to monitor in 27 Member States (MSs) on long term. For this reason a set of main common indicators are needed. The Core Indicators, moreover, will also be useful for the end of 2013 EC Report and for the annual State of Art of EUCERD RD from 2014.

At last, a proposal was suggested for a structure of the Recommendation and the Drafting Group composition.

INDICATORS SELECTION ACCORDING TO THE DELPHI TECHNIQUE (EUROPLAN "A"): PRESENTATION OF RESULTS - *RITA MARIA FERRELLI, AMALIA EGLE GENTILE, MARTA DE SANTIS* *(EUROPLAN 2012-2015 Coordinating Team, Italian National Institute of Health, Rome)*

For selecting a limited number of indicators two processes were utilized: a Delphi process, carried out by ISS, and the EURORDIS approach. The results of both processes werediscussed during this meeting. Delphi is a qualitative research technique for consensus building wherein every expert can express his/her own opinion anonymously.

Usefulness and feasibility of data collection werethe two main criteria that EUROPLAN II identified for selecting. Data analysis reports the mean, median and range of the values.
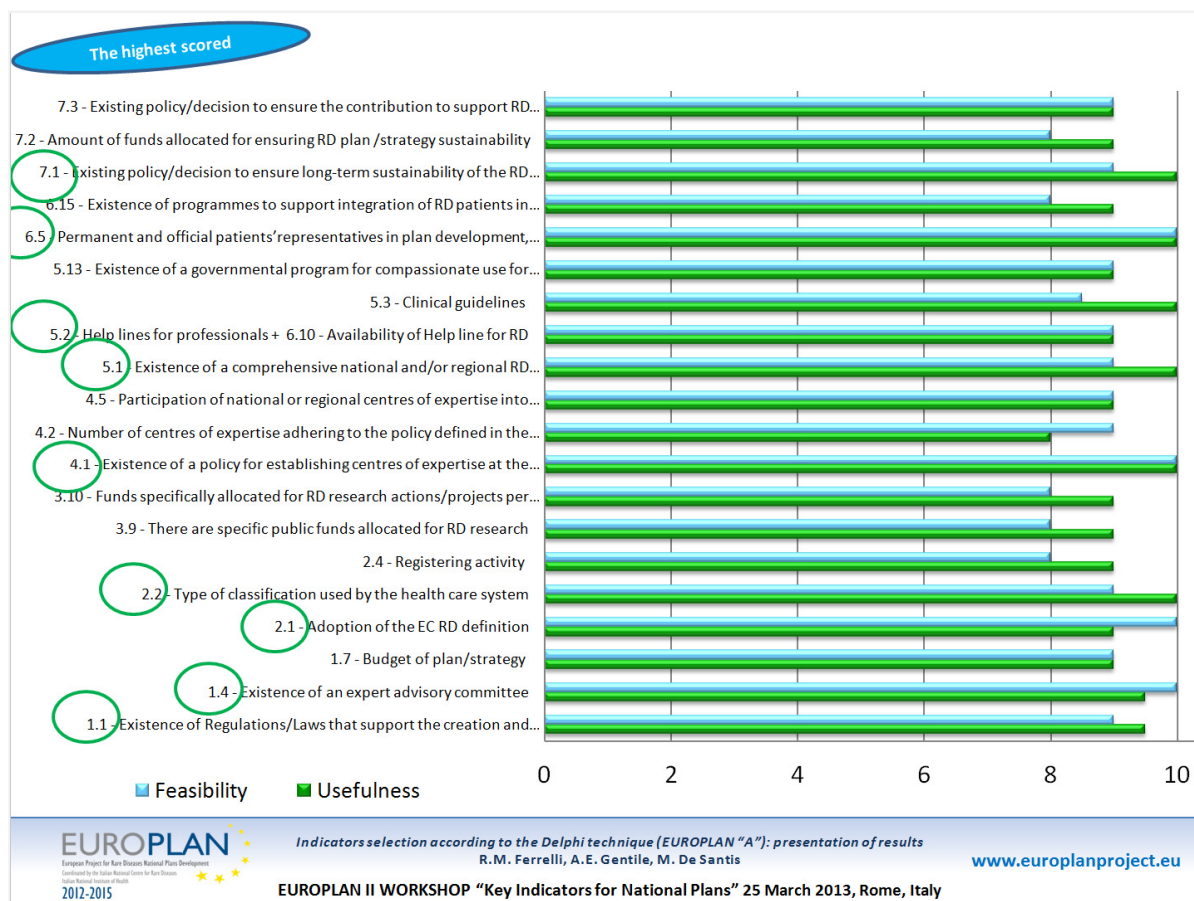Only the highest median [10/10 and 10/9 (in green) and 9/9 and 9/8 (in yellow)] has been taken into account for selecting the indicators.

In order to facilitate the process and to ensure homogeneity the EUROPLAN team prepared a USER MANUAL, available on EUROPLAN website (Members area).
Delphi generally proceeds in a series of rounds, but in this selection process a satisfactory level of agreement was reached at the first round.

Among those who were invited to participate in the selection process, the following attended it: 26 EU MS MoH Representatives (instead 27- Germany deemed the indicators process selection to be in a early stage and decided not rake part into the process); 2 non-EU Countries MoH Representatives ;4 Experts; 6 EURORDIS Advisors (out of a total of 10).

The following table describes in brief the main results:

The highest scored

Indicators selection according to the Delphi technique (EUROPLAN "A"): presentation of results
R.M. Ferrelli, A.E. Gentile, M. De Santis
www.europlanproject.eu
EUROPLAN II WORKSHOP "Key Indicators for National Plans" 25 March 2013, Rome, Italy

INDICATORS "CROSS-OVER" SELECTIONACCORDING TO THE EURORDIS APPROACH (EUROPLAN "B"): PRESENTATION OF RESULTS - *YANN LE CAM, VALENTINA BOTTARELLI* (EURORDIS - European Organisation for Rare Diseases)

The EURORDIS approach to identify core indicators for National Plans has involved EURORDIS staff, EURORDIS-EUROPLAN Advisors with their National Alliances and MoH representativesinvolved in National RD Plan/Strategy.

The methodology entailed two levels: national and group. An initial approach to the establishment of a set of core common indicators (tested in France)was based on first-hand experience of EUROPLAN Advisors and their MoH contacts in RD policy implementation.

In detail, each EURORDIS-EUROPLAN advisor assessed the 59 EUROPLAN Indicators according to the selected criteria (Usefulness for Patients and Care ProvidersPolitical Feasibility Usefulness) and on the basis of their direct experience with their NP/NS and RD policy. EURORDIS-EUROPLAN advisors

worked with their MoH contacts to perform the assessment and submitted to EURORDIS the scores assigned to the 59 EUROPLAN indicators. Each Indicator scored from 1 to 5 for each criterion.

At group level, the 10 EURORDIS-EUROPLAN Advisors compiled the results with the highest scores highlighted, held some group discussion to come up with a list of selected core. The EURORDIS-EUROPLAN Advisors group selected a list of 22 Core Indicators and 6 Desirable Indicators, on the basis of: Highest scores (group results); and Group discussion (keeping an eye at the preliminary results of the ISS Delphi method. Additionally, it was proposed to reword some Indicators.

The following tables (1&2) show the results:

Overall results - Core

1.1 - Existence of Regulations/Laws that support the creation and development of a RD plan
1.4 - Existence of an expert advisory committee
1.7 - Budget of plan/strategy
2.2 - Type of classification used by the health care system
2.4 – Registering activity
3.2 - RD research programme monitoring
3.6 - Including public health and social research in the field of rare diseases
3.7 - Research platforms and other infrastructures are also funded by the research programme
3.9 - There are specific public funds allocated for RD research
3.10 - Funds specifically allocated for RD research actions/projects per year since then plan started
4.1 - Existence of a policy for establishing centres of expertise at the national/regional level
4.2 - Number of centres of expertise adhering to the policy defined in the country
4.5 - Participation of national or regional centres of expertise into European reference networks
5.1 - Existence of a comprehensive national and/or regional RD information system supported by the govern.
5.2 - Help lines for professionals + 6.10 - Availability of Help line for RD
5.3 - Clinical guidelines
5.9 - Number of OMPs granted a market authorisation by EMA, available in the country (priced & reimbursed)
5.13 - Existence of a governmental program for compassionate use for Rare Diseases
6.5 - Permanent and official patients' representatives in plan development, monitoring and assessment
6.7 - Participation of patient organisations in RD centres of expertise designation and evaluation
6.15 - Existence of programmes to support integration of RD patients in their daily life
7.2 - Amount of funds allocated for ensuring RD plan /strategy sustainability

Table 1.

Table 2

PRESENTATION OF DRAFT EUCERD RECOMMENDATION PLANS/STRATEGIES FOR RARE DISEASE - *YANN LE CAM* (EUCERD Vice-Chair, Paris)

A smaller set of core/common indicators are needed for a good vision across 27 MSs and on long term so to monitor process and progress of common policies on RDs; potentially useful for the EC Report of end 2013 and surely useful for the annual EUCERD State of Art of RD.

The overall objective of the Core Indicators for Rare Disease National Plans and Strategies is to capture relevant data and information on the process of planning, following up and implementing of these plans and strategies. The other objectives are to agree on this common core set of indicators and commit to its collection and to streamline and align the follow up of the implementation of RD NP/NS amongst EU MSs in areas defined in the Council Recommendation and guided by EUCERD Recommendations.

The preliminary results of the two paths of selection (Delphi and EURORDIS approach) were presented during EUCERD 7th Meeting (31st January 13), together with the structure of the potential EUCERD Recommendation:

- Background & Methodology & Target Groups

- Objectives & vision & scope
- List of 21 core indicators
- In annex detailed guidance for good understanding and quality collection of data
- Key reference documents.

The EUCERD Recommendation on Core Indicators - as All EUCERD Recommendations - is considered as experimental and will need revision based on feedback from field work in MSs. This Reco will be experimented for few years and may be revised if needed based on experience gained in the collection and analysis of this indicators. It may also be revised based on further work to revise the full sets of indicators for national strategies on rare diseases based on actual plans and EUCERD JA/WP 4 EUROPLAN II action in the coming year.

DISCUSSION OF THE FINAL SELECTION/FINAL WORDING OF ABOUT 20 MAIN INDICATORS FOR NATIONAL PLANS/STRATEGIES FOR RARE DISEASES"
MODERATORS: DOMENICA TARUSCIO (EUROPLAN Leader, Rome), YANN LE CAM (EURORDIS Leader, Paris)

The discussion about the main selected indicators was extended for hours and it reached a general agreement. Each attendee actively participated suggesting possible changes and improvements that have been included in the third Draft of EUCERD Recommendation on Indicators for National Plans/Strategies for Rare Diseases. Twenty one indicators have been agreed, with the option of successive integrations based upon the experience gained in EU Member States with the adoption and implementation of National Plans and Strategies on Rare Diseases, as commended by the Council Recommendation. In addition to the list of Core Indicators, a grid with definitions and associated answers for the practical data collectionhas been shared and worked as well.

EUROPLAN NATIONAL CONFERENCES AND POST-NCS' ACTIVITIES IN SUPPORT TO MEMBER STATES - *EURORDIS TEAM - EUROPLAN TEAM*

The "Debrief Session" is a specific activity post-National Conference (NC), with the following objectives:

- To identify and collect needs, challenges and the most pressing issues emerged during the EUROPLAN National Conference (NC) in relation to the development of Rare Diseases National Plans and Strategies (NP/NS);
- To suggest dedicated solutions to national authorities.

The long-term objective is to create a "hub" within the EUROPLAN Coordinating Team for support and information for national authorities on NP/NS, based on the expertise built from the direct contacts with national authorities.
A common form will be used to identify the main needs/gaps for each Country (MoH Representative). These sessions (lasting approximately 1 hour-1 ½ hour) should involve:

- 1/2 EUROPLAN Coordinating Team member/s (who participate/s to the NC);

- 1 EURORDIS Advisor;
- 1 EUCERD JA Coordination representative;
- 1/2 Ministry of Health (MoH) representative/s (and key persons involved in elaboration/implementation of NP, including contact person of EUROPLAN "A", identified in collaboration with EURORDIS and National Alliances).

The EUROPLAN Coordinating Team, according to the identified needs and the existing resources - in collaboration with EURORDIS and the National Alliances and EJA Coordination, will arrange for each Country a dedicated proposal in order to provide connections to experts in other Countries (with same solved problems) and existing "guidance" documents (e.g. EUCERD Reports, Recommendations, etc.) regarding identified needs. Specific instruments and methods (e.g. conference calls, Skype-calls, workshops) will be selected considering the individual cases.

NEXT STEPS

25 April 2013 – **3ndDraft EUCERD Recommendation sent to EUCERD members**(mettiamo qui la consultazione con epaacvisto che non è stata fatta o si cancella?)

5-6 June 2013 - **Possible adoption EUCERD Plenary**

ANNEXES

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

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

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