

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



Co-funded by the



STRENGTHS AND WEAKNESSES IN DEVELOPING NATIONAL PLANS: RESULTS OF EUROPLAN 2012-2015 SURVEY

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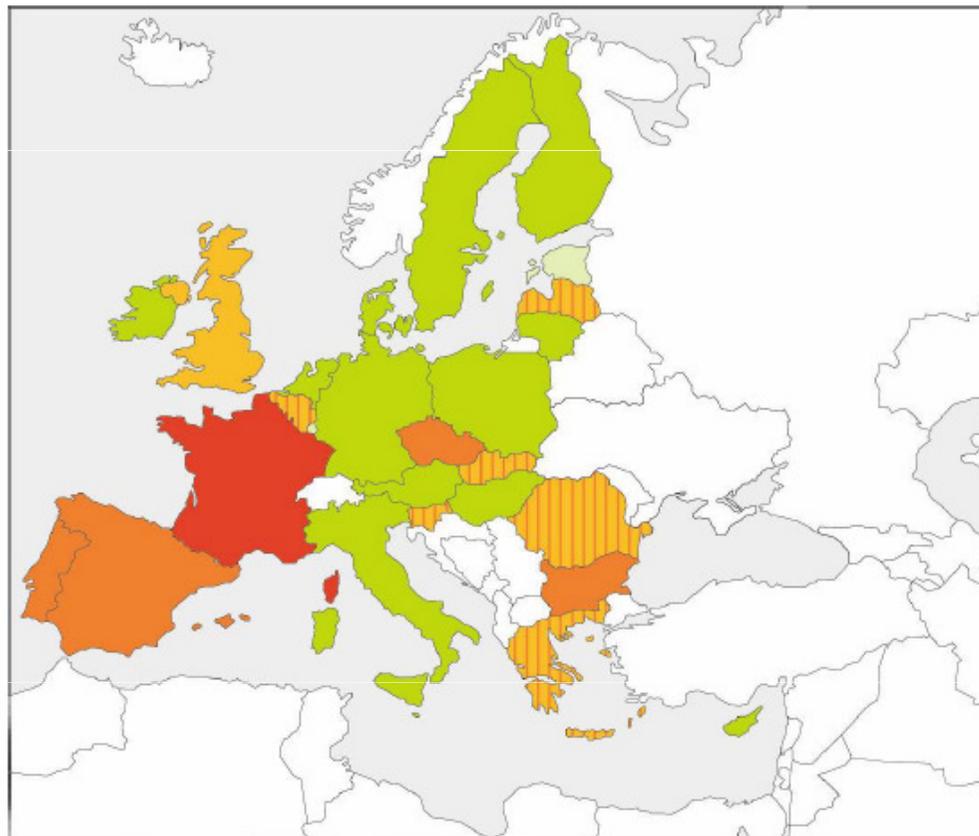
Istituto Superiore di Sanità
Italy

INCEPTION WORKSHOP ON NATIONAL PLANNING FOR RARE DISEASES

10-11 September 2012, Rome, Italy

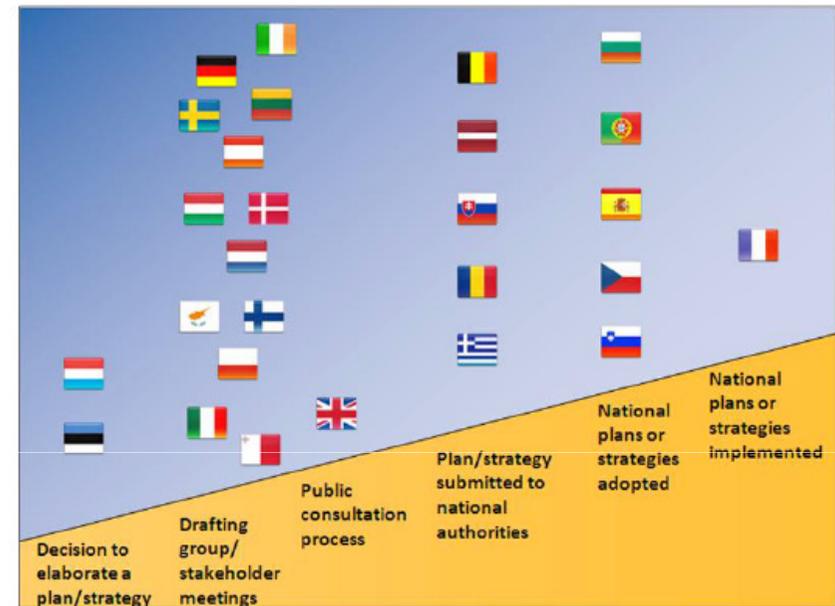


DEVELOPMENT OF NP/NS IN EU MS



- National plans or strategies implemented
- National plans or strategies adopted
- Public consultation process
- Drafting group/stakeholder meetings
- Plan/strategy submitted to national authorities
- Decision to elaborate a plan/strategy

www.eucerd.eu



EUROPLAN 2012 - 2015

- Continuation of EUROPLAN 2008 - 2011
 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
- WP4 of EUCERD Joint Action: Working for Rare Diseases
- AIM: to support the establishment of national plans and strategies for rare Diseases at Member State level
- METHOD: dedicated workshops, interactive EU RD policy makers public health network



INCEPTION WORKSHOP

- OBJECTIVES

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

- PREPARATORY WORK

- Questionnaire distributed to all participants and filled in by July 10, 2012 on www.surveymonkey.com



QUESTIONNAIRE

Summary

The inception workshop is part of the starting activities of EUROPLAN (Work Package 4 of the EUCERD Joint Action for Rare Diseases - EJA). The following info is requested in order to prepare workshop activities. We thank you in advance for your collaboration.

The EUROPLAN team

1. Name and Surname

2. Position

3. Institution Name

4. Please specify which of following categories you belong to:

Health Authority

Health Professional

Patient organization

Other (please specify)

5. Contacts

Telephone

E-mail

6. Strengths highlighted during the planning process

7. Difficulties faced in the process of developing the National Plan for Rare Diseases

8. Other observations



RESULTS

- The questionnaire was sent to the stakeholders for developing NP/NS:
 - the 27 MS policy-makers identified by the project and confirmed by EUCERD as contacts points for developing NP/NS,
 - plus Norway and Croatia and
 - the non EU collaborating partners: Armenia, Georgia, Russia
- Response rate 97%
 - 31 out of 32 filled in the questionnaire



STRENGTHS: 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



STRENGTHS: 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



STRENGTHS: 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



DIFFICULTIES FACED IN NP: local features and legal framework

- ECONOMIC CONSTRAINTS
 - Budget/economic crisis/lack of funds/
- LOCAL FEATURES
 - Large Country/small population
 - Devolution: build on what is already there/fragmented health system
- LEGAL FRAMEWORK
 - Changes of State Administration/uncertain environment/lack of MoH stability
 - Inadequate legislative framework
 - Policy on pricing and reimbursement of OD / Accessibility of orphan drugs



DIFFICULTIES FACED IN NP: knowledge and networking

NETWORKING AND COMMUNICATION

- Getting the involvement of all stakeholders/ each stakeholder has its own interest and viewpoint / bad communication among some stakeholders
- Lack of multidisciplinary teams to cover all needs
- Different interests of different institutions
- Fragmentation of competencies for RD
- Lack of a national network of specialised medical centres

KNOWLEDGE

- Statistical data gathering and analysis
- Low level of research
- Finding good solutions on how to run registers
- Awareness of doctors on early diagnosis
- Low knowledge of RD in GP/lack of clinical pathways



OTHER OBSERVATIONS

- Involvement of different stakeholders
- European interest in action for RD
- Identification of the correct indicator (concrete measures) with targets and timeline to evaluate the effectiveness of the implemented recommendations to improve health and quality of life of patients with RD
- Elaboration of good indicators to evaluate the NP
- Societal crisis against handicapped, sick and old people



CONCLUSIONS

- Knowledge of the local situation, in addition to the technical knowledge on RD, represent the basis for NP
- Stakeholders involvement and networking are the key factors for developing a NP
- Political willingness is a must: the existence of an inducive legal and institutional environment facilitates NP



LET'S BE CAREFUL!!

- Networking, stakeholders involvement and communication represent key factors for developing NP/NS and deserve special attention
- Elaboration of good indicators is still a weak point in NP for RD

