

NATIONAL PLANS/STRATEGIES FOR RARE DISEASES: ADHERENCE to the “EC RECOMMENDATION on an action in the field of rare diseases”

OBJECTIVE

To analyse the adherence of National Plans (NP) and National Strategies (NS) for rare diseases (RD) adopted in Europe to the areas of the “Recommendation on an action in the field of rare diseases” (2009/C 151/02) of the European Council, in the framework of EUROPLAN (WP4 of EUCERD Joint Action). [Fig. 1. NP/S for RD (September 2015)]. EUROPLAN (European Project for Rare Disease National Plans Development) was launched in 2008 and foresaw 2 implementation phases: phase 1 (2008-2011) to build the consensus definition of operational tools (recommendations and indicators) and the phase 2 (2012-2015), embedded in the EUCERD Joint Action, with the main goal of establishing an interactive network of stakeholders to support Rare Disease National Plans through scientific and technical assistance.

METHOD

Selection criteria of documents to be included:

- Document type: NP and NS (published by August 2015) [If available, for the selected States with NP/S, other documents (such as Recommendations) have been included, if necessary].
- Language: English, French, Spanish and Italian.

RESULTS

Fourteen EU States have been included:

- 13 Member States (Belgium, Bulgaria, Czech Republic, France, Germany, Hungary, Ireland, Italy, Romania, Slovak Republic, Slovenia, Spain, United Kingdom);
- one EEA/EFTA Country (Switzerland). A table, considering all Council Recommendation areas, has been elaborated for each State, in order to identify parts of the documents relative to the Recommendation.

A double check (two researchers) has been done for each State included in the study and an analysis of data has been performed. Data analysis highlighted that all Recommendation areas were considered in these NP/S.

More specifically, area by area main evidence was the following:

- Area 1. PLANS AND STRATEGIES IN THE FIELD OF RD. All considered documents took into account the presence of a panel/committee of experts and indicated objectives of NP/S.
- Area 2. ADEQUATE DEFINITION, CODIFICATION AND INVENTORYING OF RD. National Registry for RD was included in all NP/S. Coding types mentioned most often were ICD (78%) and Orphacode (57%).
- Area 3. RESEARCH ON RD. In all documents Countries fostered participation to research projects on RD. In 79% research needs and priorities were included.
- Area 4. CENTRES OF EXPERTISE AND EUROPEAN REFERENCE NETWORKS FOR RD. Centres of Expertise and European Reference Networks were included in all NP/S. In 86% Exchange of expertise and multidisciplinary approach as well as healthcare pathways were considered.
- Area 5. GATHERING THE EXPERTISE ON RD AT EUROPEAN LEVEL. Training for health professionals and Orphan Drugs and medicines were included in all NP/S.
- Area 6. EMPOWERMENT OF PATIENT ORGANISATIONS. Consultation of Patient Organisations, in terms of inclusion in committees, were included in all NP/S, as well as information for Patients.
- Area 7. SUSTAINABILITY. The documents took into account general funds (57%) and specific funds (36%) for ensuring the sustainability of NP/S.

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Fig. 1. NP/S for RD (September 2015).

CONCLUSION

National Plans and Strategies for Rare Diseases are the common denominator of modern day public health policy concerns on rare diseases across the Europe. They conjugate a) the European common objective to ensure equal access and availability of prevention, diagnosis, treatment and rehabilitation for people with RD, with b) the National Authorities right to choose which specific measures for adoption and implementation. “The Council Recommendation on European Action in the field of Rare Diseases”, adopted by the EU Council in June 2009, recommended Member States to adopt National Plans or Strategies for rare diseases before end of 2013. By August 2015, 78% out of them satisfied this recommendation on time, while 22% adopted NP/S later. Coverage of all areas, although NP/S were published at different times, highlights the relevance of the EC Recommendation and adds value to the work done in the EUROPLAN project. It was quoted in the 86% of the NP/S published between August 2009 and August 2015. The EU co-funded project was launched in 2008. It foresaw 2 implementation phases: phase 1 (2008-2011) to build the consensus definition of operational tools (recommendations and indicators), and phase 2 (2012-2015, as Work Package 4 of the EUCERD Joint Action), mainly aimed at capacity building with the proactive involvement of multilevel stakeholders. EUROPLAN facilitated and accelerated the implementation of National Plans in almost all EU and several non-EU Countries. It contributed a) to bring the EC Recommendation to the attention of all stakeholders in EU Countries and b) to build a “double level” approach, respectful of national decisions but expected to ensure a coherent and consistent progress in EU rare diseases care. Even after the project officially ended in 2015, EUROPLAN website and Twitter profile are continuing to disseminate information about NP/S on RD. After all, EUROPLAN, as already stated, is a European and international process more than a project!