



Overview of the Rare Disease National Plans/Strategies in Europe

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New priorities after Commission Communication and Council Recommendation

Plans and strategies in the field of rare diseases

- **The Member States are invited to establish national or regional action plans for RD before End 2013 in order to implement the actions suggested in the Commission Communication and the Council Recommendation and to provide an annual report on the progress made toward this objective**
- **The Commission is providing European guidelines for the elaboration of these action plans for RD (EUROPLAN Project selected for funding for the period 2008-2011). National conferences have been organised by EURORDIS.**
- **A national plan/strategy (NP/NS) can be defined as the sum of integrated and comprehensive health policy actions for RD, to be developed and implemented at national level. A plan has: a) well specified objectives and b) actions that have to be supported by a budget, implemented within a time frame, evaluated with specific indicators (EUROPLAN Definition).**



New priorities after Commission Communication and Council Recommendation

Plans and strategies in the field of rare diseases

This definition includes two central concepts of the Council Recommendation on RD. '*Integrated*' refers to the fact that strategies should be developed in a way to identify complementarities, maximize synergies and avoid duplications. '*Comprehensive*' refers to the fact that the actions foreseen in the plan should fulfill all main patients' needs (e.g. quality of care but also social services and centers of expertise).

Definition of common and harmonized indicators to appraise and evaluate the national plan/strategy (NP/NS) of rare diseases.

The EUROPLAN Recommendations also includes the international dimension not possible to accomplish at national level (e.g. classification and codification, reference networks, orphan drugs, research, etc.).

Objectives for action: In the Council Recommendation Member States committed themselves to adopt a plan or strategy as soon as possible, preferably by the end of 2013 at the latest.

In 2009, a focus on rare diseases was relatively new and innovative in most EU Member States.

Before the adoption of the Council recommendation, most Member States based work on rare diseases on specific genetic services and strategies for patients with disabilities. The lack of initiatives and health policies specifically targeted at rare diseases resulted in delayed diagnosis and difficult access to treatment and care.

While being different in their clinical nature with specific pathogenic and clinical features, rare diseases share common issues from a health care systems perspective and require specifically targeted policies.

The existence of comprehensive and integrated approaches to the delivery of health care for rare diseases set down in national plans is crucial for improving access to high quality health care for patients. Healthcare services are usually not tailored to the needs of patients with rare diseases. National plans are considered as a key tool to address this situation and significantly improve healthcare pathways for rare diseases patients.

Before 2009 only a few Member States had national plans in place. These were Bulgaria, France, Portugal and Spain.

Implemented actions: Approaches adopted by Member States vary significantly and choices of specific measures are related to differences in the organisation of health care systems.

Currently (in May 2014) 18 Member States have adopted national plans or strategies.

Five more countries are well advanced in the development of their plans/strategies.

Countries vary considerably in their level of the implementation these plans. This is partially due to the fact that several countries like the UK, Germany, the Netherlands and Belgium only recently officially adopted their plans/strategies. Only one country, France, has already finished implementing the first plan and adopted a second national plan.

Most Member States have no dedicated specific budget for the implementation of national plans. Funding is usually provided as part of overall health spending.

Countries do provide occasional budgets for the implementation of specific projects. Some countries reported that budgets are under additional strain as a result of the economic crisis.

Despite their comprehensiveness and inter-sectorial approach, all plans were adopted at the level of the Ministry of Health. In the Czech Republic, in addition, the plan was also endorsed by the Prime Minister.

The scope of the rare diseases plans differs between countries. For example, while rare cancers are an important part of the rare diseases spectrum, several plans/strategies do not cover this group of diseases. This is true for Germany, France, Belgium, Denmark and Portugal. Denmark does not consider infectious diseases as rare diseases.

Fourteen countries have run information campaigns to raise awareness on rare diseases. Germany, Croatia, Cyprus and Latvia are currently preparing their campaigns.

Other countries with plans in place (Croatia, France, Lithuania, Portugal and Spain) base their monitoring strategy on EUROPLAN indicators. Bulgaria and Slovakia have no monitoring strategy. In the remaining countries monitoring strategies are under development.

b. European Commission activities

In order to support Member States in the process of developing national plans and strategies, the European Commission co-financed the EUROPLAN project from the Health programme.

The project, running from April 2008 to March 2011, involved representatives of national health authorities of 21 Member States. Some of EUROPLAN activities, especially those related to technical assistance to Member States with particular difficulties in the preparation of their national plan or strategy, are covered further by a specific work package of the EUCERD Joint Action.

Some conclusions

National Plans are of four main categories:

- Declarative**
 - Resolving problems between competent authorities (regions, tc.)**
 - Giving additional funding to public health, research or orphan medicinal products**
 - Integrated**
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- A serious problem persists in the fact that certain RD are part of othe national plans (cancer, others)**
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- Insufficient International or European dimension.**



DG SANCO priorities on rare diseases Web site

Public health actions

http://ec.europa.eu/health/rare_diseases/policy/index_en.htm

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Research actions

http://ec.europa.eu/research/health/medical-research/rare-diseases/index_en.html

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