



European Union action in the field of Rare Diseases: European Reference Networks

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Legal basis for the developments of the EU Public Health Policy

■ Based on Article 152 of the EU Treaty

■ A Community action programme on Rare Diseases, including genetic diseases, was adopted for the period of 1 January 1999 to 31 December 2003 with the aim of ensuring a high level of health protection in relation to RD. As the first EU effort in this area, specific attention was given to improving knowledge and facilitating access to information about these diseases.

■ Orphan Medicinal Product Regulation (Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products, was proposed to set up the criteria for orphan designation in the EU and describes the incentives (e.g. 10-year market exclusivity, protocol assistance, access to the Centralised Procedure for Marketing Authorisation) to encourage the research, development and marketing of medicines to treat, prevent or diagnose rare diseases.

■ For the period 2008-2013 the Commission has adopted the [White Paper COM\(2007\) 630 final "Together for Health: A Strategic Approach for the EU 2008-2013"](#) of 23 October 2007 developing the EU Health Strategy. Actions under Objective 1 of this EU Strategy cover a Communication on European Action in the Field of Rare Diseases and in point 4.1 of this EU Strategy it is suggested to put forward EC-level structured cooperation mechanisms to advise the Commission and to promote cooperation between the Member States.

Legal basis for the developments of the EU Public Health Policy

- **As a consequence Rare diseases are now one of the priorities in the [EU Health Programme 2008-2013](#). According to the DG SANCO Work Plans for the implementation of the Public Health Programme, the two main lines of action are the exchange of information via existing European information networks on rare diseases, and the development of strategies and mechanisms for information exchange and co-ordination at EU level to encourage continuity of work and trans-national co-operation.**
- **In the current Framework Programme, the FP7, the Health Theme of the "Cooperation" Specific Programme, is designed to support multinational collaborative research in different forms. The main focus of the Health theme in the rare diseases area are Europe-wide studies of natural history, pathophysiology, and the development of preventive, diagnostic and therapeutic interventions.**
- **Commission Communication COM (2008) 679/2 to the European Parliament, the Council, the Economic and Social Committee and the Committee of the Regions on Rare diseases: Europe's challenges creating an integrated approach for the EU action in the field of rare diseases. **Adopted 11th November 2008.****
- **Council Recommendation on a European action in the field of rare diseases recommending actions at national level to implement the EU action (e.g. National Plans for Rare Diseases). **Adopted 9th June 2009.****

Future legal basis for the developments of the EU Public Health Policy

■ **Decision of the Commission creating a European Union Committee of Experts on Rare Diseases during 2009. To be composed by 51 members representing Member States, patient's organisations, industry, FP Projects, Health Programme projects, etc.**

Adopted 30h November 2009.

■ **Directive of the European Parliament and of the Council on the application of patients' rights in cross-border healthcare (COM(2008)414) provides for the development of European reference networks (ERNs) to be facilitated by the Member States. The ERN for Rare Diseases will have a strategic role in the improvement of quality treatment for all patients throughout the European Union as called by the patients' organisations. To be adopted before the End 2010 (tbc).**



Directive of the European Parliament and of the Council on the application of patients' rights in cross-border healthcare (COM(2008)414)

Article 13: European reference networks

1. The Commission shall support Member States in the development of European reference networks between healthcare providers and expertise in the Member States. The networks shall be based on voluntary participation by its members, which shall participate and contribute to the networks activities according to the legislation of the Member State where they are established.

2. The aim of European reference networks shall be to help to:

- (a) realise the potential of European cooperation regarding highly specialised healthcare for patients and for healthcare systems from innovations in medical science and health technologies;**
- (b) facilitate access to diagnosis and to high quality and cost-effective healthcare for all patients with a medical condition requiring a particular concentration of expertise;**
- (c) maximise cost-effective use of resources;**
- (d) reinforce research, epidemiological surveillance like registries and provide training for health professionals;**
- (e) facilitate mobility of expertise, virtually or physically and to develop, share and spread information, knowledge and best practice within and outside the networks;**

Directive of the European Parliament and of the Council on the application of patients' rights in cross-border healthcare (COM(2008)414)

(f) Member States with an insufficient number of patients with a particular medical condition or lacking technology or expertise to provide highly specialised services.

3. For the purposes of paragraph 1, the Commission shall:

- (a) develop and publish criteria and conditions that the European reference networks should fulfil;**
 - (b) develop and publish criteria for evaluating European reference networks**
 - (c) facilitate the exchange of information and expertise in relation to the establishment of networks and their evaluation.**
- 4. The criteria and conditions referred to in paragraph 3 (a) and (b) shall be adopted in accordance with the procedure referred to in Article 16(2).**
- 5. Measures adopted according to this Article shall not harmonize any laws or regulations of the Member States and shall fully respect the responsibilities of the Member States for the organisation and delivery of health services and medical care.**

The Commission Communication and the Council Recommendation on rare diseases

- **There is probably no other area in public health in which 27 national approaches could be considered to be so inefficient and ineffective as with rare diseases. The reduced number of patients for these diseases and the need to mobilise resources could be only efficient if done in a coordinated European way.**
- **Article 152 provides for the adoption by qualified majority by the Council of Recommendations, on the basis of Commission proposals, for the purposes set out in that article.**
- **These Recommendations are the only legislative tool provided for in Article 152 on public health except for the few areas where measures or incentive measures may be adopted (see Article 152.4).**

New priorities after Commission Communication and Council Recommendation

Action to support implementation of National Plans for Rare Diseases

- **The Member States are invited to establish national or regional action plans for RD before 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 will provide European guidelines for the elaboration of these action plans for RD (EUROPLAN Project selected for funding on 2007). Appropriate international conferences will be organised (twelve national conferences scheduled during 2010).**
- **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

Action to support implementation of National Plans for Rare Diseases

- **This definition includes two central concepts of the Council Recommendations 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).**
- **Five Member States have adopted National Plans/Strategies for Rare Diseases: France, Bulgaria, Portugal, Greece, Spain.**
- **Three are in an advanced stage for adoption: Italy, Romania, Czech Republic**
- **Eighth have structures in place for the definition of a plan: United Kingdom, Germany, The Netherlands, Cyprus, Luxembourg, Belgium, Hungary, Austria.**

Still now priority was given to Pilot reference networks (centres of reference) for rare diseases

The suggested criteria to be fulfilled by the European reference networks are:

- **Sufficient activity and capacity to provide relevant services and maintain quality of the services provided**
- **Capacity to provide expert advice, diagnosis or confirmation of diagnosis, to produce and adhere to good practice guidelines and to implement outcome measures and quality control**
- **Demonstration of a multi-disciplinary approach;**
- **High level of expertise and experience documented through publications, grants or honorific positions, teaching and training activities**
- **Strong contribution to research**
- **Involvement in epidemiological surveillance, such as registries**
- **Close links and collaboration with other expert centres at national and international level and capacity to network**
- **Close links and collaboration with patients associations where they exist.**
- **Appropriate arrangements for referrals of patients from other Member States established within a framework.**
- **Appropriate capacities to diagnose, to follow-up and manage patients with evidence of good outcomes so far as applicable.**

Still now priority was given to Pilot reference networks (centres of reference) for rare diseases

The Work Plan 2006 for the implementation of the EU public health programme, introduces as a priority in the area of rare diseases: to develop European Networks of Centres of Reference for Rare Diseases. According to this priority 10 Projects have been selected for funding:

- **European Centres of Reference Network for Cystic Fibrosis with the Klinikum der Johann Wolfgang Goethe-Universität (DE) as Project Leader,**
- **European Network of Centres of Reference for Dysmorphology with The University of Manchester (UK) as Project Leader,**
- **Patient Associations and Alpha1 International Registry with the Stichting Alpha1 International Registry (NL) as Project Leader,**
- **European Porphyria Network: providing better healthcare for patients and their families with the Assistance Publique - Hôpitaux de Paris (FR) as Project Leader,**
- **Establishment of a European Network of Rare Bleeding Disorders, with the Università degli Studi di Milano (IT) as Project Leader.**
- **European network of paediatric Hodgkin's lymphoma – European-wide organisation of quality controlled treatment with the University of Leipzig (D) as Project Leader.**
- **European Network of Reference for Rare Paediatric Neurological Diseases (NEUROPED) with the European Network for Research on Alternating Hemiplegia (AT) as Project Leader.**
- **A reference network for Langerhans cell histiocytosis and associated syndrome in EU with Assistance Publique Hôpitaux de Paris (FR) as Project Leader.**
- **Improving Health Care and Social Support for Patients and Family affected by Severe Genodermatoses – TogetherAgainstGenodermatoses (TAG) with Fondation René Touraine (FR) as Project Leader.**
- **European Reference Network of expert centres in rare anaemias (ENERCA 3) with Hospital Clínic de Barcelona (ES) as Project Leader.**

New priorities after Commission Communication and Council Recommendation

Actions to develop national/regional centres of expertise and establish EU reference networks

- **To repertory in an EU list the existing Centres of Expertise identified throughout the Member States by the end of 2010;**
- **To establish a procedure for designation and accreditation methodology of EU Reference Networks for Rare Diseases (according to the future Directive on Cross-border health care);**
- **To provide adequate, long-term public funding to Centres of Expertise in order to ensure their sustainability and continuity of care for patients;**
- **To provide adequate, long-term public funding to European Reference Networks Centres of Expertise in order to ensure their sustainability and continuity of care for patients;**
- **To recommend inclusion in the National Plan for Rare Diseases provisions on the recognition and funding of Centres of expertise and their participation in European Reference Networks;**
- **To recommend the adoption of national initiatives in the National Plans for Rare Diseases on specialised social services;**
- **To provide financial support to networks of specialised social service;**

New priorities after Commission Communication and Council Recommendation

Actions to coordinate the policies and initiatives at EU level

- **An EU Committee of Experts on Rare Disease (EUECRD) will be created, by Commission Decision, in order to advise the European Commission. Members should be nominated by the national authorities. Experts leading Health Programme and FP initiatives should also be members. Observers from the patient's organisations and from the industry will also be proposed.**
- **The European Conferences on Rare Diseases will be organised every two or three years. Funds will be provided by the Health Programme. Next one in 2010 (Krakow, Poland, 13-16 May).**
- **Every four years the Commission should produce an Implementation report on the Commission Communication and Council Recommendation addressed to the Council, the Parliament, the Social and Economic Committee and the Committee of the Regions (Commission)**

DG SANCO priorities on rare diseases

Web site

http://ec.europa.eu/health/ph_threats/non_com/rare_diseases_en.htm





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