EU policy on rare diseases

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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.

Legal basis for the developments of the EU Policy on rare diseases

- **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.
Legal basis for the developments of the EU Policy on rare diseases

- **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 8th June 2009.

- **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 30th November 2009.

Legal basis for the developments of the EU Policy on rare diseases

- **Directive of the European Parliament and of the Council** of 9 March 2011 on the application of patients’ rights in cross-border healthcare (2011/24/EU) provides for the development of European reference networks (ERNs) by Commission and Member States. The ERN can improve the access to diagnosis and the provision of high-quality healthcare to patients who have conditions requiring a particular concentration of resources or expertise, especially for rare diseases. Deadline for transposition the 23th of October of 2013.
The Commission Communication and the Council Recommendation on rare diseases – Main priorities

I. Plans and strategies in the field of rare diseases
Calls on the MS to elaborate and adopt a plan or strategy by the end of 2013.

II. Adequate definition, codification and inventorying of rare diseases
Evokes the common definition of a rare disease as a condition affecting no more than 5 per 10 000 persons; aims to ensure that rare diseases are adequately coded and traceable in all health information systems based on the ICD and in respect of national procedures; and encourages MS to contribute actively to the inventory of rare diseases based on the Orphanet network.

III. Research on rare diseases
Calls for the identification and fostering of rare disease research at all levels.

IV. Centres of expertise and European reference networks for rare diseases
Asks the MS to identify and facilitate networks of expertise based on a multidisciplinary approach to care, and foster the diffusion and mobility of expertise and knowledge.

V. Gathering the expertise on rare diseases at European level
MS should share best practices, develop medical training relevant to the diagnosis and management of rare diseases, coordinate European guidelines, and, to minimise the delay in access to orphan drugs, MS should share clinical/therapeutic added-value assessment reports at the Community level.

VI. Empowerment of patient organisations
MS should consult patient representatives on policy development; facilitate patient access to updated information on rare diseases; promote patient organisation activities.

VII. Sustainability
Long-term sustainability in the field of information, research and healthcare of infrastructures must be ensured.
The Directive intends to clarify patients’ rights to access safe and good quality healthcare in another Member State (MS), and be reimbursed for it.

- Increase transparency by making mandatory for MS and healthcare providers to make public comprehensive and accurate information on the services, the possible treatment options, the prices, and the quality and safety of the services they provide.

- This Directive will increase cooperation between national health authorities:
  - National Contact Points
  - Cross-border recognition of prescriptions
  - EU structures to implement projects on European reference, eHealth and health technology assessment networks

**Art 12. ERN**

- Art. 12 of the Directive notably foresees enhanced cooperation of Member States in the area of European reference networks (ERN).

- Main goal is to facilitate improvements in the diagnosis and treatment of certain diseases of conditions across the EU:
  - By the delivery of high-quality, accessible and cost-effective healthcare
  - For patients suffering of medical conditions which could require a particular concentration of expertise or resources, particularly in medical domains where expertise is rare.
The Commission shall support MS in the development of ERN between healthcare providers and Centers of expertise in the Member States.

Participation in the ERN shall be voluntary. Its members shall participate and contribute to the networks' activities in accordance with the MS legislation where the members are established.

ERN shall be open to new healthcare providers which might wish to join them, provided that such healthcare providers fulfil all the required conditions and criteria.

Milestones and timeline for the implementation (ERN):

- Delegated Acts (Art. 17)
  - Experts group
  - Adoption of a list of criteria and conditions for the CR & ERN to fulfil Art. 12.2
- Implementing acts (Art. 16)
  - Committee
  - Facilitate the exchange of information and expertise for ERN Art. 12.4(c)
  - Develop and publish criteria for establishing and evaluating ERN Art. 12.4(b)
- Permanent supporting mechanism:
  - Coordination & Management
    - 1st identification
      - Centres of Reference at EU level
  - Designation
    - CR fulfilling the Criteria
  - Launching
    - 1st ERN
  - Evaluation & Management ERN
    - Evaluation existing RC & Designation new CR
    - Update of criteria, Conditions & identification Centers of Reference
Recommendations for Centres of Expertise adopted unanimously by the European Union Committee of Experts on Rare Diseases

Adopted on 24 of October 2011

Art 13. Rare diseases

The Commission shall support Member States in cooperating in the development of diagnosis and treatment capacity in particular by aiming to:

(a) make health professionals aware of the tools available to them at Union level to assist them in the correct diagnosis of rare diseases, in particular the Orphanet database, and the European reference networks;

(b) make patients, health professionals and payers of healthcare aware of the possibilities offered by Regulation (EC) No 883/2004 for referral of patients with rare diseases to other Member States even for diagnosis and treatments which are not available in the Member State of affiliation.
To translate the eHealth research into eHealth solutions for enhancing better and safer access to healthcare

- **Developing guidelines** of set of data to be included in patient summaries to be accessible in cross border healthcare by patients and doctors
- **Developing methodologies** for reuse of data from patients summaries or EHR for medical and public health research, including by establishing cross-border patient registries
- Supporting common **e-identification** and **authentication** measures for the health sector
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