European Union action in the field of Rare Diseases: funding opportunities

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Health and Consumers

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Defining and quantifying rare diseases

- **In EU countries, any disease affecting fewer than 5 people in 10 000 is considered rare according to the definitions adopted in the Orphan Drugs Regulation (EC) No 141/2000 and in the Commission Communication COM (2008) 679/2 on Rare diseases: Europe's challenges.**
- **That number may seem small, but it translates into approximately 246 000 people throughout the EU's 27 member countries.**
- **Most patients suffer from even rarer diseases affecting 1 person in 100 000 or more.**
- **It is estimated that today in the EU, 6000-8000 distinct rare diseases affect 6-8% of the population – between 27 and 36 million people.**
- **In the United States, the Rare Disease Act of 2002 defines rare disease strictly according to prevalence, specifically "any disease or condition that affects less than 200,000 persons in the United States,"or about 1 in 1,500 people.**
- **In Japan, the legal definition of a rare disease is one that affects fewer than 50,000 patients in Japan, or about 1 in 2,500 people**

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Legal basis for the developments of the EU Public Health Policy

Based on new Article 168 (former 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

Based on new Article 168 (former 152) of the EU Treaty

- **As a consequence Rare diseases are now one of the priorities in the Second EU Health Programme 2008-2013. According to the DG SANCO Work Plans for the implementation of the Public Health Programme, main lines of action and priorities are choosed very year. Work Plan for 2012 adopted.**

- **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 8th June 2009.**

Legal basis for the developments of the EU Public Health Policy

Based on new Article 168 (former 152) of the EU Treaty

- **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.**

- **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. Adopted 9th March 2011.**

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The Commission Communication and the Council Recommendation on rare diseases

The European Union approach

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.

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The Commission Communication and the Council Recommendation on rare diseases

The European Union approach

1. **Plans and strategies in the field of rare diseases**
2. **Adequate definition, codification and inventorying of rare diseases**
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**

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 (twelve conferences 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

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.).

The Continuity of EUROPLAN 2011-2013 (under the form of a Joint Action) is scheduled prioritising technical assistance and training for Member States with precise needs. Advice to be provided to non EU countries.

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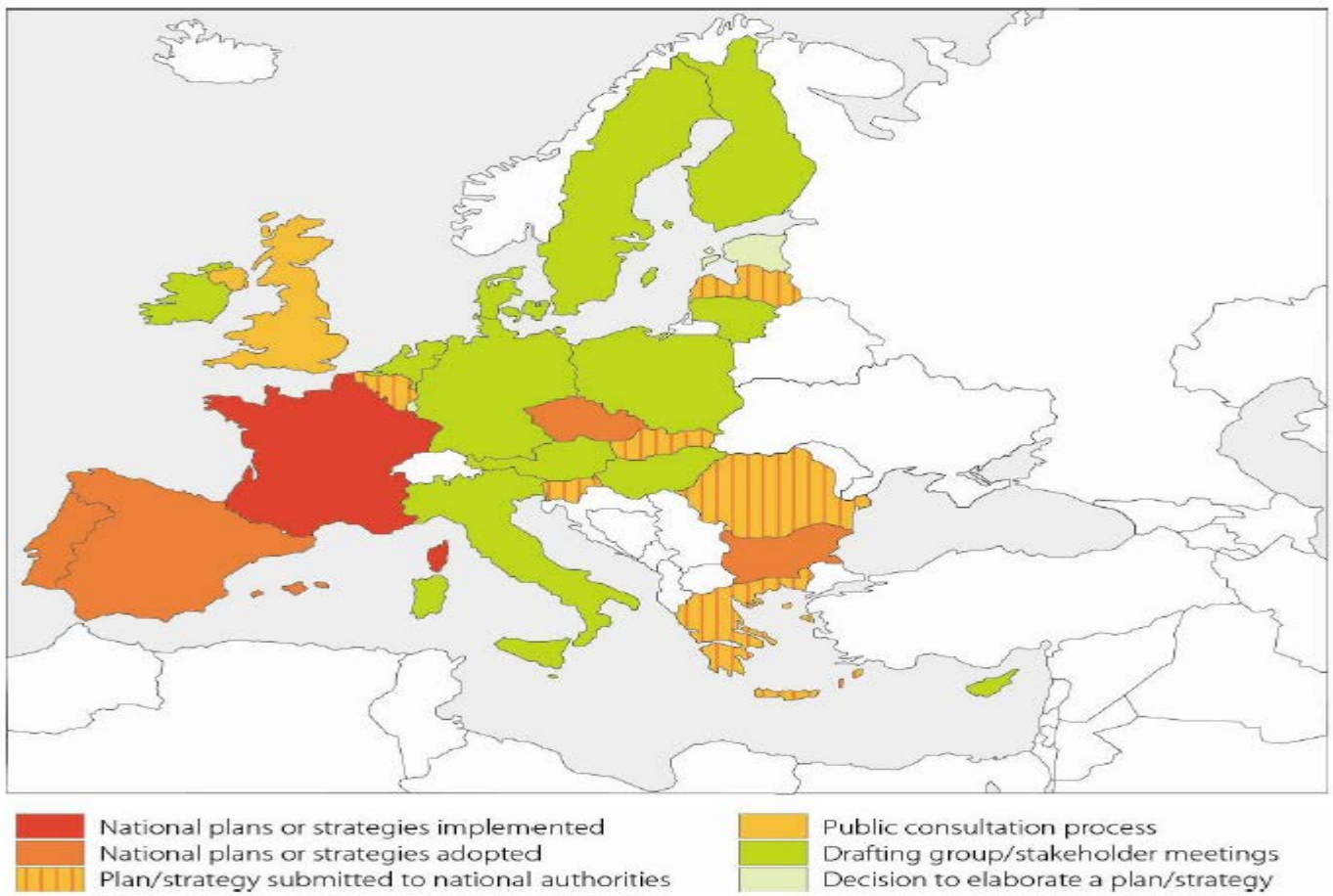
New priorities after Commission Communication and Council Recommendation

Plans and strategies in the field of rare diseases



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New priorities after Commission Communication and Council Recommendation

II. How to finance national plans on rare diseases

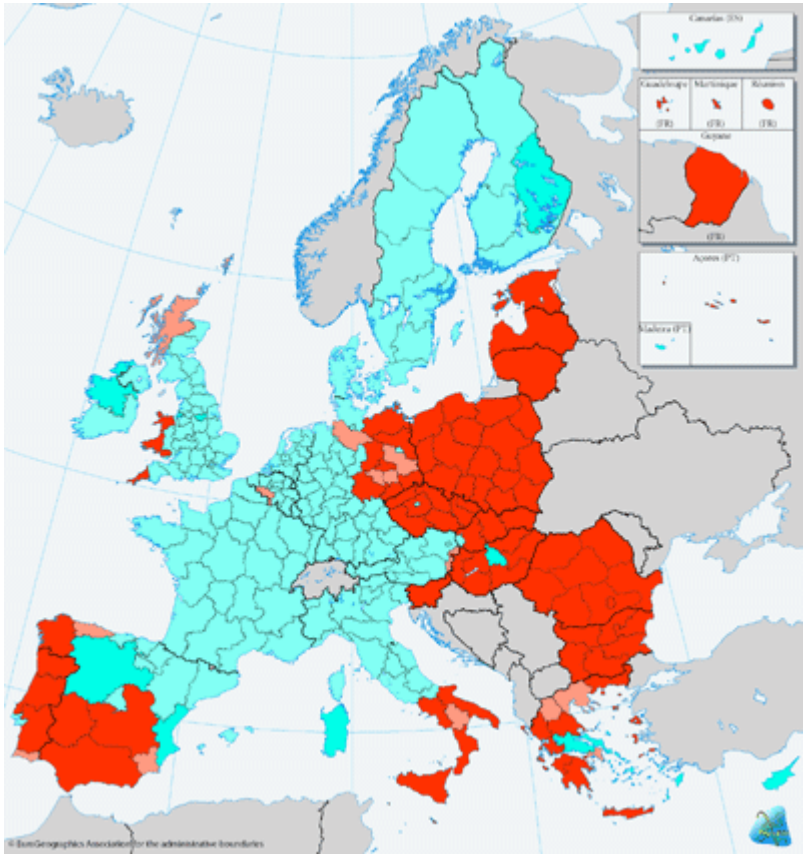
- **Structural Funds Regulation 2014-2020**
- **Horizon 2020**
- **Health Programme 2014-2020**
- **ERIC**
- **JASPERS**

European Commission

New priorities after Commission Communication and Council Recommendation

II. How to finance national plans on rare diseases

Convergence Regions
Phasing-out Regions
Phasing-in Regions
Competitiveness and Employment Regions



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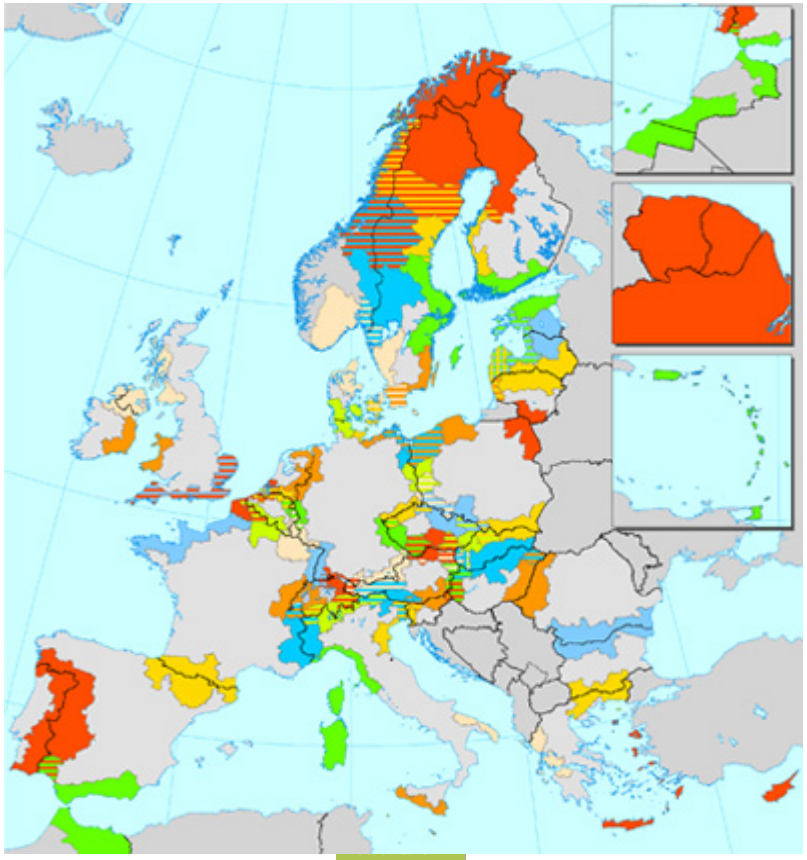
New priorities after Commission Communication and Council Recommendation

II. How to finance national plans on rare diseases: cross-border cooperation



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New priorities after Commission Communication and Council Recommendation

II. How to finance national plans on rare diseases:

Draft proposal launched in October 2011

Includes:

Common Provision Regulation and 5 specific Regulations on

- **European Regional Development Fund**
- **European Social Fund**
- **Cohesion Fund**
- **European Agricultural Fund for Rural Development**
- **European Maritime and Fisheries Fund**

EUR 376 billion 2014 - 2020

European Commission

New priorities after Commission Communication and Council Recommendation

II. How to finance national plans on rare diseases:

- **Scope of Support** Investments in social and educational infrastructure; in infrastructure providing basic services to citizens in transport and ICT
- **Investment Priorities** in health and social infrastructure which contribute to (...) transition from institutional to community-based services
- Common **indicators** to be used to measure the results of investments in growth and jobs

New priorities after Commission Communication and Council Recommendation

II. How to finance national plans on rare diseases:

Thematic objectives for the CSF Funds and Common Strategic Framework, article 9(9) of the

REGULATION OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL laying down common provisions on the European Regional Development Fund, the European Social Fund, the Cohesion Fund, the European Agricultural Fund for Rural Development and the European Maritime and Fisheries Fund covered by the Common Strategic Framework and laying down general provisions on the European Regional Development Fund, the European Social Fund and the Cohesion Fund and repealing Council Regulation (EC) No 1083/2006

In your country

Eligible areas

Programmes

Beneficiaries

Managing authorities

Managing authorities

The detailed management of programmes which receive support from the Structural Funds is the responsibility of the Member States. For every programme, they designate a managing authority (at national, regional or another level) which will inform potential beneficiaries, select the projects and generally monitor implementation.

Select a country and the contact information for the managing authorities of the various programmes will appear below on this page.

- Belgique - België
- България - Bulgaria
- Česká Republika
- Danmark
- Deutschland
- Eesti
- Ireland - Eire
- Ελλάδα - Ellada
- España
- France
- Italia
- Κύπρος - Kýpros
- Latvija
- Lietuva
- Luxembourg
- Magyarország
- Malta
- Nederland
- Österreich
- Polska
- Portugal
- România
- Slovenija
- Slovensko
- Suomi - Finland
- Sverige
- United Kingdom

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EU Regional Policy: Stay informed

New priorities after Commission Communication and Council Recommendation

III. Research on rare diseases

- *FP5 (1998-2002): 47 projects funded, € 64 million in total*
- *FP6 (2002-2006): 59 projects funded, € 230 million in total*
- *FP7 (2007-2013) 66 ongoing projects: EC support around € 325 million*

Horizon 2020 is the financial instrument implementing the Innovation Union, a Europe 2020 flagship initiative aimed at securing Europe's global competitiveness. Running from 2014 to 2020 with an €80 billion budget, the EU's new programme for research and innovation is part of the drive to create new growth and jobs in Europe.

Horizon 2020 provides major simplification through a single set of rules. It will combine all research and innovation funding currently provided through the Framework Programmes for Research and Technical Development, the innovation related activities of the Competitiveness and Innovation Framework Programme (CIP) and the European Institute of Innovation and Technology (EIT).

New priorities after Commission Communication and Council Recommendation

III. Research on rare diseases

The proposed support for research and innovation under Horizon 2020 will:

- **Strengthen the EU's position in science with a dedicated budget of € 24 598 million. This will provide a boost to top-level research in Europe, including an increase in funding of 77% for the very successful European Research Council (ERC).**

- **Strengthen industrial leadership in innovation € 17 938 million. This includes major investment in key technologies, greater access to capital and support for SMEs.**

- **Provide € 31 748 million to help address major concerns shared by all Europeans such as climate change, developing sustainable transport and mobility, making renewable energy more affordable, ensuring food safety and security, or coping with the challenge of an ageing population.**

New priorities after Commission Communication and Council Recommendation

IV. JASPERS (Joint Assistance to Support Projects in European Regions)

JASPERS ('Joint Assistance to Support Projects in European Regions') is a technical assistance partnership between the European Commission (DG Regional Policy), the European Investment Bank, the European Bank for Reconstruction and Development and Germany's Kreditanstalt für Wiederaufbau.

JASPERS provides technical expertise to thirteen countries: the twelve EU Member States which joined the EU in 2004 and 2007 (Bulgaria, Czech Republic, Cyprus, Estonia, Hungary, Latvia, Lithuania, Malta, Poland, Romania, Slovakia and Slovenia) and, since 2011, Croatia, in anticipation of EU accession, scheduled for mid-2013.

JASPERS operates on the basis of Annual Action Plans for each Member State prepared in cooperation with the national authority concerned and the European Commission. A Managing Authority acts as a central coordinator for each country and it can request assistance from JASPERS.

New priorities after Commission Communication and Council Recommendation

IV. ERIC (European Research Infraestructure)

An ERIC has legal personality based on EU law (Article 171 of the EC Treaty). Its main tasks are establish and operate a research infrastructure.

The ERIC is an easy-to-use legal instrument providing:

the spirit of a truly European venture (also allowing the participation of non-European countries)

- **a legal personality recognised in all EU Member States**
- **flexibility to adapt to the specific requirements of each infrastructure**
- **some privileges/exemptions allowed for intergovernmental organisations**
- **a faster and more cost-efficient process than creating an international organisation**

An ERIC can benefit from exemptions from VAT and excise duty in all EU Member States and it may adopt its own procurement procedures, which have to respect the principles of transparency, non-discrimination and competition but are not subject to public procurement procedures.

New priorities after Commission Communication and Council Recommendation

IV. ERIC (European Research Infraestructure)

Members will be states and intergovernmental organisations.

At least three Member States agree to establish and operate together a research infrastructure. Associated countries, third countries and intergovernmental organisations may also be members.

The members agree on statutes ruling governance, IPR policy, financing, etc.

The seat has to be in an EU-Member State or in a country associated to the EU Framework programmes.

The members submit the file to the Commission, which, with the aid of independent experts, examines whether the conditions of the ERIC Regulation are fulfilled. After that, a committee composed of representatives of the EU Member States gives an opinion on the file by qualified majority, following which the Commission decides on the application.

New priorities after Commission Communication and Council Recommendation

Governance and monitoring

- The Commission is assisted by an EU Committee of Experts on Rare Diseases (EUCERD) to advise on implementation of the Communication and the Recommendation.
- The Committee is chaired by Ségolène Aymée (INSERM, FR) and assisted by a Scientific Secretariat, supported through the Health Programme.
- Composed by 51 members representing Member States (27+EFTA+ Candidates), patient's organisations, Pharmaceutical industry, FP Projects, Health Programme projects and ECDC + 12 Commission and EU agencies representatives (SANCO, RTD, ENTR, EMA, COMP, EAHC).
- This committee will replace the existing EU Rare Diseases Task Force.
- The EUCERD adopted a Road Map 2011-2013 submitted by the European Commission. An specific Working Plan for the EUCERD is also adopted.

New priorities after Commission Communication and Council Recommendation

Governance and monitoring

The EUCERD is a World Reference in the participation of all stakeholders in the definition and collaboration in Rare Diseases Policy.

Applications to cooperate with the EUCERD received from non EU countries (Japan, Russia, others). US is creating a similar committee following EU model.

The EUCERD newsletter 'Orphanews' is disseminated two times per month.

Workshops from EUCERD:

- **Iniciatives and incentives**
- **Registers**
- **Accesibility to orphan drugs**
- **Specialised social services**
- **Standards of care**
- **Newborn screening**
- **Classification and codification**
- **E-health and rare diseases (tbc)**

DG SANCO priorities on rare diseases
Web site

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

Contact points at DG SANCO
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Research actions
http://ec.europa.eu/research/health/medical-research/rare-diseases/index_en.html

Contact point at DG RTD
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