



EURORDIS
Rare Diseases Europe

C O N T R I B U T I O N

**EURORDIS' RESPONSE
TO THE DISCUSSION DOCUMENT
FOR A HEALTH STRATEGY**



12 February 2007

EURORDIS - the European Organisation for Rare Diseases – is pleased to send its contribution to the Commission’s Discussion Document for a Health Strategy “Health in Europe: A Strategic Approach”.

EURORDIS represents more than 270 rare disease organisations from 32 countries, 19 of which are EU member states, and thereby reflects the voice of an estimated 30 million patients affected by rare diseases in the European Union. Rare diseases are those affecting less than 5 out of 10,000 citizens.

1. Legitimacy of Community action in the field of Public Health and rare diseases

Rare disease patients strongly feel that the European Community is legitimately expected to take action in health-related matters. In fact, according to the principle of subsidiarity, “*the Union does not take action (except in the areas which fall within its exclusive competence) unless it is more effective than action taken at national, regional or local level*”. The legitimacy of Community action in the Health arena clearly appears when combining the principle of subsidiarity as defined above with the legal basis for EU action in the area of Public Health (art. 152¹). Consequently, Eurordis considers that the Community has a very important role to play.

It is also important to stress that the specificities of rare diseases make them a unique area of very high European added-value: given both the limited number of patients and the limited knowledge and expertise on rare diseases in a country, actions in the area of rare diseases at the national level, while being necessary, cannot be regarded as sufficient. In the last few years, the necessity for tackling the issue of rare diseases at the EU level has been progressively acknowledged both by national and European authorities, as well as all by concerned stakeholders.

Moreover, the “leverage effect” of activities in the area of rare diseases, together with their high European added-value, are fundamental elements in the strengthening of the legitimacy of Community action in this specific field. The diversity of more than 6,000 rare diseases, all severe to very severe, chronic, often degenerative and life-threatening, opens an endless field for research and innovation.

¹ “A high level of human health protection shall be ensured in the definition and implementation of all Community policies and activities. Community action, which shall complement national policies, shall be directed towards improving public health, preventing human illnesses and diseases, and obviating sources of danger to human health”.

By nature, rare diseases are at the forefront of future progress in the following areas:

- Reduction of inequalities in access to health services;
- Comprehensive approach of care to patients;
- Compensation of inequalities and disabilities;
- New therapies.

The Discussion Document on Health Strategy elaborated by the Commission covers a broad scope. It aims at stimulating responses for concrete objectives and ways of implementing those objectives. EURORDIS' contribution will therefore focus exclusively on key and concrete proposals in the area of rare diseases.

EURORDIS' objective, through the present contribution, is two fold:

First **to consolidate** the position of rare diseases as a priority in the political agenda of European decision-makers, by capitalising on existing achievements, and second, **to go beyond** what has already been accomplished in the area of rare diseases.

2. Concrete overarching objectives

2.1 Fighting against the impact of rare diseases on the lives of patients, particularly through the improvement of access to diagnosis, information, treatment and care, and the exchange of good practices; and the improvement of quality of life through patient support, social, and educational services;

2.2 Promoting research at both fundamental and clinical levels to develop medicinal products and therapies aimed at treating, relieving and curing rare diseases patients.

We strongly feel that these objectives should be shared by the European Community as a whole, and therefore included within the new strategic framework to guide the Commission's future work in the field of Public Health².

3. Consolidation and improvements

It must be stressed that much was achieved for rare diseases in the last decade. Patients living with rare diseases have come out of the shadow; the relentless work of patient organisations has ensured progress: in healthcare and social assistance provided to patients and families; in the development of treatments; and in raising public awareness on rare diseases.

² « Rare Diseases : understanding this Public Health Priority », http://www.eurordis.org/IMG/pdf/princeps_document-EN.pdf

Since 1999 the European Commission has gradually developed a progressive approach towards rare diseases. This includes the Orphan Drugs Regulation, the priority on rare diseases in the 7th Framework Programme of Research & Development, and in the Community Action Programme on Public Health. In addition, some Member States have developed specific policies on rare diseases at national level.

These policies at both European and national levels provide a solid basis on which further policy development can be fostered for the benefit of European “patients-citizens” because much remains to be done. It is also important to stress that time should be looked at differently for rare diseases than for more widespread diseases: because of lack of information and expertise on rare diseases and scarcity of patients, an action that lasts 5 years is still too short for rare diseases.

4. Proposed instruments and methods:

4.1 Methods

Eurordis wishes to recommend two parallel methods to follow simultaneously in order to secure the best possible results in nearing to our final objectives (as defined in paragraph 2):

- On the one hand, promote national policies in Member States and increase cooperation between Member States;
- And at the same time, coordinate action at EU level, guided by common guidelines.

4.2 Concrete instruments

The instruments that EURORDIS proposes are aimed at promoting national policies for rare diseases within EU Member States, while linking these national efforts with a common strategy at European level. This “double-level” approach will ensure that progress is globally coherent and follows common orientations throughout Europe.

EURORDIS’ concrete proposals are as follows:

4.2.1 The elaboration of a Commission Communication and Council Recommendations on rare diseases

This step is essential because it will ensure that **common policy guidelines** are shared everywhere in Europe and that specific actions - in areas such as research, centres of expertise, access to information,

incentives for the development of orphan drugs, screening, etc. –take place within an overall **minimum common strategy** on rare diseases.

We also believe that this instrument will reinforce **cooperation** between Member States, within a Community framework. In this context, the European Community has a fundamental role to play in the **establishment of different types of Networks**, such as European networks of Reference Centres, European research platforms, European information web server (Orphanet), European network of rare disease help lines, European networks of rare disease patients (Eurordis), and European networks of services to patients.

4.2.2 The creation of an EU High Level Group on rare diseases (HLGRD)

The HLGRD will represent the **political body** where Member States - working together and with the European Commission to implement the Commission Communication and Council recommendations - will be represented and given an appropriate platform/forum for discussion. The HLGRD will regularly **stimulate action** in line with the agreed common policy guidelines and could also serve as a **monitoring** body. The assessment exercise would be performed according to specific indicators, which still need to be defined.

The HLGRD will strengthen Community policies and allow for a closer cooperation between EU Member States willing to engage in such a process. It would include representatives of the Member States taking part in this process and rare disease patient organisations.

4.2.3 The creation of a Community Agency for Rare Diseases (CARD)

The Community Agency for Rare Diseases will respond to the need to establish a permanent, sustainable instrument for the long-term implementation of rare diseases policies at Community level.

According to the definition, “A Community agency is a body governed by European public law. It is distinct from the Community Institutions and has its own legal personality. It is set up by an act of secondary legislation in order to accomplish a **very specific technical, scientific or managerial task**”.

EURORDIS believes that an Agency could be an excellent instrument to ensure the permanence and coherence of relevant strategies at EU level in

areas such as patient registries, biobanks, clinical trials, information on rare diseases, networks of centres of reference, quality assessment and consensus clinical care recommendations, etc.

5. Health in all policies

As underlined by the Discussion Document, EURORDIS believes that it is very important **to explore synergies and work with other relevant policy areas** to ensure that actions performed with a health impact in other sectors are consistent with the main objectives as defined in paragraph 2, and do not have any counterproductive effects on these objectives.

The sectors currently identified as the most relevant for taking rare diseases into due account are the following ones:

- **Pharmaceutical policy** (orphan drugs, paediatric drugs, advanced therapies medicinal products, compassionate use, long term risk management...,etc.). An important aspect related to medicinal products is also the need to develop instruments at EU level aimed at monitoring and removing delays in accessing new drugs and treatments;
- **Research policy** (biomedical, public health policy, social, ethics, etc...);
- Strategy for Community action on **Health Services** (including patient mobility and mobility of health professionals) and for the establishment of European Networks of Centres of Reference. In this area, EURORDIS wishes to underline the specific need for rare diseases patients to have a privileged access to mobility across border and the need for clarification of patients' rights when seeking second opinion and medical treatment in countries different than the one of origin. Legal security is also needed with regard to procedures and levels of reimbursement when returning "home". It is also necessary to develop standards for a comprehensive approach to care for rare diseases patients across the EU;
- **Information policy**, including information to patients and health professionals, but also the strategy for the creation of an EU "information society" as a whole;
- **Social policies**, including disability policy, compensation of inequalities, social assistance, residential care and respite services;
- **Education policy**, including integration at school and "life-long learning", as well as integration at the work place.

6. Global issues

As already expressed in the EURORDIS Position Paper on the “WHO Report on Priority Medicines for Europe and the World - November 2004³”, rare diseases represent a global challenge and call for Public Health answers. Patients living with a rare disease share the same fundamental concerns wherever they live: access to diagnosis and information, access to care, social integration and recognition, etc.

Research is global, drug development and markets are global, even more so in the “niche” of drugs for treating rare diseases. Therapeutic advances in Europe will benefit people in countries throughout the world.

It is also worth stressing that rare diseases can be extremely useful as models to understand other much more widespread diseases, as it is already the case for cholesterol, malaria, plague, obesity and mental illnesses. “Furthermore, many orphan medicinal products are innovative biotechnological products that have been the start for several small biotech-companies⁴”.

EURORDIS believes that the European Community also has an important role to play in speeding up the transfer of designated orphan drugs from the EU to other continents to improve access and availability worldwide.

Because patient representatives and relevant stakeholders have already experienced the need for, and the outcomes of, coordinated international actions in Europe, Eurordis (together with key public and private players) actively participates in the establishment of an International Coordination for Orphan Drugs and Rare Diseases (ICORD).

Europe at large has a key responsibility in liaising with its North-American, as well as Eurasian and Mediterranean counterparts, to promote rare disease health strategies worldwide, including developing countries.

In a nutshell, the 30 million patients living with rare diseases in Europe are waiting for the EU to take much needed action on their behalf. Eurordis is willing to be a partner in the establishment of this new policy.

³ http://www.eurordis.org/IMG/pdf/eurordis_position_WHO__priority_medicines_dec04.pdf

⁴ Background Paper on rare diseases used for the elaboration of the “WHO Report on Priority Medicines for Europe and the World - November 2004”.