European Commission
Health and Consumer Protection Directive-General
Rare Diseases consultation
HTC 01/198
11, Rue Eugène Ruppert
L-2557 Luxembourg

Brussels, 14th February 2007
Ref.008-030

Dear Madam/Sir,

Re: Position of the European Disability Forum on the Public consultation of the European Commission “Rare diseases: Europe’s challenges”

The European Disability Forum (EDF) welcomes the possibility to participate in the public consultation on “Rare Diseases: Europe’s challenges”.

The European Disability Forum (EDF) is the European umbrella organisation representing the interests of 50 million disabled citizens in Europe. EDF membership includes national umbrella organisations of disabled people from all EU/EEA countries, accession countries and other European countries, as well as European NGOs representing the different types of disabilities. The mission of the European Disability Forum is to ensure disabled people full access to fundamental and human rights through their active involvement in policy development and implementation in Europe.

EDF promotes the Human Rights approach to disability. EDF believes that the inclusion of people with disabilities in European societies should be based on legal frameworks to prevent discrimination against people with disabilities and on the promotion of positive action. The most important legal development on this area took place in December 2006 with the adoption of the UN Convention on the Rights of people with disabilities.

The disability movement is inclusive of all people that due to barriers in society face discrimination to access health, education, services, employment, etc. This is the reason why the European disability movement represents also people with disabilities that have been traditionally excluded from the disability strategies. Examples of those excluded movements are people with psycho-social disabilities and also people with a chronic illness, rare or invisible diseases. EDF has fought for 10 years against the narrow definition of disability that, in many countries, is excluding the groups mentioned before from the protection of anti-discrimination legislation as well as from the social inclusion strategies. People with a rare disease can therefore suffer a second type of discrimination. People with rare diseases can develop impairments. Since this impairment is developed in the frame of the disease, some public authorities do not consider people with rare diseases as a person with a disability. This excludes persons with a rare disease from any legal protection, benefits, etc…

All stakeholders on the field of health have to endorse the needs of people with a rare disease to overcome the medical model that considers just their condition as patients and not as citizens with the same rights than other fellow citizens in all areas of life. Access to health is a pre-condition to social inclusion for people with a rare disease and therefore all health strategies should be embedded on the greater objective of full participation in society of people with a rare disease. EDF much welcome the reference to the fight against discrimination on the working document of the European Commission.

People with a rare disease are citizens, which in some cases face invisible disabling conditions, just like other disabled people. This situation drives them to experience the lack of understanding of other people in society and the lack of support from public authorities. In some cases degrading treatment exists where the law does not already protect people with rare diseases. Therefore the inclusion of people with rare diseases into non discrimination legislation is fundamental.

People depending on a long term treatment should fully participate in the decisions regarding their treatment. Health systems should take into account the views of people with a rare disease and of their representative organisations in adopting health policies. Moreover, dignity and confidentiality of data are still unprotected in many countries of the European Union. Measures proposed in the Community action on the field of cooperation, collection and exchange of data and information should be carefully analysed in the perspective of dignity and confidentiality.

Increasing the capacity and coordination of European member states will benefit the life expectancy and condition of people with a rare disease. The improvement of social services for people with a rare disease and encouraging industry and public authorities to tackle the needs of “orphan diseases” are key steps on the enhancement of the quality of the lives of people with a rare disease.

The capacity of the organisations representing people with rare diseases should be supported at European and at national level. Civil society is one of the main elements to empower persons with rare diseases. Empowerment of persons with rare diseases is a key element in the success of a European strategy. This will ensure more success and sustainability of the strategy. EDF much welcomes the inclusion of this aspect in the document presented by the Commission. It is nevertheless important to overcome considering people with rare disease just patients, in order to look at them as citizens facing needs other than just health care and facing barriers other than just in accessing health services.

EDF much welcomes the initiative to include efforts on the field of research in the FP6 and FP7 programmes. Research is one of the key areas to prevent rare diseases and to improve quality of life of persons with those diseases.

EDF considers than in the objectives of the strategy there should have been included, better participation of persons with a rare disease in the process through their representative organisations. It should be required also better non-discrimination approaches and active inclusion policies to guarantee proper health services and ensure better participation of people with a rare disease in the society.

After consultation with the EDF Chronic Illness committee, and members of EDF representing persons with rare diseases, EDF has issued this response tackling some of the questions raised by the European Commission.

Main Points:

Question 1: Is the current definition of a rare disease satisfactory?

Members of EDF agree with the current definition and believe that it is a good framework for all EU countries. It is important that the definition does not move to a more restrictive definition that would leave out the rare diseases with more persons affected.

EDF insists also on the need that the protection on anti-discrimination principles in favour of people with disabilities should be extended to all people with a chronic illnesses and a rare disease. It is important to understand that people with a rare disease can develop impairments that could affect their daily living. The barriers in society and the effects of the impairment are the same than the ones faced by other disabled persons. It is not understandable why there are still national legislations leaving out of the protection on antidiscrimination and benefits support people with rare diseases developing impairment and suffering social barriers.

Question 8: Do you envisage the solution to the orphan drugs accessibility problem on a national scale or on an EU scale?

To answer this question, an EDF member from Estonia answered the following: *“this is important for us and I think for the other small countries as well. In Estonia we cannot buy drugs for one Estonian child with a concrete diagnosis of a rare disease. The Estonian Sick Fund argues that there is not enough budget to cover such an expensive treatment”*

There is an urgent need to guarantee access to all drugs in Europe equally to all citizens. This requires an EU scale response to support persons with rare diseases living in countries where treatments are not accessible. A more unified and clear market of “orphan drugs” would benefit the users and the industry.

EDF member DEBRA Europe proposes: “*Crucially, accessibility will require national commitment to implementation, for example: to allow licensing appraisal processes to be tailored to rare diseases rather than applying standard methods where cost-effectiveness drives the outcome and may not be representative of the value of the treatment and to ensure all prescribing physicians are made aware when innovative products become available so that all patients will be treated with the right medicine at the right time. Both resources and expertise will be needed to secure this commitment at national level.”*

Question 10: What kind of specialised social and educational services for RD patients and their families should be recommended at EU level and at national level?

People with rare disease should have equal opportunities and rights to use all social resources – participate in the educational system, have a similar employment rate than other non-disabled people, enjoy new technologies, healthcare and social services, sports and leisure activities, consumer based benefits and services.

It is infinitely important, that people with a rare disease are aware about their rights in the employment system, social sector, access to transportation and cultural life. Social model recognises that people with a rare disease have the right to participate in the society. Organisations of people with a rare disease should ensure that awareness-raising strategies take place inside their organisations. It is important that organisations support the demands, based on a social approach, of their members. In order to do so, organisations should receive financial support from the public authorities in order to empower their members.

Social services for people with a rare disease have been traditionally understood as medical care. It is important to expand this concept in order to integrate all kind of services that are linked to the full inclusion of a person with a rare disease in society.

The Communication from the commission on services of general interest, including social services of general interest[1], and in reference to the Protocol annexed to the Lisbon Treaty on services of general interest, recognises important operational principles such as:

- Respecting the diversity of services, situations and needs and preferences of users
- Achieving a high level of quality, safety and affordability
- Ensuring equal treatment and promoting universal access
- Upholding user rights

All these principles are of utmost importance for people with a rare disease and their organisations will closely follow their implementation in concrete European and national policies.

Supporting professionals in order to improve their capacities to better tackled the needs of a patient with a rare disease. Educational services enhancing their connexion with European expert networks are a key element for the success of the strategy.

It is important to note that many people with rare diseases may feel excluded by society and sometime even by their family and friends. There is particularly a need to work to remove attitudinal barriers in society. Awareness raising and education for the whole society is very important.

Question 12. How do you see the role of partners (industry and charities) in an EU action on rare diseases? What model would be the most appropriate?

The main conditions to ensure a fruitful dialogue between industries, charities and public authorities should be based in the following conditions:

- Real structured dialogue with civil society: This means full involvement of organizations representing people with rare diseases in the preparation, implementation and evaluation of public policies.
- Real involvement of persons with chronic illness in all stages of their treatment.
- Full information to patients on the situation of the research and industrial capacity on a rare disease.
- Funding support to civil society to ensure full participation all along the process and to become a stable and sustainable partner.

---

[1] Communication from the Commission to the European Parliament, the Council, the European Economic and Social Committee and the Committee of the Regions – Accompanying the Communciation on “A single market for 21st century Europe” Services of general interest, including social services of general interest: a new European commitment COM (2007) 725.

- Capacity building strategies to enhance the capacity of civil society on the field of research
- Supporting projects on the field of research involving charities, industry and public authorities.

<u>Question 13: Do you agree with the idea of having action plans? If yes, should at be at national or regional level in your country?</u>

Based on the experience of action plans in other areas (National Reform Programmes on the Lisbon objectives, the National Action Plans on social inclusion or the Operational Programmes for the Structural Funds), it is important that there is an integrated approach putting together all levels of decision making.

One of the main problems of the National Action Plans is when they are not integrated in the priorities of the national authorities. We can see that in the case of the NAPs Social inclusion since in many cases they are disconnected from the priorities of the national authorities. Sometimes they look as something produced for the European Commission services, there is not a real endorsement at national level. They should include also clear targets, deadlines and national, regional and local budget allocation.

The action plans should be created at regional and local level. The evaluation of these plans should be done at European ministerial level, issuing recommendations for the countries. It is important to involve civil society all along the action plans.

<u>Question 14: Do you consider it necessary to establish a new European Agency on RD and to launch a feasibility study in 2009?</u>

The creation of an Agency and a feasibility study will certainly increase the political commitment from the European Union to address the issue of rare diseases. The European Union should nevertheless direct its efforts to create a comprehensive network at EU level where industry, persons with a rare disease and public authorities can act and ensure better life conditions for people with a rare disease.

Finally, EDF would like to welcome again this consultation and remain at your disposal for any further clarification on the content of this document.

Yours sincerely,

Yannis Vardakastanis
President of EDF