PUBLIC CONSULTATION – RARE DISEASES: EUROPE'S CHALLENGES

by

European Commission, Health & Consumer Protection Directorate-General, Unit C 2

Comments from:	**GBS Initiative e.V.**
Author:	Harald Niemann, Vice Chairman
Character of the organisation:	Registered association
Members:	> 600
Country:	Germany, with close contacts to neighbouring countries
Disease:	Guillain-Barré-Syndrom; acute or chronic disease of the peripheral nervous system. Arms and legs become inflamed and stop working. Sudden weakness, leading to limb paralysis and a loss of sensation, sometimes with heavy pain. Neither hereditary nor contagious. Causes may be throat or intestinal infection, influenza or something else a few weeks before. These infections trigger an incorrect response in the immune system which attacks the nerves.
Prevalence:	1 – 2 people per 100.000
Date:	9 February 2008

Comments

We greatly appreciate this Commission initiative. In section 1 to 3 the main problems of people suffering rare diseases are addressed. We kindly encourage the Commission to continue tackling the identified problems, in particular considering rare diseases as a priority in the EU public health programme.

We have also examined the questions in section 4 and would like to give the following comments.

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

As far as our disease is concerned, the answer is: Yes. However, we are member of EURORDIS and feel as a part of the rare disease community. We advise the Commission to follow closely EURORDIS' comments.

Question 2: Do you agree that there is a pressing need to improve coding and classification in this area?

Yes.

Question 3: Can a European inventory of rare diseases help your national / regional system to better deal with rare diseases?

Yes. Orphanet's data base can be helpful to establish that inventory.

Question 4: Should the European reference networks privilege the transfer of knowledge? The mobility of patients? Both? How

European reference networks should first of all support the exchange of knowledge. It is desirable to have a common platform of rare diseases for both physicians and patient groups. That should work as follows:

(1) If a physician fails to diagnose the symptoms he or she should have the opportunity to put the symptoms into a section of the network and gets a link to possible diseases. That would make a diagnosis easier.
(2) When the possible diagnosis appears after entering the symptoms into the platform at the same time links to the respective patient groups should appear.

With this we have two advantages: Help for right diagnosis and the possibility for the physician to recommend patient groups to his patient.

Question 5: Should on-line and electronic tools be implemented in this area?

Yes.

Question 6: What can be done to further improve access to quality testing for rare diseases?

Include the data in a European network (see answer to question 4) accessible for both scientists and patients.

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

It is vital to have the same access to orphan drugs within the EU. It is unacceptable within a common market to have different levels of health. Yes, a solution on an EU scale is a must.

Question 9: Should the EU have an orphan regulation on medical devices and diagnostics?

Yes, and it is supposed to contain equal access for all EU citizens.

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

GBS patients are often transferred to care while there is still the possibility to make them fit for a life without being subjected to care. We want to encourage physicians to treat the patients instead to transfer them to care. Thus we need better educated personnel in the hospitals.

Question 11: What model of governance and of funding scheme would be appropriate for registries, databases and biobanks?

Being affected by a rare disease is a fate. It is a matter of solidarity to tackle it. The community of insured should cover the costs, e.g. by a certain amount per insured.

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

We would like to welcome a member state's duty to establish national action plans. The EU should require such plans in every member state. They should be agreed with the national patient organisations and submitted to the EU.

Question 14: Do you consider it necessary to establish a new European Agency on rare diseases and to launch a feasibility study in 2009?

Only if the EU fails to include the specific needs of patients suffering rare diseases in the current policy. A feasibility study is superfluous: Rare disease patients need help not a feasibility study!

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