



For the attention of:  
European Commission  
Health and Consumer Protection  
Directorate-General  
Rare Diseases consultation  
HTC 01/198  
11, Rue Eugène Ruppert  
L-2557 Luxembourg

**Object: Public Consultation regarding Community action on rare diseases**

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**Page 4 – 4th bullet point**

LFB Comment: Such patients, as any other human being, have the right to all these requirements: prevention, diagnosis and treatment. The point is that if they do not get diagnosed, they will not **benefit** from prevention, diagnosis and treatment like any other patients.

It would be better worded as:

The development of a European **collaboration for the delivery of health care** and medical services to RD patients will have a major potential for bringing benefits to European citizens by: ... would allow patients with a Rare Disease to benefit from **equal prevention, diagnosis and treatment like any other patients**

**Page 10 – Question 4**

LFB comment: We agree with the transfer of knowledge rather than mobility of patients

**Page 11 – Question 7**

LFB Comment: One should always remain careful and watch for eugenics. Only for severe diseases can it be promoted. There is a fine line between relief of a big burden and subjective choice of the child to be born or not

**Page 12 – Question 8**

LFB Comment: National organisation is quite important

**Page 15: Question 12**

LFB Comment: One should be careful that the orphan designation does not make the game of industry competition only and remains for the benefit of patients

**Page 16 - Question 13 – 3<sup>rd</sup> bullet point: Creation of the EU Advisory Committee on RD:**

LFB Comment: Could the European advisory RD committee replace the national ethics committee(s) for study on RD approval?  
If a specific RD agency is created at European Community level, could it be possible to centralize the very RD clinical study approval in this agency? the main issue for RD clinical trial is often the obligation to "open" one country, for one investigational site and for one or two patients

**Page 17: Question 14**

LFB Comment: What mission for such an Agency? Sure not for assessment and granting Orphan designations and diseases. Favourable for implementing programmes.

Sincerely,

No further comments from

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