The European Commision
Health & Consumer Protection Directorate-General
Rare Diseases consulatation
HTC 01/198
11, rue Eugene Ruppert
L-2557 Luxenbourg

e-mail: sanco-rarediseases-consultation@ec.europa.eu

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Dear Sir

Concerning: RARE DISEASES: EUROPE’S CHALLENGES

On behalf of the two National Centres for Rare Diseases in Denmark we have the following comments:
The figures refer to the figures of the paper: Rare Diseases: Europe´s challenges.

01. A common EU definition is recommended. 5 per 10.000 seem wide, but acceptable as it also I current for the orphan drug regulation.

02. Yes, indeed. A well established coding and classification system may in some aspects reduce the need for national or international databases and would give a better basis for research.

03. With a well established European definition of RD, we don’t think it is necessary. Furthermore, the list will be rather huge (about 9.000 diagnosis) and it might therefore be difficult to deal with.

04. The transfer of knowledge is important, and to some extent is current today. The Centres in Denmark find it very important that it is the national centres which do the referrals.

05. Yes, on-line and electronic tools are important and should be implemented more than today. However, it is time-consuming and financial support is very important.

06. Transborder flow is important through clearly stated, transparent standards, as is proficiency testing, registers for laboratories (e.g. EDDNAL) and firms making coordinated offers (e.g. Gendia).

07. Yes, but is important that the focus is on the diseases more than on the technical issues.

08. An EU assessment for orphan drugs accessibility would be fine, but also very difficult due to different health priorities and fiscal policy between the countries.

09. Yes.

10. The specialised social and educational services should principally be recommended at the national level.

11. Long-term financial investment is needed. The activities should be nationally founded.

12. Cooperation between private companies and researchers is good, but is should not be a pretext for doing nothing for the national governments. National and totally independent supported research is necessary and in particular in rare disorders in which basic research still is needed.

13. Due to the small number of inhabitants in Denmark, the action plans should be national (and not regional).

14. Yes, we can support the establishment of a Community Agency for rare diseases.

Yours Sincerely

John R. Ostergaard, M.D., DMSci.
Centre for Rare Diseases
Department of Paediatrics
Aarhus University Hospital, Skejby
Brendstrupgaardvej 100
DK-8200 Aarhus N
DENMARK
e-mail: joa@sks.aaa.dk

Allan Lund, M.D., DMSci
Unit for Rare Diseases
Department of Clinical Genetics
University Hospital of Copenhagen
Rigshospitalet
DK-2100 Copenhagen
DENMARK