From: Lupas Delia [delia_pupaza@yahoo.com]
Sent: dimanche 23 décembre 2007 14:45
To: SANCO RAREDISEASES CONSULTATION
Subject: hello

1. Is the current EU definition of a rare disease satisfactory?
-I think the current EU definition of rare disease is good. Also, I think that it will be better if all MS will be agree with this definition or find another one that will be satisfactory for all of them.

2. Do you agree that there is a pressing need to improve coding and classification in this area?
-Yes, I agree. And I think that the EU should cooperate with WHO in the process of revising the existing International Classification of Diseases in order to ensure that RD can be adequately coded to be traceable in all healts information systems.

3. Can a European inventory of rare diseases help your national/regional system to better deal with RD?
-Yes, because is necessary to provide the community with an accurate inventory of RD, classified by medical speciality, so as to maximize awareness and to provide documentary support to research and data storage in general.

4. Should the European Reference Networks privilege the transfer of knowledge? The mobility of patients? Both? How?
-Yes, the European Reference Networks should privilege the transfer of knowledge and the mobility of patients with improving diagnosis and care in the field of RD, to provide accurate information in a format adapted to the needs of professionals and of affected person.

5.Should on-line and electronic tools be implemented in this area?
-Yes, I think that on-line and electronic tools should be implemented in that area because they are very efficient and should be a strong part of EU strategy on RD.

6. What can be done to further improve access to quality testing for RD?
-To improve the further access to quality testing for RD, I think it should do the evaluation of population sceening, including neonatal screening, strategies for RD, primary preventive measure when it is possible and practices on RD.

7. Do you see a major need in having an EU level assessment of population screening for RD?
-Yes, because the organisation of population or targeted screening is conditioned by many issues such as the quality and reliability of the test, the availability of an effective treatment/intervention for those screened, the prevalence of the disease and its severity and the choice/value that society attributes to the screening.

8. Do you envisage the solution to the orphan drugs accessibility problem on a national scale or on an EU scale?
-Yes, I do.

9. Should the EU have an orphan regulation on medical devices and diagnostics?
- Yes, by presenting a report to the Council and the Parliament indentifiying these bottlenecks (delays, marketing, acces, reimbursement, pries, etc) every two years, proposing the necesary legislative modifications in order to guarantee equal acces to orphan drugs troughout the EU.

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?

-The specialized social and educational services for the RD patients and their families are important to improve the quality of life people living with a RD. I think that the services should be: respite care services, information services and help lines, therapeutic recreation programmes for children and young adults, financial support and psychological support.

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

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 role of partners (industry and charietes) in an EU action on RD, are very important because without this, the data protection, networks of research for rare disease, coordonation between member state funding agencies and intesifiying research it couldn’t be done.

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

-Yes, I agree with this idea and I think it should be at national level in my country.

14. Do you consider it necessary to establish a new European Agency on RD and to launch a feasibility study in 2009?

-Yes, I consider it necessary. I think the EC should launch a feasibility study in 2009 for the creation of a European Agency on RD because, this Agency would address the need to establish a permanent, sustainable instrument for the long-term implementation of RD policies at Community level.