PUBLIC CONSULTATION
RARE DISEASES: EUROPE'S CHALLENGES

Question1: Is the current definition of rare disease satisfactory?
Yes.

Question 2: Do you agree that there is 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 RD?
Yes.

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

If it is possible it would be better the Orphanet database to be offered at 26 languages.
The European Reference Network should privilege the transfer of knowledge and biological material for analysis by establishing general criteria at European level and the reference centres and laboratories designate in the internet space what kind of services they offer and what the conditions of performance such activities are.
This would limit unnecessary transfer of patients.

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 RD?

Reference centres and laboratories to be designate within the frames of the National Program.
It is necessary, criteria at national level to be built regarding the shipment of biological material for analysis abroad and the financial mechanism to support such actions.

Question 7: Do you see a major need in having an EU level assessment of potential population screening for RD?
Yes.

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

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

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

Programs at national level to be recommended; it is also crucial to propose at European level a mechanism of facilitation patients mobility in the purposes of attending seminars and work meetings between patients and professionals.
Specialized services to be designate/organize at national level.

Question 11: What model of governance and of funding scheme would be appropriate for registries, databases, and biobanks?
Centralized model of management at national level; predominantly a Government model of financial support.

Question 12: How do you see the role of partners (industry and charities) in an EU action on rare diseases?
Upon participation in joint infrastructural, research ant other projects at all levels.

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?
Yes.

Question 14: Do you consider it necessary to establish a new European Agency on RD and to launch a feasibility study in 2009?
Yes.
In Bulgaria a national Program for Diagnostics and Prophylactics of Rare Diseases has been built at national level.

Sincerely yours
Prof. Ivo Kremensky