



International Cooperation Indispensable in the Battle against “Rare Diseases”

- **Enormous potential for increase in quality and efficiency**
- **Centralise research and know-how, treatment remains at local level**
- **French national plan has signal effect for EU member states**

More intensive international cooperation can make a decisive contribution to an increase in quality and efficiency in the battle against rare diseases. This is the conclusion of Ségolène Aymé, director of the French National Plan for Rare Diseases, at the 11th European Health Forum Gastein (EHFG) taking place from 1 to 4 October 2008 in Bad Hofgastein. With more than 600 participants from health policy, health administration, medicine, science and NGO, the EHFG is the leading health policy event in the EU.

“As long as countries fail to cooperate intensively and in a structured fashion, the work in the field of rare diseases simply cannot be carried out efficiently,” according to Aymé. “It is the nature of rare diseases that the case numbers are too small to establish laboratories, educational institutions and an adequately large pool of experts in every single country to work cost efficiently and ensure the best possible quality of treatment.” Yet the treatment can and should remain organised at the local level. “Efficiency certainly does not mean patients have to fly back and forth across the continent.”

In addition to the international network, Aymé, who with the French National Plan directs the world’s most comprehensive programme for the treatment of rare diseases, demands primarily a systematisation of activities: “There are very ambitious activities in an entire range of EU countries, yet they are not incorporated into an overall programme comparable to the French initiative.”

Thus, the French initiative for rare diseases is now a highly regarded model for other countries within the EU. Important cornerstones of the more than EUR 100 million programme are:

- A network of 132 national competence centres for various rare diseases
- Comprehensive online patient information at the website www.orpha.net (now available in five languages)
- Innovative pricing for medicines for rare diseases (“orphan drugs”)
- Rare diseases are standard components of doctors’ university training

“Patient satisfaction has risen in the last four years since the start of the programme,” says Aymé. “In the coming years we will also analyse the influence of the national plan on the aetiopathology of disease and the fatality rates of the patients.”

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