Response to the Public Consultation 2008 of EURORDIS on Rare Diseases: Europe's challenges

EURORDIS is performing a great and successful campaign for the equal availability, pricing and reimbursement of orphan drugs for rare diseases in Europe.

We would like to draw the attention to a related issue for a disease which has no drug treatment. Von Hippel-Lindau, abbreviated VHL, is a genetic condition, which according to EURORDIS categorisation belongs to the very rare RDs, affecting one in 100 000 people or less such as haemophilias, Ewing Sarcoma, Duchenne muscular dystrophy.

VHL syndrome is a chronic disease, causing a series of tumors throughout one's lifetime. **The only treatment for these tumours is surgery**. An individual may need 2-20 surgeries during his lifetime. If the surgery is done well, people can usually manage quite nicely. If the surgery does not go well, the patient may become blind or disabled and need longer term treatment or social care. It is therefore **vitally important** - both to the patient's quality of life and to the long-term financial cost - that these surgeries be carried out with the **best technique and experience**. There are currently **only three centres in Europe** where a significant amount of clinical research has been conducted with hundreds of patients for 20 or more years.

Therefore in the first place we would propose to make a recommendation for the national governments to make **a treaty among the EU countries enhancing the free choice of centres for these patients**, such that - if necessary - the patient has to reimburse only the difference between the prices of the foreign and national medical centres.

We would also like to make another proposal, which is closely connected with the main goal of EURORDIS' activity: cost-effective use of resources by concentrating them where appropriate; helping to share knowledge and provide training for health professionals on the field of RDs.

On one side in countries where there is a **technically highly competent surgical centre** (like Switzerland, for example, or Sweden) that has limited experience with VHL, the country might develop a **focus on VHL** at that centre.

On the other hand while accepting the importance of long term research for the future we should not forget the problems of the present day. So we would suggest to allocate a relatively small amount from the financial support of the huge EU research grants for funding **short term grants for doctors** - practising in small or poor European countries **- to get knowledge and practice in highly specialized RD centres** or to attend medical meetings on rare diseases to improve their understanding of these RD's.

I hope that EURORDIS will find our suggestions worthy of attention.

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