

PUBLIC CONSULTATION
RARE DISEASES: EUROPE'S CHALLENGES
Eucomed contribution

Eucomed represents 4,500 designers, manufacturers and suppliers of medical technology used in the diagnosis, prevention, treatment and amelioration of diseases and disability. Most of these companies are SMEs. The mission of Eucomed is to improve patient and clinician access to modern, innovative and cost-effective medical technology. Members employ approximately 435,000 people and generate annual sales of 63 billion Euros, playing a major role in Europe's economy at a cost of less than .7% of the GNP. In addition, the medical technology sector continually introduces innovative and life-saving products that address unmet clinical needs.

Eucomed is pleased to contribute to this important and much needed public consultation on rare diseases, and looks forward to seeing the proposals later this year. We are especially happy that the consultation document recognises the importance of medical devices and medical technology in tackling rare diseases.

Question 3: Can a European inventory of rare diseases help your national/regional system to better deal with RD?

The EU should work towards a European inventory of rare diseases. Initiatives such as the Rare Bleeding Disorders Database (RBDD) exist already for certain types of rare disease, but sharing knowledge on all areas of rare diseases can only be beneficial. This is especially important for General Practitioners whose expertise in treating rare diseases can assume to be spread thin, by the very nature of the conditions being rare. GPs could turn to the database for help in diagnosis and treatment, and then the patient can be properly informed and referred to the relevant specialist. A European inventory of rare diseases would provide a genuine and tangible added value to Member State health systems from the European Union.

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

ERN is a network of reference centres with the objective to provide both healthcare professionals and patients access across Europe to high-level, shared expertise in a given field of RDs¹. The idea is that the expertise, rather than the patients, should travel - although patients should also be able to travel to the centres if required. Eucomed agrees that the primary goal should be one of knowledge exchange for example through electronic solutions without limiting patient mobility.

¹ EC, Report on the work of the High Level Group, 2006, HLG/2006/8 FINAL

Question 5: Should on-line and electronic tools be implemented in this area?

Eucomed agrees that the use of information technology can greatly aid patients' and healthcare professionals' access to innovative technologies. In that sense, introducing electronic solutions to facilitate knowledge transfer within the frame of a European Reference Network would share knowledge about diagnosis and treatment options. There are currently a number of public-private partnership reference networks that do this effectively for specific rare diseases, and they benefit patients greatly. In the age of cross-border healthcare the co-ordination of patients and doctors across Europe is vitally important.

Already now the medical technology for home use plays an increasingly central role in the diagnosis and treatment of rare diseases, especially chronic diseases. Care at home will be increasingly feasible with innovative systems off-site from healthcare facilities linked to doctors for monitoring. The "Health development in Germany up until 2037" study estimates that by 2037, up to 1.400 billion euros in direct and indirect costs will be saved through innovations in healthcare. Delivery of medicines and treatment remotely will be more common. Homecare is the fastest growing sector of healthcare: 19% vs. growing 5-6% of Medical Devices Industry across Europe (global market for tele-health was 7.7 billion USD in 2006 up from 3.2 billion USD in 2003, 241% increase).

A European Reference Network could form the foundations of such a system, and will provide benefits to EU citizens for years to come.

Question 6: What can be done to further improve access to quality testing for RD?

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

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

The EU should work towards a greater availability and accessibility of accurate diagnostic tests where possible. Eucomed therefore welcomes the DG SANCO consideration of evaluating population screening strategies. For many rare diseases accurate screening and diagnosis is already possible, and the EU should encourage Member States to make more use of the possibility, especially in cases with a prior family history of a rare disease.

A greater exchange of best practice between Member States in terms of screening and diagnostics would also be beneficial.

Eucomed believes the healthcare sector would benefit enormously from a specific policy for the funding of medical devices for rare diseases which would create industry incentives for research as well as a market interest. Concerning the opportunity for a special regulation on Orphan medical devices, it would be appropriate to analyse, and possibly review, the current requirements, contained in Directives 90/385/EEC and 93/42/EEC, for custom made devices and those at art. 11.13 (known also as the “compassionate use” clause).

These requirements allow non mass-produced devices to be put on the market without a full conformity assessment procedure, but, in particular art. 11.13 leave to Member States their practical implementation resulting in a natural lack of harmonization. Patients with rare diseases would benefit as well from such harmonization, as more diagnosis would be possible and better delivery methods for treatment available.

Estimations of diagnosis levels for rare diseases in Europe are somehow fragmented, and an orphan regulation on medical devices and diagnostics is a vital facet of addressing this.

In addition the future definition of rare diseases should not only include patients with exotic syndromes but also focus on the (comparatively few) patients with rather frequent diseases but exhausted standard therapeutic options. Such patients with severely impaired health status may eventually profit from extracorporeal therapy (‘last resort’ option).

Question 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?

In addition to the regular conferences on RD that takes place every two years, we believe that a more structured dialogue is needed where patients, healthcare professionals, industry and scientific experts, the European Commission and European Parliament can exchange views and work towards a better EU policy would be most beneficial. Eucomed would be delighted to contribute to such a group which could take the form of a stakeholder alliance such as Health First Europe. Health First Europe was established in March 2004 as an awareness-raising platform for patient groups, healthcare workers, academics, experts and the medical technology industry. The alliance seeks to encourage reflection and dialogue on the future of healthcare in Europe at a time of demographic and technological revolution. We would emphasise that SMEs should be fully represented. The European Commission should take a lead in driving such a dialogue.

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?

In the EU healthcare arena, subsidiarity is a major consideration. Having action plans and taking measures at EU level may be fruitless if the Member States do not have plans in place to tackle rare diseases themselves. As to whether the action plans should be national or regional, this depends on

the country as some Member States have centrally operated health systems and some hand over control to the regional government or regional health system management. Member States are in the best position to judge which is most effective.

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

Rare diseases and a need to improve their diagnosis and treatment require greater recognition at EU and Member State level. Eucomed welcomes this consultation as it suggests that DG SANCO has accepted the need to react to the current situation. However, a new agency should only be established if it can add value to what is already being done. A feasibility study should be carried out to see if an agency on rare diseases is possible, and if it would be worthwhile. A superfluous agency could prove counter-productive, but if the rare diseases situation can be tangibly improved by the establishment of an agency then it would undoubtedly be a step in the right direction.