MYELOMA EURONET

Evropská sít' organizací sdružujících pacienty s mnohočetným myelomem / Europäisches Netzwerk von Myelom-Patientengruppen / European Network of Myeloma Patient Groups / Red Europea de Grupos de Ayuda para Pacientes con Mieloma / Réseau européen de groupes de patients atteints du myélome multiple / Rete europea di gruppi di assistenza per pazienti affetti da mieloma multiplo / Europejska sieć grup pacjentów chorych na szpiczaka / A rede europeia dos grupos de doentes afectados do mieloma múltiplo / Reteaua Europeana a pacientilor cu mielom / Miyelom Hastaları Grubu Avrupa Ağı / الشبكة الاوروبية لمجموعة مرضى المايلوما

www.myeloma-euronet.org | 4 February 2008

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COMMENTS ON THE EC PUBLIC CONSULTATION "RARE DISEASES: EUROPE'S CHALLENGES"

Myeloma Euronet, the European Network of Myeloma Patient Groups (www.myeloma-euronet.org), is an international not-for-profit organisation consisting of 30 member groups from 17 European countries dedicated to raising the awareness of multiple myeloma, a rare but increasingly common form of bone marrow cancer. Myeloma Euronet also seeks to provide information on the diagnosis, treatment and care of persons living with multiple myeloma as well as support for their families and loved ones.

Given the fact that more and more frequent cancers are being subdivided into specific but rarer cancer types, Myeloma Euronet welcomes the initiative of the European Commission to make rare diseases a priority within the EU's future public health policy. Belonging to the non-Hodgkin group of lymphomas, multiple myeloma is an incurable but treatable disease affecting more than 77,000 people in Europe at any one time. Following are our comments related to some of the questions raised in the Public Consultation document:

Question 1: Is the current EU definition of a rare disease satisfactory?

We feel that the current EU definition of rare diseases is satisfactory because it is the one that is most widely used across European institutions and the focus should now be on doing something about rare diseases rather than on spending time on changing a widely accepted definition.

Question2: Do you agree that there is a pressing need to improve coding and classification in this area?

While there is a need to improve the traditional coding and classification approach in the field of rare diseases, it must not be forgotten that a disease should not only be defined by means of aetiological and pathophysiological facts but also by patients' needs regarding the provision of health services.

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

It is important to have up-to-date rare disease inventories at the European as well as at national levels but these inventories should not only be "classified by medical specialty, by prevalence, by mechanism, by aetiology" but also by patients' needs regarding the provision of health services.

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

We believe that knowledge transfer and patient mobility both deserve to be privileged. Information dissemination should be expanded to far more language groups and should take into consideration existing networks of different stakeholder groups (e.g., European patient networks). More multi-disciplinary information networks should be established internationally, also involving patients and/or patient representatives. The issue of centres of reference is also very important for rare disease patients, as is the obligation of General Practicioners to work together with these reference centres.

Information networks and reference centres should also–with assistance from the European Agency on rare diseases, see question 14 below–make national and European political agencies and decision makers aware of their existence and work together with them so as to make sure that rare disease patients have access to the best possible treatment and care from a multi-disciplinary team of experts, no matter where in Europe this team is based.

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

These tools are important for patients communities but it should be taken into consideration that many more language groups need to be addressed and existing networks of stakeholder groups (e.g., patient networks) already providing multi-lingual on-line and electronic tools should be taken into consideration.

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

As our recent survey has shown, it is very important to train General Practitioners and, especially in the case of **multiple myeloma**, orthopaedic surgeons in the field of diagnostic testing, as many of them don't think of a rare disease such as myeloma when a patient sees them and reports that he or she is suffering from back pain. A simple blood test would already make a huge difference for patients who otherwise might go undiagnosed for years and–to make matters worse–receive orthopaedic treatment causing a lot of unnecessary suffering on the side of the patient.

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

Before considering costly (and potentially harmful) population screening programmes for rare diseases, it should be carefully assessed which diseases justify a systematic screening approach and why.

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

Drug accessibility problems can only be solved on both EU and national scales. In fact, as our recent survey has shown, slow drug approval procedures at EU and national levels constitute one of the most significant barriers to treatment and care for myeloma patients.

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

In order to increase the motivation of the industry to develop medical devices and diagnostics for rare diseases, we feel it is important to have an orphan regulation on medical devices and diagnostics modeled after the regulation for orphan medicinal products, also making sure that the patient perspective is appropriately represented during the assessment/approval process.

Question 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?

Specialised services depend on the specific needs of rare disease patients and families as well as on the situation in the respective country/region. However, it can be said for many rare diseases, if not all, that any specialised social and educational programme and service that helps to empower patients and families and increase their quality of life, level of self-determination, and scope of choices is recommended at both EU and national levels. In this respect, it is very important that also rare disease patient groups and organisations are supported at both levels. Irrespective of the recommended service, it is necessary to involve rare disease patients and/or patient representatives and patient support groups during the development of such services to make sure that such services are appropriate and meet the needs of the respective patients and families.

As for multiple myeloma, accurate, easily understood information about myeloma and its diagnosis and treatment is needed for every patient and his or her family, irrespective of their country, language group or social status. This is recommended at EU and national levels. At the European level, patient group networks should be strengthened and further expanded and print and online information should be provided in as many languages as possible to ensure its accessibility also for patients from language groups or countries where it is not readily available. At the national level, all stakeholders must work together to make sure that appropriate support services for patients and caregivers are available and easily accessible to all who choose them. For example, the growth of new multiple myeloma patient groups throughout Europe should be encouraged, especially in cities and countries where they are not now found.

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

We strongly feel that patients and/or patient representatives should play an prominent role in the management of patient registries and databases. Patients should not be reduced to being pure "material providers" but should be actively involved in the governance of registries, databases and biobanks.

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?

Both partners have important roles to play in the rare disease arena and they should work together with national and international institutions and networks to intensify research and drug development in this field. Constant and transnational dialogue is key to ensure optimal outcomes and regular public-private forums for rare diseases that include the aforementioned stakeholders as well as European and national research funding agencies can be one way to facilitate this 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?

Concrete action plans should be developed and implemented in all EU countries. The question whether this should be done at national or regional level has to be decided by each country individually but it has to be made sure that those action plans include the patient perspective and are coherent with other health care action plans in the respective country or region. For example, if a country has developed a national cancer plan that also addresses rare cancer types, this should be taken into consideration when developing an action plan to address rare diseases in this country.

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

As already stated before (question 4), the creation of a new European Agency on Rare Diseases would be useful to help co-ordinate relevant European activities and the ongoing dialogue between all different stakeholder groups. This agency would also ensure long-term funding and sustainability of essential activities in the field of rare diseases.

Closing remark: Evidence-based medicine rather than economic considerations should be the determining factor for treating patients. Low funding means little research in the field of rare diseases, which is why we are lacking studies proving the need for orphan as well as no-label drugs.