Rare diseases: European action

2008/0218(CNS) - 23/04/2009 - Text adopted by Parliament, 1st reading/single reading

The European Parliament adopted by 383 votes to 116, with 15 abstentions, a legislative resolution amending, under the consultation procedure, the proposal for a Council recommendation on a European action in the field of rare diseases. Parliament wants financial support at European level for this Recommendation. It also wants Member States to adopt a comprehensive and integrated strategy, by the end of 2010 (rather than 2011).

Recommendations to Member States are amended as follows:

- national plans must also ensure the **rehabilitation** for those living with the disease;
- priority actions within the national plan must contain substantial and clearly designated funding;
- Member States must declare whether they have any specialised centres and compile a catalogue of experts.

New recommendations to Member States are inserted in the text. Member States should:

- declare whether they have any specialised centres and compile a catalogue of experts;
- encourage efforts to avoid rare diseases which are **hereditary**, and which will lead finally to the eradication of those rare diseases, through: genetic counselling of carrier parents; and where appropriate and not contrary to existing national laws and always on a voluntary basis, through pre-implantation selection of healthy embryos;
- provide for exceptional measures within the national plans in relation to making available **medicinal products which have no marketing authorisation**, when there is a real public health need; and, in the absence of appropriate and available therapeutic alternatives in a Member State, and when the risk/benefit balance is presumed to be positive, ensure that patients affected by rare diseases have access to the medicinal products in question;
- establish at the national level multi- stakeholder advisory groups to guide governments in the setting up and implementation of national action plans for rare diseases;
- encourage treatments for rare diseases to be **funded at national level**. Where Member States may not wish or may not be able to have Centres of Excellence, this central national funding should be used to ensure that patients can travel to a Centre in another country. However, it is also vital that this separate budget is annually reviewed and adapted on the basis of the knowledge about patients needing treatment in that given year, and about eventual new therapies to be added. This should be done with the input of the multi-stakeholder advisory committees;
- support, in particular by **financial means,** at European, national or regional level specific disease information networks, registries and databases, including regularly-updated information, which is accessible to the public, on the internet;
- foster knowledge-sharing between researchers, laboratories and research projects in the EU and similar institutions in third countries, to bring global benefits not only to the EU but also to poorer countries;
- make use of the possibilities offered by Regulation (EC) No 141/2000 on orphan medicinal products;
- provide **adequate and long-term funding**, for example through public-private partnerships, so as to support research efforts at national and European level and guarantee the sustainability thereof;
- encourage, possibly with **EU funding or co-funding**, centres and hospitals of expertise to create specific training for professionals in certain rare diseases and allow them to acquire relevant expertise;

- European guidelines on population screening and diagnostic tests should include genetic tests like heterozygote testing and polar body diagnosis, ensuring high- quality testing and appropriate genetic counselling while respecting ethical diversity in the Member States;
- there must be structural support for investment in the **Orphanet database**;
- funding for patient organisations which is not directly linked to single pharmaceutical companies should be provided. Member States should facilitate patient access to information existing at European level concerning medicines, or treatment centres in Member States or third countries providing medical care specifically suited to their illnesses;
- Member States should ensure that national plans provide for the identification of national or regional centres of expertise and for the compilation of catalogues of experts on rare diseases.

A **new recommendation to the Commission** is inserted in the proposal. The Commission should support, in a sustainable way, "Orphanet", a European website and "one-stop shop" providing the following information: on the existence of specific research into rare diseases, the findings thereof and their availability to patients; on available medicines for each rare disease; on the treatment existing in each Member State for each rare disease; on existing specialist medical centres in Member States or third countries for each rare disease.

Lastly, the Parliament states that the **implementation report** must be produced before the end of 2012. Accordingly. the Commission must produce, by the end of 2012, the year in which it will propose the implementing actions covering inter alia: a) the budgetary measures necessary for the Community Programme on Rare Diseases to be effective; b) the creation of relevant networks of centres of expertise; c) the collection of epidemiological data on rare diseases; d) the mobility of experts and professionals; e) the mobility of patients; and f) consideration of the need for other actions to improve the lives of patients affected by rare diseases and those of their families.