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Committee on Industry, Research and Energy

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OPINION

of the Committee on Industry, Research and Energy

for the Committee on the Environment, Public Health and Food Safety

on the proposal for a Council recommendation on a European action in the field of rare diseases
(COM(2008)0726 – C6-0455/2008 – 2008/0218(CNS))

Rapporteur: Françoise Grossetête

SHORT JUSTIFICATION

Rare diseases affect some 30 million Europeans. Each disease affects a small number of individuals, sometimes fewer than a dozen in any given country. The average is one in 2000. Over 7000 rare diseases are known to exist today, and two new pathologies are described every week in medical publications. Eighty per cent of rare diseases are genetic in origin, and 65% are paediatric.

It is therefore essential that rare diseases be made a priority at European level, in order to deal properly with the needs of several million citizens suffering from misdiagnosis and thus receiving no treatment to cure their diseases.

Unlike other public health or research issues, what is at stake here is not merely making up for lost time with regard to a neglected problem, but devising tools or permanent arrangements which will enable the specific features of these diseases to be taken into due account for all time to come.

Their rarity spawns specific challenges in scientific, economic and organisational terms, and these challenges cannot be tackled with the tools that are generally applied. What is called for is a global, consistent and sustainable approach, which can only be achieved by simultaneously mobilising all those concerned: politicians, institutions, patients' associations, health professionals, sufferers' associations, carers, researchers and industry.

The tiny numbers involved mean that sufferers cannot be dealt with at local or regional level, while the illnesses are far too numerous to be taught to health professionals; the result is that expert knowledge is as rare as the diseases. Since no EU Member State is capable of combating rare diseases on its own, access to information, diagnostics, carers and research needs to be organised into coherent and coordinated networks - usable by all those involved - of methodologies and shared approaches at national and European level. The Europe-wide approach is therefore essential when it comes to organising the fight against rare diseases and to mutualising and implementing resources and wherewithal.

On 16 December 1999, the European Parliament and the Council of Ministers adopted Regulation (EC) No 141/2000 on orphan medicinal products, which urges the pharmaceutical and biotechnology industry to develop and market orphan medicinal products (tax incentives, protocol assistance, 10 years' exclusivity, etc.). A Committee for orphan medicinal products (COMP) was set up within the European Agency for the Evaluation of Medicinal Products (EMEA), with the remit of examining applications for designation and assisting the Commission in discussions concerning orphan medicinal products.

In October 2008, 569 medicinal products had been granted 'orphan' designation in Europe, whereof 54 have already received Community marketing authorisation; almost 3 million patients are concerned.

The adoption of the European Regulation on orphan medicinal products created a favourable framework for companies involved in developing these products, in the shape of the aforementioned tax incentives, commercial exclusivity, etc., and has proved a boost to work on developing orphan medicinal products.

The pharmaceutical industry is disinclined to develop such products under normal market conditions: certain illnesses are so rare that the costs of development and marketing the product would never be recouped by potential sales.

There are many examples of how progress made as a result of work on rare diseases has greatly benefited less-rare diseases and will continue to do so. The range of therapeutic approaches is multiplying: genetic and cellular therapies, new molecules, 'à la carte' treatments, etc. The innovative therapies which are developed for rare diseases are a foretaste of the ever more personalised medicine of tomorrow.

In economic terms, the explosive growth of opportunities for clinical development in the field of rare diseases opens up fresh prospects, and here too, all available means to exploit these opportunities need to be coordinated at European level. Taken individually, these diseases do not constitute viable markets. Despite the existence of European measures encouraging the development of orphan medicinal products, the pharmaceutical industry is even less willing to take risks in the initial stages of developing treatments (from preclinical to phase II) than it is with more common diseases.

For the industry, the relationship between financial risks and profit prospects becomes attractive only late on in the process of developing new treatments. Between the proof of concept and the investment of the substantial private funding required before the marketing authorisation stage is reached, rare diseases have to go through a 'death valley' much longer than that faced by commoner diseases, with their much greater market potential.

It is therefore necessary, on the one hand, to construct tailor-made development tools and to optimise the development cycle for treatments so as to minimise costs, and, on the other, to invent new tools for sharing risks between private and public funding, which will enable these stages to be funded. At European level, developing networks and stepping up collaboration are unquestionably major levers in attaining this end. If measures are not taken quickly, there is a real risk that certain treatments will never see the light of day and that the only treatments which will be marketed will come from the other side of the Atlantic, at prohibitive prices.

The proposal for a Council recommendation on a European action in the field of rare diseases has a particularly important role to play with regard to the drafting and adoption of national rare diseases strategies and plans. In all the Member States these strategies and plans should have the same scope; they should be coordinated between the various Member States, and be consistently and effectively linked into Community policies.

AMENDMENTS

The Committee on Industry, Research and Energy calls on the Committee on the Environment, Public Health and Food Safety, as the committee responsible, to incorporate the following amendments in its report:

Amendment 1

Proposal for a recommendation
Recital 5

Text proposed by the Commission	*Amendment*
(5) Because of their low prevalence and their specificity, rare diseases call for a global approach based on special and combined efforts to prevent significant morbidity or avoidable premature mortality, and to improve quality of life and socio-economic potential of affected persons.	(5) Because of their low prevalence and their specificity, rare diseases call for a global approach based on special and combined efforts***, including in partnership with third countries such as the United States,*** to prevent significant morbidity or avoidable premature mortality, and to improve quality of life and socio-economic potential of affected persons ***in developed and developing countries.***

Justification

The specificities of rare diseases - limited number of patients and scarcity of relevant knowledge and expertise - means that cooperation with countries outside the EU can bring benefits, not just to the EU but also in knowledge sharing with poorer and developing countries.

Amendment 2

Proposal for a recommendation
Recital 14 a (new)

Text proposed by the Commission	*Amendment*
	(14a) On 12 October 2008 the Pharmaceutical Forum adopted its final report, which proposes guidelines to enable Member States, stakeholders and the Commission to step up their efforts to guarantee easier and swifter access to orphan medicinal products within the European Union.

Justification

Following the conclusions of the Pharmaceutical Forum, the Member States have given a political undertaking to eliminate bottlenecks in respect of access to orphan medicinal products via the Community approval procedure. The guidelines set out in the Pharmaceutical Forum's final report need to be given specific mention in the proposal for a recommendation.

Amendment 3

Proposal for a recommendation
Recommendations to Member States – paragraph 1 – point 1

Text proposed by the Commission	*Amendment*
(1) elaborate and adopt a comprehensive and integrated strategy, by the end of ***2011***, aimed at guiding and structuring all relevant actions in the field of rare diseases in the form of a national plan for rare diseases;	(1) elaborate and adopt a comprehensive and integrated strategy, by the end of ***2010***, aimed at guiding and structuring all relevant actions in the field of rare diseases in the form of a national plan for rare diseases;

Justification

It is important for Member States to elaborate and adopt by the end of 2010 a comprehensive and integrated strategy aimed at guiding and structuring all relevant actions in the field of rare diseases in the form of a national plan for rare diseases;

Amendment 4

Proposal for a recommendation
Recommendations to Member States – paragraph 1 – point 3

Text proposed by the Commission	*Amendment*
(3) define a limited number of priority actions within the national plan for rare diseases, with concrete objectives, clear deadlines, management structures and regular reports;	(3) define a limited number of priority actions within the national plan for rare diseases, with concrete objectives, clear deadlines, ***substantial and clearly identified funding,*** management structures and regular reports;

Amendment 5

Proposal for a recommendation
Recommendations to Member States – paragraph 1 – point 5 a (new)

Text proposed by the Commission	*Amendment*
	(5a) provide for exceptional measures within the national plans when it comes to making available medicinal products which have no marketing authorisation, when there is a real public health need. In

the absence of appropriate and available therapeutic alternatives in a Member State, and when the risk/benefit balance is presumed to be positive, patients affected by rare diseases shall have access to the medicinal products in question.

Justification

The national plans also need to provide for the possibility of providing and reimbursing temporary access to treatments or medicinal products which are in the process of securing marketing authorisation, or which do not have such authorisation, in extremely specific cases where the use of these products could be beneficial to patients suffering from a rare disease. This possibility exists via the compassionate use ('Autorisation Temporaire d'Utilisation' - ATU) procedure.

Amendment 6

Proposal for a recommendation
Recommendations to Member States – paragraph 2 – point 4

Text proposed by the Commission	*Amendment*
(4) support at national or regional level specific disease information networks, registries and databases.	(4) support***, in particular by financial means,*** at ***European,*** national or regional level specific disease information networks, registries and databases.

Amendment 7

Proposal for a recommendation
Recommendations to Member States – paragraph 3 – point 3 a (new)

Text proposed by the Commission	*Amendment*
	(3a) put in place adequate, long-term funding, by means , for example, of public/private partnerships, to support research at national and European level, so as to guarantee its viability;

Amendment 8

Proposal for a recommendation
Recommendations to Member States – paragraph 3 – point 3 b (new)

Text proposed by the Commission	*Amendment*
	(3b) foster knowledge-sharing and cooperation between researchers, laboratories and research projects in the European Union and similar institutions in third countries, to bring global benefits not only to the European Union but also to poorer and developing countries, which are less well placed to provide resources for research into rare diseases;

Justification

The specificities of rare diseases - limited number of patients and scarcity of relevant knowledge and expertise - means that cooperation with countries outside the EU can bring benefits, not just to the EU but also in knowledge sharing with poorer and developing countries.

Amendment 9

Proposal for a recommendation
Recommendations to Member States – paragraph 4 – point 3

Text proposed by the Commission	*Amendment*
(3) organise healthcare pathways for patients through the establishment of cooperation with relevant experts within the country or from abroad when necessary; cross-border healthcare, including mobility of patients, health professionals and providers and provision of services through information and communication technologies should be supported where it is necessary to ensure universal access to the specific healthcare needed;	(3) organise ***European*** healthcare pathways for ***those*** patients ***suffering from rare diseases*** through the establishment of cooperation with relevant experts within the country or from abroad when necessary; cross-border healthcare, including mobility of patients, health professionals and providers and provision of services through information and communication technologies should be supported where it is necessary to ensure universal access to the specific healthcare needed;

Justification

It is important to organise healthcare pathways for patients suffering from rare diseases through the establishment of cooperation with relevant experts within the country or from abroad when necessary.

Amendment 10

Proposal for a recommendation
Recommendations to Member States – paragraph 4 – point 5

Text proposed by the Commission	*Amendment*
(5) ensure that national or regional centres of expertise adhere to the standards defined by the European reference networks for rare diseases taking into due account the needs and expectations of patients and professionals.	(5) ensure that national or regional centres of expertise adhere to the standards defined by the European reference networks for rare diseases taking into due account the needs and expectations of patients and professionals ***and involving the patients in the activities, management and evaluation of the centres***.

Amendment 11

Proposal for a recommendation
Recommendations to Member States – paragraph 5 – point 1 – point c

Text proposed by the Commission	*Amendment*
(c) sharing ***Member State's*** assessment reports on the ***therapeutic*** added value of orphan drugs at EU level, in order to minimise delays for access to orphan drugs for rare disease patients;	(c) sharing ***Member States'*** assessment reports on the ***clinical*** added value of orphan drugs at EU level ***within the European Medicines Agency, which brings together European expertise in this field***, in order to minimise delays for access to orphan drugs for rare disease patients;

Justification

The EMEA is the most appropriate platform for reducing the waiting times and centralising the evaluation reports.

Amendment 12

Proposal for a recommendation
Recommendations to Member States – paragraph 6 – point 2 a (new)

Text proposed by the Commission	*Amendment*
	(2a) facilitate patient access to information existing at European level

concerning medicines, treatments or treatment centres in the Member States or third countries providing medical care specifically suited to their illnesses;

Justification

It is important to facilitate patient access to information existing at European level concerning medicines, treatments or treatment centres in the Member States or third countries providing medical care specifically suited to their illnesses.

Amendment 13

Proposal for a recommendation
Recommendations to the Commission – paragraph -1 (new)

Text proposed by the Commission	*Amendment*
	(-1) To support, in a sustainable way, "Orphanet", a European website and "one-stop shop" providing the following information:
	(a) on the existence of specific research into rare diseases, the findings thereof and their availability to patients,
	(b) on available medicines for each rare disease,
	(c) on the treatment existing in each Member State for each rare disease,
	(d) on existing specialist medical centres in Member States or third countries for each rare disease.

PROCEDURE

Title	European action in the field of rare diseases
References	COM(2008)0726 – C6-0455/2008 – 2008/0218(CNS)
Committee responsible	ENVI
Opinion by Date announced in plenary	ITRE 4.12.2008
Rapporteur Date appointed	Françoise Grossetête 2.12.2008
Discussed in committee	11.2.2009
Date adopted	9.3.2009
Result of final vote	+: 43 –: 0 0: 0
Members present for the final vote	Jan Březina, Giles Chichester, Dragoş Florin David, Pilar del Castillo Vera, Den Dover, Lena Ek, Norbert Glante, Umberto Guidoni, Fiona Hall, David Hammerstein, Rebecca Harms, Erna Hennicot-Schoepges, Mary Honeyball, Romana Jordan Cizelj, Werner Langen, Anne Laperrouze, Pia Elda Locatelli, Eluned Morgan, Reino Paasilinna, Atanas Paparizov, Francisca Pleguezuelos Aguilar, Anni Podimata, Herbert Reul, Teresa Riera Madurell, Paul Rübig, Andres Tarand, Catherine Trautmann, Claude Turmes, Nikolaos Vakalis, Adina-Ioana Vălean, Dominique Vlasto
Substitute(s) present for the final vote	Alexander Alvaro, Pilar Ayuso, Ivo Belet, Françoise Grossetête, Marie-Noëlle Lienemann, Erika Mann, Vittorio Prodi, Esko Seppänen, Vladimir Urutchev, Lambert van Nistelrooij
Substitute(s) under Rule 178(2) present for the final vote	Elisabetta Gardini, Ulrike Rodust