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Committee on the Environment, Public Health and Food Safety

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AMENDMENTS 13 - 46

Draft report
Antonios Trakatellis
(PE420.052v01-00)

A European action in the field of rare diseases

Proposal for a recommendation
(COM(2008)0726 – C6-0455/2008 – 2008/0218(CNS))

AM_Com_LegReport

Amendment 13
Urszula Krupa

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 ***the high cost of treating rare diseases*** and their specificity, ***they*** call for ***international programmes and*** 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.

Or. pl

Amendment 14
Urszula Krupa

Proposal for a recommendation
Recital 8

Text proposed by the Commission	*Amendment*
(8) In order to improve the coordination and coherence of national, regional and local initiatives addressing rare diseases, all relevant national actions in the field of rare diseases should be integrated into national plans for rare diseases.	(8) In order to improve the coordination and coherence of national, regional and local initiatives ***and cooperation between research centres*** addressing rare diseases, all relevant national actions in the field of rare diseases should be integrated into national plans for rare diseases.

Or. pl

Amendment 15
Daciana Octavia Sârbu

Proposal for a recommendation
Recital 13

Text proposed by the Commission	*Amendment*
(13) The Community added-value of European reference networks is particularly high for rare diseases by reason of the rarity of these conditions, which implies both limited number of patients and scarcity of expertise within a single country. Gathering expertise at European level is therefore paramount to ensure equal access to high quality care to rare disease patients.	(13) The Community added-value of European reference networks is particularly high for rare diseases by reason of the rarity of these conditions, which implies both limited number of patients and scarcity of expertise within a single country. Gathering expertise at European level is therefore paramount to ensure equal access ***to accurate information, appropriate and timely diagnosis and*** to high quality care to rare disease patients.

Or. en

Amendment 16
Daciana Octavia Sârbu

Proposal for a recommendation
Recital 16 a (new)

Text proposed by the Commission	*Amendment*
	(16a) Specialised social services, including those that are therapeutically and psychologically focused, should be established in order to avoid social exclusion and discrimination.

Or. en

Amendment 17
Antonios Trakatellis, Adamos Adamou

Proposal for a recommendation
Recital 20

Text proposed by the Commission	*Amendment*
(20) Patients and patients' representatives should therefore be involved at all steps of the policy and decision-making processes. Their activities should be actively promoted and supported, including financially, in each Member State.	(20) Patients and patients' representatives should therefore be involved at all steps of the policy and decision-making processes. Their activities should be actively promoted and supported, including financially, in each Member State***, but also on an EU level in terms of pan-EU patient support networks for specific rare diseases***.

Or. en

Justification

Because of the rarity of these diseases, many patient organisations are working in pan-European networks to communicate and support each other across the EU 27.

Amendment 18
Evangelia Tzampazi

Proposal for a recommendation
Recommendations to Member States – Paragraph 1 – introductory part

Text proposed by the Commission	*Amendment*
1. Establish national plans for rare diseases in order to ensure to patients with rare diseases universal access to high quality care, including diagnostics, treatments and orphan drugs throughout their national territory on the basis of equity and solidarity throughout the EU, and in particular:	1. Establish national plans for rare diseases in order to ensure to patients with rare diseases universal access to high quality care, including diagnostics, treatments and orphan drugs***, as well as rehabilitation and habilitation for those living with the disease*** throughout their national territory on the basis of equity and solidarity throughout the EU, and in particular:

Or. el

Justification

It is important to note that many sufferers of rare diseases will be forced to live with their ailment for many years. For this reason, research and other activities in this area should also seek to ensure the social integration of those suffering from rare diseases in accordance with Article 26 of the UN Convention on the Rights of Persons with Disabilities.

Amendment 19
Frédérique Ries

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 designated funding***, management structures and regular reports;

Or. fr

Amendment 20
Evangelia Tzampazi

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

Text proposed by the Commission	*Amendment*
(5) include in the national plans provisions designed to ensure equitable access to high quality care, including diagnostics, treatments and orphan drugs, **to** all rare disease patients throughout their national territory with a view to ensuring equitable access to quality care on the basis of equity and solidarity throughout the European Union.	(5) include in the national plans provisions designed to ensure equitable access to high quality care, including diagnostics, treatments and orphan drugs, ***together with rehabilitation and habilitation for those living with the disease for the benefit of*** all rare disease patients throughout their national territory with a view to ensuring equitable access to quality care on the basis of equity and solidarity throughout the European Union.

Or. el

Justification

It is important to note that many sufferers of rare diseases will be forced to live with their ailment for many years. For this reason, research and other activities in this area should also seek to ensure the social integration of those suffering from rare diseases in accordance with Article 26 of the UN Convention on the Rights of Persons with Disabilities.

Amendment 21
Avril Doyle, Alojz Peterle

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

Text proposed by the Commission	*Amendment*
(5) include in the national plans provisions designed to ensure equitable access to high quality care, including diagnostics, treatments and orphan drugs, to all rare disease patients throughout their national territory with a view to ensuring equitable access to quality care on the basis of equity and solidarity throughout the European Union.	(5) include in the national plans provisions designed to ensure equitable access to high quality care, including diagnostics, treatments and orphan drugs, to all rare disease patients throughout their national territory with a view to ensuring equitable access to quality care on the basis of equity and solidarity throughout the European Union ***following the principles agreed in the High Level Pharmaceutical Forum paper "Improving Access to Orphan Medicines for all Affected EU citizens"***.

Or. en

Amendment 22
Daciana Octavia Sârbu

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

Text proposed by the Commission	*Amendment*
(5) include in the national plans provisions designed to ensure equitable access to high quality care, including diagnostics, treatments and orphan drugs, to all rare	(5) include in the national plans provisions designed to ensure equitable access to high quality care, including diagnostics, ***primary preventive measures,*** treatments

disease patients throughout their national territory with a view to ensuring equitable access to quality care on the basis of equity and solidarity throughout the European Union.	and orphan drugs, to all rare disease patients throughout their national territory with a view to ensuring equitable access to quality care on the basis of equity and solidarity throughout the European Union.

Or. en

Amendment 23
Urszula Krupa

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

Text proposed by the Commission	*Amendment*
(5) include in the national plans provisions designed to ensure ***equitable*** access to high quality care, including diagnostics, treatments and orphan drugs, to all rare disease patients throughout their national territory with a view to ensuring equitable access to quality care on the basis of equity and solidarity throughout the European Union.	(5) include in the national plans provisions designed to ensure ***free*** access to high quality care, including diagnostics, treatments and orphan drugs, to all rare disease patients throughout their national territory with a view to ensuring equitable access to quality care on the basis of equity and solidarity throughout the European Union.

Or. pl

Amendment 24
Antonios Trakatellis, Dorette Corbey, Frédérique Ries, Adamos Adamou

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

Text proposed by the Commission	*Amendment*
	(5a) encourage efforts to avoid rare diseases which are hereditary, and which will lead finally to the eradication of those rare diseases, through:
	(a) genetic counselling of carrier parents; and
	(b) where appropriate and not contrary to

existing national laws and always on a voluntary basis, through pre-implantation selection of healthy embryos.

Or. en

Amendment 25
Adamos Adamou

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

Text proposed by the Commission	*Amendment*
	(5a) establish at the national level multi-stakeholder advisory groups comprising all interested stakeholders to guide governments in the setting up and implementation of national action plans for rare diseases. These should ensure that governments are well-informed and that the decisions taken at national level reflect the views and needs of society.

Or. en

Justification

In order to minimise the risk of loosing expertise on such a matter of specific nature due to changes in government and administration, there should be standing committees comprising all interested and experienced parties i.e. patients , treating physicians, payers, academia and industry. These parties should offer guidance for the political decision takers and policy makers in the set up and implementation of national action plans.

Amendment 26
Thomas Ulmer

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

Text proposed by the Commission	*Amendment*
	(5a) establish at the national level multi-stakeholder advisory groups comprising

all interested stakeholders should be established at the national level to guide governments in the implementation of national action plans for rare diseases. These should ensure that governments are well-informed and that the decisions taken at a national level reflect the views of society.

Or. en

Justification

Rare disease expertise is scattered and the specific nature of rare diseases creates specific issues. There should be standing committees comprising all stakeholders: patients, treating physicians, payers, academia and industry amongst them. These would be guardians of the expertise irrespective of governmental or administrative changes. Their input should guide the administration and government of the day regarding policies affecting the rare disease community.

Amendment 27
Adamos Adamou

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

Text proposed by the Commission	*Amendment*
	(5b) 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 fund 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.

Or. en

Justification

Treating centres or hospitals should be encouraged in diagnosing and treating patients with rare disease . If they have to pay from their own budgets, they might not be able to bare the financial burden. Funding at national level can give the opportunity to the patients to have access to the appropriate medical care.

Amendment 28
Antonios Trakatellis, Dorette Corbey, Frédérique Ries, Adamos Adamou

Proposal for a recommendation
Recommendations to Member States – Paragraph 2 – point 1

Text proposed by the Commission	*Amendment*
(1) implement a European Union common definition of rare diseases as those diseases affecting no more than 5 per 10 000 persons;	(1) implement a European Union common definition of rare diseases as those diseases affecting no more than 5 per 10 000 persons***, as a number for the whole European Union, but it is very important to know the exact distribution for each Member State***;

Or. en

Amendment 29
Daciana Octavia Sârbu

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 at national or regional level specific disease information networks, registries and databases***, including regularly-updated information, which is accessible to the public, on the internet*** .

Or. en

Amendment 30
Frédérique Ries

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 ***financially and in other ways*** at national or regional level specific disease information networks, registries and databases.

Or. fr

Amendment 31
Dorette Corbey

Proposal for a recommendation
Recommendations to Member States – Paragraph 3 – point 3

Text proposed by the Commission	*Amendment*
(3) foster participation of national researchers and laboratories in research projects on rare diseases funded at Community level;	(3) foster participation of national researchers and laboratories in research projects on rare diseases funded at Community level ***and make use of the possibilities offered by Regulation (EC) No 141/2000 on orphan medicinal products***;

Or. nl

Amendment 32
Evangelia Tzampazi

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

Text proposed by the Commission	*Amendment*
(4) include in the national plan for rare diseases provisions aimed at fostering research, including public health and social research, in the field of rare diseases,	(4) include in the national plan for rare diseases provisions aimed at fostering research, including public health and social research, in the field of rare diseases,

especially with a view to the development of tools such as transversal infrastructures as well as disease-specific projects**.**	especially with a view to the development of tools such as transversal infrastructures as well as disease-specific projects***, and rehabilitation and habilitation programmes for the duration of a rare disease***.

Or. el

Justification

It is important to note that many sufferers of rare diseases will be forced to live with their ailment for many years. For this reason, research and other activities in this area should also seek to ensure the social integration of those suffering from rare diseases in accordance with Article 26 of the UN Convention on the Rights of Persons with Disabilities.

Amendment 33
Adamos Adamou

Proposal for a recommendation
Recommendations to Member States – Paragraph 3 – point 4 a (new)

Text proposed by the Commission	*Amendment*
	(4a) provide industry, which is one of the main funding providers on clinical research, with adequate incentives, in order for it to invest in genetic research.

Or. en

Amendment 34
Frédérique Ries

Proposal for a recommendation
Recommendations to Member States – Paragraph 3 – point 4 a (new)

Text proposed by the Commission	*Amendment*
	(4a) 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;

Or. fr

Amendment 35
Frédérique Ries

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 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 ***and expertise through data-mobility support***, 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;

Or. fr

Amendment 36
Frédérique Ries

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***, by involving patients in the activities, administration and assessment of these centres***;

Or. fr

Amendment 37
Peter Liese, Thomas Ulmer

Proposal for a recommendation
Recommendations to Member States – Paragraph 5 – point 1 – subpoint b

Text proposed by the Commission	*Amendment*
(b) European guidelines on population screening and diagnostic tests;	(b) European guidelines on population screening and diagnostic tests***, including 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***;

Or. en

Amendment 38
Antonios Trakatellis, Dorette Corbey, Frédérique Ries, Adamos Adamou

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

Text proposed by the Commission	*Amendment*
(c) ***sharing*** Member *States'* 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) ***establishing*** Member *States'* assessment reports on the therapeutic added value of orphan drugs at EU level ***within the EMEA where the relevant European knowledge and expertise is gathered***, in order to minimise delays for access to orphan drugs for rare disease patients;

Or. en

Amendment 39
Avril Doyle, Alojz Peterle

Proposal for a recommendation
Recommendations to Member States – Paragraph 5 – point 1 – subpoint 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 State's assessment reports on the ***clinical*** added value of orphan drugs at EU level, in order to minimise delays for access to orphan drugs for rare disease patients;

Or. en

Amendment 40
Dorette Corbey

Proposal for a recommendation
Recommendations to Member States – Paragraph 5 – point 1 – subpoint c a (new)

Text proposed by the Commission	*Amendment*
	(ca) structural support for investment in the Orphanet database to ensure ease of access to information concerning rare diseases;

Or. nl

Amendment 41
Frieda Brepoels

Proposal for a recommendation
Recommendations to Member States – Paragraph 5 – point 1 – subpoint c a (new)

Text proposed by the Commission	*Amendment*
	(c a) development of specialist medical training in fields relevant to the diagnosis and management of rare diseases (for instance genetics, immunology, neurology, oncology, paediatrics);

Or. en

Justification

80% of the rare diseases are genetic. Most often, genetic specialists are the first contact points, able to diagnose correctly and refer the patient to the appropriate medical specialism for treatment. Though the criteria for recognition still differ to some extent between Member States, "clinical or medical genetics" is recognised as a specialism in most EU countries, except for Greece, Spain and Belgium. In the latter two, a request for recognition is ready. Currently, there is however no recognition at EU level. This recognition is crucial so that patients in every Member State can find the correct expertise and the mobility of the genetic doctors within the EU is guaranteed.

Amendment 42
Antonios Trakatellis, Peter Liese, Thomas Ulmer

Proposal for a recommendation
Recommendations to Member States – Paragraph 6

Text proposed by the Commission	*Amendment*
6. Empowerment of patient organisations	6. Empowerment of ***independent*** patient organisations
(1) take action to ensure that patients and patients' representatives are duly consulted at all steps of the policy and decision-making processes in the field of rare diseases, including for the establishment and management of centres of expertise and of European reference networks and for the elaboration of national plans;	(1) take action to ensure that patients and ***independent*** patients' representatives are duly consulted at all steps of the policy and decision-making processes in the field of rare diseases, including for the establishment and management of centres of expertise and of European reference networks and for the elaboration of national plans;
(2) support the activities performed by patient organisations, such as awareness-raising, capacity-building and training, exchange of information and best practices, networking, outreach to very isolated patients;	(2) support the activities performed by ***independent*** patient organisations, such as awareness-raising, capacity-building and training, exchange of information and best practices, networking, outreach to very isolated patients;
(3) include in the national plans for rare diseases provisions on the support to and the consultation of patient organisations as referred to in paragraphs (1) and (2).	(3) include in the national plans for rare diseases provisions on the support to and the consultation of patient organisations as referred to in paragraphs (1) and (2).

Or. en

Amendment 43
Dorette Corbey

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

Text proposed by the Commission	*Amendment*
	(2a) Provide funding for patient organisations so that they can continue to operate as independently as possible;

Or. nl

Amendment 44
Antonios Trakatellis, Peter Liese, Thomas Ulmer

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

Text proposed by the Commission	*Amendment*
	(2a) ensure that funding for patient organisations which is not directly linked to single pharmaceutical companies is provided;

Or. en

Amendment 45
Antonios Trakatellis, Peter Liese, Thomas Ulmer

Proposal for a recommendation
Recommendations to Member States – Paragraph 6 – point 3

Text proposed by the Commission	*Amendment*
(3) include in the national plans for rare diseases provisions on the support to and the consultation of patient organisations as referred to in paragraphs (1) and (2).	(3) include in the national plans for rare diseases provisions on the support to and the consultation of ***independent*** patient organisations as referred to in paragraphs (1) and (2).

Or. en

Amendment 46
Frédérique Ries

Proposal for a recommendation
Hereby invites the Commissions – Paragraph 1

Text proposed by the Commission	*Amendment*
1. To produce an implementation report on this Recommendation addressed to the Council, the European Parliament, the European Economic and Social Committee and the Committee of the Regions on the basis of the information provided by the Member States, not later than in the end of the fifth year after the date of adoption of this Recommendation, to consider the extent to which the proposed measures are working effectively, and to consider the need for further action.	1. To produce an implementation report on this Recommendation addressed to the Council, the European Parliament, the European Economic and Social Committee and the Committee of the Regions on the basis of the information provided by the Member States, not later than in the end of the fifth year after the date of adoption of this Recommendation, to consider the extent to which the proposed measures are working effectively ***to improve the lives of patients affected by rare diseases and those of their families*** and to consider the need for further action.

Or. fr