EUROPEAN PARLIAMENT

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Committee on the Environment, Public Health and Food Safety

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DRAFT REPORT

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))

Committee on the Environment, Public Health and Food Safety

Rapporteur: Antonios Trakatellis

Symbols for procedures

*	Consultation procedure *majority of the votes cast*
**I	Cooperation procedure (first reading) *majority of the votes cast*
**II	Cooperation procedure (second reading) *majority of the votes cast, to approve the common position* *majority of Parliament's component Members, to reject or amend the common position*
***	Assent procedure *majority of Parliament's component Members except in cases covered by Articles 105, 107, 161 and 300 of the EC Treaty and Article 7 of the EU Treaty*
***I	Codecision procedure (first reading) *majority of the votes cast*
***II	Codecision procedure (second reading) *majority of the votes cast, to approve the common position* *majority of Parliament's component Members, to reject or amend the common position*
***III	Codecision procedure (third reading) *majority of the votes cast, to approve the joint text*

(The type of procedure depends on the legal basis proposed by the Commission.)

Amendments to a legislative text

In amendments by Parliament, amended text is highlighted in ***bold italics*.** In the case of amending acts, passages in an existing provision that the Commission has left unchanged, but that Parliament wishes to amend, are highlighted in **bold**. Any deletions that Parliament wishes to make in passages of this kind are indicated thus: [...]. Highlighting in *normal italics* is an indication for the relevant departments showing parts of the legislative text for which a correction is proposed, to assist preparation of the final text (for instance, obvious errors or omissions in a given language version). Suggested corrections of this kind are subject to the agreement of the departments concerned.

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DRAFT EUROPEAN PARLIAMENT LEGISLATIVE RESOLUTION

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))

(Consultation procedure)

The European Parliament,

– having regard to the Commission proposal to the Council (COM(2008)0726),

– having regard to Article 152(4), second subparagraph of the EC Treaty, pursuant to which the Council consulted Parliament (C6-0455/2008),

– having regard to Rule 51 of its Rules of Procedure,

– having regard to the report of the Committee on the Environment, Public Health and Food Safety and the opinion of the Committee on Industry, Research and Energy (A6-0000/2009),

1. Approves the Commission proposal as amended;

2. Calls on the Commission to alter its proposal accordingly, pursuant to Article 250(2) of the EC Treaty;

3. Calls on the Council to notify Parliament if it intends to depart from the text approved by Parliament;

4. Asks the Council to consult Parliament again if it intends to amend the Commission proposal substantially;

5. Instructs its President to forward its position to the Council and Commission.

Amendment 1

Proposal for a recommendation
Recital 1

Text proposed by the Commission	*Amendment*
(1) Rare diseases are a threat to the health of European citizens insofar as they are life-threatening or chronically debilitating diseases with a low prevalence and a high level of complexity.	(1) Rare diseases are a threat to the health of European citizens insofar as they are life-threatening or chronically debilitating diseases with a low prevalence *and* a high level of complexity, ***but since there are so many different types of rare disease, the total number of people affected is quite high***.

Or. en

Amendment 2

Proposal for a recommendation
Recital 2

Text proposed by the Commission	*Amendment*
(2) A Community Action Programme on Rare Diseases, including genetic diseases, was adopted for the period 1 January 1999 to 31 December 2003. This programme defined the prevalence for a rare disease as affecting no more than 5 per 10 000 persons in the European Union.	(2) A Community Action Programme on Rare Diseases, including genetic diseases, was adopted for the period 1 January 1999 to 31 December 2003. This programme defined the prevalence for a rare disease as affecting no more than 5 per 10 000 persons in the European Union***, a number to be judged on a statistical basis subject to a scientific review***.

Or. en

Justification

More flexibility is required to avoid dilemmas arising from diseases with incidence 5,1 or 5,2 etc. per 10 000 persons.

Amendment 3

Proposal for a recommendation
Recital 2 a (new)

Text proposed by the Commission	*Amendment*
	(2a) Based on this statistical incidence, rare diseases should be meticulously catalogued and reviewed regularly by a scientific committee to determine the need for possible additions.

Or. en

Justification

It is an absolute necessity to compile the catalogue of rare diseases in the European Union.

Amendment 4

Proposal for a recommendation
Recital 4

Text proposed by the Commission	*Amendment*
(4) It is estimated that between 5 000 and 8 000 distinct rare diseases exist today, affecting between 6% and 8% of the population in the course of their lives. In other words, between 27 and 36 million people in the European Union. Most of them suffer from less frequently-occurring diseases affecting one in 100 000 people or less.	(4) It is estimated that between 5 000 and 8 000 distinct rare diseases exist today, affecting between 6% and 8% of the population in the course of their lives. In other words, ***although rare diseases are characterised by low prevalence for each one of them, the total number of people affected is quite high, ranging*** between 27 and 36 million people in the European Union. Most of them suffer from less frequently-occurring diseases affecting one in 100 000 people or less.

Or. en

Amendment 5

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***,*** their specificity ***and the high total number of cases***, 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.

Or. en

Amendment 6

Proposal for a recommendation
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 ***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.***

Or. en

Amendment 7

Proposal for a recommendation
Paragraph 1 – point 3 a (new)

Text proposed by the Commission	*Amendment*
	(3a) Declare whether they have any specialised centres and compile a catalogue of experts;

Or. en

Amendment 8

Proposal for a recommendation
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; and compile a catalogue of rare diseases and have a permanent scientific committee to determine whether a disease can be considered as a rare disease even with an incidence of more than 5 per 10 000 persons***;

Or. en

Amendment 9

Proposal for a recommendation
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, especially with a view to the development of tools such as transversal infrastructures as well as disease-specific projects.	(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, ***as well as research on diagnostic tests and tools;***

Or. en

Amendment 10

Proposal for a recommendation
Paragraph 4 – point 1

Text proposed by the Commission	*Amendment*
(1) identify national or regional centres of expertise throughout their national territory by the end of 2011, and foster the creation of centres of expertise where they do not exist notably by including in their national plan for rare diseases provisions on the creation of national or regional centres of expertise;	(1) identify national or regional centres of expertise throughout their national territory by the end of 2011, and foster the creation of centres of expertise where they do not exist notably by including in their national plan for rare diseases provisions on the creation of national or regional centres of expertise; ***help compile catalogues of rare diseases and rare diseases experts;***

Or. en

Amendment 11

Proposal for a recommendation
Paragraph 4 – point 5 a (new)

Text proposed by the Commission	*Amendment*
	(5a) encourage, possibly with European Union 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;

Or. en

Justification

It is essential to train more professionals in the field of rare diseases in order to combat them efficiently.

Amendment 12

Proposal for a recommendation
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 patient organisations as referred to in paragraphs (1) and (2); ***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;***

Or. en

EXPLANATORY STATEMENT

General introduction and appraisal of the Proposal for a Council Recommendation

This Council Recommendation is welcome because a concerted action in the field of rare diseases at the European Union level and at the national level is an **absolute necessity**. This is because although the incidence for each rare disease is very low, millions of people are affected across the European Union because these diseases are measured in thousands.

However this Proposal at hand is really insufficient as in its current state and it is not possible to produce a viable programme out of it. This is because it does not describe, at least in general terms, **the necessary funding** from EU and the co funding by EU and Member States or other organisations. Thus cannot effectively promote certain essential aspects on rare diseases i.e. **creation of networks of centres of expertise, cataloguing of diseases, special research required etc.**

The text asks for an implementation proposal from the Commission five years after the adoption, which is a long period during which nothing can be practically done as there is no funding provided.

Therefore as a Rapporteur, I propose that the Commission be asked to **provide the implementation proposal** at the latest by the end of 2012, since by this date the required data from Member States on expert centres and expertise on rare diseases will be at hand (2011).

In this implementation specific mention should be made for funding/co funding etc. in the areas of:
a.) the collection of epidemiological data and compiling a catalogue of rare diseases, as this is necessary in order to have a clear picture of the field of these diseases in the EU;
b.) the formation of relevant networks;
c.) the creation of expert centres in Member States which lack such centres;
d.) the creation of special training courses in the existing centres for professionals to acquire expertise;
e.) the mobilisation of experts and professionals in order to create the necessary conditions for advancing existing knowledge;
f.) research on diagnostic tools and tests on rare diseases and especially on genetic ones.

We should considering this proposal for a Council Recommendation as a roadmap to create helpful conditions in the field of rare diseases, and we should understand that it is of a general nature but I would like to stress once more that in order to be efficiently and successfully applied, the Proposal needs to be more precise and definite in the calendar (years) of implementation.

Important aspects to be mentioned
in the Commission's proposal for implementation

1. As it is evident from the Council Recommendation, the field of rare diseases in the European Union is **not well defined** :
-neither as a registry cataloguing the diseases one by one,
- nor as far as the accuracy of the statistical data is concerned.
This is evident from the great **spread** in the number of patients (27 to 36 million people) and the number of rare diseases (5 000 to 10 000 rare diseases).

2. It is also important to note that we should approach the subject with **flexibility** because a definition of rare disease as affecting less than 5 persons per 10 000 is **too restrictive**, as you may have numbers slightly above this ratio and be confronted with the dilemma of not considering as a rare disease one if the ratio is for example 5,1 . Also, a rare disease may have a different distribution from one Member State to another.

3. Due to the great number of affected persons but the low incidence for each disease, a concerted action across the European Union level is of absolute necessity.
For this reason, I believe that it is appropriate that the final implementation proposal should try to **compile the specialized centres and hospitals** for some of these diseases, as well as the **expertise** existing in each country and **connect them in a network.**

4. It is also obvious that to combat rare diseases, one need to regulate **mobility of patients** because there is not all the expertise in each Member State.
Therefore as long as there are no expertise and centres which can take care of the patients, this should be corrected by their mobility. Here the issue of funding should be left for consideration to the Member States.

5. **Mobility of professionals** is another very important aspect, i.e.:
a.) mobility of experts across the European Union in order to help the creation of new centres in other Member States
b.) mobility of professionals in order to acquire expertise on certain rare diseases in existing centres across the European Union. This action requires the creation of training courses and clinical practice in order to train more experts on these diseases.
Therefore the mission of these centres will not only be to combat rare diseases but to create the necessary environment for transmission of knowledge.

6. Finally because many of the rare diseases are of hereditary nature, **research and innovation** is absolutely essential in order to enhance the **diagnostic ability through genetic tests**.

7. There are cases for which genetic tests exist and can be applied to **pre-implantation procedures** in order to avoid these diseases in embryos and consequently eradicate some of these diseases. This should be done in concerted action with genetic counselling and networks of information. This is in practice in some Member States for certain diseases, and it should be considered and taken into account as the application of these actions will eradicate in the end some of these diseases.