



COMMISSION OF THE EUROPEAN COMMUNITIES

Brussels, 15.04.1998
COM(1998) 232 final

97/0146 (COD)

Amended proposal for a

EUROPEAN PARLIAMENT AND COUNCIL DECISION

adopting a

**programme of Community action 1999-2003 on rare diseases in the context of
the framework for action in the field of public health**

(presented by the Commission pursuant to Article 189 a (2)
of the EC-Treaty)

EXPLANATORY MEMORANDUM

(1) The initial proposal for a European Parliament and Council decision adopting a programme of Community action on rare diseases was adopted by the Commission on 26 May 1997¹.

(2) The Economic and Social Committee² and the Committee of the Regions³ delivered favourable opinions on this initial Commission proposal.

(3) In the light of the European Parliament's opinion after its first reading on 10 March 1998 the Commission, in accordance with article 189a (2) of the treaty, is now submitting an amended proposal for a decision. The text incorporates amendments that clarify and broaden the actions covered by the programme.

(4) The European Parliament adopted 28 amendments of which 16 can be accepted by the Commission: 1 in toto, 2 with slight rewording and 13 partially (matched with a modified proposition).

(5) The 12 amendments which are not acceptable by the Commission can be grouped as follows :

- 6 concern the recitals in the preamble ;
- 5 concern the articles of the decision ;
- 1 concerns the annex ;

The reasons for rejecting these 12 amendments are the following:

- 4 amendments are not acceptable for legal reasons (6, 10, 20, 21) ;
- 1 amendment falls outside the scope of the programme (18)
- 3 amendments are unacceptably limiting (4, 15, 24)
- 2 amendments are covered elsewhere (11, 13)
- 2 amendments are unacceptable due to budgetary limitations (14, 19).

¹ OJ N°C 0203 of 03.07.97

² Opinion delivered on 29 October 1997, OJ N°C 19 21.1.98

³ Opinion delivered on 20 November 1997, OJ N°C 64 27.2.98

Amended proposal for a European Parliament and Council Decision adopting a programme of Community action 1999-2003 on rare diseases in the context of the framework for action in the field of public health

**THE EUROPEAN PARLIAMENT AND
THE COUNCIL OF THE EUROPEAN
UNION**

Having regard to the Treaty establishing the European Community, and in particular Article 129 thereof,

Having regard to the proposal from the Commission,

Having regard to the opinion of the Economic and Social Committee,

Having regard to the opinion of the Committee of the Regions,

Acting in accordance with the procedure referred to in Article 189b of the Treaty,

1. Whereas, the very fact of the rareness of the low-prevalence diseases and conditions and the consequent lack of information about them can lead to people affected by these conditions not receiving the health resources and services they need;

Having regard to the proposal from the Commission,¹

Having regard to the opinion of the Economic and Social Committee,²

Having regard to the opinion of the Committee of the Regions,³

1a) Whereas Community actions should be directed towards the prevention of diseases and whereas action by the Community can provide unique added value to tackling problems, the scale of which in individual countries is too small to allow the necessary analysis or proper action;

1b) Whereas the number of people affected by individual rare diseases may, by definition, be relatively small, but whereas the aggregation of all patients with rare diseases amounts to a substantial number in the population;

¹ OJ N° C 203 of 03.07.97

² OJ N° C 19 of 21.01.98

³ OJ N° C 64 of 27.02.98

2. Whereas, for the purposes of this programme rare diseases will be defined as life-threatening or chronically debilitating diseases which are of such low prevalence that special combined efforts are needed to address them;

3. Whereas, in accordance with point (o) of Article 3 of the Treaty, Community action shall include contribution towards the attainment of a high level of health protection;

4. Whereas Article 129 expressly provides for Community competence in this field in so far as the Community contributes to it by encouraging co-operation between the Member States and, if necessary, lending support to their action; promoting co-ordination of their policies and programmes, and fostering co-operation with third countries and international organisations competent in the sphere of public health; whereas Community action should be directed towards the prevention of diseases, and the promotion of health education and information;

1c) Whereas rare diseases are considered to have little impact on society as a whole due to their low prevalence individually, but they pose serious difficulties for sufferers and their families;

2a) Whereas understanding of rare diseases needs to be improved, since they constitute warning signs from a public health perspective to the extent that problems shown to affect a small section of the population could, as time passed, spread to the community as a whole if the incidence of the causes were to become more frequent;

5. Whereas rare diseases have been identified as a priority area for Community action within the framework for action in the field of public health¹

6. Whereas in its Resolution (A4-0311/95) on the Medium-Term Social Action Programme 1995-1997² the Parliament asked the Commission to present, under the proper procedures, the action programme for rare diseases foreseen in the Commission's framework communication on public health;

7. Whereas, in accordance with the principle of subsidiarity, action on matters not under the exclusive competence of the Community, such as action on rare diseases, must be undertaken by the Community only if and in so far as, by reason of its scale or effects, it may be better achieved at Community level;

¹. COM(93) 559 final
². OJ N° C 32 of 05.02.96

5. Whereas rare diseases have been identified as a priority area for Community action within the framework for action in the field of public health⁴

5a) Whereas a programme of Community action on rare diseases must be undertaken as a part of a coherent strategy which also includes provisions for orphan medicinal products and research on rare diseases and whereas a Community databank should be compiled to store updated information on rare diseases and whereas detection and control systems should likewise be set up;

6. Whereas in its Resolution (A4-0311/95) on the Medium-Term Social Action Programme 1995-1997⁴ the Parliament asked the Commission to present, under the proper procedures, the action programme for rare diseases foreseen in the Commission's framework communication on public health;

⁴. COM(93) 559 final
⁵. OJ N° C 32 of 05.02.96

8. Whereas, the Community can provide added value to the actions of Member States concerning rare diseases, through the co-ordination of national measures, the dissemination of information and experiences, the joint establishment of priorities, the development of networking as appropriate, selection of European Community-wide projects and the motivation and mobilisation of all involved;

in particular health professionals and patients' and support group associations;

8a)

Whereas, in accordance with the social and human rights model of disability supported in the Commission communication on equality of opportunity for people with disabilities (COM(96)0406), groups and organisations of persons with rare diseases and their families should be associated with the implementation of the programme.

9. Whereas co-operation with the international organisations competent in the field of public health and with third countries should be fostered;

10. Whereas, by providing support for acquiring better knowledge and understanding of, and wider dissemination of information about rare diseases and by developing actions complementary to existing Community programmes and actions, while avoiding unnecessary duplication, the programme will contribute to the achievement of the Community objectives set out in Article 129;

10a)

Whereas the high level of technology currently available can contribute significantly to the acquisition of better knowledge and

understanding of, and the wider dissemination of information about rare diseases, as stated above, and whereas this technology should be used to enhance the achievement of the objectives and actions envisaged under the programme;

11. Whereas, in order to increase the value and impact of the programme, a continuous assessment of the actions undertaken should be carried out, with particular regard to their effectiveness and the achievement of the objectives set and, with a view where appropriate, to making the necessary adjustments;
12. Whereas this programme should be of five-year duration in order to allow sufficient time for actions to be implemented to achieve the objectives set;
13. Whereas the introduction of specific Community arrangements will help to ensure that all Member States are swiftly informed in the event of an emergency situation, so that the protection of the population can be ensured;
14. Whereas these Community arrangements for the rapid exchange of information do not affect the Member States' rights and obligations under Treaties or bilateral and multilateral conventions;
15. Whereas an agreement on a *modus vivendi* between the European Parliament, the Council and the Commission concerning measures for the implementation of acts adopted under the procedure laid down in Article 189b of the Treaty was reached on 20 December 1994.

16. Whereas this decision lays down, a financial framework constituting the principal point of reference, within the meaning of point 1 of the Declaration of the European Parliament, the Council and the Commission of 6 March 1995, for the budgetary authority during the annual budgetary procedure;
17. Whereas the Community's financial perspective is valid up until 1999 and will have to be revised for the period beyond that date;
18. Whereas the financial framework for the last four years of the programme (2000-2003) shall be determined after the establishment of the future financial perspectives;

HAVE DECIDED AS FOLLOWS:

Article 1

Establishment of the programme

1. A programme of Community action on rare diseases hereinafter referred to as "this programme", is hereby adopted for the period 1 January 1999 to 31 December 2003 in the context of the framework for action in the field of public health.
2. The aim of this programme is to contribute towards ensuring a high level of health protection in relation to rare diseases by providing knowledge about these diseases by promoting and strengthening patient support groups and by fostering the setting up of cluster response teams.
2. The aim of this programme is to contribute towards ensuring a high level of health protection in relation to rare diseases by providing knowledge about these diseases, especially for patients and health professionals, by promoting and strengthening patient support groups, and by fostering the setting up of cluster response teams.
3. The actions to be implemented under this programme and their specific

objectives are set out in the Annex under the headings:

- 1) Actions on Community information on rare diseases
- 2) Actions in support of patient and family support groups
- 3) Actions on handling rare diseases clusters

3) Actions on identifying handling and responding rapidly to rare diseases clusters.

Article 2

Implementation

1. The Commission shall ensure implementation, in close co-operation with the Member States, of the actions set out in the Annex.
2. The Commission shall co-operate with institutions and organisations active in the field of rare diseases.

Article 3

Budget

1. The financial framework for the implementation of the programme for the year 1999 shall be ECU 1,3 Million, in keeping with current financial perspectives. The financial framework for the final four years of the programme (2000-2003) shall be determined in detail after the establishment of the future financial perspectives.
2. The annual appropriations shall be established by the Budgetary Authority in accordance with the financial perspectives.

Article 4

Consistency and complementarity

The Commission shall ensure that there is consistency and complementarity between the Community actions to be implemented under this programme and those implemented under other relevant Community programmes and actions.

Article 5

Committee

1. In implementing this action plan, the Commission shall be assisted by an advisory committee, hereinafter referred to as "the Committee", consisting of two representatives from each Member State, and chaired by the Commission representative.
2. The representative of the Commission shall submit to the Committee a draft of the measures to be taken concerning, in particular:
 - (a) the criteria, and procedures for selecting and financing projects under this programme;
 - (b) the evaluation procedure.

The Committee shall deliver its opinion on the draft, within a time limit which the chairperson may lay down according to the urgency of the matter, if necessary by taking a vote.

The opinion shall be recorded in the minutes; in addition, each Member State shall have the right to ask to have its position recorded in the minutes.

1. In implementing this action plan, the Commission shall be assisted by an advisory committee, hereinafter referred to as "the Committee", consisting of two* representatives from each Member State, and chaired by the Commission representative.

* Correction of the text due to a mistake in the English version of the proposal COM(97) 225 final

The Commission shall take the utmost account of the opinion delivered by the Committee. It shall inform the Committee on the manner in which its opinion has been taken into account.

The representative of the Commission shall keep the Committee regularly informed about Commission proposals or Community initiatives and the implementation of programmes in other policy areas which are relevant to the achievement of the objectives of this programme.

Article 6

International co-operation

1. In the course of implementing this programme, co-operation with third countries and with international organisations competent in the field of public health shall be fostered.
2. This programme shall be open to participation by the associated countries of Central Europe (CEC), in accordance with the conditions laid down in the Association Agreements or Additional Protocols related thereto concerning participation in Community programmes. This programme shall be open to participation by Cyprus and Malta on the basis of additional appropriations in accordance with the same rules as those applied to the EFTA countries, in accordance with procedures to be agreed with those countries.

Article 7

Monitoring and evaluation

1. In the implementation of this Decision, the Commission shall take the necessary measures to ensure

the monitoring and continuous evaluation of the programme taking account of the general and specific objectives referred to in Article 1 and in the Annex.

2. During the third year of this programme, the Commission shall present to the European Parliament and to the Council an evaluation report
3. The Commission shall submit to the European Parliament and the Council a final report on completion of this programme.
4. The Commission shall incorporate into these two reports information on Community financing in the various fields of action and on complementarity with the other actions referred to in Article 4, as well as the results of the evaluations. It shall also send them to the Economic and Social Committee and the Committee of the Regions.

Done at Brussels,

For the European Parliament
For the Council

The President
The President.

ANNEX

SPECIFIC OBJECTIVES AND ACTIONS

I. ACTIONS ON COMMUNITY INFORMATION ON RARE DISEASES

Objective: to provide knowledge about rare diseases especially for patients, health professionals and researchers.

Objective : to provide knowledge about rare diseases especially for patients and their relatives, health professionals and researchers.

1. Encouragement and support for the establishment of a European rare diseases database, with entries listing the disease name, synonyms, a general description of the disorder, symptoms, causes, affected population, standard treatments, investigational treatments (when available) and a list of resources that can be contacted for further information about the condition.

2. Promoting access to information and co-ordinating existing information systems and services by supporting the setting up and strengthening of networks at local, regional, national and community level.

3. Organising consensus meetings among health professionals in order to improve the early detection, recognition, intervention and prevention of rare diseases.

II. ACTIONS IN SUPPORT OF PATIENT AND FAMILY SUPPORT GROUPS

Objective: to establish, foster and strengthen voluntary organisations involved in supporting people directly or indirectly affected by rare diseases.

4. Promoting the establishment of groups of persons with the same rare conditions or those professionally involved in order to disseminate their experience, to facilitate training and to

1. Encouragement and support for the establishment of a European rare diseases database, with entries listing the disease name, synonyms, a general description of the disorder, symptoms, causes, affected population, standard treatments, investigational treatments (when available) and a list of resources that can be contacted for further information about the condition. The information collected in the central European database described above will be made as widely available as possible.

co-ordinate their activities at national and Community level.

5. Promoting the groups' collaboration and networking and the setting up and fostering of umbrella bodies, focusing particularly on efforts to encourage the continuity of work and cross-national co-operation.

III. ACTIONS ON HANDLING RARE DISEASES CLUSTERS

Objective: to ensure an efficient handling of the problem of clusters, which is of key importance for rare diseases.

6. Supporting the monitoring (sentinel) of rare diseases, including birth defects, genetic disorders or diseases of different organ systems and the appropriate techniques for low-prevalence diseases, in order to meet on the one hand the demands of detection, treatment and research and on the other hand the demands of relevant statistical monitoring.
7. Promoting the creation of rare diseases response teams and of specialised training courses for those investigating clusters.
8. Supporting surveillance and early warning systems for clusters.
9. Encouraging the exchange of expertise in the evaluation, assessment, communication and management of clusters of rare diseases that are associated with exogenic causes.

Objective : to have in place an effective system of monitoring in order to ensure the rapid identification, assessment and handling of rare diseases and diseases clusters.

6. Promoting the development of systems for the monitoring of rare diseases at community level, including birth defects, genetic disorders or diseases of different organ systems and the appropriate techniques for low-prevalence diseases, in order to meet on the one hand the demands of detection, treatment and research and on the other hand the demands of relevant statistical monitoring.
7. Promoting the creation and development of structures for providing appropriate responses for rare diseases and of networking and training for the investigation and handling of clusters.

ISSN 0254-1475

COM(98) 232 final

DOCUMENTS

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05 15 16 01

Catalogue number : CB-CO-98-245-EN-C

ISBN 92-78-35074-5