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DRAFT

PROGRAMME OF COMMUNITY ACTION

ON RARE DISEASES

(1999 – 2003)

**Decision No 1295/99/EC of the European Parliament and
of the Council of 29 April 1999**

(O.J. n° L 155 of 22.06.1999, p. 1)

ANNUAL WORK PROGRAMME FOR 2001

(Art. 5(2) of Decision 1295/99/EC)

ANNUAL WORK PROGRAMME FOR 2001

1. INTRODUCTION

For the purposes of this programme, rare diseases, including those of genetic origin, are life-threatening or chronically debilitating diseases which are of such low prevalence that special combined efforts are needed to address them so as to prevent significant morbidity or perinatal or early mortality or a considerable reduction in an individual's quality of life or socio-economic potential;

As a guide, low prevalence can be understood as meaning prevalence which is generally recognised as less than 5 per 10 000 in the Community.

European Parliament and Council Decision No 1295/99/EC of 29 April 1999¹ adopting a programme of Community action on rare diseases (1999-2003) provides that the Commission representative shall submit to the Committee an annual work programme indicating the priorities for action (Article 5(2b)).

Article 1 of Decision No 1295/99/EC defines the aim of the programme as follows:

- “2. The aim of this programme is to contribute, in coordination with other Community measures, towards ensuring a high level of health protection in relation to rare diseases by improving knowledge, for example by promoting the setting-up of a coherent and complementary European information network on rare diseases, and facilitating access to information about these diseases, in particular for health professionals, researchers and those affected directly or indirectly by these diseases, by encouraging and strengthening transnational cooperation between voluntary and professional support groups for those concerned, and by ensuring optimum handling of clusters and by promoting the surveillance of rare diseases.”
3. The actions to be implemented under this programme are set out in the Annex.”

The Annex to Decision 1295/99/EC lists four actions:

- “1. Promote the development of, and access to, a coherent and complementary European information network on rare diseases, using the existing databases, among other things. The information is to comprise entries listing the disease name, synonyms, a general description of the disorder, symptoms, causes, epidemiological data, preventive measures, standard treatments, clinical trials, diagnostic laboratories and specialised consultations, research programmes and a list of sources that can be contacted for further information about the condition. The availability of this information must be made as widely known as possible, including via the Internet.

¹ OJ n° L 155 of 22.06.1999

2. Contribute to training and refresher courses for professionals in order to improve early detection, recognition, intervention and prevention in the field of rare diseases.
3. Promote transnational collaboration and networking between groups of persons directly or indirectly affected by the same rare conditions or volunteers and professionals involved and coordination at Community level in order to encourage continuity of work and trans-national cooperation.
4. Support at Community level the monitoring of rare diseases in the Member States and early warning systems for clusters, and promote the networking and training of experts concerned with the handling of rare diseases and with rapid response to the phenomenon of clusters.”

These actions constitute the main reference framework for the implementation of the programme and the establishment of priorities.

In the next pages and in the context of the programme implementation, these actions will be referred to using the following abbreviations:

Action 1 “European information network on rare diseases”;

Action 2: “Training on rare diseases”;

Action 3 “Transnational collaboration on rare diseases”;

Action 4 “Monitoring, surveillance, early warning for clusters of rare diseases”.

The present work programme identifies the actions that should be implemented as a matter of priority in 2001. For this year, the projects selected for funding will be chosen from the applications submitted in reply to a specific single call for proposals with 15 September 2000 as deadline for submission.

2. PRIORITIES FOR ACTION 2001

2.1 General directions and balances of the programme

Community added value. In order to generate added value for the European Community, priority will be given to projects and to networks with a substantial European dimension that are likely to make a significant contribution towards the attainment of the programme’s objectives. In this context, the programme’s primary purpose is not to provide direct support to local fieldwork activities or structures but to encourage initiatives having the largest possible Community dimension, contributing to Community policies.

Horizontal approach. The budget allocation of €6.5 million for the period 1999-2003 (an average of €1.3 million per year) is too limited to allow a proper use of a disease-specific approach, considering that there are more than 5.000 rare diseases. In particular for actions 1, 2 and 3, priority will be given to projects addressing or providing for action on rare diseases in general, important groups of diseases or at least a considerable number of them.

Priority actions All programme actions (1, 2, 3 and 4) will be implemented during 2001, with the same level of priority. In the selection of projects, preference will be given to projects that are likely to contribute to the new Community health strategy as defined in the Commission communication COM 285(2000) of 16/5/2000 and in the proposal for a Community action Programme in the field of public health (2001-2006).

2.2 Priorities by action

2.2.1. Action 1 “European information network on rare diseases”

Priority will be given to projects based on existing databases and using Internet technologies (World Wide Web), in order to make it available to the general public; these databases should as far as possible address rare diseases in general, important groups of rare diseases (such as genetic diseases) or at least a considerable number of them.

The databases should consist on disease-based entries (list of rare diseases). The information in the databases should comprise the following content: disease name, synonyms, a general description of the disorder, symptoms, causes, epidemiological data, preventive measures, standard treatments, clinical trials, diagnostic laboratories and specialised consultations, research programmes and a list of sources that can be contacted for further information about the condition.

The information in these databases should not comprise any personal data of patients affected by rare diseases.

The information in the databases will have to be validated and constantly brought up-to-date. The beneficiary of the grant must arrange such validation with the assistance of scientific-health experts. The information should be consistent with the general criteria developed to assess the quality of health information on the Internet.

The Internet home page of the European information network on rare diseases will be available on the Europa server (the main server of the European Commission), with links established to the external databases making up the network. An adequate disclaimer clauses’ policy will be adopted.

2.2.2. Action 2: “Training on rare diseases”

Priority will be given to projects organising training and refresher courses for professionals aimed at improving early detection, recognition, intervention and prevention in the field of rare diseases. These courses should as far as possible address rare diseases in general, important groups of rare diseases (such as genetic diseases) or at least a considerable number of them.

2.2.3. Action 3 “Transnational collaboration on rare diseases”

Priority will be given to projects of transnational collaboration and networking promoted by “umbrella organisations” (organisations pooling together and/or representing other organisations). These organisations should as far as possible cover rare diseases in general, important groups of rare diseases (such as genetic diseases) or at least a considerable number of them.

2.2.4. Action 4 “Monitoring, surveillance, early warning for clusters of rare diseases”

Priority will be given to projects of monitoring and surveillance of rare diseases, in order to improve the systematic collection, analysis, interpretation and dissemination of epidemiological data on rare diseases; furthermore, priority will be given to projects concerning early warning and rapid response to rare diseases clusters. The personal data used in these projects should be held in a manner consistent with Directive 95/46/EC.

3. IMPLEMENTATION OF THE PROGRAMME

3.1 Committee meetings

Article 2 and 5 of Decision 1295/99/EC stipulate that, for the purposes of implementing the programme, the Commission shall be assisted by a Committee consisting of two representatives of each Member State and chaired by a representative of the Commission. For 2001, the Commission will make arrangements for the Committee to meet twice.

The Commission foresees to submit to the Committee by year 2001 the following drafts of the measures to be taken in accordance to article 5(2):

- (a) the evaluation procedure;
- (b) the arrangements for dissemination and transfer of results;
- (c) the procedures for coordination with programmes and initiatives which are of direct relevance to achievement of the aim of this programme;
- (d) the arrangements for cooperating with the institutions and organisations referred to in Article 2(2).

3.2 Working parties

The Committee's rules of procedure provide that working parties may be set up in order to deal with specific aspects of the action programme.

Such working parties could provide a focal point for exchanges of experience and information between Member States and the Commission. They could also formulate proposals for the Committee concerning the direction to be taken and priorities to be given to certain actions of the programme. The creation of such working parties, and their mandates, will be determined in consultation with the Committee.

3.3 Calls for proposals

In order to select projects for years 1999 and 2000, a call for proposals has been published in the Official Journal of the European Communities with 15 March 2000 as deadline for submission. For the following years, it is envisaged to publish a call for proposals each year, with 15 September as deadline for submission of applications (15 September 2000 for year 2001).

3.4 Information on the action programme - Dissemination of results

On the basis of article 5(2e), the Commission will submit a specific draft measure to the Committee presenting the arrangements to make sure that the general public, those directly or indirectly affected and the health professional dealing with rare diseases are informed and the results of the programme are properly disseminated.

The Commission recommends the production of brochures explaining the programme, the publication and circulation of documents and reports, the attachment of greater importance to the exploitation/dissemination criterion when selecting projects, the publication of a directory of the projects financed by the programme.

The Internet site of the European Commission will feature all relevant information concerning the programme. As stated above under 2.2.1., the Internet home page of the European information network on rare diseases will be created on the public health pages of the Europa server (the main server of the European Commission), with links established to the external databases making up the network.

The address of the home page of the Europa server is (subject to possible future modifications):

<http://europa.eu.int>

4. CONSISTENCY AND COMPLEMENTARITY

As foreseen in article 3 of Decision No 1295/99/EC, the Commission shall ensure that there is consistency and complementarity between the actions to be implemented under this programme and the following other Community programmes and actions:

4.1 Regulation (EC) No 141/2000 of the European Parliament and of the Council on orphan medicinal products

This Regulation was recently adopted by the EP and Council². Its purpose is to lay down a Community procedure for the designation of certain medicinal products as orphan medicinal products and to provide incentives for research into and the development and placing on the market of designated orphan medicinal products.

A Commission Regulation (EC No 847/2000) laying down the provisions for implementation of the criteria for designation of a medicinal product as an orphan product has been recently adopted³. It also contains provisions regarding the relation with other Community activities on rare diseases: as far as the prevalence of a condition as criteria for designation is concerned, the Regulation provides the following (article 2(1d):

² OJ L. 18 of 22.01.2000

³ OJ L 103 of 28.4.2000

“where a diseases or condition has been considered within the framework of other Community activities on rare diseases, this information shall be provided. In case of diseases or conditions included in projects financially supported by the Community in order to improve information on rare diseases, a relevant extract from this information, including in particular details of the prevalence of the diseases or condition in question, shall be provided.”

The Commission has also appointed 6 Members of the Orphan Drugs Committee, 3 in representation of the EMEA and 3 in representation of patients’ organisations. The three representatives of the patients’ organisations may be consulted also for matters concerning the implementation of the rare diseases programme. At this regard, the Commission will present, as foreseen by article 5(2g) of Decision No 1295/99/EC, a specific draft measure concerning the arrangements for cooperating with institutions and organisations active in the field of rare diseases.

4.2 Community research

Community research on rare diseases is currently being carried out under the 5th Framework (1998-2002) programme for research and technological development. In the context of the specific Programme-“Quality of live and management of living resources”, research on rare diseases is being carried out mainly under activity 7. “Chronic and Degenerative Diseases, cancer, diabetes, cardiovascular diseases and rare diseases”. Also activities 8 “Research into Genomes and diseases of genetic origin” and 9. “Neurosciences” are particularly relevant for rare diseases.

The rare diseases programme will take into account these important research activities, and will contribute, particularly through action 1 “European information network on rare diseases” to the dissemination of the results of the research on rare diseases. The possibility of creating direct Internet links with the Cordis database will be examined. Furthermore, the Commission, assisted by the rare diseases Committee, will consider how the result of those research projects most directly related to the rare diseases programme, supported under the Fifth Framework Programmes might be used as an input in the attainment of the objectives of the rare diseases programme.

The rare diseases programme will also take into account the Community research carried out in the field of Health Telematics, considering its importance for action 1 “European information network on rare diseases” and action 4 “Monitoring, surveillance, early warning for clusters of rare diseases”.

Appropriate co-ordination arrangements will be established in order to avoid any duplication and to ensure complementarity as far as funding of projects is concerned.

4.3 Programme of Community action on health monitoring (1997 to 2001)

Decision No 1295/99/EC clearly provides that the systematic collection of health data is carried out within the framework of the programme of Community action on health monitoring and that a regular exchange of information and data must therefore be ensured between the health monitoring and the rare diseases programmes (recital 20).

4.4 Programme of Community action on the prevention of AIDS and certain other communicable diseases (1996-2000)²; Network for the epidemiological surveillance and control of communicable diseases in the Community

Several important communicable diseases are also often rare in the Community and therefore could be in principle addressed both by the Programme-Network on communicable diseases or by the rare diseases programme.

As a general line to ensure proper coordination, the Commission recommends that rare communicable diseases should be in first instance treated as communicable diseases, that is to say their surveillance-early warning should be carried out in the context of the Community Network on communicable diseases, and applications for funding should be directed towards the Programme for the prevention of AIDS and certain other communicable diseases.

4.5 Action plan to combat cancer (1996-2000)²

Also many types of cancer are rare. As a general line to ensure proper coordination, the Commission recommends that rare cancers should be in first instance treated under the action plan to combat cancer (1996-2000).

4.6 IDA (Interchange of Data between Administration)

The IDA-EUPHIN project (European Union Public Health Information Network) is already providing the telecommunication infrastructure for two public health Community actions, that is to say the Health Monitoring Programme and the Network on Communicable Diseases. As soon as possible it will also be extended to cover the rare diseases programme (IDA-EUPHIN-rare diseases), in particular action 1 "European information network on rare diseases" and action 4 "Monitoring, surveillance, early warning for clusters of rare diseases". During the second half of year 2000, the preparatory phase will start.

5. BUDGET

The Commission has proposed to the budgetary authority that the annual budget for year 2001 is €2 million (€1.3 million increased with €0.7 million deriving from the budget 1999 that was not executed. The remaining portion (€0.6 million) could be transferred to the year 2002).

² This programme is likely to be prolonged to year 2001.

6. INTERNATIONAL COOPERATION

The participation of candidate countries in Community programmes is an important component of the enlargement strategy. The Commission has recently presented a Communication to the Council reviewing in a comprehensive way the participation of candidate countries in Community programmes, agencies and committees (COM(99)710 of 20 December 1999).

Article 6(2) of Decision 1295/99/EC provides for the participation by the associated countries of central Europe in the rare diseases programme. The opening of the programme to those countries is subject to the official expression of their willingness to participate. Once this has been expressed, the implementation of the opening can be prepared, and it will be effective with the entry into force of specific Association Council Decisions. The actual implementation will have to take into account the specific rules concerning the allocation of credits as well as the budget available.

The approach envisaged comprises the identification of suitable institutions as coordinators of the programme and the definition of a work plan including priorities in the participating countries. In general, this participation should focus on joining the most important European projects/networks set-up and supported by the Community programme.

The participation of candidate countries in the rare diseases programme should also be considered in the context of the proposal for a new public health programme 2001-2006 (COM 285/2000) that would repeal all existing public health programmes. The Commission recommends that applicant countries participate in this new programme right from the beginning.

7. MONITORING AND EVALUATION

Article 7(1) of Decision No 1295/99/EC provides that the Commission shall take the necessary measure to ensure the monitoring and the continuous evaluation of this programme.

In particular, Article 7(2) provides that the Commission shall submit to the Council and the European Parliament an interim report during the third year of the programme and a final report upon completion of the programme. These reports will incorporate, among other things, the results of the evaluation.

On the basis of article 5(2d) the Commission will submit as soon as possible a specific draft measure to the Committee concerning the evaluation procedure.