EURORDIS

European Organization for Rare Disorders April 23rd, 2001

Intermediate Activity Report
Agreement N° SI 2.288939 (2000 CV G4 –801)

To create and animate a European transnational network on rare diseases around key themes : Orphan Medicinal Products to the service of patients affected by rare diseases ».

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1. Introduction

This project is the first part of a global long term project covering 2000/2003 period : to create and animate a European transnational network on rare diseases around key themes. The first theme developed is : Orphan Medicinal Products to the service of patients affected by rare diseases ». The main objectives are :

- to strengthen existing national alliances
- to strengthen collaboration at Community level among associations on rare disorders
- to develop new national alliances in European Countries (Belgium, Netherlands, Portugal).
- To develop partnerhips among all alliances.

This project is the opportunity for members and national alliances :

- to get to know more about Orphan Medicinal Products theme
- to develop communication between national alliances at the European level and to know each other better through borders and pathologies.

In this intermediate report we will develop :

- summary of progress to date against planned schedule
- key findings to date
- next steps until the end of the project

2. Summary of progress

2.1. Generality and launching of project

Generally speaking and up to now, the present project is achieved according to planned schedule.
The major steps are the following :

⇒ **Kick off meeting** (see agenda enclosed)
Date : October 20th, 2000
Location : Brussels
Number of participants : 21 persons - 1 coordinator per national alliance (7) Denmark, Germany, Italy, France, Spain, Sweden, UK, 1 coordinator per potential national alliance (3) Belgium, Netherlands, Portugal, Board members of EURORDIS, representative of Gemini Consulting, EURORDIS coaches
Objective : Present the project, obtain ownership from each National alliance and potential ones, design a methodology for the workshops at national level, develop partnerships between alliances and potential alliances, help in the preparation of workshops with coordinators of alliances.

The kick-off meeting has fulfilled our objectives which were to put on-board each alliance involved and have them fully motivated for this ambitious project.

After each participant gave their expectations of the meeting, we presented the project as well as some update information of the work achieved at COMP (Committee on Orphan Medicinal Products) in order for our participants to get the same background on the subject but also to see the links between the COMP and this present project. The second part of the meeting was more a training session to

give to participants some methodology so that they can develop more easily their workshops in their respectives countries. Some coaches were designated to help and advise each national alliance all along the project. A plan of actions as well as a planning were drawn (see enclosed) with the participants during the meeting. All national workshops were scheduled according to the present plan of action, in January/February 2001. We gave to coordinators some guidelines to build the agenda of the workshops and later on guidelines to write the reports after the workshop. The goal was for each national alliance to develop 2 days workshop in their respective countries in January/February 2001.
Then we developed a small brainstorming exercise by using metaplan to give them some tool to be used at their workshops.

2.2. National Workshop

Time frame : January/February 2001 – as planned
7 Member states : Denmark, France, Germany, Italy, Spain, Sweden, UK
Agendas : See agendas enclosed
Participants : see list of participants
Objectives : To develop 2 days workshop and brainstorming session on Orphan Medicinal Products. To explore the situation at national level, find about the availability of Orphan Medicinal Products, Research…., make recommendations and start a plan of action
Methodology : Preparation of national workshops in coordination with EURORDIS - agenda, list of participants, logistic aspects for 10 participants - invitation of pharmaceutical industries representatives to participate in the discussion. After these two days workshops, all national alliances have written a report giving their recommendations and plan of action acccording to guidelines sent previously to them.
Coaching : Steering committee meetings were organized. The first one, on December 8th, 2000 in order to review all the agendas of workshops prepared by National Alliances. At the issue of that steering committee meeting, we drafted a sample agenda to help those alliances having difficulties to make one and asked each of them to draft a new agenda for December 21st 2000. The purpose of having a good agenda was to help national alliances to really prepare as much as possible their workshops so that the brainstorming days can bring good and valuable recommendations at the end.
Comments : All the workshops were developed in the 7 member states as scheduled. We received very positive feedback from all of them. The liveliness of the recommendations shows that great motivation and creativity were developed within national alliances in a good team spirit. We will develop more about the results in the key findings (see chapter 3) to give a first feedback on content.

2.3. European Workshop

Time frame : June 7th, 8th, 2001 *
Location : Brussels
Agendas : See draft agenda enclosed
10 Member states : Belgium, Denmark, France, Germany, Italy, Netherlands, Portugal, Spain, Sweden, UK
Objectives : To develop 2 days workshop, common understanding at European level, identify the needs and problems in Europe, share best practices and create synergies in Europe, make recommendations.

Coaching : A second steering committee meeting was hold after the National Workshops on March 20th, 2001 in London with Coaches to study the reports and make analysis of recommendations sent by national alliances.

We are now working on the agenda of this european workshop. The first day will be focussed on the reporting of each national alliance about their key findings both at national and european level. The 3 potential alliances will report on progress of building an alliance in their country. By the end of the day, some key issues will be identified and ready to be presented to Pharmaceutical Industries the next day.

The second day, after listening the different point of views of international organizations, european pharmaceutical industries, European Commission, the key issues identified by patients groups will be addressed to those representatives. The Vice-President of COMP will then present the recommendations which are currently implemented through that Committee.

At this point, with all these new elements a discussion on key issues with the present stakeholders will be then developed and animated by our partner, Gemini Consulting.

In the afternoon, the patients groups will be able to make a synthesis of this workshop and to select, prioritize the recommendations which will be delivered to the EU towards the end of the project.

* A request was sent to the European Commission, Mr A. Cluzeau, on April 2nd, 2001 to get an additional clause to the present contract, asking to extend the duration by 2 months which will allow us to organize the European Workshop in June instead of April as written in the initial contract.

3. Key findings

At a first glance of analysis, we have two levels of recommendations :
⇒ at national level (to date around 20 recommendations)
⇒ at european level (to date around 13 recommendations)

At national level, one of the recommendations which appears strongly in 4 national alliances out of 7 is to identify specific incentives and national funds for research on rare disorders, to implement the regulation on Orphan Medicinal Products and promote research. National Alliances are very concerned by the fact that the OMP regulation is not yet implemented in their member states.

At the European level, a first strong recommendation appears clearly as the most important to national alliances : to make an inventory of existing orphan medicinal products and to make them available to patients in all member states.

We have made a synthesis of recommendations (see attached document) according to the following axes :
⇒ Treatment non available/Existing treatment
⇒ Access to treatment/No access to treatment.

This synthesis gives us a good view of the type of recommendations which were reported to EURORDIS after the national workshops.

4. Next step until the end of the project

After the european workshop scheduled in June 2001, a synthesis document will be written and sent to all the stakeholders who participated to the workshop. In this synthesis document, the prioritized recommendations will be listed.

We will then enter into the phase IV of the project (September/October) which consists of developing one day national workshop in the 7 national alliances as well as in the 3 potential alliances to report to other patients groups and make analysis of the European workshop held in Brussels in June 2001. It will be then appropriate for each National Alliance to make action plans in their respective country regarding the recommendations already selected by the group at national level.

For the final phase – Phase V - Each National Alliance will submit to EURORDIS a synthesis paper of recommendations at national level, with a possible plan of action.
With all the elements gathered all through the project, EURORDIS will write final report and is planning to submit it to the European Commission by the end of year 2001.