Orphan Medicinal Products to the service of patients affected by rare disorders
PARD 1

A project conducted by

EURORDIS
European Organization for Rare Disorders

Under the

Programme of Community Action on Rare Diseases

Contract n° SI2.288939 (2000CVG4-811)

Executive summary
March 2002

The Annexes to the Final Activity Report are provided in a separate document
also dated March 2002 under the same reference.

I - Executive Summary

Around 5000 rare diseases affect daily the life of 20 million people in the European Union. Due to the very low prevalence of these disorders, the organisation, networking and strengthening of patient organisations at national level and even more at European level is extremely challenging. It is estimated that over 1000 local or national patient organisations exist in the European Union. The vast majority of these organisations are isolated, with very reduced membership for most rare disorders, extremely limited resources hence limited access to information, best practices and knowledge on treatments, new therapies, research, etc.

A European partnership is therefore essential to reach a critical mass of patients, experience, resources, and knowledge. In the context of the implementation of the EU Regulation on Orphan Medicinal Products (OMPs), it was considered that creating and animating a European trans-national network would be a critical factor to put OMPs to the service of patients affected by rare diseases. To this end, the project was structured to achieve four core objectives:

1. to strengthen existing national alliances and to develop new national alliances in European countries, around the theme "Orphan Medicinal Products"
2. to strengthen collaboration at European level among patient organisations
3. to develop partnerships among all alliances
4. to develop European recommendations and national action plans.

The project was conducted from October 2000 to December 2001 and was structured in four phases, built upon a range of European co-ordination meetings and workshops and National workshops. Across the four phases, these workshops involved over 200 distinct persons, among which:

- 75% representing patient organisations,
- 11% representing pharmaceutical industry,
- 10% representing universities, government or international agencies
- 4% representing national health systems.

Through this approach each national alliance was able for the first time to put in common, in a structured and co-ordinated way, their experience and best practices. This led to the development and dissemination of recommendations for both European and National authorities and of national action plans aiming at co-ordinating the implementation of these recommendations and strengthening activities of national organisations.

Benefited directly from the experience of existing national alliances, four new national organisations were set up in Germany, in The Netherlands, in Portugal and in Belgium.

It is estimated that 500 associations were put in contact or touched by communication on the results of this project. This directly resulted in enhanced exchanges and links between patient groups.

The development of a partnership between patients/industry and competent authorities is also one of the most promising achievement of this project. As reported by the European Agency for the Evaluation of Medicinal Products in its press release of February 27th, 2002 “this project has achieved a common understanding among patients groups for rare diseases in Europe and established a platform for addressing issues regarding orphan medicinal products”.