EURORDIS
European Organization for Rare Disorders

Final Conclusion
Minutes of the project leader's presentation
European workshop – Brussels, 8th June 2001

Terkel Andersen, the project Manager made the following conclusion remarks :

In the different reportings heard at this European workshops, we have made significant progress. Patients voice will be more easily heard in the future. The proposals, recommendations have been presented by the national alliances and we have now an overview of issues related to rare diseases that was not available until today. Three new national alliances are under formation. Making advocacy and involvement of patients organisations more easily. Rare Disorders have increased visibility and recognition as an important health issue. This is very much due to the contribution of national alliances organising national and European conferences and by putting increasing pressure on governments. The latest expression of this recognition came up this morning from Luca Martinelli's presentation : « the new health programme will comprise an explicit mention of rare disorders » this is a very important signal sent by the health ministers. On the other hand the inventory established by COMP clearly shows a lack of concrete actions from most European Union state members.

We have discussed the recommendations and obtained a broad and more common understanding of the issues. Most alliances have stressed the need for access to information, in particular information on Orphan Medicinal Products and the need for a shortening of the process from product development to availability to patients. Other important priorities have been the need for improved epidemiological data and a better system – national and European – to ensure rapid diagnostic and agreement on quick lines for treatment. Research and care would benefit from the creation of networks of centres of excellence and continuous assessment of these in a European context.

We have learnt from today's presentation and roundtable that many of our ideas and concerns are shared by the Commission and the EMEA/COMP representatives. The Orphan Medicinal Products availability at national level should be improved and Patrick Le Courtois from EMEA/COMP mentioned the current investigation into the status of Orphan Medicinal Products with Members States in Europe. The compassionate use issue was raised by several speakers. EURORDIS will take immediate action to comment on the revision of the European Union medical legislation.

During these two days, many have addressed the problems related to clinical trials and rare diseases. It was pointed out that close collaborations are essential to speed up the running of clinical trials. The new clinical trials directive should be promoted by patients organisations. It is important that information on clinical trials will soon be available online and we hope that this will allow patients to retrieve information on new drugs and therapies. It has been underlined by a number of speakers that post marketing follow-up is essential with orphan drugs in order to match the need for quick marketing with optimal safety.

It has become clear that the COMP is putting more emphasis on serving as a communication platform between all interested parties ensuring a strategic approach to other needs of patients with rare diseases. This development will be welcomed by people with rare diseases throughout Europe. We need such instruments to ensure a close monitoring of progress and new challenges.

This morning, we heard in the video from NORD, Marlene Haffner stating that « Rare Disorders are not rare », thus pinpointing the fact that the total number of people affected by one of the many rare diseases is very high. But rare disorders still need more recognition and visibility and for this reason patient involvement is essential to raise awareness and educate authorities, governments and public on rare diseases as a major health issue. Luckily patients do not stand alone. These two days have demonstrated that our goals and strategies coincide largely with other actors in the field and that we can benefit largely from collaboration and exchange.