EURORDIS CONTRIBUTION TO THE WORKING DOCUMENT ON THE ORPHAN DRUG REGULATION
MARCH 2006

EURORDIS - the European Organisation for Rare Diseases - represents 260 rare disease organisations from 29 countries, 18 of which are EU member states, and thereby reflects the voice of an estimated 30 million patients affected by rare diseases in the European Union.

In response to the Commission's open consultation on its working document on the experience acquired as a result of the application of the Regulation on orphan medicinal products and account of the public health benefits obtained, EURORDIS is pleased to send its contribution.

After the first five years of application of the Regulation on orphan medicinal products, EURORDIS and its Members welcome its success and agree with main conclusions of the Commission's report. EURORDIS considers that the orphan legislation has indeed been applied without any major difficulties and applauds the successful development of the twenty-four orphan medicinal products placed on the market, which may potentially benefit to more than one million patients.

Concerning the two objectives of the Regulation where progress to be made has been identified (e.g. access to authorised orphan drugs and effectiveness of incentives), EURORDIS would like to put forward the following proposals, which do not intend to modify the current legislative framework, but aim at improving its effective implementation through adequate policies:

1. On access to authorised orphan drugs

Unacceptable delays (far beyond the 180 days) in the availability of authorised orphan drugs and different levels of access to treatment for patients across the European Union have been reported to the COMP and the Commission. Considerable differences in access to orphan drugs have been observed between different EU Member States, but also between different regions within the same country.

For some products, and despite their designation for significant benefit, member states refuse their reimbursement, based on their assessment of the therapeutic added value, thus contradicting the COMP opinion.

The hurdle of access to orphan drugs throughout the EU can be partly explained by the difficulties encountered by national authorities to establish appropriate price and reimbursement levels for orphan medicinal products. Therefore, by pooling expertise to assess the therapeutic added value of orphan drugs and by accelerating the price negotiations, access to orphan drugs will be greatly improved.

This is why, EURORDIS has been advocating in recent years, in favour of the establishment of a new EU Committee (or a subgroup of the EU Transparency Committee).

The new Committee would help assessing the Therapeutic Added Value (TAV) of each orphan drug and discussing a European ex factory catalogue price with the marketing authorisation holder determining such reference price, which would help evaluating price level and reimbursement at national level, in the form of an advisory opinion.

This procedure would be implemented by Member States on a voluntary basis in order for competent authorities to draw on the reference price for their national decisions.

The procedure could start immediately after the positive opinion of the CHMP, before the Commission decision. Implementation of this measure is only a policy decision which does not require a new piece of legislation.

Another way to compensate for long post marketing authorisation delays is the possibility to open compassionate use programmes during the final phase of the product evaluation. However, for 73.5% of designated products there is no compassionate use programme. Furthermore, for many designated product, it is not clear whether or not the clinical development has actually started.

2. On incentives at EU and national levels

- Incentives at EU level:

EURORDIS believes that Commission's research grants, through the EU Framework Programmes, for performing clinical trials on orphan drugs would constitute an important incentive for the development of orphan medicinal products.

This could be achieved following the model of the research fund for paediatric medicines: a specific budget line in the 7th Research Framework Programme would be dedicated to phase 1 and phase 2 clinical trials for designated orphan drugs; the COMP would be the expert panel responsible for the evaluation of the projects submitted for funding. In this way, the COMP would play the same role for the development of orphan drugs than the one assigned to the Paediatric Committee for the development of paediatric medicines. Also, this measure would mirror the US FDA Programme for orphan drug clinical trial grants.

- Incentives at national level:

At national level, incentives mainly take the form of favourable tax policies and reductions.

EURORDIS believes that increasing the visibility of national best practices through the elaboration of comparative tables to be updated on a regular basis, would help encouraging EU Member States to improve measures at national level.

EURORDIS suggests that the Commission with the support of COMP develop a more pro-active approach to stimulate Member States to implement further incentives. The first step could be a workshop with Commission, Member States and COMP on this specific issue.

Conclusions

- EURORDIS strongly believes that the proposed actions would help improve the effectiveness of the implementation of the orphan drugs Regulation, while fully respecting the spirit of the legislation, which has proven to greatly contribute to the development of orphan medicinal products in the European Union.

- EURORDIS encourages the writing of recommendations to be addressed to Member States and national authorities to help reaching the full effectiveness of the Regulation as soon as possible, within the framework of the current legislation.

- In this context, EURORDIS calls for the organisation in the European Parliament of a Public Hearing on Orphan Drugs, gathering all relevant stakeholders.