

**EURORDIS CONTRIBUTION TO THE CONSULTATION
ON THE FUTURE OF THE EU SINGLE MARKET IN PHARMACEUTICALS
FOR HUMAN USE
OCTOBER 2007**

EURORDIS - the European Organisation for Rare Diseases - represents 300 rare disease organisations from 34 countries, 23 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 consultation on the future of the EU single market in pharmaceuticals for human use, EURORDIS is pleased to send its contribution, focused on the future of the Single Market in Orphan Drugs and based on previous Papers, such as "EURORDIS contribution to the open consultation on the Report on the Orphan Drugs Regulation".

1. Do you agree with the analysis of the main challenges outlined above? Do you see other challenges?

EURORDIS agrees with the broad analysis of the main challenges outlined in the consultation paper. Nevertheless, concerning the future of the EU Single Market in the specific field of Orphan Medicinal Products, EURORDIS has identified the following two additional challenges:

- To secure patients' access to authorised orphan drugs in the whole EU;
- To improve the effectiveness of existing incentives.

2. Do you see other areas than those already targeted by the Commission where regulatory action should be taken?

And

5. What can be done to foster convergence and transparency as regards pricing and reimbursement in the EU?

Without changing the current legislative framework, EURORDIS believes that two further steps are needed in order to address the specific challenges mentioned above in the Single Market for Orphan Medicinal Products, thereby improving the effective implementation of the Orphan Drug Regulation through adequate policies:

- Creation of a (sub)Committee on Therapeutic Added Value:

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, as well as to patient organisations. 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.

These measures are “policy decisions” aimed at accompanying and improving the implementation of an existing Regulation and therefore do not require the elaboration of a new piece of legislation.

- Upgrading incentives at EU and national level:

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.

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 the Commission, Member States and COMP on this specific issue.

<p>3. What would you suggest as concrete measures to ensure the safety of medicines supplied in the EU, addressing in particular counterfeit medicines, and provision of high quality and affordable medicines also to third countries?</p>

Drugs for the prevention, diagnosis and treatment of rare diseases and neglected diseases have to face the same challenge represented by the lack of interest from

the pharmaceutical companies given to the non-profitability of the markets concerned (either too narrow or too poor). It has therefore been acknowledged that there is a need for economic regulation in these fields.

Not only research must be encouraged into conditions which, for whatever reason, are not focused on by pharmaceutical companies, but it also has to be underlined that there is no such a thing as a geographical frontier when it comes to rare diseases and that being affected by a rare disease while living in a developing country does make you the poorest of the poor, exacerbating health, social and economic vulnerabilities. This is why EURORDIS has been advocating in favour of a global approach to orphan drugs, without limiting the reflection to the developed world, especially because - as pointed out by the Commission's consultation document - we do live in a "globalised economy" where research is global, drug development and market are global, and this is even truer in the "niche" of drugs for treating rare diseases.

EURORDIS believes that international health authorities, together with patient organisations, should increasingly support orphan drugs development on a global scale and promote orphan drugs regulation worldwide, either at regional and national or at international levels. We think that WHO should encourage actions aimed at speeding up transfer of designated orphan drugs from the EU or the US to other continents to improve access to patients and availability worldwide.

We do believe that it is a dangerous mistake to limit the reflection on orphan drugs only to certain regions of the world. We have to think global. We have to escape the equation: "rare diseases only refer to very expensive orphan drugs and therefore, we raise the problem of rare diseases only in the rich countries of the EU and in the US". If we do not come out of this circle, we will continue to build and reinforce the creation of a health for the rich and a health for the poor, based on geographical and political frontiers regardless of their social, economic and cultural environment and individual abilities, ignoring that poor families are struggling to access existing treatments in rich countries, as well as rich families in poor countries.

4. What can be done to improve Europe's international competitiveness?

EURORDIS believes that additional effort have to be made in terms of financing innovation, by supporting activities such as the Innovative Medicines Initiative.

EU efforts should also focus on the "valorisation" of research results performed by the academic world by stimulating and increasing the links between public research sector (academy) and private pharmaceutical companies (industry).

6. Do you think the current EU regulatory framework can accommodate emerging technologies like regenerative and personalised medicine, as well as nanobiotechnology?

EURORDIS has strongly welcomed the adoption of an EU Regulation on Advanced Therapies Medicinal Products and calls for the same kind of "principle" to be applied to other emerging technologies. This principle is: a legislative framework establishing a centralised EU system for the assessment of efficacy, quality and safety of innovative products, for the benefit of all European citizens, while allowing freedom of research within regulatory boundaries.