HOW CAN COOPERATION AT EUROPEAN LEVEL FACILITATE NATIONAL DECISIONS AND IMPROVE ACCESS TO ORPHAN DRUGS?

The regulatory framework established with the Orphan Drug Regulation, aimed at enhancing the development and placing on the market of Orphan Medicinal Products, has proven to be successful. In fact, the Orphan Drugs Regulation has allowed, up to July 2009, the designation of 584 Orphan Drugs, among which 49 have been granted a Marketing Authorisation.

Despite this successful outcome, the main problem of the overall European strategy on Orphan Drugs lies in the actual access to these drugs: rare diseases patients do not have equitable and timely access to the approved Orphan Drugs they need.

One major element hampering real and equitable access to Orphan Drugs is represented by national/regional delays in placing them on the market, often far beyond the legal timeframe of 180 days after the Marketing Authorisation has been granted. These delays at national level must be reduced in order to improve patients' access.

Through several recent policy documents (the EU High Level Pharmaceutical Forum conclusions and recommendations: "Improving Access to Orphan Medicines for all affected EU citizens", the Commission Communication on "Rare Diseases: Europe's Challenges" and the Council Recommendation on a European Action in the field of Rare Diseases), the European Commission and the Member States have called for an increased cooperation between EU level authorities and Member States in order to improve timely access to Orphan Drugs. This means that they both acknowledge that European level cooperation may facilitate - without replacing or imposing them - decisions on pricing and reimbursement that have to be taken at national level in order to allow for national market access.

In this context, EURORDIS and other stakeholders are proposing to create a Working Party for the assessment of the Clinical Added Value on Orphan Drugs (CAVOD) and to

locate this Working Party where the relevant knowledge and expertise on Orphan Drugs is gathered, namely at the EMEA.

The Working Party on the CAVOD will be a key instrument for an increased cooperation between Member States and EU level authorities to produce:

1. **Scientific Common Assessment Reports (CARs) on the CAVOD** to provide a non-binding basis for the national level to take appropriate, well-informed decisions on pricing and reimbursement within the legal timeframe, based on expert opinions that will support and speed-up national decisions;

2. **The Annex** to the CARs on CAVOD to provide a non-binding basis for agreement on the post-marketing studies required by Member States.

This approach will support the promotion of conditional pricing and reimbursement which can be reviewed in the following years based on data generated to better define the place of the medicine in the therapeutic strategy of the rare condition in real life setting.

In order to underline the link between the Working Party and the Member States, the role and importance of the Common Assessment Reports will be mentioned in the National Plans on Rare Diseases as a tool to minimise delays and improve timely patients' access to Orphan Drugs.

The success of this collaboration will largely depend on precisely defined role, mandate and composition of the Working Party. EURORDIS, together with other stakeholders, have developed a set of 27 recommendations to guide the establishment of a Working Party for the scientific assessment of the CAVOD. These recommendations aim at ensuring that the proposed process will neither weaken, nor interfere with the normal EU-centralised regulatory approval process and will rather complement it by bridging the gap between the European Commission / EMEA, and the EU Member States.

The 27 proposed recommendations cover the rationale, scope, mandate, roles and responsibilities of the Working Party, the format of the Common Assessment Reports, and finally the composition and essential rules of procedures of the Working Party.

Because there is a need for a strong link to the Member States to encourage them to use the Common Assessment Reports, EURORDIS also developed a set of proposals to the Member States in order to ensure their commitment to use these reports in their individual National Plans on Rare Diseases.

29 October 2009