13th Workshop
Eurordis Round Table of Companies

"Patients'Access to OMPs, Innovative Pricing Schemes and National Measures in a Global Financial & Economic Crisis Environment"

13th December 2010

Les Salons de l'Aéro-Club de France
6, rue Galilée – 75116 Paris

Concept Paper

The EURORDIS past surveys on Orphan Drug availability in Europe had pointed out unacceptable delays and inequalities in rare disease patients' access to their medicinal products. As also shown by other independent studies, like the one performed by Alcimed for the EC in 2004, EURORDIS has shown how different factors, like sponsors' market strategies and local NCA practices and procedures, result in a disparity of access to OMPs across Europe.

Many initiatives are in progress at the EU level to improve this situation and, in particular, European collaborations among Member States (MS) are ongoing or are being planned on different aspects of the post-marketing life of Orphan Drugs.

Among these, the EUnetHTA aims to create a sustainable system of HTA knowledge sharing, and to promote good practice in HTA methods and processes, while an ongoing EC/DG Health and Consumers tender will study "…the creation of a mechanism for the exchange of knowledge between Member States and European authorities on the scientific assessment of the clinical added value of orphan medicines…".

In 2008, among the conclusions and recommendations of the High Level Pharmaceutical Forum we can read that "Member State authorities, stakeholders and the Commission should strengthen their efforts to ensure access to orphan medicines in all EU Member States. They are therefore called upon to take up the appropriate ideas developed in the Working Group Pricing regarding i) early dialogue on research and development, ii) exchange of knowledge on the scientific assessment of the clinical added value, iii) specific pricing & reimbursement mechanisms and iv) increased awareness on orphan diseases."

Soon after the release of these conclusions, the global financial crisis hit the world and today, with this 13th ERTC workshop, EURORDIS would like to evaluate how the different stakeholders have implemented the above recommendations and what impact, if any, the financial crisis has had on patients' access to OMPs.

The morning session of this 13th workshop will be dedicated to an update of the state-of- the- art on the situation of patients' access to Orphan Drugs in Europe through two different studies, one

conducted in collaboration with the EU network of hospital pharmacists and the second conducted by EURORDIS for the first time, with the contribution of the Rare Disease National Alliances.

Three different pharmaceutical companies will be given the opportunity to report on their recent experiences on pricing and reimbursement at national level, highlighting in particular any differences in the national negotiation approaches and attitudes towards new orphan drugs accessing their market.

A panel discussion will end the morning session and will explore what pharmaceutical companies and National Competent Authorities (NCA) are ready to do or accept in order to ensure access to patients in a timely and affordable manner. It will also be the opportunity to hear about the new initiative of the Belgian Presidency of the EU: "Corporate Responsibility in the Field of Pharmaceuticals" and in particular on the new "Mechanism of Coordinated Access to Orphan Medicinal Products".

During the afternoon sessions, the attendees will be given the opportunity to discuss on different related and sensitive issues in parallel working groups:

1. *Considering the first innovative approaches and concrete experiences, what new pricing scheme could be implemented for orphan drugs in the upcoming years? (e.g. secured sale volumes, risk sharing, ….)* Is a single pricing scheme an acceptable option for all Member States?

2. *Which coordinated mechanism of access to orphan drugs is needed at the European level?*
   Bearing in mind that decisions on pricing and reimbursement are the exclusive competence of Member States, some common approaches could nevertheless be shared on a voluntary basis and be beneficial for:
   - A more rapid patient access to Orphan Drugs
   - A clearer view on expenditures for health systems while maintaining proportional costs for each Member State.
   - An increased predictability of sale volumes and accelerated market entry

   The recent Belgian Presidency initiative could be the occasion for Member States and other stakeholders to collaborate to achieve such an objective.

3. *Some price containment measures are put in place in some Member States: how to handle the specific case of Orphan Drugs?*
   In the context of the global economic crisis, countries like Germany or Spain have undertaken price containment measures. In most cases orphan drugs were not concerned by these measures and thus, there was no impact on patients' access.
   The group is asked to discuss if such an approach can be adopted by all Member States or if more adapted containment measures could be developed for Orphan Drugs.

4. *What best practices should be implemented at national level to ensure that stakeholders have a real interaction and participation in the different aspects related to patients' access to orphan drugs?*
   Nowadays, patients are involved in most of the regulatory processes taking a drug to the market authorisation, but only in very few cases are they requested to express their views and opinions on how they value the potential benefit of a new medicine and on what they consider the place of the new medicine to be in the context of their global treatment.

   Few HTA bodies, or similar institutions, have already started taking into account the opinion of the future « users » of a new treatment. A dedicated HTA group is specifically working on

patients'/citizens involvement in HTA activities. Based on these pioneering experiences, the group is expected to discuss how to concretely involve patients in the post-marketing phases at the national level.

The reports from these four working groups will feed the final discussion that will conclude this ERTC meeting.