11th Workshop
Eurordis Round Table of Companies

"Improving Access to Orphan Drugs for all Patients Affected by Rare Diseases in Europe: EU Assessment of Clinical Added-Value of Orphan Drugs (CAVOD)"

December 11th, 2009
Paris, France

Les Salons de l'Aéro-Club de France

Concept Paper

It has been acknowledged over recent years that, while the EU Orphan Drugs Regulation 141/2000 has stimulated research and development of orphan medicinal products in the EU, **equitable and timely access to approved Orphan Drugs for rare diseases patients remains an issue.** As underlined by the final conclusions and recommendations on Pricing & Reimbursement of the EU High Level Pharmaceutical Forum, "Effective market access and utilisation vary strongly between and within Member States".

To address this issue, several policy documents[1] have recently called for an **increased cooperation between EU level authorities and Member States in order to improve access to Orphan Drugs** for people living with rare diseases.

[1] 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.

As acknowledged in the Pharmaceutical Forum conclusions, "…the know-how to make the value assessment of Orphan Drugs is fragmented over national procedures within the Member States and their regions…The disconnection of national and regional processes from the knowledge and experience gathered upfront in the centralised EU processes does add to this fragmentation". This situation generates detrimental delays in the national decision-making process aimed at making Orphan Drugs available to patients on national markets.

Faced by this major challenge, interested parties - patients and industry, as well as EU and national decision-makers - have identified **the creation at the EMEA of a Working Party for the Assessment of the Clinical Added Value of Orphan Drugs (CAVOD)** as being a key instrument for an increased collaboration between Member States and EU-level authorities.
This centralised assessment at the time of the marketing authorisation is expected to minimise delays in patients' access to Orphan Drugs, while fully respecting national competences for pricing & reimbursement decisions within their respective healthcare and economic environment.

With its **"Proposal for the Practical Implementation of Policy Principles to Improve Access to Orphan Drugs in the EU"**, EURORDIS has set a list of recommendations for the establishment of an EMEA Working Party for the scientific assessment of the clinical added value of orphan drugs. This document includes practical suggestions regarding:

a) The ideal composition of the new Working Party, its specific role and responsibilities.
b) The procedure and timeline
c) The content of the non-binding scientific assessment report that the CAVOD Working Party will release on the relative effectiveness of the newly authorised orphan drug.
d) The content of the Annex to coordinate post-marketing studies expected by national authorities and relative effectiveness studies

Eurordis recently started the implementation phase of the proposal by presenting it to the different stakeholders involved: the European Commission, the EMEA, the pharmaceutical industry, the patient groups as well as key projects such as EuroPlan and national conferences.

In this context, the 11th ERTC Workshop will be an opportunity to invite all interested parties to discuss how to translate such proposal into a concrete process.
The morning session will be dedicated to the presentation of the issues that prompted EURORDIS to advocate for a EU coordinated action to reduce delays in access to orphan drugs for rare disease patients, while during the afternoon session, attendees will be invited to discuss practical aspects of the future expected evaluation process such as:

- What information and data the common assessment report should contain?
- What kind of recommendations should it give in terms of “post-marketing life” of the product?
- How to involve the national institutions that are supposed to take advantage of this report?
- What is the role of the sponsor in this process?
- How to take into account the divergent opinions of the members of the working party but also of those interested parties not officially member of the WP?
- What data should be collected for the revision of the report and in which time frame?
- What indicators should be used to evaluate the efficacy of the CAVOD centralised evaluation?

The success of this newly proposed collaboration at the EU level will depend on carefully, precisely and realistically defining the role, mandate and composition of the Working Party. The link between the Working Party and the EU Member States needs to be stated clearly and ensured to be implemented, thanks to national plans on rare diseases and to the Commission’s political support.

Overall, it has to be ensured that any newly created process will not interfere with the normal regulatory approval processes as this might create additional delays in access to orphan drugs for patients, instead of facilitating such access.

This initiative comes at a time when the European Union is actively developing its policy framework on relative effectiveness, while the USA is doing the same on comparative effectiveness.

By involving all stakeholders in the reflection on the practical implementation of the centralised CAVOD assessment, we want to ensure that all of them will respect their engagements and contribute to the success of this collaborative process for the ultimate benefit of rare disease patients.