Recommendations of the EUCERD to the European Commission and Member States
on
Improving Informed Decisions Based on the Clinical Added Value
of Orphan Medicinal Products (CAVOMP) Information Flow

Executive Summary

It has been acknowledged over recent years that, while the EU Regulation on Orphan Medicinal Products EC 141/2000 has stimulated research and development of orphan medicinal products in the EU, equitable and timely access to approved orphan medicinal products for rare diseases patients remains an issue. Large disparities in access exist between and even within the European Member State countries.

To address this issue, several policy documents have called for an increased cooperation between EU-level authorities and Member States in order to improve access to Orphan Medicinal Products for people living with rare diseases:

- The EU Regulation on Orphan Medicinal Products (16 December 1999)
- Final Conclusions and Recommendations of the EU High Level Pharmaceutical Forum[1]
- The Commission Communication on "Rare Diseases: Europe's Challenges" (11 November 2008)
- The Council Recommendation on a European Action in the Field of Rare Diseases (8 June 2009).

The EUCERD was asked to make recommendations to the European Commission on potential ways to facilitate scientific information exchange on orphan medicinal products, in order to support the Member States in their processes of making informed decisions on the scientific assessment of the clinical effectiveness of an orphan medicinal product.

This EUCERD recommendation highlights the fact that the lifecycle of an orphan medicinal product is a continuum of evidence generation, which is needed by assessors and decision makers, as well as being necessary to improve the good use of medicines and thereby optimising the use of limited resources.

The EUCERD recommendation encourages the creation of an Information Flow between individual Member States and between Member States and the EU bodies, which would bridge existing knowledge gaps, especially at the time of marketing authorisation. This information flow and sharing fits into existing regulatory, clinical, Health Technology Assessment (HTA), pricing and reimbursement processes, while avoiding additional burdens.

[1] http://ec.europa.eu/enterprise/sectors/healthcare/files/docs/pharmaforum_final_conclusions_en.pdf

The CAVOMP information flow recommended by the EUCERD includes the four following time points:
Timepoint 1: Early dialogue
Timepoint 2: Compilation Report and evidence definition / Evidence Generation Plan (EGP)
Timepoint 3: Follow-up of the EGP
Timepoint 4: Assessment of relative effectiveness

The recommendation capitalises on existing mechanisms, procedures and regulatory frameworks, and does not bring new obstacles to the assessment process: each element within the Information Flow would be maintained by the corresponding institution responsible for that activity during each particular point in time.

The EUCERD Recommendation on *Improving Informed Decisions Based on the Clinical Added Value of Orphan Medicinal Products (CAVOMP) Information Flow* will ultimately accelerate access to approved orphan medicinal products, by providing the most robust set of information possible, while encouraging pricing and reimbursement decisions based on the value of the OMP and promoting good medical practices throughout the EU.

The Recommendation has been submitted to the European Commission.

Consult the text of the recommendation