

**Process on Corporate Social Responsibility
in the Field of Pharmaceuticals
Platform on Access to Medicines in Europe
Working Group on Mechanism of Coordinated Access to Orphan Medicinal
Products (MoCA-OMP)**

KEY CONCLUSIONS AND RECOMMENDATIONS¹

In September 2010 the European Commission launched the Process on Corporate Responsibility in the Field of Pharmaceuticals² focusing on, amongst others areas, non-regulatory conditions for a better access to medicines following their marketing authorisation.

Under its Platform "Access to Medicines in Europe", EU Member States, countries of the European Economic Area and relevant stakeholders were invited to participate in a project group to develop the concept of a coordinated access to orphan medicinal products based on the set up of programmes between companies and groups of competent authorities, and on a mechanism for the assessment of clinical added value of orphan medicinal products. The results of the project were intended to be a potential mechanism for approaching this on a collaborative, voluntary basis. The initial idea was to set up a pilot project in a second stage.

Following this call – which was stimulated by the initiative of the Belgian EU Presidency in 2010 “Unmet medical need and solidarity in Europe: a mechanism for coordinated access to orphan medicinal products (OMP)” – a number of Member States, experts, patient organisations, industry representatives and other relevant stakeholders volunteered to participate in the so-called “MoCA” (Mechanism of Coordinated Access to Orphan Medicinal Products) Working Group.

The purpose of the MoCA Working Group was to develop proposals as to how to create a future voluntary European collaboration, as well as a pilot project on voluntary basis, to improve access to orphan medicinal product in Europe.

This paper represents the collaborative outcomes from discussions of the MoCA working group that was formed by volunteers from Austria, Belgium, Estonia, Finland, France, Hungary, Italy, Malta, Netherlands, Portugal, Spain, European Patient Forum (EPF represented by the European Organisation for Rare Diseases, EURORDIS), Standing Committee of European Doctors (CPME), European Social Insurance Platform (ESIP), Association Internationale de la Mutualité (AIM), European Federation of Pharmaceutical Industries and Associations (EFPIA), European Association for Bio-industries (EuropaBio), and European Confederation of Pharmaceutical Entrepreneurs (EUCOPE), and the European Commission, with support from the EC-funded project Eminent.

This paper reflects the conclusions and recommendations on which consensus has been found within the group.

Pricing and reimbursement authorities often lack sufficient, robust and trusted information on which to base their evaluation of the inclusion of an orphan medicinal product in their national healthcare systems, which

¹The present document is without prejudice to any existing or future EU/ national and international legislation.

²<http://europa.eu/rapid/pressReleasesAction.do?reference=MEMO/10/442&format=HTML&aged=1&language=en&guiLanguage=en>

would provide the effective access for patients. Collaboration between all stakeholders along the development chain could facilitate effective gathering of information about the real-life outcomes in a clinical setting.

Enhanced collaboration and dialogue could also benefit industry by increasing predictability (at different stages in the product development).

- The MoCA group concluded that enhancing collaboration and coordination could lead to an added value in the process of improving access to OMPs for patients.
- Cooperation should be possible within the current legislative framework. Since the provision of healthcare is the responsibility of the individual Member States, no changes to the national law are proposed.
- Decisions on pricing and reimbursement are the exclusive competence of the Member States. The participation in and commitment to any future actions concerning the mechanism of coordinated access on a European level are expressed on a strictly voluntary basis by all stakeholders, including Member States and the Marketing Authorisation Holder (MAH). Decisions taken are non-prejudicial for Member States or other processes and are non-binding up to and until a formal agreement is signed by all parties interested. The “opt-out”-option exists during preliminary negotiations and all prior processes.
- Existing expert groups such as the EMA (COMP) and EUCERD, networks such as EUNetHTA and on-going and proposed initiatives for creation of knowledge and exchange of information such as CAVOMP-IF, Orphanet, ERNs, as well as on-going developments on Database and Registries Platforms, should be taken into consideration.
- Enhancing mutual understanding and trust at the EU level between all the stakeholders when exchanging relevant information to support well-informed decision-making at national level, where the decisions remain, is crucial.
- An important aspect for access (in addition to others, such as the organisation of diagnosis and care, as well as affordability and budget impact) is defining/identifying the (added) value of a new orphan medicinal product. The group is aware that this value may change over time, depending on evidence generated (i.e., value determination is a dynamic process). Coordination and/or collaboration in that perspective – e.g., using a Transparent Value Framework, or in follow-up studies – is expected to have added value for individual Member States and stakeholders.
- The Transparent Value Framework (TVF) should help to coordinate access pathways for orphan medicinal products in EU Member States by providing a simple and consistent terminology and methodology.
- It is important that the processes and elements proposed in the MoCA project take account of and are linked with existing, on-going projects. In particular, there should be a coordinated approach for post-marketing authorisation research activities and further evidence generation. In the proposed MoCA elements, the proposals for evidence generation, such as those exemplified by the CAVOMP and for early dialogue, to be continued throughout the development of an orphan medicinal product, will be a vital element of ensuring that projects in the field of orphan medicinal products are consolidated and “joined up”. The Centres of Expertise, according to the Recommendations adopted by the EUCERD³, which will be gathered into European Reference Networks (ERNs) as laid down in the EU Cross-Border Healthcare Directive, will play a vital role.⁴
- When it comes to individual access the MoCA group concluded that, based on the current existing legislative and political environment, MoCA should complement the following activities:
 - The strategy on Centres of Expertise and European Reference Networks for Rare Diseases;
 - The establishment of patient data bases and registries; and
 - The elaboration of standards of diagnosis and care.

³ http://www.eucerd.eu/?post_type=document&p=1224

⁴ http://www.eucerd.eu/?page_id=163#CEERN

- The implementation of National Plans for Rare Diseases, as promoted by EUROPLAN.
- It is only through the enactment of a comprehensive strategy that the voluntary collaborative efforts of the MoCA will deliver equitable access and benefit to patients in a real-life setting in an affordable and sustainable way.
- The Working Group agreed on the necessity of a continued voluntary collaboration between stakeholders focusing on:
 - The continuation of the discussions on the items identified in this report
 - The checking on a regular basis for which activities initiatives have been taken or are envisaged regarding the identified pathways
 - The organisation of pilot projects.MEDEV (Medicines Evaluation Group, the informal group of experts from competent authorities on reimbursement hosted by ESIP) has offered to take the project forward and work toward a pilot.

To that purpose, activities could be managed by an ad hoc taskforce. The taskforce would be composed of all volunteering stakeholders (industry, patients, payers, regulators, healthcare providers, healthcare authorities, Member States...) and its main activities would be the abovementioned.