EURORDIS
Rare Diseases Europe

Early Dialogue between Payers and Developers of Medicinal Products: Patients' Perspective

Yann Le Cam, CEO

European Commission's Expert Group on Rare Diseases
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MAPPs and Early Dialogue with Payers: An Urgent Need for Patients

Some key principles from our point of view:

- **Patients are demanding faster access to new medicines** ⇨ The MAPPs concept reflects the need for a more flexible, adaptive approach to the medicines development pathway.
- In today's system, either for MA or reimbursement, a "yes/no" decision often happens after **as much as 10 years** of research and studies.
- For us, MAPPs must open new pathways for medicines to reach patients at **a much earlier stage than today** – typically with an early authorisation for a well-defined and targeted population, coupled with adaptive clinical trial design, patient-centric benefit/risk assessments and **continuous re-evaluation** as new evidence becomes available **throughout the entire life cycle of a medicine.**

Early Dialogue with Payers: Not a new idea…

- Back in 2008 already, the notion of early dialogue was at the core of the recommendations of the **High Level Pharmaceutical Forum**:

*« National authorities and companies should also consider ways of having **early dialogue during product development to improve the generation of appropriate data as far as possible**. » (Recommendation #6)*

« 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 1) **early dialogue on research and development**, 2) exchange of knowledge on the scientific assessment of the clinical added value, 3) specific pricing & reimbursement mechanisms and 4) increased awareness on orphan diseases. » (Recommendation #7)*

Membership

The Pharmaceutical Forum was composed of the European Commission, the 27 Member States, three representatives from the European Parliament nominated in their personal capacities, EFTA representatives and key stakeholders from the public and private sectors:

- European Patients Forum - EPF
- Standing Committee of European Doctors - CPME
- Pharmaceutical Group of the European Union - PGEU
- Association Internationale de la Mutualité - AIM
- European Social Insurance Platform - ESIP
- European Federation of Pharmaceutical Industries & Associations - EFPIA
- European Generic medicines Association - EGA
- European Self-Medication Industry - AESGP
- European Association for Bioindustries - EuropaBio
- European Association of Full-Line Wholesalers - GIRP

A number of other stakeholders were also invited as observers for certain specific discussions.

The secretariat of the Pharmaceutical Forum was provided by the European Commission's services in Directorates General Enterprise & Industry and Health & Consumers.

Early Dialogue with Payers: Not a new idea…

- Idea taken up again and further explored in the **« Process on Corporate Social Responsibility in the Field of Pharmaceuticals »** (2010-2013)

IP/10/1170

Brussels, 24 September 2010

An innovative pharmaceutical industry which meets the needs of society

On the occasion of the Belgian Presidency's ministerial conference on innovation and solidarity in the field of pharmaceuticals held today, Commission Vice-President Antonio Tajani announced the launch of a process on corporate responsibility in the pharmaceutical industry. A distinction is made between three platforms – (1) ethics and transparency, (2) access to medicines in Africa and (3) access to medicines will examine the major challenges of access Africa in the light of the issu opportunities for innovative European level, issues of ethi addressed.

Antonio Tajani, Vice-President entrepreneurship, said "*I attach gr the field of pharmaceuticals it is all to be in line with the general intere a commercial market. I think that European level in this sector so tha the needs of society.*"

In the light of the contribution that thi important to ensure that strategies a stakeholders are prepared to take re exchange between the national author civil society stakeholders.

The pharmaceutical industry makes a **employment in Europe**. The Europea in this field, employing more than 600 value of some € 190 billion.

The process will be divided into th transparency, (2) access to medicines Europe.

1. The objective of the **platform on ethic** information and establish a common de
2. The goal of the **platform on access to** contribution made by European co challenges with which they are faced. will not duplicate the work already departments or international organisation

Belgian Presidency EU Council 2010

Process on corporate responsibility in the field of pharmaceuticals – Platform on access to medicines in

Terms of reference

UNMET MEDICAL NEED AND SOLIDARITY IN EUROPE:

A MECHANISM FOR COORDINATED ACCESS TO ORPHAN MEDICINAL PRODUCTS

a call for Collaboration on Innovating Pricing and Reimbursement Policies

INTRODUCTION

Decisions on Pricing and Reimbursement are the exclusive competence of the Member States European Union. Nevertheless, these Member States foster the same undisputed principles of and solidarity, face common challenges when providing indispensable medicines for their and suffer similar burdens when organizing this access.

All of the issues become even more explicit when limited numbers of patients are concern possible answers to meet the Unmet Needs of these patients are scarce and expensive, as is with Rare Diseases and Orphan Drugs.

The European Commission, Member States, patient organizations, the pharmaceutical indus other stakeholders have recognized the importance to join forces. A number of project therefore been initiated by Member States and the Commission to coordinate investm evaluation and assessment of new medicines and in exchange of information and knowledge.

In the framework of the process on corporate responsibility in the field of pharmaceuticals platform on access to medicines in Europe, the Belgian Presidency now invites the Member St take the next step forward. The Be Presidency hereby invites the Members of the platform to on creative ways of collaboration in order to provide a coordinated access to an orphan dr reflection should be conducted within the existing legal framework.

The Belgian Presidency invites hereby the volunteering Members of the platform to colla participate and/or engage in a Pilot Project designed for this purpose, putting study, intentio ideas in a tangible, operational form and in this way materializing the value of equity and so within and between Member States, and in particular looking at how they apply to private and entities.

Participation in the project is on a voluntary basis and should not commit organisations in actions/initiatives outside the project. To conclude the project, and based on the experience members of the project will be invited to propose recommendations for adoption by the S Group. None of the discussions should commit members of the project.

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 RECOMMENDATIONS[1]

In September 2010 the European Commission launched the Process on Corporate Responsibility in the Field of Pharmaceuticals[2] 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 for Coordinated Access to Orphan Medicinal Products) Working Group.

The purpose of the MoCA Working Group was to develop proposals for a European collaboration, as well as a pilot project on voluntary basis, for a product in Europe.

This paper represents the collaborative outcomes from discussions formed by volunteers from Austria, Belgium, Estonia, Finland, France, tugal, Spain, European Patient Forum (EPF represented by the Euro RORDIS), Standing Committee of European Doctors (CPME), European tion Internationale de la Mutualité (AIM), European Federation of (EFPIA), European Association for Bio-Industries (EuropaBio), and Entrepreneurs (EUCOPE), and the European Commission, with supp

This paper reflects the conclusions and recommendations on w group.

Pricing and reimbursement authorities often lack sufficient, rob their evaluation of the inclusion of an orphan medicinal produ

[1]The present document is without prejudice to any existing or future

[2]http://europa.eu/rapid/pressReleasesAction.do?reference=MEMO/ guage=en

Outcome: A set of recommendations for voluntary payer-led proposals for engagement at all stages of the process, cross-border, on a continuum, using existing specific tools & processes for OMPs

3.3. Step 3: Early dialogue

The existing EU regulatory framework for review and approval of OMP foresees many opportunities for early and on-going dialogue between stakeholders on a voluntary and non-binding basis. This starts as early as at the time of orphan designation. This orphan designation can occur at any time in the development of a medicinal product, on the sponsors' request, as early as proof of concept with medical plausibility.

The recommendations for the Clinical Added Value of Orphan Medicinal Products Information Flow (CAVOMP-IF, previously known as CAVOD)[12], adopted by the EU Committee of Experts on Rare Diseases (EUCERD) describes – in Time-Point 1 – the basis on how such early dialogue / interactions could be articulated in the future.

The highest added value would be achieved by having the opportunity for coordinated input from both regulators and HTA agencies at the same time. This "coordinated parallel" scientific advice will allow the sponsor to fine-tune the relevancy of a programme for the clinical development phase.

These early dialogue initiatives are an opportunity to develop needed flexible value assessment approaches for new emerging rare disease treatments that incorporate scientific and technological innovation based upon unmet medical need and patient outcomes. *This value could be enhanced by having such input from different EU Member States' competent authorities in the same forum. Ideally, payers' representatives might also be invited to sit at the same table, to be aware of the information on a research project as early as possible, on an informal basis and where this is possible within national healthcare systems*. It is understood that this might not be possible in all Member States, but as the process is voluntary, it should not impact those countries where such an engagement is indeed possible. This also needs to be considered in the existing legal framework that separates the role of the Centralised Procedure / EMA in assessing quality, safety and efficacy from evaluating "economic and other considerations".[13] Nevertheless, the value of facilitating such early information exchanges will be high, even if it is necessarily on an informal basis.

2010 2011 2012 2013

Early Dialogue with Payers: Not a new idea…

- Another tool delivered by MoCA: the **European Transparent Value Framework**
  - Basis for structured discussion between all stakeholders around the value of an individual OMP – similar language?
  - Taking into account unmet need, degree of net benefit, response rates, degree of certainty, etc
  - Post-Pilot: number of patients, burden of disease
  - Where possible: Rarity – increased complexity at all stages
  - Create shared understanding for starting national pricing & reimbursement discussions

Criterion	Lower Degree	Medium Degree	High Degree
Available Alternatives/ Unmet Need, including non-pharmaceutical treatment options	yes, new medicine does not address unmet need	yes, but major unmet need still remains	no alternatives except best supportive care - new drug addresses major unmet need
(Relative) Effectiveness, Degree of Net Benefit (Clinical Improvement, QoL, etc. vs. side effects, societal impact, etc.) relative to alternatives, including no treatment.	incremental	major	curative
Response Rate (based on best available clinically relevant criteria)	<30%	30-60%	>60%
Degree of Certainty (Documentation)	promising but not well-documented	plausible	unequivocal

MoCA: Where are we today?

- Who?
  A panel of selected EU Member States' authorities, patients (EURORDIS) and industry representatives (EFPIA-EuropaBio TF on OMPs and RDs)

MoCA: Where are we today?

- What?
  MoCA pilots have been implemented since 2014 by MEDEV with:
  - **5** pilots already initiated
  - **4** requests submitted for new pilots

MoCA: Where are we today?

- **A (typical) example of a current pilot:**
  - Early dialogue on **a targeted gene therapy** for **a very small population** (~ 10,000 patients in Europe)
  - **Very complex therapy** (80 days min for all treatment steps + 6 months of active follow-up)
  - **Almost impossible to set up a Europe-wide network to serve all Member States** => treatment will be limited to **a few selected "Centers of Excellence"** across Europe (similarity with ERNs)
  - If all European patients are to have access to treatment, huge implications in terms of:
    - enabling genuine **cross-border patient mobility**,
    - obtaining **administrative pre-authorisations** for treatment,
    - securing **national payers' acceptance of need for, + price of, treatment**
    - etc…

MoCA: … And where should we go tomorrow?

- Key concepts in MoCA now mature and agreed: New series of pilots to be implemented in a more structured and better supported way to generate concrete results and new learnings

- In our **Call to EU National Competent Authorities for Pricing and Reimbursement (May 2015)**, we ask all members of CAPR to support MoCA as the specific early dialogue platform with payers on Orphan Drugs and to support future pilots

For discussion

- MoCA only one part of the whole picture…
- … but a **« proof of concept »** that shows growing appetite for dialogue between developers and payers
- Need to integrate that effort and its outcomes in other ongoing initiatives:
  - In MAPPs – Payers must have a role to play in the development of adaptive pathways
  - In all current debates (STAMP, CAPR…) about access, pricing and sustainability (e.g. European Reference Pricing, differential pricing, etc)
- **How can we better factor these new ideas into national realities?**
- **Are you aware of our proposal to set up a « table for price negotiation »?**