Mechanism of coordinated access to Orphan Medicinal Products

MoCA

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Context

The Issue:

… Delays and disparities in access to OMP's

Decisions on Pricing and Reimbursement

… exclusive competence of Member States

Still Member States :

… foster same principles of equity and solidarity

… face common challenges when providing indispensable medicines

… bare similar burdens when organizing this access

Objectives and Scope

… Provide **real** access to **real** solutions for patients with **real** unmet medical needs

… Identify possible options to create a mechanism of coordinated access to OMPs

… Based on a voluntary, non-legislative, non-regulatory and non-binding collaboration

The Project

Platform on access to medicines in Europe

Austria, Belgium, Estonia, Finland, France, Hungary, Italy, Malta, Netherlands, Portugal, Spain (+ Sweden participated but could not endorse conclusions)

EPF/Eurordis, CPME, ESIP, AIM, EFPIA, EuropaBio, Eucope, EU Commission, Eminet

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Outcome - primary

EU Member States endorsed april 2013

http://ec.europa.eu/enterprise/sectors/healthcare/files/docs/orphans_report_en.pdf

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 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.

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Outcome - primary

- **Voluntary payer-led proposal for engagement** at all stages of the process, cross-border, on a continuum:
  - Horizon-scanning & early dialogue
  - Early Access Programmes
  - Patient selection
  - Pricing
  - Clinical development
  - Therapeutic Scientific Compilation Reports
  - Transparent Value framework
- Using **existing specific tools & processes** for OMPs
- Possible **collective value assessment** + **potential purchasing agreements**
- Basis for **structured discussion between all stakeholders** on the value of an individual OMP – similar language?
- Taking into account:
  - Unmet need: *availability of other treatments*
  - Degree of net benefit: *Response rates: variable, important determinant*
  - Degree of certainty: *compelling evidence available?*
  - Where possible: *Rarity – increased complexity at all stages*
- Post-Pilot: number of patients, burden of disease
- Create **shared understanding** for starting Pricing & Reimbursement discussions in-country

TVF: Transparent Value Framework tool

"..First steps to agree on criteria on which value could be assessed.."

Pharmaceutical Industry: A Strategic Sector for the European Economy
Commission Staff Working Document
june 2014

TVF: Transparent Value Framework tool

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

Recommendations and Conclusions

Continue the debate on feasibility and desirability on issues where no agreement (yet) could be established

- … post marketing and evidence generation plans
- … Discussions with companies on a price/cost framework
- … Negotiated agreement on the price/cost framework in the MoCA report
- … Joint cost and budget impact assessment
- … Joint procurement or comprehensive treatment packages.

Implementation through an **ad hoc taskforce**

- … Coordination of the continued debate
- … Checking on a regular basis new/ ongoing activities and initiatives for the identified pathways
- … Organising pilot projects

MEDEV has taken the project forward and is working on pilots

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The Pilot

Sobi

Pediatric indication

Phase II / III development..

The Pilot - objectives

To streamline the process

To test hypotheses in the different phases

Identify strenghts and weaknesses, gaps and pitfalls

To test the Transparent Value Framework

To confront a Concept with Real Life

The Pilot: common ground for discussion (I)

- Needs and expectations of all participants/stakeholders
- Agreed agenda and calendar for discussions/feed-back
- Potentially identifiable costs: direct / indirect costs related to the healthcare system
- Protocol design: feedback to potentially be taken into account in programme design
- Top-line data: review and gap identification
- First run of Transparent Value Framework (TVF) with top-line data
- Review proposed economic models
- Identify collaborative opportunities with other initiatives (EUnetHTA, EMA early dialogue,..)

The Pilot: common ground for discussion (II)

- Common agreement about key elements in dossier
- Second run of TVF
- Explore points in the regulatory process to align with other review bodies, e.g., COMP, CHMP, PRAC – elements to be included in regulatory follow-up measures
- Explore areas for agreement on other elements, e.g., price-volume, potential conditional scenarios, joint procurement mechanisms,..
- Patient organizations involvement

The Pilot – Key elements for success

Trust – Confidentiality

Open dialogue

All Stakeholders involvement and active engagement

The Pilot - Conclusion

MoCA

Intended to be a *mechanism*

Turns out to be an concrete trans-national, multi-stakeholders *platform*,

providing with unique opportunities to exchange .. explore ..

.. develop .. discuss .. challenge ..broaden.. ideas and concepts on all aspects of real access to OMP (and beyond..)

from MoCA to PoCA

"..new forms of public-private cooperation to facilitate access to medicines.."

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The Pilots – the future

2nd pilot identified

Agenda for collaboration will be presented for next MEDEV meeting

3rd, 4th and 5th possible candidates are considered

The Pilots – the future: a call for action

Facilitate, upscale and broaden the scope of future pilots: promising treatments for high unmet needs, differential pricing,…

Consolidate the program - Governance

- Building Knowledge – Expertise – Experience..
- Take forward the Lessons Learned..

  … in relation to other developments (HTA network, adaptive licensing,..)
  … widening the scope of/for collaborative mechanisms (early dialogue, MEA, assessments, conditional reimbursement, registries, procurement mechanisms,..)
  … speed up and materialize access to innovation, fair pricing,..

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thank you