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

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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)
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)

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.
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

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