MoCA Concept and Pilot Project

Feedback from the process around the first pilot project

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Slides based on the presentation given at the ECRD Berlin | 10 May 2014
Shared objective: healthcare systems committed to be able to treat patient populations in a timely, equitable + sustainable way

• Pricing & reimbursement authorities often lack sufficient, robust and trusted information on which to base their decisions

• Uncertainty increases in fields of high innovation and in limited or small populations, e.g., Orphan Medicinal Products

• Challenges are shared – across borders and between stakeholders

• Solutions can be better explored collaboratively, rather than unilaterally
The 4 “Ws” of MoCA

- **Who?**
  11 + 1 EU Member States; industry; patients + other stakeholders – European Commission convenes

- **When?**
  December 2010 to April 2013
  Pilots start September 2013 onwards...

- **What?**
  - Recommendations
  - Transparent Value Framework draft tool

- **Why?**
Challenges in evaluation – even more apparent in OMPs

CHMP
Regulatory Evaluation

PRAC
Safety & Efficacy Follow-up plans

HTA
Relative Effectiveness Evaluations

Challenges in evaluation – even more apparent in OMPs

- CHMP: Regulatory Evaluation
- PRAC: Safety & Efficacy Follow-up plans
- HTA: Relative Effectiveness Evaluations
EU invitation – Member State / country initiative

INITIATIVE

DIALOGUE

Endorsed by EU Member States – April 2013

OUTCOME

Testing it out
Pilot Projects

• “Bottom-up” approach
• Chaired by Belgium – leadership from countries
• Next steps fully country-led
Result of the dialogue part: Recommendations...

- Voluntary payer-led proposal for engagement at all stages of the process, cross-border, on a continuum:
  - Horizon-scanning & early dialogue
  - Clinical development
  - Early Access Programmes
  - Therapeutic Scientific Compilation Reports
  - Patient selection
  - Transparent Value framework
  - Pricing

- Using existing specific tools & processes for OMPs

- Possible collective value assessment + potential purchasing agreements
Basis for structured discussion between all stakeholders around the value of an individual OMP – similar language?

Taking into account:
- **Unmet need**: availability of other treatments
- **Degree of net benefit**: Impact of new treatment against other treatments
- **Response rates**: variable, important determinant
- **Degree of certainty**: compelling evidence available?

Post-Pilot: number of patients, burden of disease

Where possible: Rarity – increased complexity at all stages

Create shared understanding for starting Pricing & Reimbursement discussions in-country

...and a draft tool: Transparent Value Framework

What could be valued / value-able?
## Transparent Value Framework (TVF)

<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>
</tr>
<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>
No sausages!

- Areas identified – weighting to be agreed in-country
- Basis to explore – acceptable levels of uncertainty – how to manage effectively to bring treatments to patients, e.g., managed entry agreements
From dialogue to action: Pilot Projects – testing it out

- Test fundamental assumptions
- Identify “+” and “-” of different proposals
- Test the different steps
- Streamline the process / elements
- Identify any gaps

Most important:
- Evaluate the real-life ability to deliver on ambitions and aims
Pilot Project initiated September 2013

- July 2013 – kick-off, “go /no-go”, RIZIV-INAMI, Belgium
- MEDEV agrees to host process
- September 2013 – MEDEV meeting, Rome
- October 2013 – Scoping meeting + workplan, Brussels
- 6 countries, 5 “observers” – resources
- Permanent rare disease patient involvement – EURORDIS
- Specialised therapeutic area patient representative groups
- Links prepared to other groups and/or initiatives
- Communications plans – rolling basis / “bulletin” – MEDEV, other stakeholders
10 Potential areas for collaboration

1. Potentially identifiable costs: direct / indirect costs related to the healthcare system
2. Protocol design: feedback to potentially be taken into account in programme design
3. Top-line data: review and gap identification
4. First run of Transparent Value Framework (TVF) with top-line data
5. Review proposed economic models
6. Identify collaborative opportunities with other initiatives
10 Potential areas for collaboration

7. Common agreement about key elements in dossier

8. Second run of TVF

9. 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

AND – in the plan but will need further work + elaboration

10. Explore areas and time-points for exploration and potential agreement on other elements
   - E.g., price-volume agreements, potential conditional scenarios
   - To support step to individual negotiations in-country
An important + highly relevant forum and opportunity

- **Singular opportunity** + forum for shared dialogue with a group of payers from across Europe
- Chance for **open, comprehensive, early dialogue** on development programmes
- Provides **shared insights**:
  - **Payers** on the realities of drug development – cost, risk, commercial considerations
  - **Industry** on payer concerns, constraints, motivations
- Could be a **safe forum** to explore:
  - Data collection, monitoring and follow up agreements
  - Models for early market entry & uptake, including pricing
Learning from experience: some practical considerations

- **Confidentiality:** time needed to secure agreements; case-by-case basis; almost all agencies covered in employment contracts

- **Timelines:** materials to participants in good time – allow review and national positions, robust interactions

- **Updates:** non-MoCA Task Force members and external stakeholders

- **Patient representatives:** briefing, background, bring up to speed – crucial element

- **RESOURCES!** The project is on top of the work already being done by MEDEV members. Company must be prepared to “step up”, drive & coordinate with MEDEV leadership & MoCA Task Force
To continue + what would we do differently next time?

😊 Open, honest + constructive – helped us improve our planning based on input
😊 Feedback in advance of our Clinical Trial design – allowed us to adapt
😊 Helped us understand the requirements of the countries – similarities, differences; we could plan for a coordinated baseline + then tailor for local requirements
❓ Timing – confidentiality + need to make sure that we provide the information in advance; time to review inside agencies for meaningful conversations
❓ Too early to tell – did not yet get to the EUenetHTA + regulatory interfaces
What will we need to work on more in the next round?

Helpful to have better clarity on the role + mandate from the payers’ representatives in the room – mandate and authority varies

How do we secure that the conversations had in the MoCA Task Force go on to truly have traction locally?

What would be the legal and regulatory elements needed if we were to arrive at a price-volume agreement or conditional pricing scenarios?
Bringing forward the traditional interaction timepoints: could it reduce time for patient access?

Testing a hypothesis: Could earlier & sustained dialogue result in timelier & more affordable access?
MEDEV: Pilot project for MoCA
Call to Action

Introduction

“Decisions on Pricing and Reimbursement are the exclusive competence of the Member States of the European Union. Nevertheless, these Member States foster the same undisputed principles of equity and solidarity, face common challenges when providing indispensable medicines for their patients and suffer similar burdens when organizing this access. All of the issues become even more explicit when limited numbers of patients are concerned and possible answers to meet the Unmet Needs of these patients are scarce and expensive, as is the case with Rare Diseases and Orphan Drugs.”

In the framework of the Process on Corporate Responsibility in the Field of Pharmaceuticals, the Belgian EU Presidency in 2010 invited the members of the Platform on Access to Medicines in Europe to reflect on how to promote the meeting of the medical needs of different segments of the population.
Conclusions

1. Timely and sustainable patient access to treatments needs us to work together to align on challenges and to explore solutions

2. All relevant stakeholders must be involved: patients, payers, industry, HTA bodies...

3. Early engagement with a multilateral payer forum at a minimum:
   - Develop awareness and understanding for a programme
   - “Design in” payer-driven elements to the clinical design

4. A positive experience and a solid foundation for building further in the future