Update on MoCA - Mechanism of Coordinated Access to Orphan Medicinal Products

Anna Bucsics MoCA Project Advisor

Disclaimer: This presentation reflects the work of all the stakeholders involved in MoCA. Their creative input is gratefully acknowledged.

However, the opinions expressed here are exclusively mine and cannot be attributed to any organisation participating in MoCA.
What is MoCA?

It is a
- voluntary
- non-legislative,
- non-regulatory and
- non-binding collaboration

among stakeholders who are willing to work together to provide real to access to a real solution for real patients with real unmet medical needs

http://www.eurordis.org/content/moca
History of MoCA

Sept. 2010 The European Commission launched the Process on Corporate Responsibility in the Field of Pharmaceuticals

Dec. 2010 Platform „Access to Medicines in Europe“; under the Belgian EU Presidency, stakeholders and Member States are invited to participate in a Project Group

2011-2013 Working Group develops MoCA
...Identifying and assessing relevant OMPs (“horizon Scanning”)
...Structural Access and the Transparent Value Framework
...Treatment (individual access)

2014-- MoCA initiative by EURORDIS, MEDEV (an informal group of experts from statutory health insurance institutions in Europe, see www.medev-com.eu) and participating companies

2016 MoCA Revised Terms of Reference published

2018 EMA and EUnetHTA as Observers
Why Do We Need MoCA

- OMPs often have high acquisition costs
- Insufficient knowledge about the disease – epidemiology, clinical pathways, lack of natural history data, lack of comparative data, experts with conflicts of interest
- Small sample sizes, single-arm study design
- Limited data on clinical endpoints, PRO/HRQoL
- Limited duration of trials
- Subgroup data not reliable
- Assessing orphans often fails to show cost-effectiveness*
- Uncertainties in cost-effectiveness modelling
- High acquisition costs
- Poor value for money
- Concerns about budget impact & opportunity costs
- Limited Access

How does MoCA work?

MoCA has patient input at every step of the process and at every stage of the pilot.

MoCA TIMELINE

- **FIRST PILOT MEETING**
  - Product development plan
  - Challenges in pricing reimbursement access
- **SECOND PILOT MEETING**
  - Define working plan
  - List of issues
  - Proactive approach with specific countries
- **PILOT COMPLETED**
  - Participation is voluntary, confidential, non-binding!
  - Opt-out possible
  - Common agreement on most issues

Any company with an OMP/rare disease therapy at any stage of Development can contact MoCA

With an orphan designation or not From non-clinical to post-marketing phase

MoCA has patient input at every step of the process and at every stage of the pilot

Chart courtesy of EURORDIS

Pharma Pricing & Market Access 20-Mar-19
Dynamics of a MoCA meeting

- Company overview
- Disease overview
- Patient journey

Mechanism of action
Method of administration – does it have an impact on access?

Timelines of the development programme

Data requirements – endpoints, PROs
Country-specific reimbursement models - feasibility

Chart courtesy of EURORDIS
MoCA Interaction Pre-Launch

MoCA Discussion: Is this product of interest to patients & payers? Can it address an important unmet need?

Drug Discovery ➔ Pre-clinical ➔ Early clinical ➔ Late clinical ➔ Apply for MA

MoCA Discussion on design of pivotal trials – patient & payer input

Scientific Advice & Protocol Assistance

Parallel scientific advice/Early dialogue w. HTA

Establish Registry, if not already established

PRIME: continuous support and guidance

MoCA Discussion of the value proposition (target population)

MoCA Discussion about product delivery, centers, registries and post-approval data collection

Pharma Pricing & Market Access

20-Mar-19
MoCA Interaction Peri-Launch

- **Apply for MA**
- **Regulatory Review**
- **Positive CHMP decision**
- **MA by EC**
- **Patient Access**
  - **Initiate HTA review/Scoping**
  - **HTA (rapid REA)**
  - **HTA Recommendation**
  - **Price Negotiations**
  - **Develop and Negotiate Managed Entry Framework**

- **Forming a „Coalition of the Willing“**
- **Populating the TVF**

Ensure registries in place and delivery defined

Pharma Pricing & Market Access

20-Mar-19
MoCA Interaction Post-Launch

- Patient Access
- Collect and Analyze Data
- MoCA Input for Data Analysis
- Re-evaluate Product
- Renegotiate Reimbursement
- (Modified) "Coalition of the Willing"
- Managed Entry Agreement
- (Modified) Access
What is the Transparent Value Framework?

- The TVF was introduced as one of the deliverables of the MoCA Working Group of the Process on Corporate Social Responsibility of the EC.
- It was developed as a framework for discussions between pharmaceutical companies and other stakeholders, e.g., groups of payers, about the value of new orphan medicinal products.
- The TVF contains only items which were agreed upon by all participating stakeholders.
- Ideally, the agreement about the values to be assigned in the TVF should facilitate subsequent negotiations about the reimbursement and pricing of the discussed product.
The Transparent Value Framework

<table>
<thead>
<tr>
<th>Criterion</th>
<th>Lower Degree</th>
<th>Medium Degree</th>
<th>High Degree</th>
</tr>
</thead>
<tbody>
<tr>
<td>Lack of Alternatives/Unmet Need, including non-pharmaceutical treatment options</td>
<td>yes, there are alternatives: 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) relative to alternatives, including no treatment, societal impact, etc.</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>
General Comments on the TVF

- TVF is a way to discuss value aspects of a product, not a tool for quantification.
- There are already a number of tools for quantifying drug value, like the ICER.
- A distinction should be made between an assessment of value (is this product a valuable addition to our medicine chest or not) and aspects of cost effectiveness and affordability.
MoCA Stats as of March 2019

<table>
<thead>
<tr>
<th>Metric</th>
<th>Value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Number of participating companies/consortia:</td>
<td>16</td>
</tr>
<tr>
<td>Number of Products Discussed:</td>
<td>19</td>
</tr>
<tr>
<td>Number of payer-representing institutions that attended at least 1 meeting:</td>
<td>19</td>
</tr>
</tbody>
</table>

Other Participating institutions: EMA, EUnetHTA, Academia
<table>
<thead>
<tr>
<th>Status at “first contact”</th>
<th>No.</th>
<th>Current Status</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pre-Clinical Stage</td>
<td>3</td>
<td>1 progressed to clinical stage, 1 discontinued, 1 in development</td>
</tr>
<tr>
<td>Phase 1/2</td>
<td>3</td>
<td>Development ongoing</td>
</tr>
<tr>
<td>Phase 2</td>
<td>3</td>
<td>1 approved by EMA, 1 terminated, 1 in development</td>
</tr>
<tr>
<td>Phase 3</td>
<td>5</td>
<td>1 approved by EMA, 1 terminated, 3 in development</td>
</tr>
<tr>
<td>MAA submitted</td>
<td>2</td>
<td>Both approved by EMA</td>
</tr>
<tr>
<td>Already authorised</td>
<td>3</td>
<td>1 additional indication in development</td>
</tr>
</tbody>
</table>
# (Potential) Benefits of MoCA Interaction

<table>
<thead>
<tr>
<th>COMPANIES</th>
<th>PAYERS</th>
<th>PATIENTS</th>
</tr>
</thead>
<tbody>
<tr>
<td>Increased predictability</td>
<td>Better prediction of patient numbers</td>
<td>Quicker and broader availability of the product</td>
</tr>
<tr>
<td>Better understanding of EU payers’ expectations</td>
<td>Better budget impact – predictability</td>
<td>Increased equity across MS</td>
</tr>
<tr>
<td>More effective data gathering</td>
<td>Sharing of expertise with different MS</td>
<td>Better, coordinated follow-up and collection of PROs and real-life experiences</td>
</tr>
<tr>
<td>MoCA is charges no fees.</td>
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</tbody>
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MoCA is charges no fees.
Other Collaborations in Europe
based on WHO study on impact and benefits of cross-border collaboration in European Region,
Infarmed Conference, 29-30 November 2018, by S. Vogler, F. Suleiman

<table>
<thead>
<tr>
<th>Initiative</th>
<th>Horizon Scanning</th>
<th>HTA</th>
<th>Procurement</th>
<th>Joint Negotiations</th>
</tr>
</thead>
<tbody>
<tr>
<td>Baltic Procurement Initiative</td>
<td></td>
<td></td>
<td>+</td>
<td></td>
</tr>
<tr>
<td>BeNeLuxIA</td>
<td>+</td>
<td>+</td>
<td></td>
<td>+</td>
</tr>
<tr>
<td>Nordic Pharmaceutical Forum (IS, NO, SE, DK)</td>
<td>+</td>
<td></td>
<td>+</td>
<td></td>
</tr>
<tr>
<td>FINOSE (Fimea, NoMA, TLV)</td>
<td></td>
<td>+</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Valletta Declaration (IR, PT, IT, SL, HR, RO, EL)</td>
<td>+</td>
<td>+</td>
<td></td>
<td>+</td>
</tr>
<tr>
<td>Visegrad – Fair Pricing (PL, CZ, SK, HU)</td>
<td></td>
<td></td>
<td>+</td>
<td>+</td>
</tr>
</tbody>
</table>
We’re still working on...

- Outreach to SME’s
- Coordinating Initiatives (EUnetHTA, EMA’s PRIME, etc.)
- Recruiting more payers to participate: (resources, governance)
- Involving other decision-makers, not only experts
Thank you for your attention!

Any questions?

https://www.eurordis.org/content/moca
moca.omp@gmail.com