Coordinated access to orphan medicines using a dialogue-based approach between key stakeholders
The MoCA Experience

http://www.eurordis.org/content/moca
Acknowledgements

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

Disclaimer

The opinions expressed here are those of the presenters and cannot be attributed to any organisation participating in MoCA
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Anna Bucsics MoCA Project Advisor (moca.omp@gmail.com)
Wills Hughes-Wilson (ex-officio)
MoCA Explained

The Patients’ Perspective

The Payers’ Perspective

A Company Perspective
History of MoCA

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

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

2011-2013  Working Group develops MoCA

2014--  MoCA implemented 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
Why do we need MoCA?

• Despite being authorized throughout the European Union, access to orphan medicines can be delayed or limited
• Early dialogue among patients, payers and marketing authorisation applicants to anticipate access problems and develop recommendations to solve them
• „Heads-up“ for payers about new, expensive medicines
• Feedback about problematic product development paradigms for companies
• Ensuring post-marketing data collection by including all relevant stakeholders
• Laying the groundwork for joint price negotiations
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 access to a real solution for real patients with real unmet medical needs
How does MoCA work?

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

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 TIMELINE

FIRST PILOT MEETING

ROADMAP

SECOND PILOT MEETING

PILOT COMPLETED

SCOPING

ROADMAP

n+1

n+2

Product development plan
Challenges in pricing reimbursement access

Define working plan
List of issues
Proactive approach with specific countries

Opt-out possible

Participation is voluntary, confidential and non-binding!

Ca. 18 months

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Ca. 18 months
Dynamics of a MoCA meeting

Company overview
Disease overview
Patient journey

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

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

Timelines of the development programme

Preclinical  Phase I  Phase II  Phase III

Slide Courtesy of EURORDIS

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By participating in MoCA, companies can integrate **additional input from patients’ and payers’ perspectives** at any stage of product development.
MoCA input **can facilitate decision-making** at the time of marketing authorisation by enabling **safe harbor discussions on managed entry agreements**.
POST- APPROVAL

ACCESS

Collect and analyze real world data registries, CEDs
Re-evaluate product
Re-negotiate reimbursement

Managed Entry Agreement

MoCA input for data analysis – PM data collection
MoCA input renegotiation

“Coalition of the willing” redefined
### The Transparent Value Framework

New orphan medicinal products could be assessed according to how well they fulfilled the different criteria at a given point in time. This could be compared with other therapeutic alternatives and be included as one factor in pricing negotiations in Member States.

<table>
<thead>
<tr>
<th>Criterion</th>
<th>Lower Degree</th>
<th>Medium Degree</th>
<th>High Degree</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Lack of Alternatives/Unmet Need, including non-pharmaceutical treatment options</strong></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) 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>
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</table>
## Participants

<table>
<thead>
<tr>
<th>Description</th>
<th>Count</th>
</tr>
</thead>
<tbody>
<tr>
<td>Number of companies/consortia participating from 2014 – 2018</td>
<td>14</td>
</tr>
<tr>
<td>No of payer-representing institutions (attended at least 1 meeting, estimate based on 2014 and 2016 and 2017 records)</td>
<td>18</td>
</tr>
<tr>
<td>Number of patients attending</td>
<td>9</td>
</tr>
<tr>
<td>Other institutions (EMA, EUnetHTA, Academia)</td>
<td>3</td>
</tr>
</tbody>
</table>
## Meetings 2014-2018

<table>
<thead>
<tr>
<th>Description</th>
<th>Count</th>
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</thead>
<tbody>
<tr>
<td>Number of projects</td>
<td>16</td>
</tr>
<tr>
<td>Number of meetings with companies</td>
<td>34</td>
</tr>
<tr>
<td>Number of meetings with patients attending</td>
<td>15</td>
</tr>
</tbody>
</table>
## Status of product at first meeting

<table>
<thead>
<tr>
<th>Status</th>
<th>Count</th>
</tr>
</thead>
<tbody>
<tr>
<td>Authorised</td>
<td>3</td>
</tr>
<tr>
<td>MA submitted</td>
<td>2</td>
</tr>
<tr>
<td>Phase 1/2</td>
<td>4</td>
</tr>
<tr>
<td>Post phase 2/Phase 3</td>
<td>4</td>
</tr>
<tr>
<td>Pre-clinical</td>
<td>3</td>
</tr>
<tr>
<td>Indication/ATC</td>
<td>Count</td>
</tr>
<tr>
<td>------------------------</td>
<td>-------</td>
</tr>
<tr>
<td>A (alimentary &amp; metabolic)</td>
<td>4</td>
</tr>
<tr>
<td>B (Blood, hematology)</td>
<td>5</td>
</tr>
<tr>
<td>C (Cardiovascular)</td>
<td>2</td>
</tr>
<tr>
<td>N (neurological)</td>
<td>2</td>
</tr>
<tr>
<td>R (Respiratory)</td>
<td>2</td>
</tr>
<tr>
<td>S (Sensory)</td>
<td>1</td>
</tr>
</tbody>
</table>
### Types of Products Discussed

<table>
<thead>
<tr>
<th>Type</th>
<th>Count</th>
</tr>
</thead>
<tbody>
<tr>
<td>Small Molecules</td>
<td>7</td>
</tr>
<tr>
<td>Biologicals</td>
<td>3</td>
</tr>
<tr>
<td>Advanced Therapies</td>
<td>5</td>
</tr>
<tr>
<td>Other</td>
<td>1</td>
</tr>
</tbody>
</table>
Survey on Improving MoCA (2017)

Response
- Patient (participant)
- Payers
- Participating companies
- Non-participating companies

Knowledge of MoCA
- Quite familiar with MoCA
- Have read or heard about MoCA

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How is MoCA of use to Patients?
(multiple answers)

- Providing input about...
- Early dialogue helps...
- Improving study design...
- Mutual understanding...
- Other (please specify)
How is (or would be) MoCA most helpful for Payers?

(multiple answers)

- Advance Information...
- Opportunity to identify and...
- Opportunity to discuss...
- Provide input into product...
- Opportunity to give advance...
- Opportunity to coordinate...
- Other (please specify or...)

EuropaBio Patients Bio-Forum, Brussels, 25 May 2018
How is MoCA of use to companies?
(multiple answers)
Major Limitations

• For Patients: Limit in negotiating mandate

• For Payers and Companies:
  • Limited opportunities for concrete decision-making
  • Overlap with other initiatives (EMA’s parallel scientific advice, EUnetHTA, etc.) – payers would like better coordination
The Patients’ Perspective
Why MoCA is important for patients

• **Patients are able to sit at a common table with industry and payers**

• We are considered an *equal partner* in the discussion around the access of important new therapies for many group of patients.

• We provide input about **what is important to patients** to companies and payers

• Early dialogue helps **anticipate** and **solve** access problems

• MoCA can help reducing **uncertainties also in post approval phase**, particularly in case of MA with conditional approval
What is the patients’ role?

• In our role, we try to provide the right patient at the right time to give the right contribution.
• We provide also training and educational tools to enlarge the patient pool available
• Sometimes a challenge given the rarity of the conditions and the potential conflicts of interest that might prevent participation
What do we want to see moving forward

• **Early dialogue** between payers and product developers such as MoCA **should be encouraged** for a much greater number of rare disease therapies under development.

• **Such dialogue can go a long way to enable a comprehensive discussion of all aspects of patient access**, including but not limited to economic considerations

• **Scaling up the experiments** that have taken place to date requires political encouragement and financial support to lead to a truly European collaborative effort.
The Payers’ Perspective
Learnings from MoCA

• Better understanding of which outcomes matter to patients and payers
• Better understanding of payers’ needs for decision-making
• Companies are welcome anytime during the product cycle – but the earlier the better
• Understanding the challenges of complicated products, eg when a disease is so rare and the treatment so complicated that it will be limited to a few selected “Centers of Expertise” across Europe (role of ERNs)
More discussion on Prices & Pricing Needed

- Value based – what the market will bear?
- Fair return on investment - including public funds (basic research, RWD collection, etc.)
- Fair reimbursement
  - For each component of the technology
  - Compared to alternatives
  - Savings for the system should be real, not “skimmed-off“ via high prices
- Sustainable
- Equitable
- Considers uncertainty – adaptive pricing?
- ...Transparent?
Are all orphan medicines priced fairly?

- Fair reward for innovation

  BUT

- Repurposed medicines: When compared with their unbranded or unlicensed versions, the branded orphan medicines were 1.4 to 82000 times more expensive (1)

- Prevalence is inversely associated with price only for „ultra-orphans“(1)

- Expanding indications or loss of orphan status may or may not lead to lower prices

- According to EvaluatePharma, Phase 3 orphan drug development costs half that of non-orphan(2)


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Access is more than pricing...

• Who will pay?
  • Hospital budgets? Health insurance? Special funds?
  • International cooperation/Cross-border issues?

• For what? Cells, intervention, follow-up?

• Who will get the money? Company, centers?

• How much?

• When?
  • Up front?
  • Over time, based on performance?

• What will be the role of the European Centers of Expertise?
Experience from a company perspective

• Individual company / product conversations are confidential
  • Payers not “bound” by points of view explored during meetings
  • Companies can explore options in a “safe space”

Personal perspective + reflections from experience of the MoCA
Wills Hughes-Wilson

willshugheswilson@gmail.com
Development The “traditional” way = sequential over time

- Regulatory
- Health Technology Assessment (HTA)
- Payer / Budget-Holder

EVIDENCE IS REQUIRED TO MAKE A RISK-BENEFIT ANALYSIS:
- Quality
- Safety
- Efficacy

EVIDENCE IS REQUIRED TO EVALUATE VALUE TO HEALTHCARE SYSTEM:
- Cost-Benefit
- Comparative efficacy
- Real-world effectiveness

EVIDENCE IS REQUIRED TO DECIDE ON INCLUSION IN HEALTHCARE SYSTEMS:
- Pricing
- Reimbursement
Maximising interfaces between decision-makers & their evidence needs

Continuum of Evidence

- Risk-Benefit – Comparative Efficacy / Effectiveness – Pricing & Reimbursement

Continued cross-border, trans-Atlantic + Global collaboration between authorities

- Regulatory
- Health Technology Assessment (HTA)
- Payer / Budget-Holder

PROTOCOL ASSISTANCE
PARALLEL SCIENTIFIC ADVICE
(MULTI-)HTA ADVICE
- Individual
- SEED
- EUnetHTA
MoCA
(PILOT) MULTI-COUNTRY DIALOGUES
- BeNeLux + AT (+…?)
- Valetta Group
- Future: Visegrad, Nordics…?

PRIME (Priority Medicines for Europe) Road-Map

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A “buffet” for drug development . .  
You do the choosing
Questions & “issues” vary per programme...

1. Example 1 – very early phase programme – pre-clinical
   - How do we view a costly-to-produce biologic where there is no other treatment?
   - What will be measured / what should end-points be?
   - How will they be measured / what tools + what will be acceptable
   - How demonstrate / validate

2. Example 2 – Phase III was underway
   - Nature of treatment – 4 weeks for a lifetime of prevention – how evaluate? How quantify?
   - What existing costs will you include as comparator?
   - What endpoints / clinical measures will be relevant?
   - Which tools do you use?
...and the issues we are seeking to solve will vary

3. Example 3 – Phase III was underway
   • Does regulatory approval alone actually serve the patients’ needs?
   • Nature of disease + rate of progression => tailored + adapted (regulatory) study design
   • Very large number of endpoints with requirement to show positive trend on some or all – unclear
   • Guidance on how to interpret trends in a way that could be meaningfully acceptable to payers + practical suggestions on what to do to accomplish

4. Example 4 – Phase III had completed, data was available
   • Strong phase III additional efficacy outcomes data in a “well-served” field
   • What is your biggest concern + how can we address?
Current reflections + future considerations

- MoCA dialogue has allowed “early warning” / issues identification – ideally at a time when the challenges can be addressed
- BUT we can observe that value has been added, no matter what stage the dialogues have happened

Need to build on the experience to date to consider:
- Effective use of time and resources – especially for payers
  - Coming as early as possible vs. statistical likelihood of failures in drug development
- Secure no “two-speed” system
  - Linking MoCA into other – particularly regulatory – approaches, e.g., PRIME, Adaptive Pathways
- How to secure that there is something in it for all stakeholders – create a “virtuous circle”
Learnings – Critical Success Factors

1. Genuine commitment to patient access to therapy as a key objective as well as a sustainable business

2. Support from / involvement of company leadership – mandate for conversations / participation in the process

3. A genuine intention and commitment to the process / genuine openness to hearing the payers’ point of view

4. Internal collaboration across functions

5. Transparency with investors + governance bodies within the company

6. Full participation of the patient community
Thank you very much for your attention!

... Any questions?

Anna Bucsics
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