Multi-Stakeholder Late & Early Dialogue – The MoCa Experience & Potential Contribution

EMA-Payer Community Meeting, London, Sept 19, 2017
Mechanisms of Coordinated Access to Orphan Medicinal Products

- High uncertainty around products for small populations
- Fear of high price and high budget impact
- Fragmented EU market – decisions on Pricing and Reimbursement at National level
- The solution – collaborative approach between different Member States
What is MoCA?

- **MoCA** enables a **comprehensive discussion of all aspects of patient access:**
  - Rare disease: targeted indication, prevalence, standard of care
  - Rare disease therapy
  - Economic aspects (pricing scheme, potential budget impact, managed entry agreements)
  - Diagnosis and healthcare system organisation
  - Registries, real-world evidence collection
  - Research questions to reduce uncertainties on effectiveness
**Mechanism of Coordinated Access to Orphan Medicinal Products**

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

- **SCOPING**
  - FIRST PILOT MEETING
    - Product development plan
    - Challenges in pricing reimbursement access
  - ROADMAP
    - Define working plan
    - List of issues
    - Proactive approach with specific countries
  - n+1
  - n+2

18 months

- **SECOND PILOT MEETING**

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.

Participation is voluntary, confidential and non-binding!
PRE-APPROVAL

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

- Collect and analyze real world data registries, CUPs
- 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
# Benefits of MoCA Interaction

<table>
<thead>
<tr>
<th>COMPANIES</th>
<th>PAYERS</th>
<th>PATIENTS</th>
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<tbody>
<tr>
<td>Increased predictability</td>
<td>Better prediction of patient numbers</td>
<td>Quicker and broader availability of the product</td>
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<tr>
<td>Better understanding of EU payers expectations</td>
<td>Better budget impact – predictability</td>
<td>Increased equity across MS</td>
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<tr>
<td>More effective data gathering</td>
<td>Sharing of expertise with different MS</td>
<td>Better, coordinated f-up and collection of PROs and real-life experiences</td>
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## Participants

<table>
<thead>
<tr>
<th>Category</th>
<th>Count</th>
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<tbody>
<tr>
<td>Number of companies/consortia</td>
<td>11</td>
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<tr>
<td>No of payer-representing institutions (attended at least 1 meeting, estimate based on 2014 and 2016 records)</td>
<td>13</td>
</tr>
<tr>
<td>Other institutions (EMA, EUnetHTA, Academia)</td>
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Types of Products Discussed

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<th>Category</th>
<th>Count</th>
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<tr>
<td>Small Molecules</td>
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<tr>
<td>Biologicals</td>
<td>3</td>
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<tr>
<td>Advanced Therapies</td>
<td>4</td>
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<tr>
<td>Other</td>
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## Status of product at first meeting

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<td>Authorised</td>
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<tr>
<td>MA submitted</td>
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<tr>
<td>Phase 1/2</td>
<td>4</td>
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<tr>
<td>Post phase 2/Phase 3</td>
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<td>Pre-clinical</td>
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Dynamics of a MoCA meeting

Company overview
Disease overview
Patient journey

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

Preclinical  Phase I  Phase II  Phase III

Timelines of the development programme

Data requirements – endpoints, PROs
Country-specific reimbursement models - feasibility
Survey on Improving MoCA

42 Responses:

5 Patient (participant) Responses
12 Payer Responses
9 Responses from companies which participated
16 Responses from companies that had not participated in a MoCA project

64% of responders „quite familiar“ with MoCA
33% „have read or heard about it“

Products most interesting to payers: ultra-orphans, advanced therapies, hospital products
Relevant Responses 1/2

- 40% of respondents think MoCA is most useful in Stage 2-3 of development
  - 17.5% think it’s at Stage 1-2 and 15% think it’s most useful just before and during the marketing authorization process; another 15% think it depends on the product, and 1 respondent thinks anytime during and after Phase 2-3

- More than ¾ see key connections with other EU projects
  - 80% with parallel scientific advice, adaptive licensing
  - 77% with EUnetHTA
  - 46% with PRIME
  - 44% with BeNeLuxA
Relevant Responses 2/2

- 51% of respondents see overlaps or redundancies with other EU projects. Of these,
  - 74% of those who do, see it with EMA's scientific advice, parallel scientific advice, adaptive licensing EMA’s COMP activities
  - 63% with EUnetHTA
  - 37% with BeNeLuxA
  - 21% with EMA’s PRIME

- 75% of payers think better coordination with other efforts, eg those by EMA, EUnetHTA, BeNeLuxA would make MoCA more useful
How to make MoCA more useful?

- MoCA can play a key role in coordinating the input from key stakeholders (EMA, HTA, patients, payers...)
- More resources, more stakeholders (hospitals, EMA, more payers) would make MoCA more effective
- Stronger mandates for payer participants
- Better alignment [with other processes] on key questions of evaluation and assessment
- Provide more guidance on when, where and how to approach MoCA
- ...
Results of Brainstorming Session
Company Perspective

Feedback from „real“ payers to make sure that the right evidence is generated

Companies choose fora to participate in

Balancing local requirements (more detail) vs EU level (may save resources via harmonization)

Market access people may not be consulted by clinicians designing trials

MoCA is a forum for companies to be open about their own uncertainties

During trial design, look at the evidentiary uncertainties remaining

Consider

• patient numbers
• adjudicating outcomes agreements
• where to place treatment centers – cross border healthcare

Interaction of patients with payers very helpful
EMA Perspective

- Values informal part of discussions at MoCA
- Payers are important players in registries, and were invited to EMA's Registries Workshop – will publish key principles for promoting registries
  - Not product registries
  - Liaise with holders of existing registries

Luca Pani, former AIFA / former CHMP / SAWP member EMA

“Start talking with the payers early – very early. When you start thinking about end-points. And I mean payers, not only HTAs. HTAs are not useful without the payers.”
Payer Perspective

- Patients are actually more involved in advice and input than payers!
- Let's keep MoCA's Unique Selling Point:
  - Informal, brainstorming, patients' contributions always constructive
  - Formalization has the peril of confrontation
- Providing input into the development program at a time when it can be acted upon
- Opportunity to think about consequences of pricing at an early stage
- Maybe, hand off the product to another forum at some point in time?
- Will all these efforts make drugs more affordable?
- Talk also about pricing in MoCA
  - Can a company explain how the price was established
  - Can we all work on making drugs more affordable
Patient Perspective

- Patients are also concerned about affordability and budget impact
- Connectivity, not overlap
- Value of MoCa is reducing uncertainty – on
  - Relative effectiveness
  - Cost effectiveness
  - Via registries which are enriched by input necessary for all stakeholders
  - Focus on ultra-orphan and advanced therapies
EUnetHTA Perspective

- EUnetHTA can provide tools for MoCA to work with
  - Parallel consultation with EMA
  - REA
  - Help in developing registries – these need to be no more complicated than necessary
- EUnetHTA would be interested in developing registries
- Present MoCA at EUnetHTA
Registries

- Who owns the data? Who pays for them? Must payers fund registries without access to data?
- Companies have concerns about ensuring company access to data, especially in the context of products from several companies
- However, having a separate registry for each product is inefficient and NOT the way forward
- Alignment on data elements is important
- It’s unethical to deny access if a registry can provide more information about a product, and the alternative would be to make patients get treatment with less information
- If registries are just a mechanism to enhance market access, so payers should not finance them
- They have to be useful optimizing the outcomes of the healthcare system
- Can patient records substitute for registries? Can they be supplemented with data from a sentinel system
Final thoughts:

New medicines need to be affordable. A new medicine has no benefit if a patient cannot get it, because the healthcare system cannot pay for it.