Timelines for MoCA Activity

Color Legend:
Red: MoCA
Orange: Company
Blue: EMA
Green: HTA, Payer
Mechanism of Coordinated Access to Orphan Medicinal Products

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

MoCA TIMELINE

SCOPING
FIRST PILOT MEETING
Discuss challenges in
• Product development
• Pricing reimbursement access

ROADMAP
SECOND PILOT MEETING
Define working plan
List of issues
Proactive approach with participating countries

n+1
n+2
Further meetings to clarify issues and find solutions

PILOT COMPLETED
COMMON AGREEMENT ON MOST ISSUES

MoCa is
• Confidential
• Free of charge
• Voluntary
• Non-Binding (until agreements are reached)
<table>
<thead>
<tr>
<th>REGULATORY TOOLS</th>
<th>OTHER TOOLS</th>
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</thead>
<tbody>
<tr>
<td>TO FACILITATE EARLY ACCESS</td>
<td>TO AVOID FAILURE AT MAA</td>
<td>TO AVOID FAILURE AT PRICING &amp; REIMBURSEMENT</td>
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<tr>
<td>Conditional approval</td>
<td>Scientific advice protocol assistance</td>
<td>MoCA – concrete evaluation</td>
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<tr>
<td>Accelerated assessment</td>
<td>EMA-HTA parallel scientific advice</td>
<td>EUNetHTA – early dialogue</td>
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<td>Compassionate use</td>
<td>PRAC Advice</td>
<td>Regional consortia – BeNeLuxA, etc.</td>
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Companies can integrate the input provided by MoCA from patients‘ and payers‘ perspectives at any stage of product development.
Peri-Approval

Traditional route

- Application for Marketing Authorisation
- Evaluation (CHMP/COMP)
- MA by EC

MoCA input can facilitate decision-making for MAA at the time of marketing authorisation

- Scoping with HTA
- rapid REA by HTA
- Discussions on the TVF & MEAs

...by providing some of the elements of adaptive licensing discussions, such as safe harbor discussions

- Forming a “coalition of the willing”
- Discussions on the TVF & MEAs
- Framework Agreement

- Accelerated Assessment (CHMP/COMP)
- rapid REA by HTA
- Framework Agreement

- P&R negotiations

- Forming a “coalition of the willing”
- Discussions on the TVF & MEAs
- Framework Agreement

- P&R negotiations
POST-APPROVAL

ACCESS

Collect and analyze real world data registries, CUPS

Re-evaluate product

Re-negotiate reimbursement

Managed Entry Agreement

ACCESS

Re-defined

MoCA input for data analysis

MoCA input renegotiation

“Coalition of the willing” redefined

... on managed entry and postmarketing data gathering
MoCA can also complement the adaptive licensing process by providing payer and additional patient input.
Thank you very much for your attention!