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**2nd Multi-Stakeholder Symposium on Improving
Patient Access to Rare Disease Therapies**

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**MoCA's (Mechanism of
Coordinated Access to Orphan
Medicinal Products) state of play**

Disclaimer & Acknowledgements

- The materials developed for this presentation have been done in very close collaboration with Dr. Anna Bucsics, Advisor to MEDEV and the MoCA Project
- The input from Dr. Anna Bucsics is gratefully acknowledged

Shared environment, shared challenges

 Shared solutions?



- **P&R 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**
- **Delays and inequalities in access** to patients

 Challenges are shared – **across borders** and **between stakeholders**

=> **Shared objective**: healthcare systems committed to treat patients in a timely, equitable and sustainable way

 **Solutions** can be better **explored collaboratively**, rather than unilaterally

MoCA – addressing the last cornerstone: The Payers



MoCA 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



* **Patients:** EURORDIS and individual patients or patients' organisations relevant to a specific Orphan Medicine on an ad-hoc basis

* **Payers:** 13 Volunteer National competent authorities for P&R, members of MEDEV (Medicines Evaluation Committee)

* **Pharmaceutical Industry:** candidate MAA / MAH (mostly SME's, including start-ups)

How does MoCA work?



- Expression of interest by a company
- Initial presentation at a MoCA meeting
- **Dialogue** continues in a consensually established way
- Final report containing learnings and recommendations. These may concern:
 - Patient numbers and estimates of use
 - Product delivery/treatment centers
 - Registries
 - Ultimately, a framework for negotiations (eg Transparent Value Framework)
- Confidential and non-binding (unless otherwise agreed)
- „Opt-out“ anytime; currently free of charge for companies

Experience to date



Overall:

No of Participating Companies	10
No of Products Discussed	13
Small Molecules	4
Biologicals	3
Advanced Therapies	4
Other	2

Product status @ 1st dialogue:

Authorised	2
MA submitted	2
Post phase 2/Phase 3	4
Phase 1/2	4
Pre-clinical	1

Learnings from MoCA



For Patients:

- Opportunity to inform other stakeholders of patients' priorities and needs
- Understanding of payers' concerns – and their reasons

For Industry:

- Better understanding of which outcomes matter to patients and payers
- Companies are welcome anytime during the product cycle – but the earlier the better

For Payers:

- „Heads-up“ for earlier planning
- Opportunity to influence trial design so that payers' information needs can be considered

Understanding the challenges of complex technologies, for complex rare diseases, which are complex to treat

Building trust

MoCA = Multi-stakeholder collaboration

In action!!

What can be improved:

- No “single payer voice”: great heterogeneity across and within countries
- Legal issue / political issue / organisational issue: not all payers have the same mandate
- Concerns about independency: early involvement
- Payers to come at early dialogues table with regulators and HTAs: no consensus
- Greater collaboration needed not only among stakeholders, but also among payers (multi-country)
- More trust ↔ more dialogue





Thank you for your attention!

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