Improving access to treatment in rare diseases

Expert Conference on Rare Diseases
Towards a New European Policy Framework:
"Building the future together for rare diseases"

Czech Presidency of the Council of the European Union

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Mechanism of Coordinated
Access to Medicinal
Products

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How to Improve Access to Orphan Medicinal Products

- Increase Affordability
- Improve cost-effectiveness by improving the evidentiary base
- Improve awareness of disease, diagnostics and treatment
- Develop innovative and practicable payment models
- **Cooperation**
  - At the national level: among healthcare providers and payers responsible for in- and outpatient care
  - At the European level,
    - among member states, agencies and networks
  - **At the European level among stakeholders**

Mechanism of Coordinated Access to Orphan Medicinal Products

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

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

➡ Supported by…

Potential and Realized Benefits of a Multi-Stakeholder Approach in Europe

COMPANIES	Regulators	PAYERS/HTA	PATIENTS
Better insight needs and possi-bilities of other stakeholders	**Insight into potential "downstream" problems**	**Better budget impact – predictability**	Quicker and broader availability of the product, Increased equity across Member States
Increased predictability	More efficient scientific advice	Better data for HTA	**Better understanding of needs and expectations**
	(if advice is taken into account)		
More effective data gathering	Facilitation of post-marketing evidence gathering	**Sharing of expertise with different stake-holders and Member States**	Better, coordinated follow-up and collection of PROs and real-life experiences

Topics for Discussions at MoCA

- Epidemiology and population most likely to benefit

- How to measure performance?

  - At the time of marketing authorization?

  - Post-licensing?

- International cooperation/Cross-border issues?

- Is the price justified, fair and affordable?

- Who will pay?

  - Hospital budgets? Health insurance? Special funds?

- When?

- Practicability of payment models

Technical Info on MoCA

- MoCA meetings are online since 2020. They are organized by MEDEV.

- Eurordis invites patient representatives to participate.

- There is no fee for companies.

- EMA and EUnetHTA are observers.

- MoCA stats 2013-2022:

    - 47 meetings

    - 17 companies/consortia

    - 20 Products

    - 20 payer-representing institutions that attended at least 1 meeting

- Contact for payer organiations: www.medev-com.eu

- Contact for companies: moca.omp@gmail.com

- Contact for patients: www.eurordis.org