Introduction to MoCA

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Mechanisms **o**f **C**oordinated **A**ccess to Orphan Medicinal Products

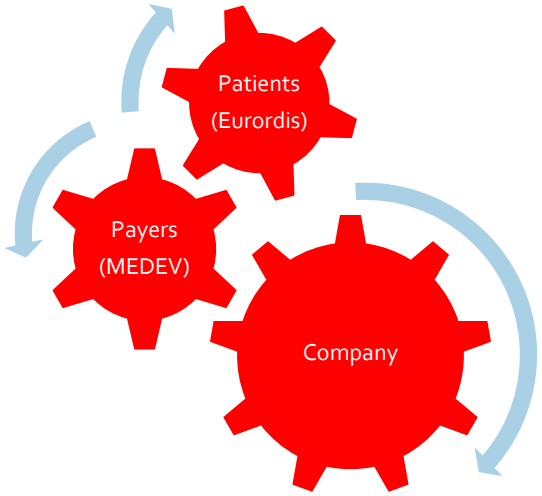
- High uncertainty around products for small populations
- Fear of high price and high budget impact
- Fragmented EU market – decisions on Pricing and Reimbursement at National level
- The solution – ***collaborative approach between different Member States***

Mechanisms **o**f **C**oordinated **A**ccess to Orphan Medicinal Products

- **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

REGULATORY TOOLS		OTHER TOOLS
TO ENHANCE DEVELOPMENT SUCCESS	**TO FACILITATE EARLY ACCESS**	**TO AVOID FAILURE AT PRICING & REIMBURSEMENT**
Scientific advice / protocol assistance	Conditional approval	**MoCA**
EMA-HTA parallel scientific advice	Accelerated assessment	EUNetHTA (methodology)
	Compassionate use	EMA-HTA parallel scientific advice

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

18 months

MoCA TIMELINE

SCOPING

FIRST PILOT MEETING

Product development plan

Challenges in pricing reimbursement access

STOP

GO

ROADMAP

SECOND PILOT MEETING

Define working plan

List of issues

Proactive approach with specific countries

n+1

n+2

PILOT COMPLETED

COMMON AGREEMENT ON MOST ISSUES

Participation is voluntary, confidential and non-binding!

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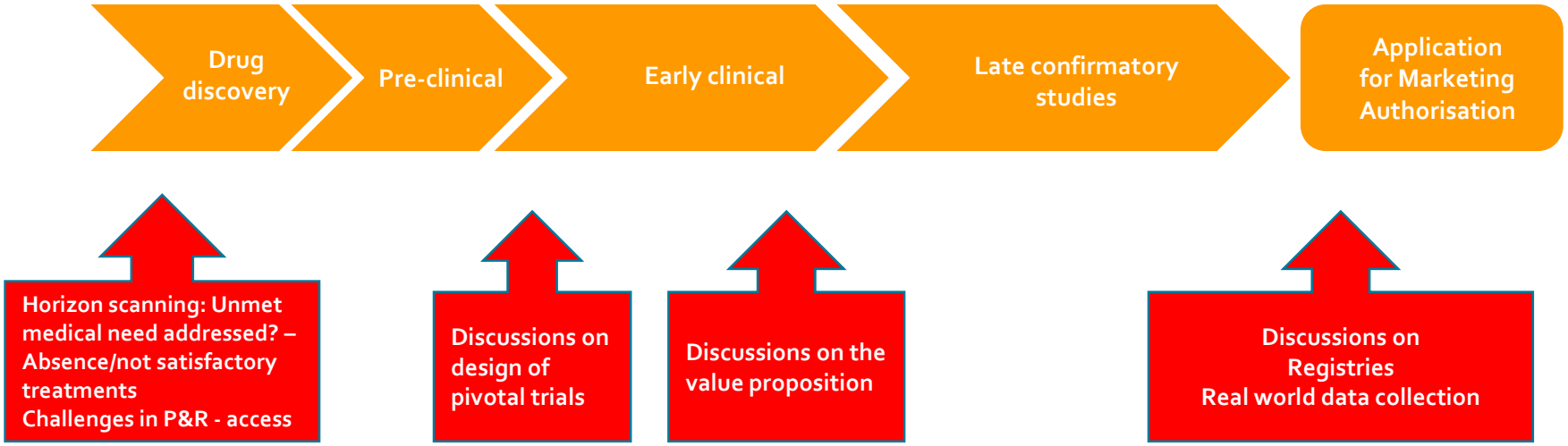
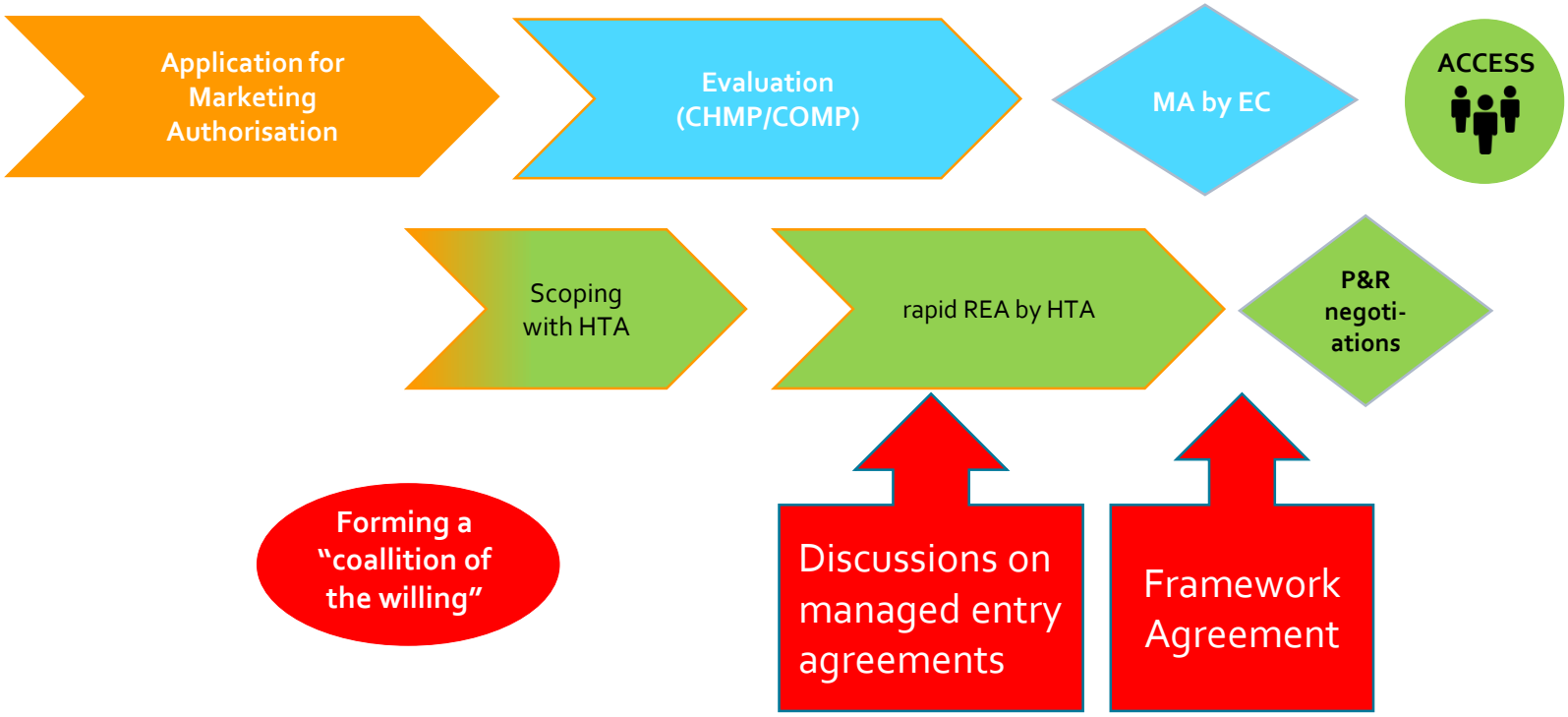
EURORDIS RARE DISEASES EUROPE

PRE- APPROVAL



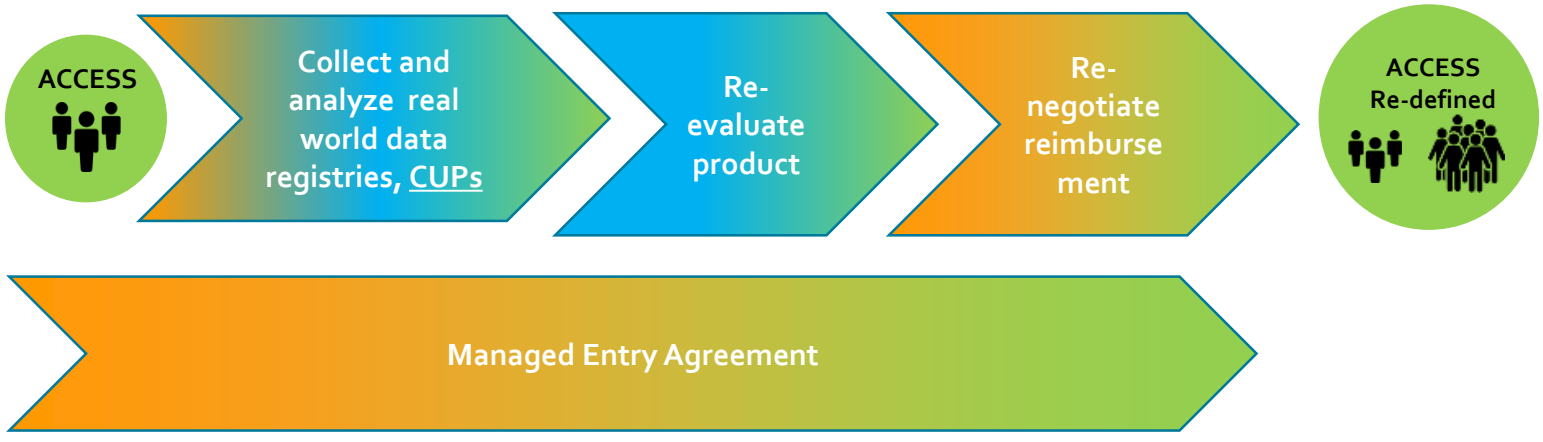
By participating in MoCA, companies can integrate **additional input from patients' and payers'** perspectives at any stage of product development

PERI-APPROVAL



MoCA input **can facilitate decision-making** at the time of marketing authorisation by enabling **safe harbor discussions on managed entry agreements**

POST- APPROVAL



MoCA input for data analysis –PM data collection

MoCA input renegotiation

"Coalition of the willing" redefined

Benefits of MoCA pilots

COMPANIES	PAYERS	PATIENTS
Increased predictability	Better prediction of patient numbers	Quicker and broader availability of the product
Better understanding of EU payers expectations	Better budget impact – **predictability**	**Increased equity across MS**
More effective data gathering	Sharing of expertise with different MS	Better, coordinated f-up and collection of PROs and real-life experiences

Example of a pilot

- Early dialogue on **a targeted gene therapy** for **a very small population** (~ 10,000 patients in Europe)
- **Very complex therapy** (80 days min for all treatment steps + 6 months of active follow-up)
- **Almost impossible to set up a Europe-wide network to serve all Member States** – treatment will be limited to **a few selected centers of excellence** across Europe (ERNs)
- If all European patients are to have access to treatment, huge implications in terms of:
    - enabling genuine **cross-border patient mobility,**
    - obtaining **administrative pre-authorisations** for treatment
    - securing **national payers' acceptance of need for treatment and its price**

Dynamics of a MoCA meeting

Company overview
Disease overview
Patient journey

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

Timelines of the development programme

Data requirements – endpoints, PROs

Country-specific reimbursement models - feasibility

Patient contribution

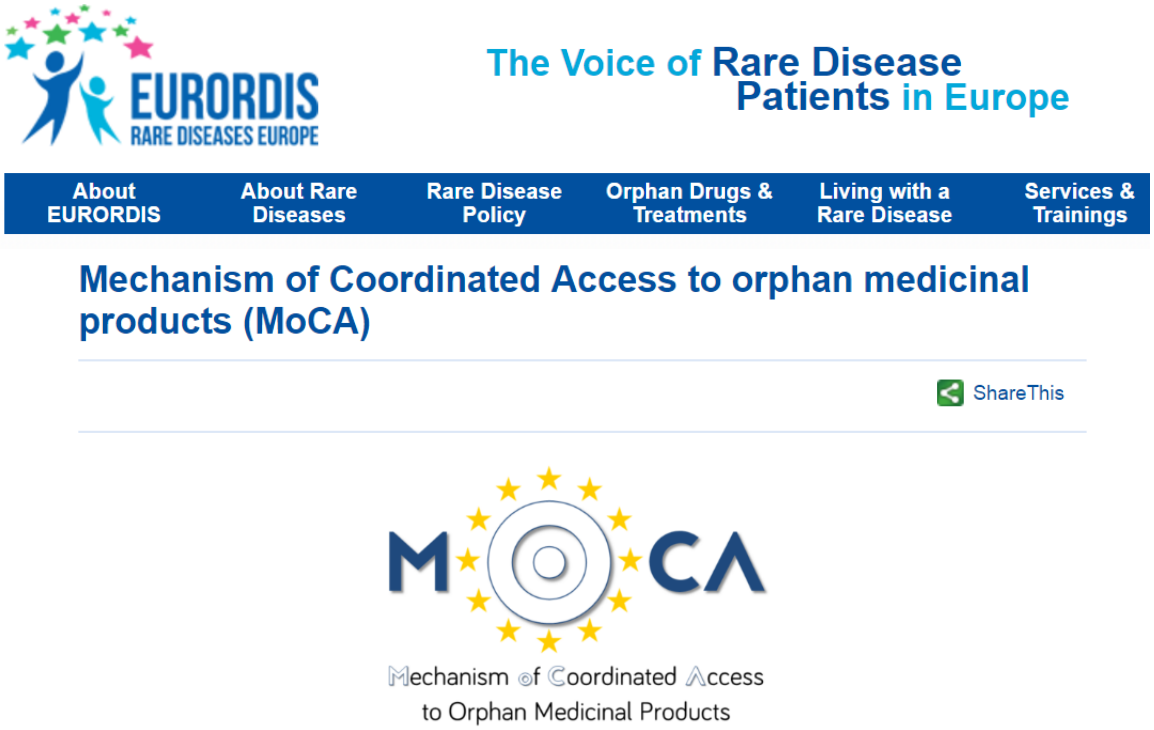
- Patient involvement in MoCA is essential to bring the patients' voice to the table as legitimate experts on:
  - The disease they are suffering from
  - The disease's impact on their daily lives
  - The solutions offered by available medicines
  - The unmet needs that new treatments should aim to fill
  - The impact of the therapy in real-life
  - Patient-relevant outcome measures
  - How patients will be affected if the medicine is only accessible in some countries and not others

Practical aspects

- Invitation by EURORDIS approximately 1 month before the meeting
- Declaration of interest and confidentiality agreement need to be signed
- Pre-reads from company and agenda distributed
- Meetings usually take place in Brussels at the European Social Insurance Platform offices
- Meetings last **1** hour (exploring remote attendance)

For more information

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



Thank you for your attention

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