MoCA Concept and Pilot Project

Feedback from the process around the first pilot project

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Slides based on the presentation given at the ECRD Berlin | 10 May 2014

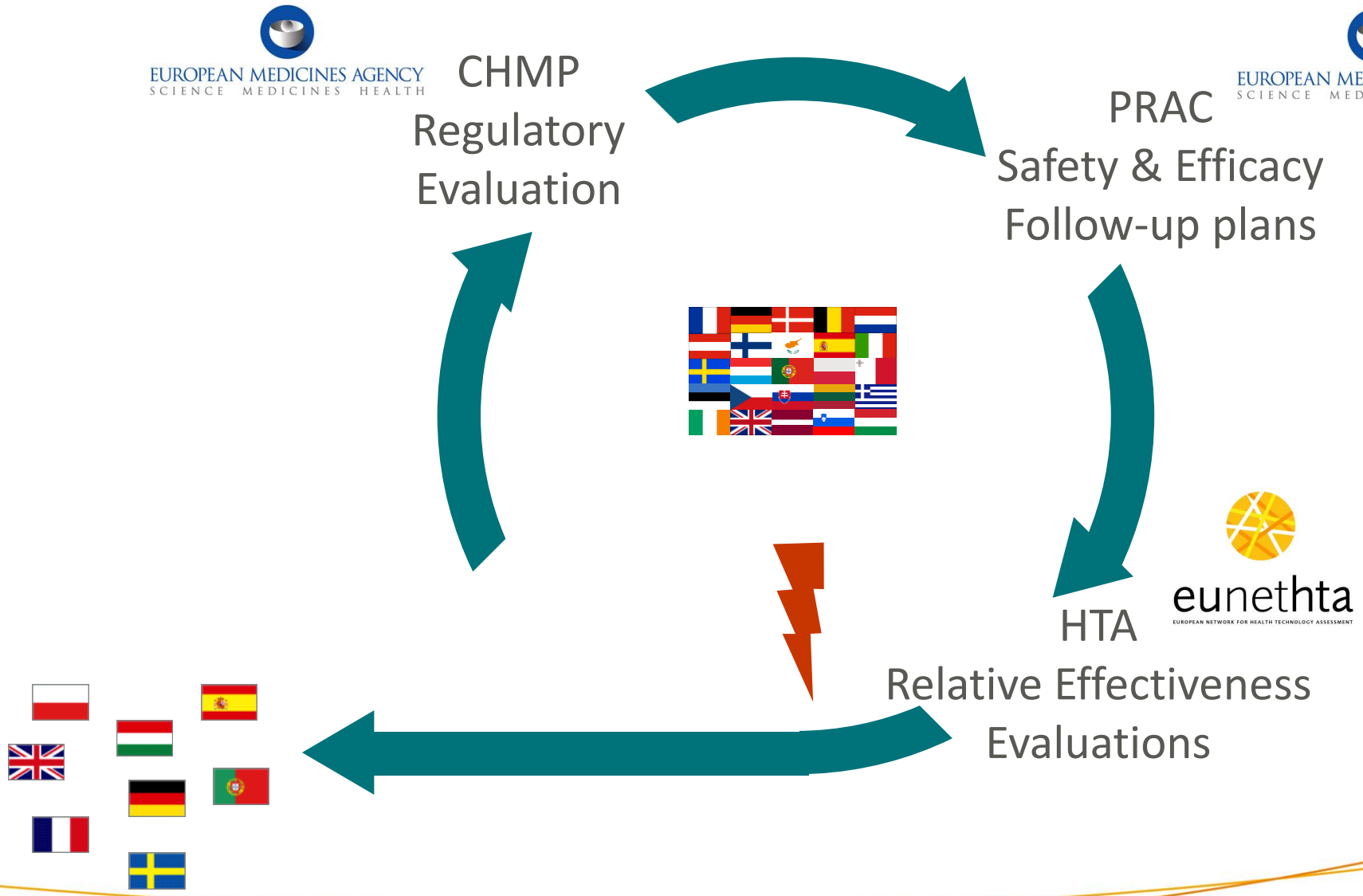
Shared environment, shared challenges?

- ➔ Shared objective: healthcare systems committed to be able to treat patient populations in a timely, equitable + sustainable way
- Pricing & reimbursement 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
- Challenges are shared – across borders and between stakeholders
- Solutions can be better explored collaboratively, rather than unilaterally

The 4 “Ws” of MoCA

- Who?
  11 + 1 EU Member States; industry; patients + other stakeholders – European Commission convenes
- When?
  December 2010 to April 2013
  Pilots start September 2013 onwards…
- What?
  - Recommendations
  - Transparent Value Framework draft tool
- Why?

Challenges in evaluation – even more apparent in OMPs



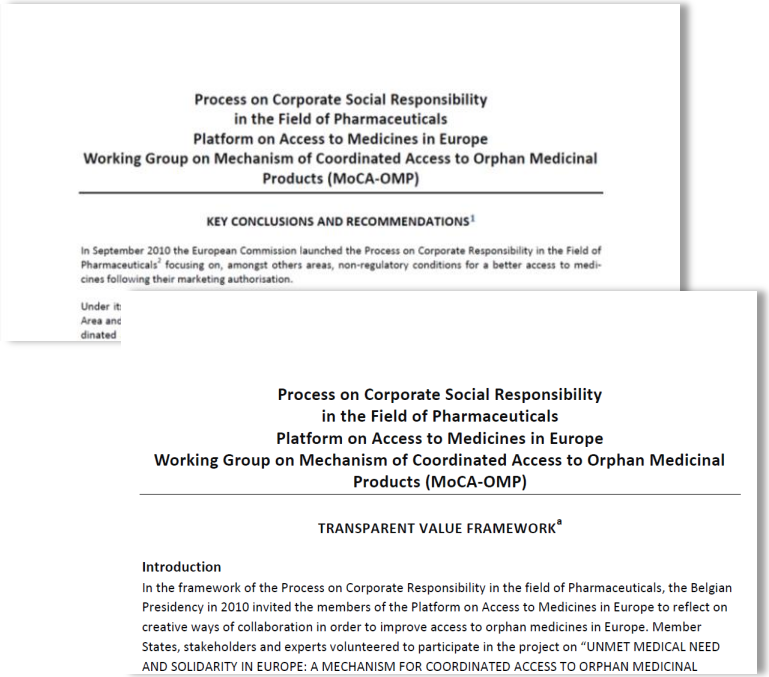
EU invitation – Member State / country initiative

INITIATIVE

DIALOGUE

Endorsed by
EU Member States – April 2013

OUTCOME



Process on Corporate Social Responsibility
in the Field of Pharmaceuticals
Platform on Access to Medicines in Europe
Working Group on Mechanism of Coordinated Access to Orphan Medicinal Products (MoCA-OMP)

KEY CONCLUSIONS AND RECOMMENDATIONS[1]

In September 2010 the European Commission launched the Process on Corporate Responsibility in the Field of Pharmaceuticals[2] focusing on, amongst others areas, non-regulatory conditions for a better access to medicines following their marketing authorisation.

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TRANSPARENT VALUE FRAMEWORK[a]

Introduction

In the framework of the Process on Corporate Responsibility in the field of Pharmaceuticals, the Belgian Presidency in 2010 invited the members of the Platform on Access to Medicines in Europe to reflect on creative ways of collaboration in order to improve access to orphan medicines in Europe. Member States, stakeholders and experts volunteered to participate in the project on "UNMET MEDICAL NEED AND SOLIDARITY IN EUROPE: A MECHANISM FOR COORDINATED ACCESS TO ORPHAN MEDICINAL

Testing it out
Pilot Projects

- "Bottom-up" approach
- Chaired by Belgium – leadership from countries
- Next steps fully country-led

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Result of the dialogue part: Recommendations…

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Under its Platform "Access to Medicines in Europe", EU Member States, countries of the European Economic Area and relevant stakeholders were invited to participate in a project group to develop the concept of a coordinated access to orphan medicinal products based on the set up of programmes between companies and

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- Voluntary payer-led proposal for engagement at all stages of the process, cross-border, on a continuum:
  - Horizon-scanning & early dialogue
  - Clinical development
  - Early Access Programmes
  - Therapeutic Scientific Compilation Reports
  - Patient selection
  - Transparent Value framework
  - Pricing
- Using existing specific tools & processes for OMPs
- Possible collective value assessment + potential purchasing agreements

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…and a draft tool: Transparent Value Framework
What could be valued / value-able?

- Basis for structured discussion between all
  around the value of an individual OMP – sir
- Taking into account:
  - **Unmet need:** availability of other treatmen
  - **Degree of net benefit:** Impact of new treatr
    against other treatments
  - **Response rates:** variable, important determinant
  - **Degree of certainty:** compelling evidence available?
- Post-Pilot: number of patients, burden of disease
- Where possible: Rarity – increased complexity at all stages
- Create shared understanding for starting Pricing & Reimbursement discussions in-country

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Transparent Value Framework (TVF)

Criterion	Lower Degree	Medium Degree	High Degree
Available Alternatives/ Unmet Need, including non-pharmaceutical treatment options	yes, new medicine does not address unmet need	yes, but major unmet need still remains	no alternatives except best supportive care - new drug addresses major unmet need
(Relative) Effectiveness, Degree of Net Benefit (Clinical Improvement, QoL, etc. vs. side effects, societal impact, etc.) relative to alternatives, including no treatment.	incremental	major	curative
Response Rate (based on best available clinically relevant criteria)	<30%	30-60%	>60%
Degree of Certainty (Documentation)	promising but not well-documented	plausible	unequivocal

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No sausages!

- Areas identified – weighting to be agreed in-country
- Basis to explore – acceptable levels of uncertainty – how to manage effectively to bring treatments to patients, e.g., managed entry agreements

From dialogue to action: Pilot Projects – testing it out

- Test fundamental assumptions
- Identify “+” and “-” of different proposals
- Test the different steps
- Streamline the process / elements
- Identify any gaps

Most important:

- Evaluate the real-life ability to deliver on ambitions and aims

Pilot Project initiated September 2013

- July 2013 – kick-off, “go /no-go”, RIZIV-INAMI, Belgium
- MEDEV agrees to host process
- September 2013 – MEDEV meeting, Rome
- October 2013 – Scoping meeting + workplan, Brussels
- 6 countries, 5 “observers” – resources
- Permanent rare disease patient involvement – EURORDIS
- Specialised therapeutic area patient representative groups
- Links prepared to other groups and/or initiatives
- Communications plans – rolling basis / “bulletin” – MEDEV, other stakeholders

10 Potential areas for collaboration

1. Potentially identifiable costs: direct / indirect costs related to the healthcare system
2. Protocol design: feedback to potentially be taken into account in programme design
3. Top-line data: review and gap identification
4. First run of Transparent Value Framework (TVF) with top-line data
5. Review proposed economic models
6. Identify collaborative opportunities with other initiatives

10 Potential areas for collaboration

7. Common agreement about key elements in dossier
8. Second run of TVF
9. Explore points in the regulatory process to align with other review bodies, e.g., COMP, CHMP, PRAC – elements to be included in regulatory follow-up measures

AND – in the plan but will need further work + elaboration

10. Explore areas and time-points for exploration and potential agreement on other elements
    - E.g., price-volume agreements, potential conditional scenarios
    - To support step to individual negotiations in-country

An important + highly relevant forum and opportunity

- **Singular opportunity** + forum for shared dialogue with a group of payers from across Europe
- Chance for **open, comprehensive, early dialogue** on development programmes
- Provides **shared insights**:
  - **Payers** on the realities of drug development – cost, risk, commercial considerations
  - **Industry** on payer concerns, constraints, motivations
- Could be a **safe forum** to explore:
  - Data collection, monitoring and follow up agreements
  - Models for early market entry & uptake, including pricing

Learning from experience: some practical considerations

- **Confidentiality:** time needed to secure agreements; case-by-case basis; almost all agencies covered in employment contracts
- **Timelines:** materials to participants in good time – allow review and national positions, robust interactions
- **Updates:** non-MoCA Task Force members and external stakeholders
- **Patient representatives:** briefing, background, bring up to speed – crucial element
- **RESOURCES!** The project is on top of the work already being done by MEDEV members. Company must be prepared to "step up", drive & coordinate with MEDEV leadership & MoCA Task Force

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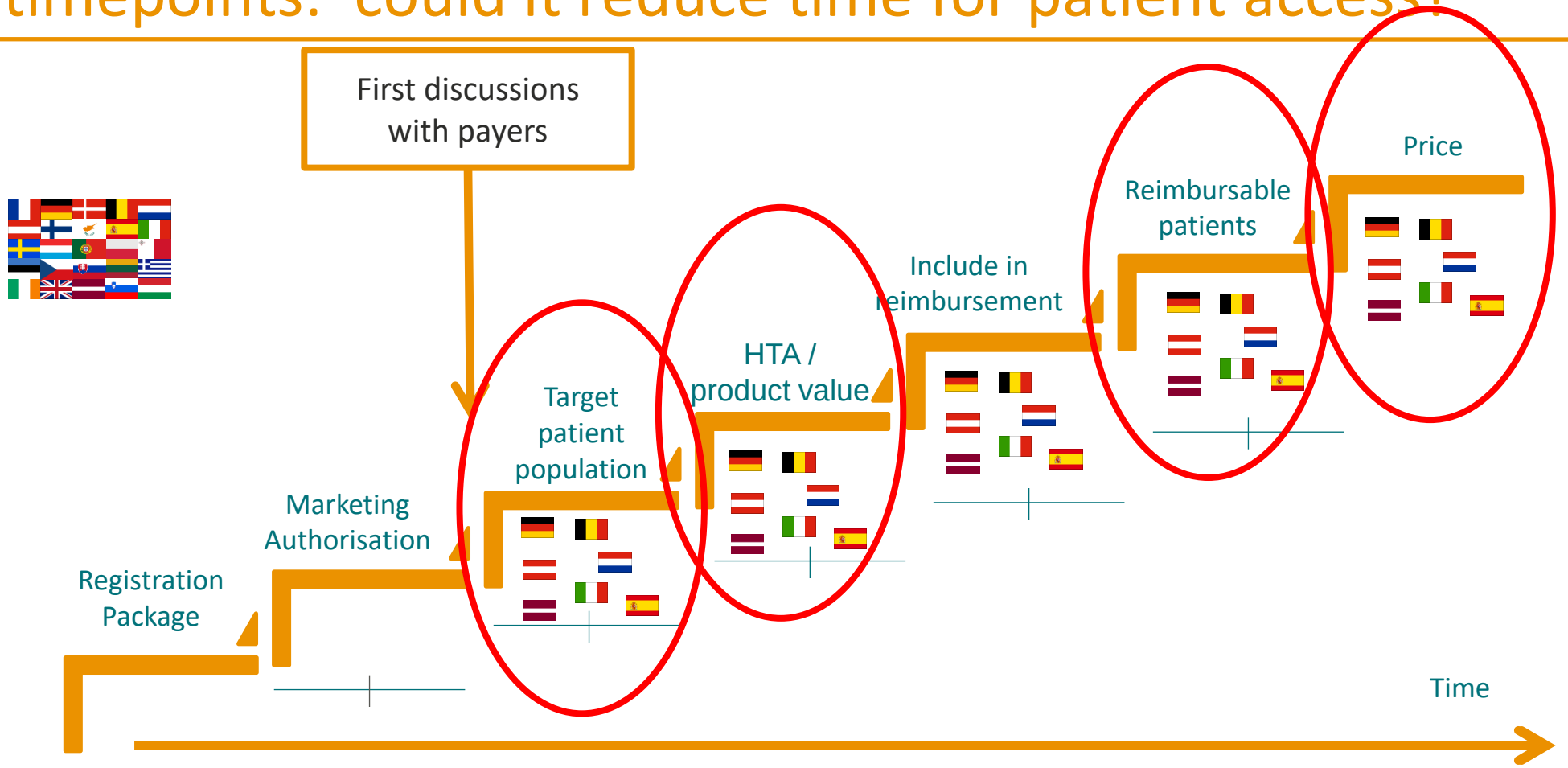
To continue + what would we do differently next time?

- ☺ Open, honest + constructive – helped us improve our planning based on input
- ☺ Feedback in advance of our Clinical Trial design – allowed us to adapt
- ☺ Helped us understand the requirements of the countries – similarities, differences; we could plan for a coordinated baseline + then tailor for local requirements
- ? Timing – confidentiality + need to make sure that we provide the information in advance; time to review inside agencies for meaningful conversations
- ? Too early to tell – did not yet get to the EUnetHTA + regulatory interfaces

What will we need to work on more in the next round?

- Helpful to have better clarity on the role + mandate from the payers' representatives in the room – mandate and authority varies
- How do we secure that the conversations had in the MoCA Task Force go on to truly have traction locally?
- What would be the legal and regulatory elements needed if we were to arrive at a price-volume agreement or conditional pricing scenarios?

Bringing forward the traditional interaction timepoints: could it reduce time for patient access?



Testing a hypothesis:

Could earlier & sustained dialogue result in timelier & more affordable access?

Pilot Projects – taking it forward from here

MOCA-CallToAction-MEDEV

MEDEV: Pilot project for MoCA
Call to Action

Introduction

"Decisions on Pricing and Reimbursement are the exclusive competence of the Member States of the European Union. Nevertheless, these Member States foster the same undisputed principles of equity and solidarity, face common challenges when providing indispensable medicines for their patients and suffer similar burdens when organizing this access. All of the issues become even more explicit when limited numbers of patients are concerned and possible answers to meet the Unmet Needs of these patients are scarce and expensive, as is the case with Rare Diseases and Orphan Drugs."[1]

In the framework of the Process on Corporate Responsibility in the Field of Pharmaceuticals, the Belgian EU Presidency in 2010 invited the members of the Platform on Access to Medicines in Europe to reflect

Conclusions

1. Timely and sustainable patient access to treatments needs us to work together to align on challenges and to explore solutions
2. All relevant stakeholders must be involved: patients, payers, industry, HTA bodies…
3. Early engagement with a multilateral payer forum at a minimum:
    - Develop awareness and understanding for a programme
    - “Design in” payer-driven elements to the clinical design
4. A positive experience and a solid foundation for building further in the future