Update on MoCA - Mechanism of Coordinated Access to Orphan Medicinal Products

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Disclaimer: This presentation reflects the work of all the stakeholders involved in MoCA. Their creative input is gratefully acknowledged.

However, the opinions expressed here are exclusively mine and cannot be attributed to any organisation participating in MoCA.

What is MoCA?

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

It is a

- **voluntary**
- **non-legislative**,
- **non-regulatory** and
- **non-binding collaboration**

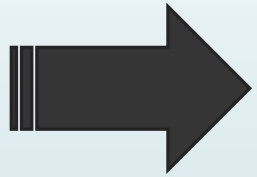
among stakeholders who are willing to work together to provide **real** to access to a **real** solution for **real** patients with **real** unmet medical needs

History of MoCA

Sept. 2010	The European Commission launched the Process on Corporate Responsibility in the Field of Pharmaceuticals
Dec. 2010	Platform „Access to Medicines in Europe“; under the Belgian EU Presidency, stakeholders and Member States are invited to participate in a Project Group
2011-2013	Working Group develops MoCA …Identifying and assessing relevant OMPs (“horizon Scanning”) …Structural Access and the Transparent Value Framework …Treatment (individual access)
2014--	MoCA initive by EURORDIS, MEDEV (an informal group of experts from statutory health insurance institutions in Europe, see www.medev-com.eu) and participating companies
2016	MoCA Revised Terms of Reference published
2018	EMA and EUnetHTA as Observers

Why Do We Need MoCA

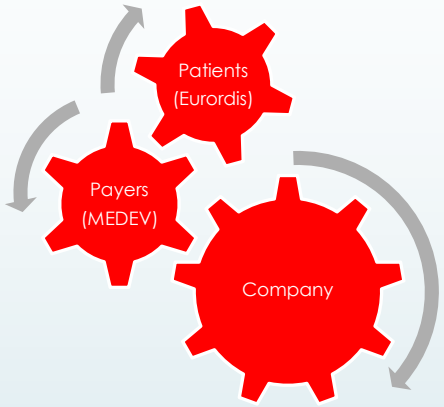
- OMPs often have high acquisition costs
- Insufficient knowledge about the disease – epidemiology, clinical pathways, lack of natural history data, lack of comparative data, experts with conflicts of interest
- Small sample sizes, single-arm study design
- Limited data on clinical endpoints, PRO/HRQoL
- Limited duration of trials
- Subgroup data not reliable



- Assessing orphans often fails to show cost-effectiveness*
- Uncertainties in cost-effectiveness modelling
- High acquisition costs
- Poor value for money
- Concerns about budget impact & opportunity costs
- Limited Access

*based on Nicod E, et al. HTA programme response to the challenges of dealing with orphan medicinal products: Process evaluation in selected European countries. Health Policy (2017), http://dx.doi.org/10.1016/j.healthpol.2017.03.009

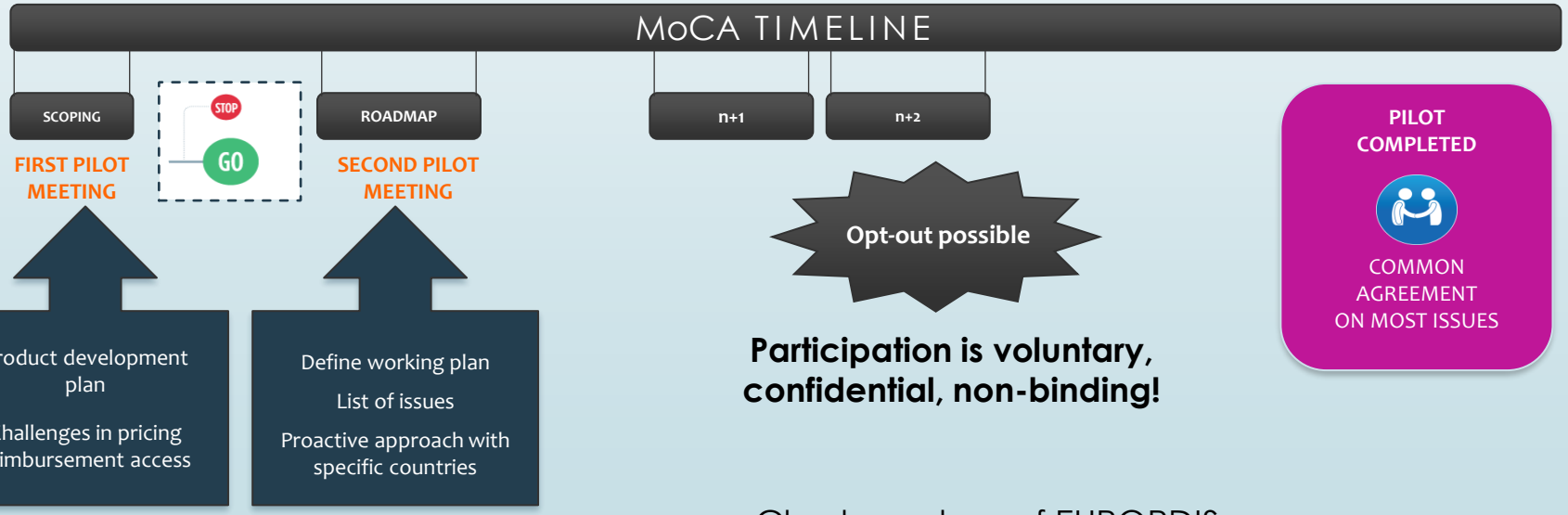
How does MoCA work?



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



Participation is voluntary, confidential, non-binding!

Chart courtesy of EURORDIS

Dynamics of a MoCA meeting

Company overview
Disease overview
Patient journey

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

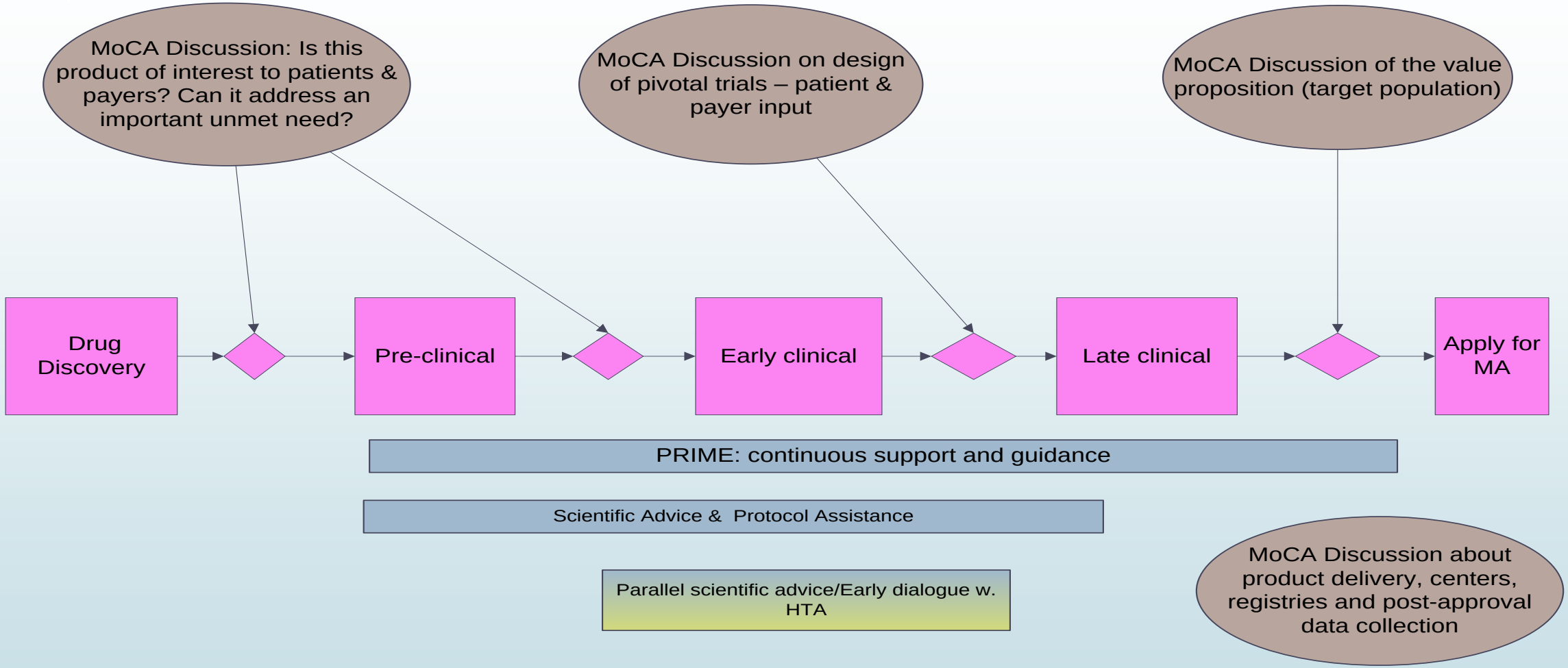
Timelines of the development programme

Data requirements – endpoints, PROs

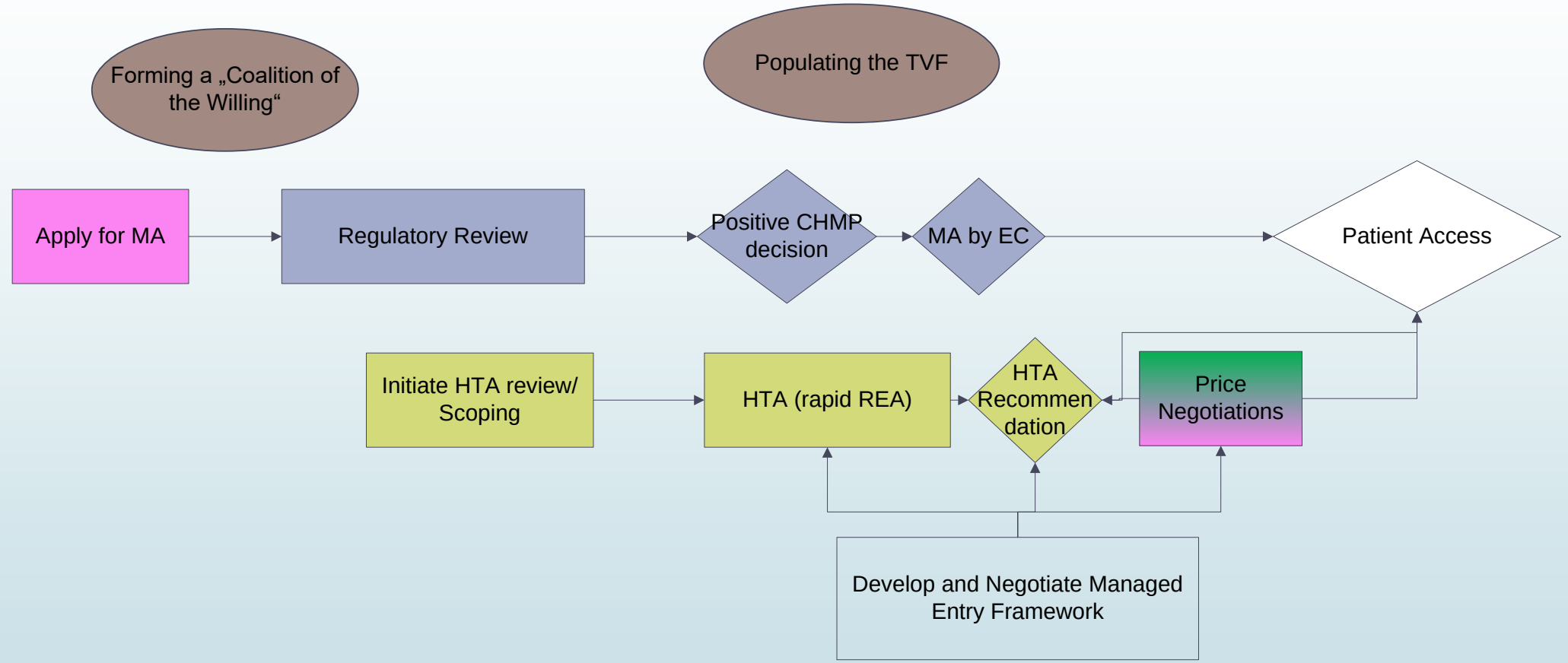
Country-specific reimbursement models - feasibility

Chart courtesy of EURORDIS

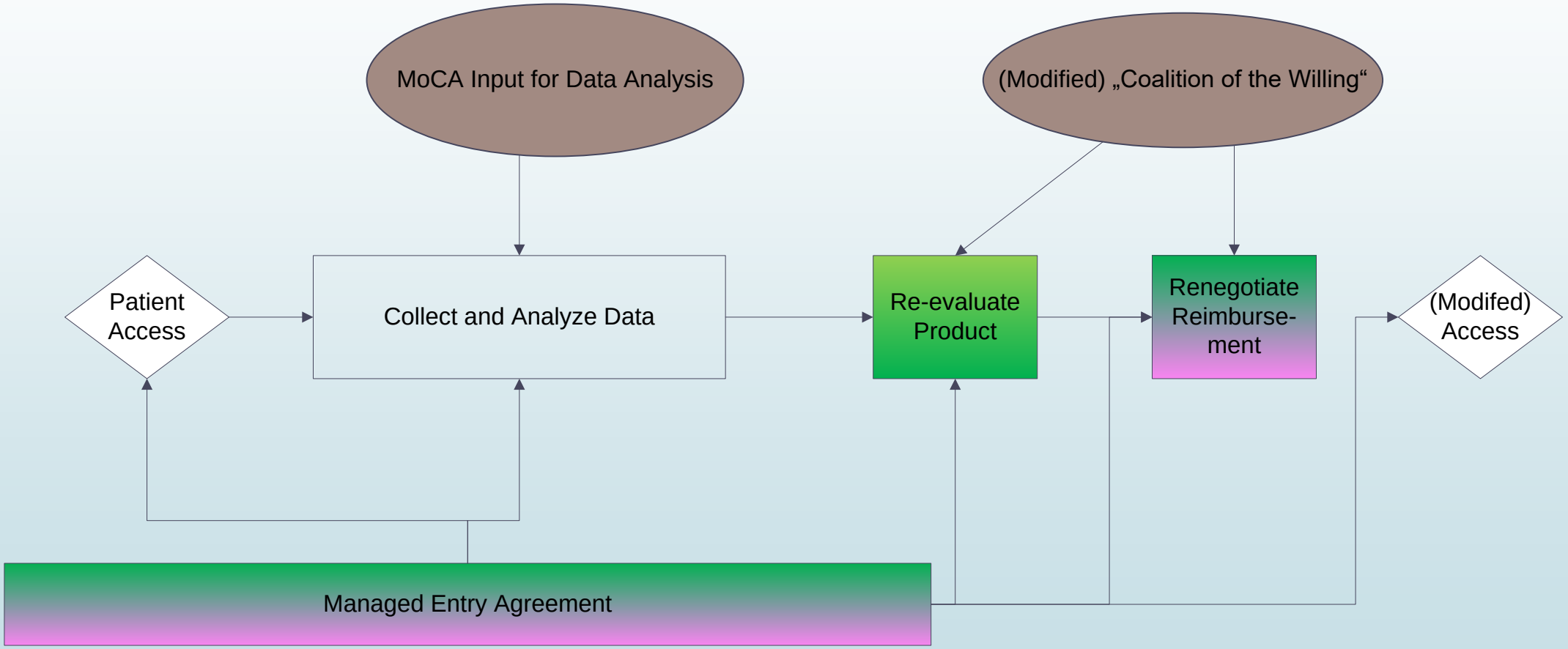
MoCA Interaction Pre-Launch



MoCA Interaction Peri-Launch



MoCA Interaction Post-Launch



What is the Transparent Value Framework?

- The TVF was introduced as one of the deliverables of the MoCA Working Group of the Process on Corporate Social Responsibility of the EC.
- It was developed as a framework for discussions between pharmaceutical companies and other stakeholders, eg groups of payers, about the value of new orphan medicinal products.
- The TVF contains only items which were agreed upon by all participating stakeholders.
- Ideally, the agreement about the values to be assigned in the TVF should facilitate subsequent negotiations about the reimbursement and pricing of the discussed product.

The Transparent Value Framework

Criterion	Lower Degree	Medium Degree	High Degree
Lack of Alternatives/Unmet Need, including non-pharmaceutical treatment options	yes, there are alternatives: 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) relative to alternatives, including no treatment, societal impact, etc.	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

General Comments on the TVF

- TVF is a way to discuss value aspects of a product, not a tool for quantification.
- There are already a number of tools for quantifying drug value, like the ICER.
- A distinction should be made between an assessment of value (is this product a valuable addition to our medicine chest or not) and aspects of cost effectiveness and affordability.

MoCA Stats as of March 2019

Number of participating companies/consortia:	16
Number of Products Discussed:	19
Number of payer-representing institutions that attended at least 1 meeting:	19

Other Participating institutions: EMA, EUnetHTA, Academia

Status at "first contact"	No.	Current Status
Pre-Clinical Stage	3	1 progressed to clinical stage 1 discontinued 1 in development
Phase 1/2	3	Development ongoing
Phase 2	3	1 approved by EMA, 1 terminated, 1 in development
Phase 3	5	1 approved by EMA, 1 terminated, 3 in development
MAA submitted	2	Both approved by EMA
Already authorised	3	1 additional indication in development

(Potential) Benefits of MoCA Interaction

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 follow-up and collection of PROs and real-life experiences
MoCA is charges no fees.		

Other Collaborations in Europe

based on WHO study on impact and benefits of cross-border collaboration in European Region, Infarmed Conference, 29-30 November 2018, by S. Vogler, F. Suleiman

Initiative	Horizon Scanning	HTA	Procurement	Joint Negotiations
Baltic Procurement Initiative			+	
BeNeLuxIA	+	+		+
Nordic Pharmaceutical Forum (IS, NO, SE, DK)	+		+	
FINOSE (Fimea, NoMA, TLV)		+		
Valletta Declaration (IR, PT, IT, SL, HR, RO, EL)	+	+		+
Visegrad – Fair Pricing (PL, CZ, SK, HU)		+		+

We're still working on…

- Outreach to SME's
- Coordinating Initiatives (EUnetHTA, EMA's PRIME, etc.)
- Recruiting more payers to participate: (resources, governance)
- Involving other decision-makers, not only experts

Thank you for your attention!

Any questions?

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

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