

Coordinated access to orphan medicines using a dialogue-based approach between key stakeholders The MoCA Experience



Mechanism of Coordinated Access
to Orphan Medicinal Products

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

Acknowledgements

This presentation reflects the work of all the stakeholders involved in MoCA.

Their creative input is gratefully acknowledged.

Disclaimer

The opinions expressed here are those of the presenters and cannot be attributed to any organisation participating in MoCA

Presenters - for the MoCA Steering Group

Simone Boselli (EURORDIS)

Anna Bucsics MoCA Project Advisor (moca.omp@gmail.com)

Wills Hughes-Wilson (ex-officio)

Topics

MoCA Explained

The Patients' Perspective

A Company Perspective

The Payers' Perspective

History of MoCA



Sept. 2010 The European Commission launched the Process on Corporate Responsibility in the Field of Pharmaceuticals

Dec. 2010 under the Belgian EU Presidency Platform „Access to Medicines in Europe“ invites stakeholders and Member States to participate in a Project Group

2011-2013 Working Group develops MoCA

2014-- MoCA implemented 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

Why do we need MoCA?

- Despite being authorized throughout the European Union, access to orphan medicines can be delayed or limited
- Early dialogue among patients, payers and marketing authorisation applicants to anticipate access problems and develop recommendations to solve them
- „Heads-up“ for payers about new, expensive medicines
- Feedback about problematic product development paradigms for companies
- Ensuring post-marketing data collection by including all relevant stakeholders
- Laying the groundwork for joint price negotiations

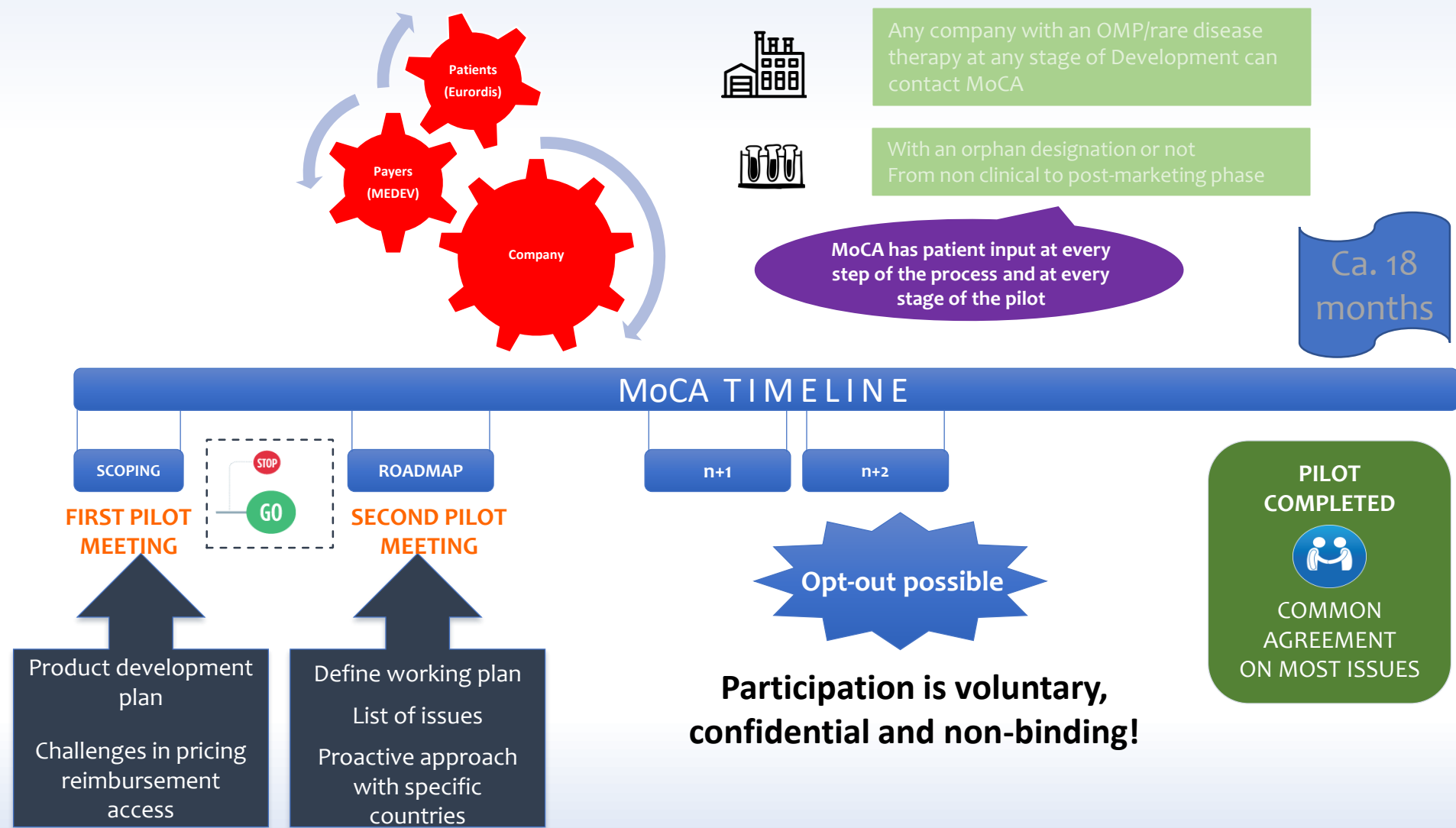
What is MoCA?

It 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

How does MoCA work?



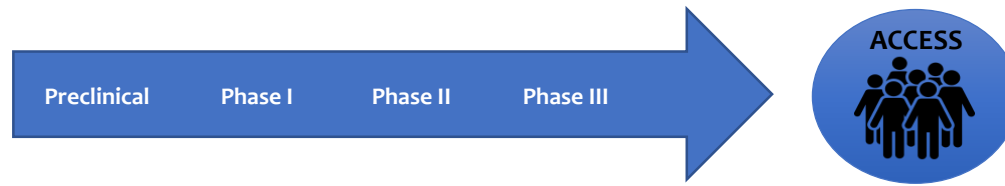
Dynamics of a MoCA meeting



Company overview
Disease overview
Patient journey



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



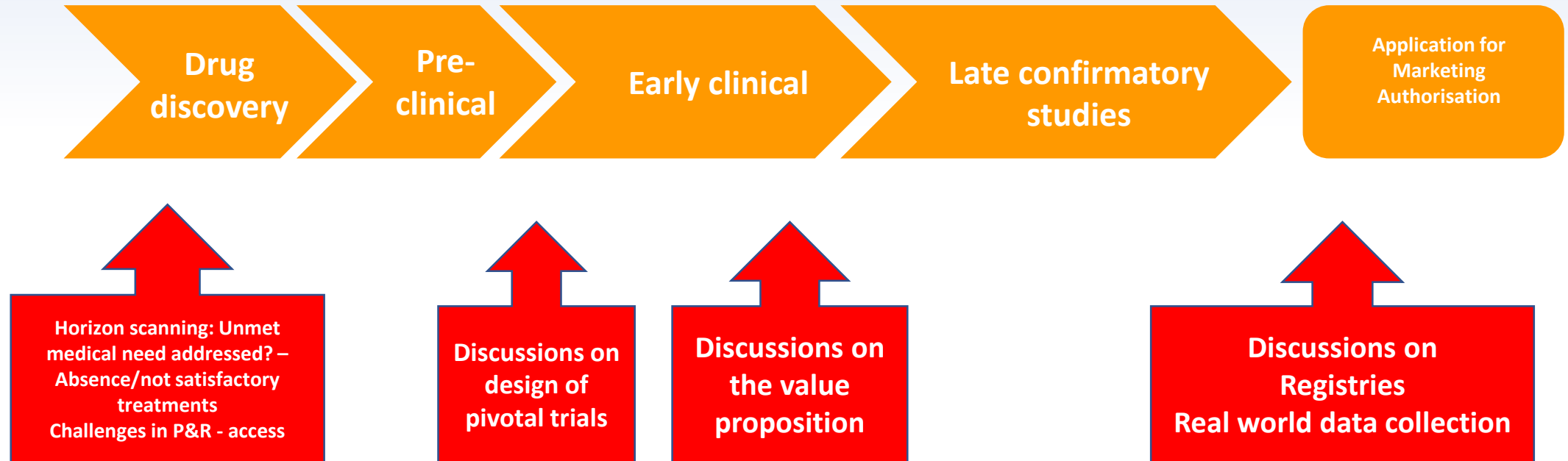
Timelines of the
development
programme

**Data requirements –
endpoints, PROs**

**Country-specific
reimbursement models -
feasibility**

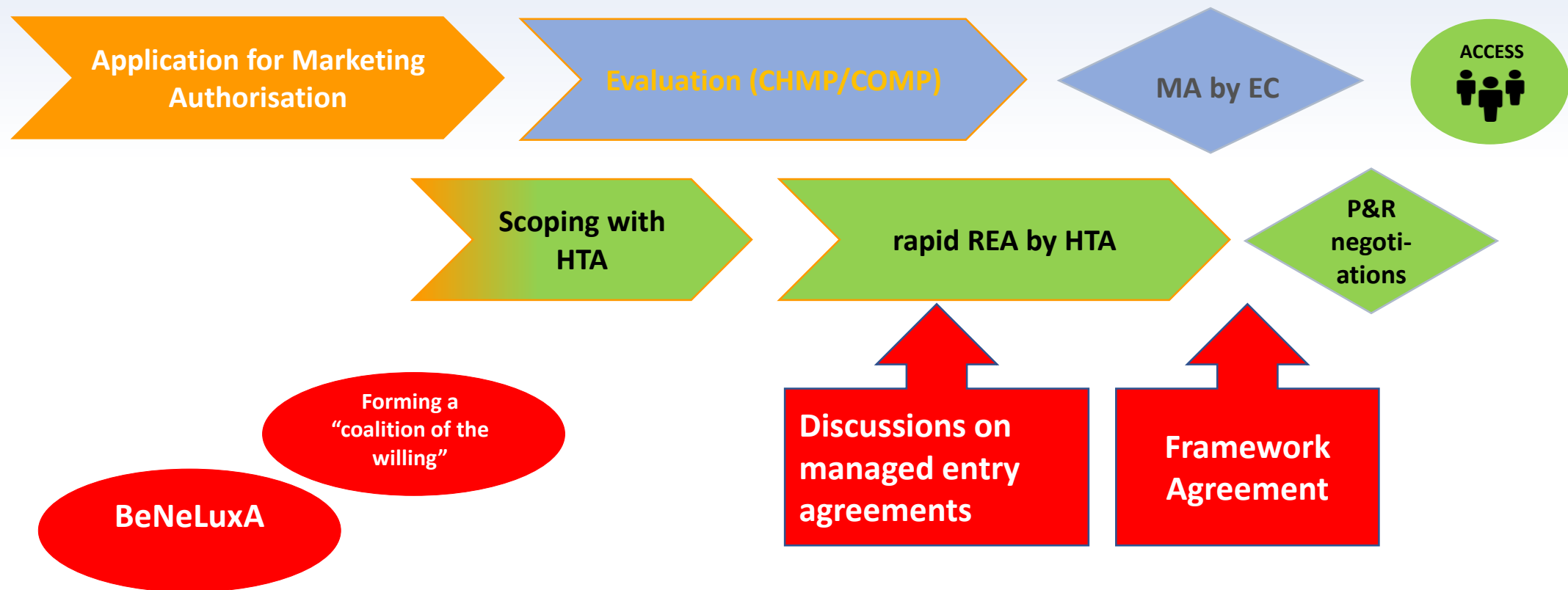


PRE- APPROVAL



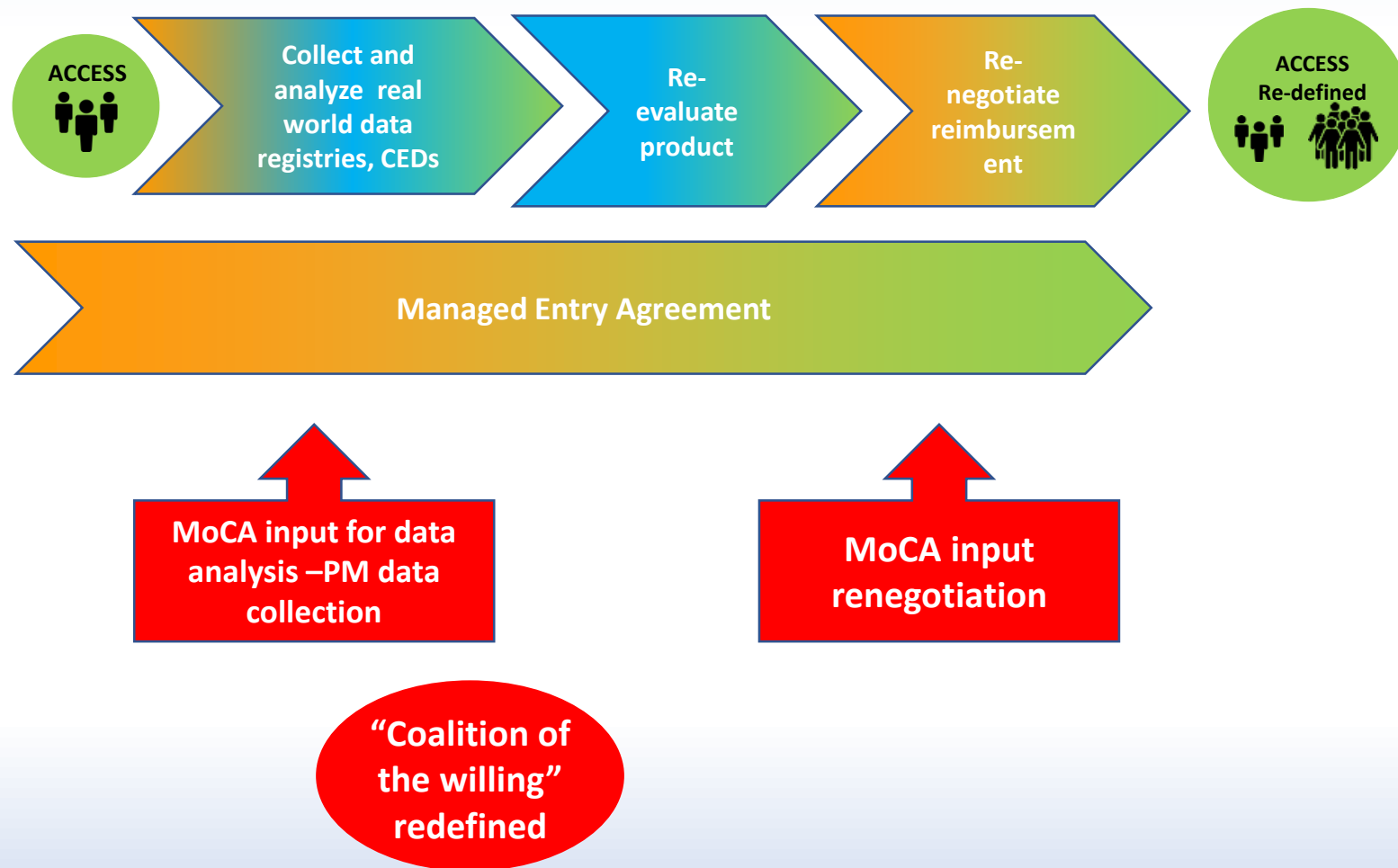
By participating in MoCA, companies can integrate **additional input from patients' and payers' perspectives** at any stage of product development

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



The Transparent Value Framework

Criterion	Lower Degree	Medium Degree	High Degree
Lack of 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) 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

New orphan medicinal products could be assessed according to how well they fulfilled the different criteria at a given point in time. This could be compared with other therapeutic alternatives and be included as one factor in pricing negotiations in Member States

Participants

Number of companies/consortia participating from 2014 – 2018	14
No of payer-representing institutions (attended at least 1 meeting, estimate based on 2014 and 2016 and 2017 records)	18
Number of patients attending	9
Other institutions (EMA, EUnetHTA, Academia)	3

Meetings 2014-2018

Number of projects	16
Number of meetings with companies	34
Number of meetings with patients attending	15

Status of product at first meeting

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

Indication/ATC

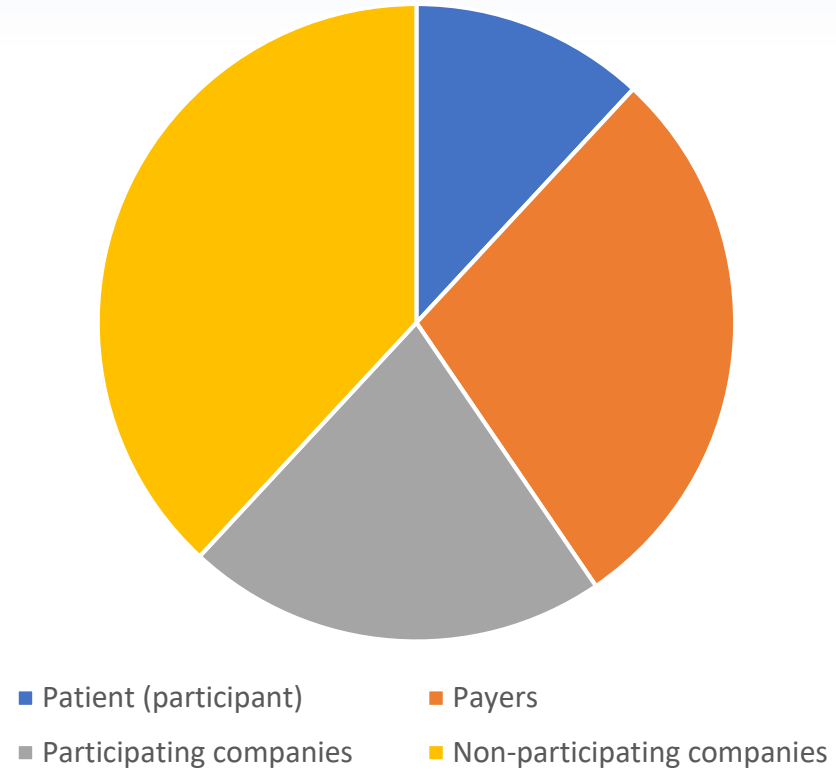
A (alimentary & metabolic)	4
B (Blood, hematology)	5
C (Cardiovascular)	2
N (neurological)	2
R (Respiratory)	2
S (Sensory)	1

Types of Products Discussed

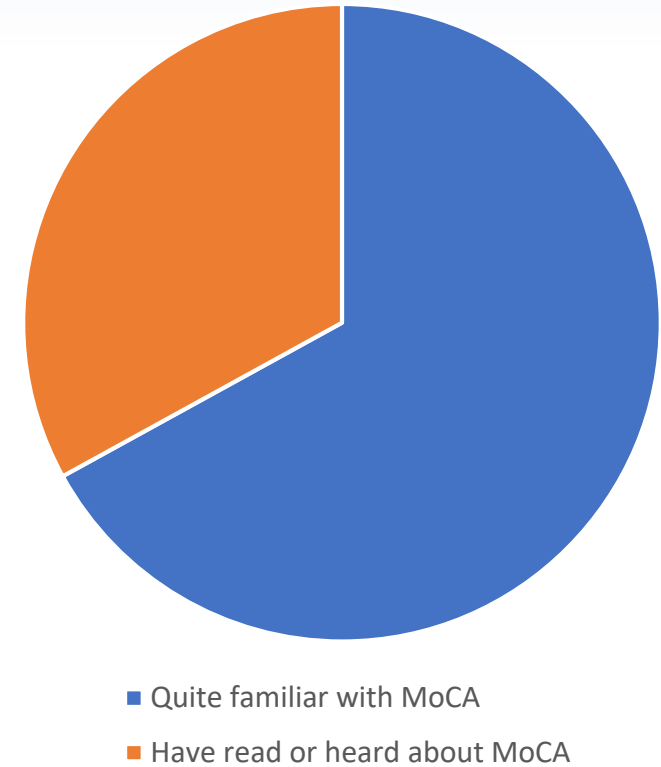
Small Molecules	7
Biologicals	3
Advanced Therapies	5
Other	1

Survey on Improving MoCA (2017)

Response

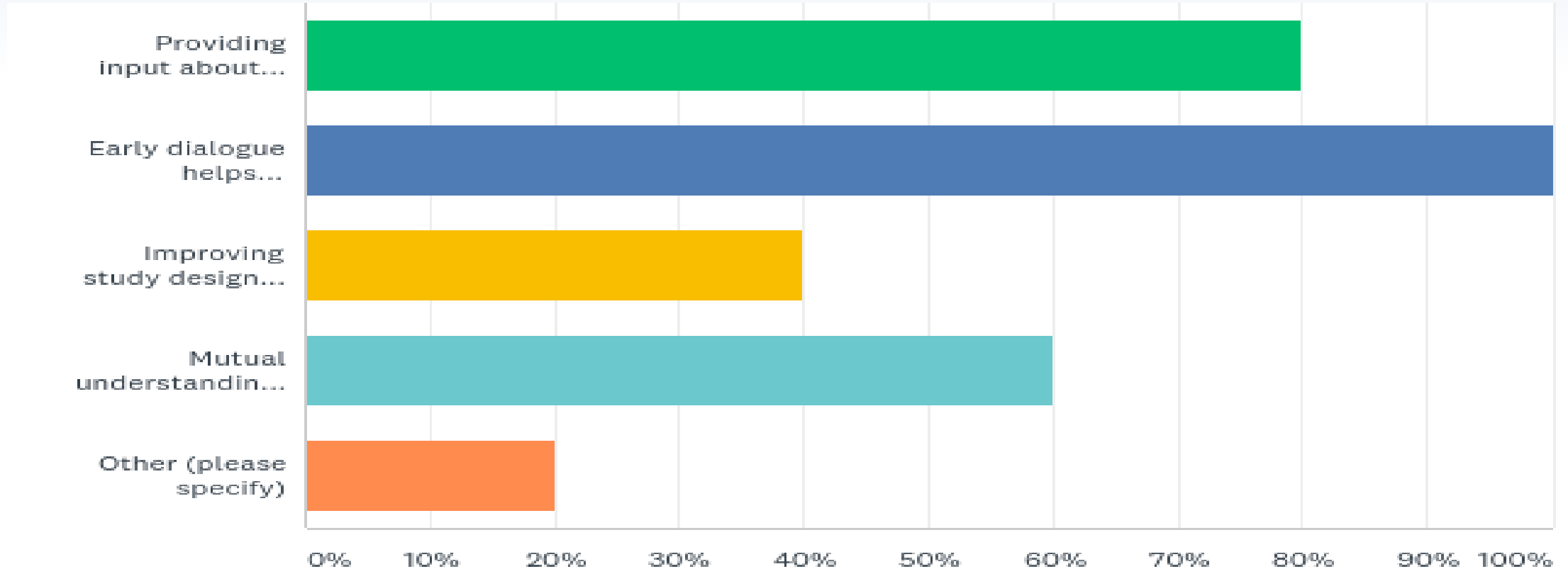


Knowledge of MoCA



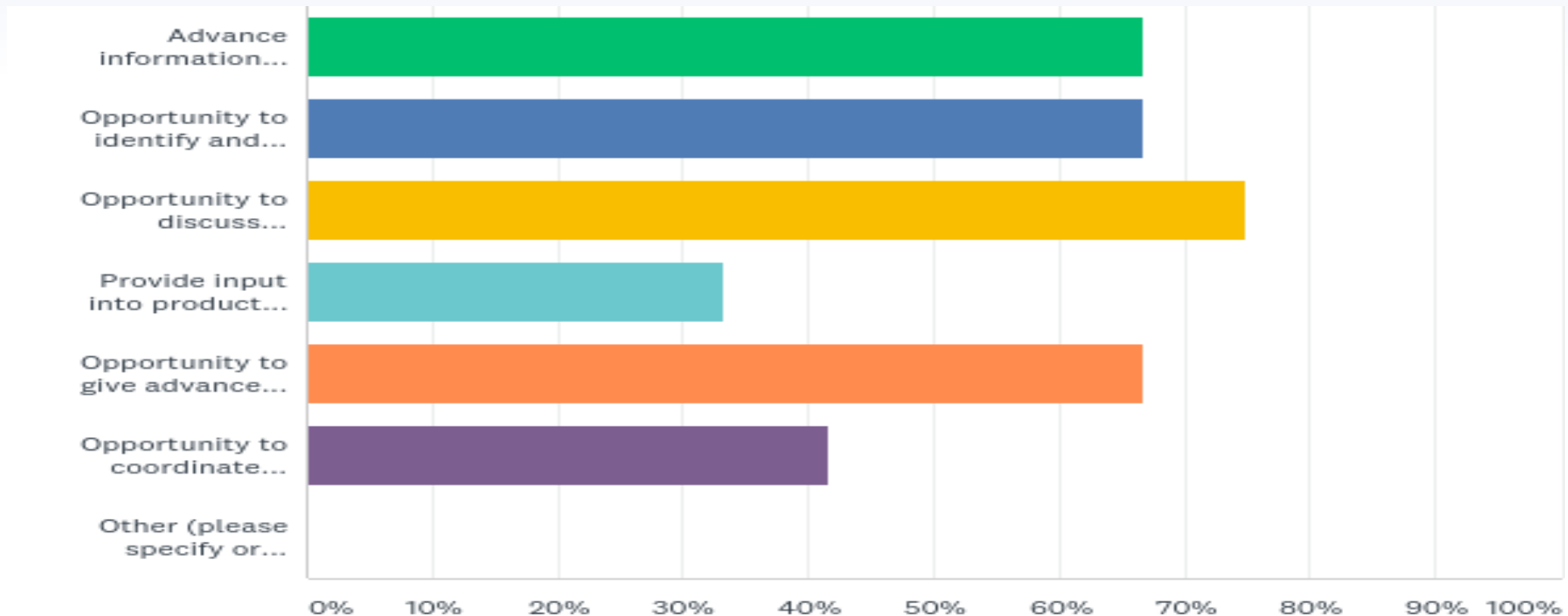
How is MoCA of use to Patients?

(multiple answers)



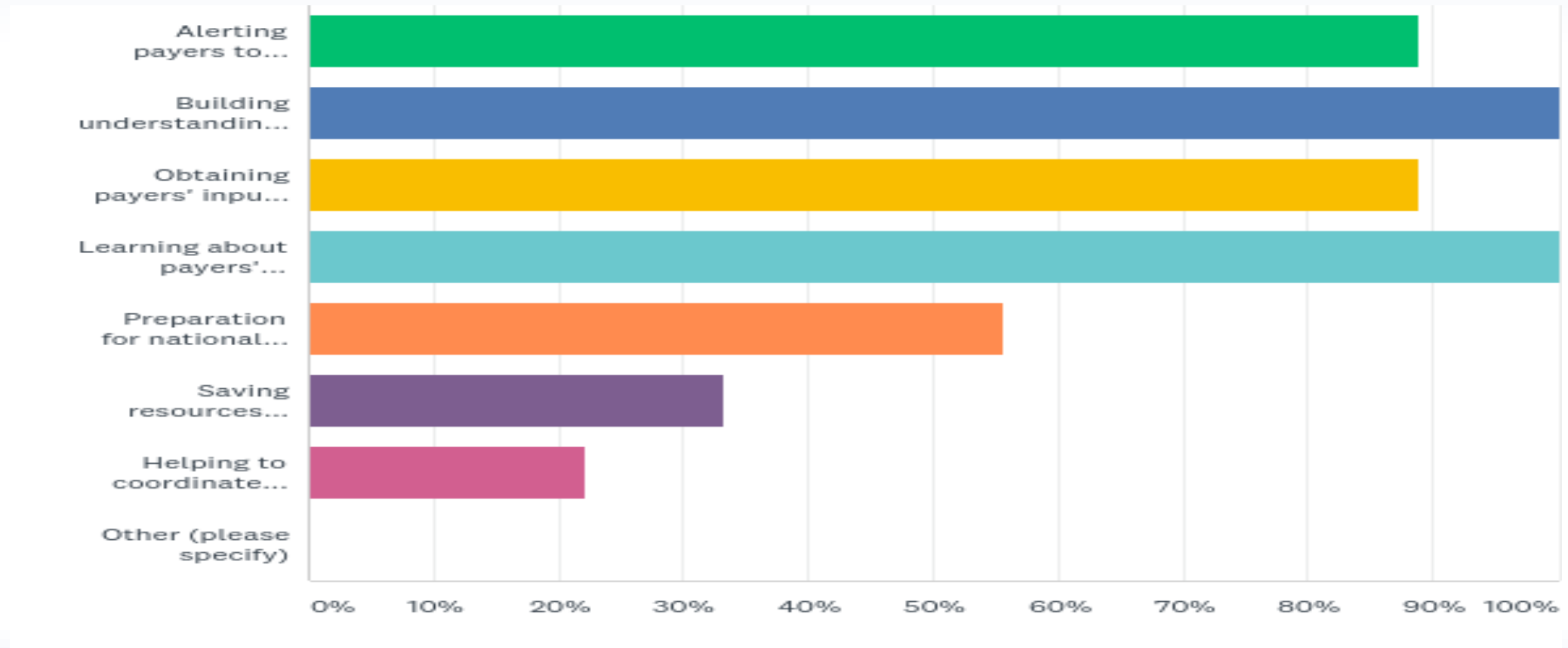
How is (or would be) MoCA most helpful for Payers?

(multiple answers)



How is MoCA of use to companies?

(multiple answers)



Major Limitations

- For Patients: Limit in negotiating mandate
- For Payers and Companies:
 - Limited opportunities for concrete decision-making
 - Overlap with other initiatives (EMA's parallel scientific advice, EUnetHTA, etc.) – payers would like better coordination

The Patients' Perspective



Why MoCA is important for patients

- **Patients are able to sit at a common table with industry and payers**
- We are considered an **equal partner** in the discussion around the access of important new therapies for many group of patients.
- We provide input about **what is important to patients** to companies and payers
- Early dialogue helps **anticipate** and **solve** access problems
- MoCA can help reducing **uncertainties also in post approval phase**, particularly in case of MA with conditional approval

What is the patients' role?

- In our role, **we try to provide the right patient at the right time to give the right contribution.**
- We provide also **training** and **educational tools** to enlarge the patient pool available
- Sometimes a **challenge** given the rarity of the conditions and the potential conflicts of interest that might prevent participation

What do we want to see moving forward

- **Early dialogue** between payers and product developers such as MoCA **should be encouraged** for a much greater number of rare disease therapies under development.
- **Such dialogue can go a long way to enable a comprehensive discussion of all aspects of patient access**, including but not limited to economic considerations
- **Scaling up the experiments** that have taken place to date requires political encouragement and financial support to lead to a truly European collaborative effort.

The Payers' Perspective

Learnings from MoCA

- Better understanding of which outcomes matter to patients and payers
- Better understanding of payers' needs for decision-making
- Companies are welcome anytime during the product cycle – but the earlier the better
- Understanding the challenges of complicated products, eg when a disease is so rare and the treatment so complicated that it will be limited to a few selected “Centers of Expertise” across Europe (role of ERNs)

More discussion on Prices & Pricing Needed

- Value based – what the market will bear?
- Fair return on investment - including public funds (basic research, RWD collection, etc.)
- Fair reimbursement
 - For each component of the technology
 - Compared to alternatives
 - Savings for the system should be real, not „skimmed-off“ via high prices
- Sustainable
- Equitable
- Considers uncertainty – adaptive pricing?
- ...Transparent?

Are all orphan medicines priced fairly?

- Fair reward for innovation

BUT

- Repurposed medicines: When compared with their unbranded or unlicensed versions, the branded orphan medicines were 1.4 to 82000 times more expensive (1)
- Prevalence is inversely associated with price only for „ultra-orphans“(1)
- Expanding indications or loss of orphan status may or may not lead to lower prices
- According to EvaluatePharma, Phase 3 orphan drug development costs half that of non-orphan(2)

(1) Onakpoya IJ, Spencer EA, Thompson MJ, et al. Effectiveness, safety and costs of orphan drugs: an evidence-based review. BMJ Open 2015;5:e007199. doi:10.1136/bmjopen-2014-007199

(2) <http://www.evaluategroup.com/public/Reports/EvaluatePharma-Orphan-Drug-Report-2015.aspx>

Access is more than pricing...

- Who will pay?
 - Hospital budgets? Health insurance? Special funds?
 - International cooperation/Cross-border issues?
- For what? Cells, intervention, follow-up?
- Who will get the money? Company, centers?
- How much?
- When?
 - Up front?
 - Over time, based on performance?
- What will be the role of the European Centers of Expertise?

Experience from a company perspective

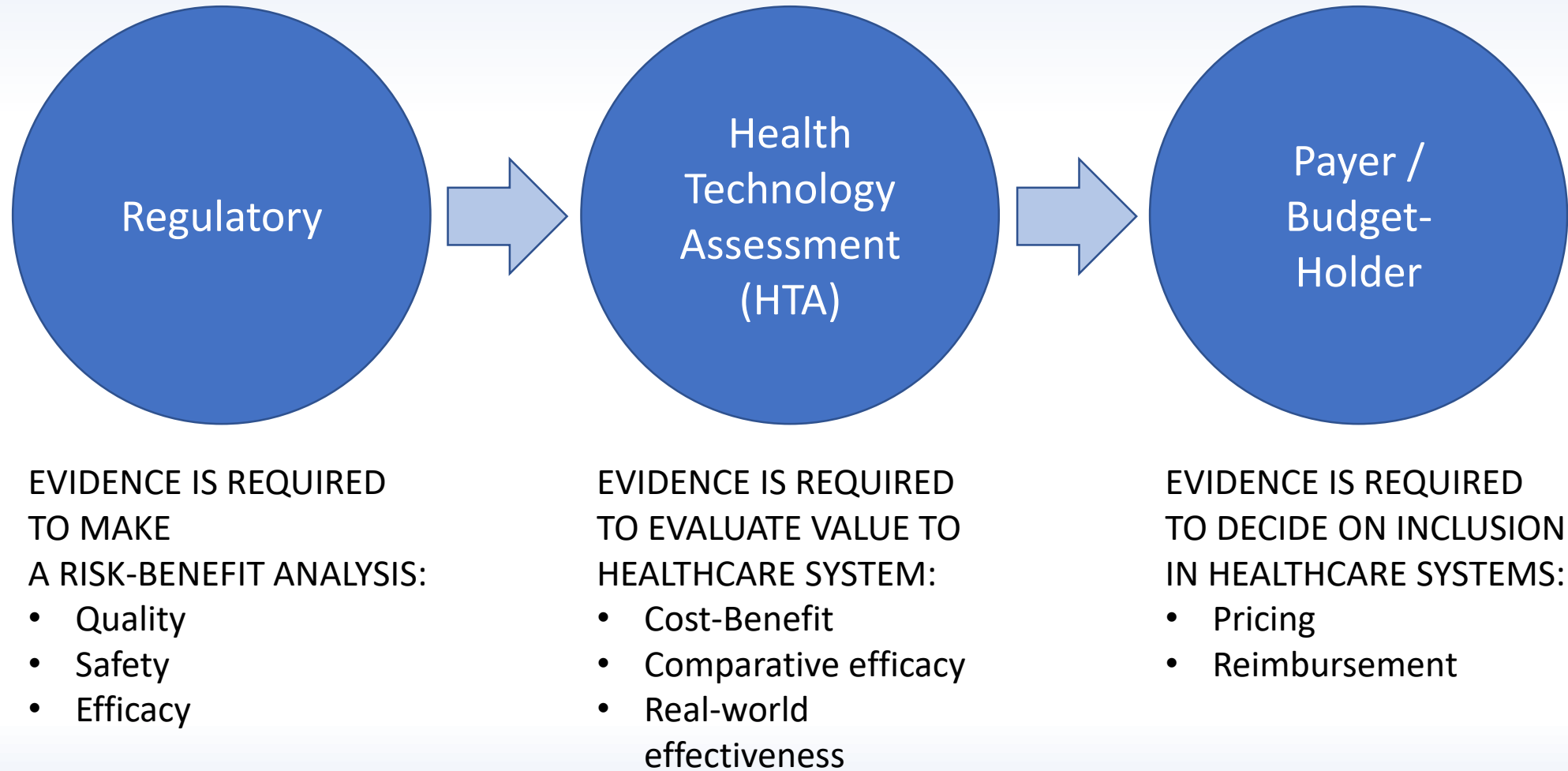
- Individual company / product conversations are confidential
 - Payers not “bound” by points of view explored during meetings
 - Companies can explore options in a “safe space”

Personal perspective + reflections from experience of the MoCA

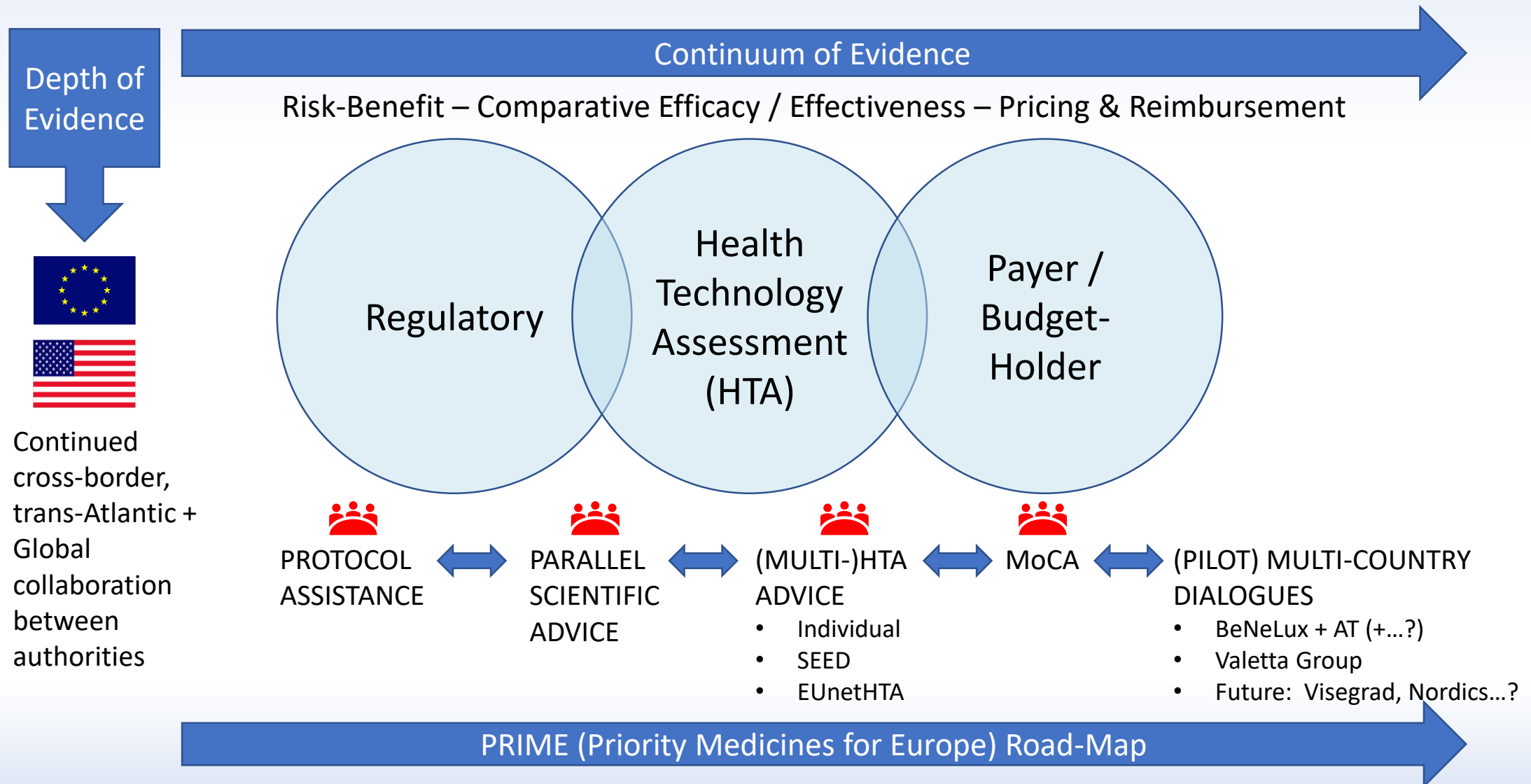
Wills Hughes-Wilson

willshugheswilson@gmail.com

Development The “traditional” way = sequential over time



Maximising interfaces between decision-makers & their evidence needs



A “buffet” for drug development . . You do the choosing

Questions & “issues” vary per programme...

1. Example 1 – very early phase programme – pre-clinical
 - How do we view a costly-to-produce biologic where there is no other treatment?
 - What will be measured / what should end-points be?
 - How will they be measured / what tools + what will be acceptable
 - How demonstrate / validate
2. Example 2 – Phase III was underway
 - Nature of treatment – 4 weeks for a lifetime of prevention – how evaluate? How quantify?
 - What existing costs will you include as comparator?
 - What endpoints / clinical measures will be relevant?
 - Which tools do you use?

...and the issues we are seeking to solve will vary

3. Example 3 – Phase III was underway

- Does regulatory approval alone actually serve the patients' needs?
- Nature of disease + rate of progression => tailored + adapted (regulatory) study design
- Very large number of endpoints with requirement to show positive trend on some or all – unclear
- Guidance on how to interpret trends in a way that could be meaningfully acceptable to payers + practical suggestions on what to do to accomplish

4. Example 4 – Phase III had completed, data was available

- Strong phase III additional efficacy outcomes data in a “well-served” field
- What is your biggest concern + how can we address?

Current reflections + future considerations

- MoCA dialogue has allowed “early warning” / issues identification – ideally at a time when the challenges can be addressed
- BUT we can observe that value has been added, no matter what stage the dialogues have happened

Need to build on the experience to date to consider:

- Effective use of time and resources – especially for payers
 - Coming as early as possible vs. statistical likelihood of failures in drug development
- Secure no “two-speed” system
 - Linking MoCA into other – particularly regulatory – approaches, e.g., PRIME, Adaptive Pathways
- How to secure that there is something in it for all stakeholders – create a “virtuous circle”

Learnings – Critical Success Factors

1. Genuine commitment to patient access to therapy as a key objective as well as a sustainable business
2. Support from / involvement of company leadership – mandate for conversations / participation in the process
3. A genuine intention and commitment to the process / genuine openness to hearing the payers' point of view
4. Internal collaboration across functions
5. Transparency with investors + governance bodies within the company
6. Full participation of the patient community

**Thank you very much
for your attention!**

...

Any questions?

Anna Bucsics
Advisor to MEDEV and the MoCA Project

moca.omp@gmail.com

EuropaBio Patients Bio-Forum, Brussels, 25 May 2018