Multi-Stakeholder Late & Early Dialogue – The MoCa Experience & Potential Contribution

EMA-Payer Community Meeting, London, Sept 19, 2017

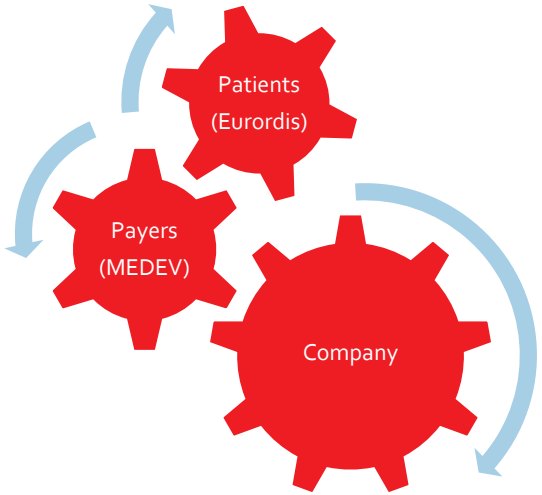
Mechanisms **o**f **C**oordinated **A**ccess to Orphan Medicinal Products

- 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***

What is MoCA?

- **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

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

STOP / GO

ROADMAP

SECOND PILOT MEETING

n+1

n+2

PILOT COMPLETED

COMMON AGREEMENT ON MOST ISSUES

Product development plan

Challenges in pricing reimbursement access

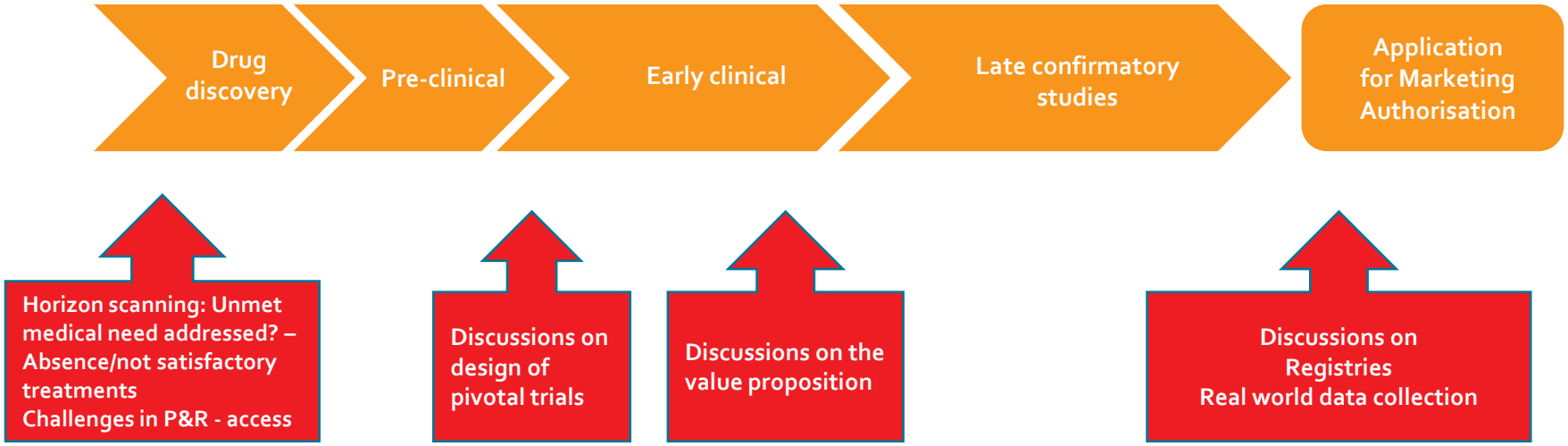
Define working plan

List of issues

Proactive approach with specific countries

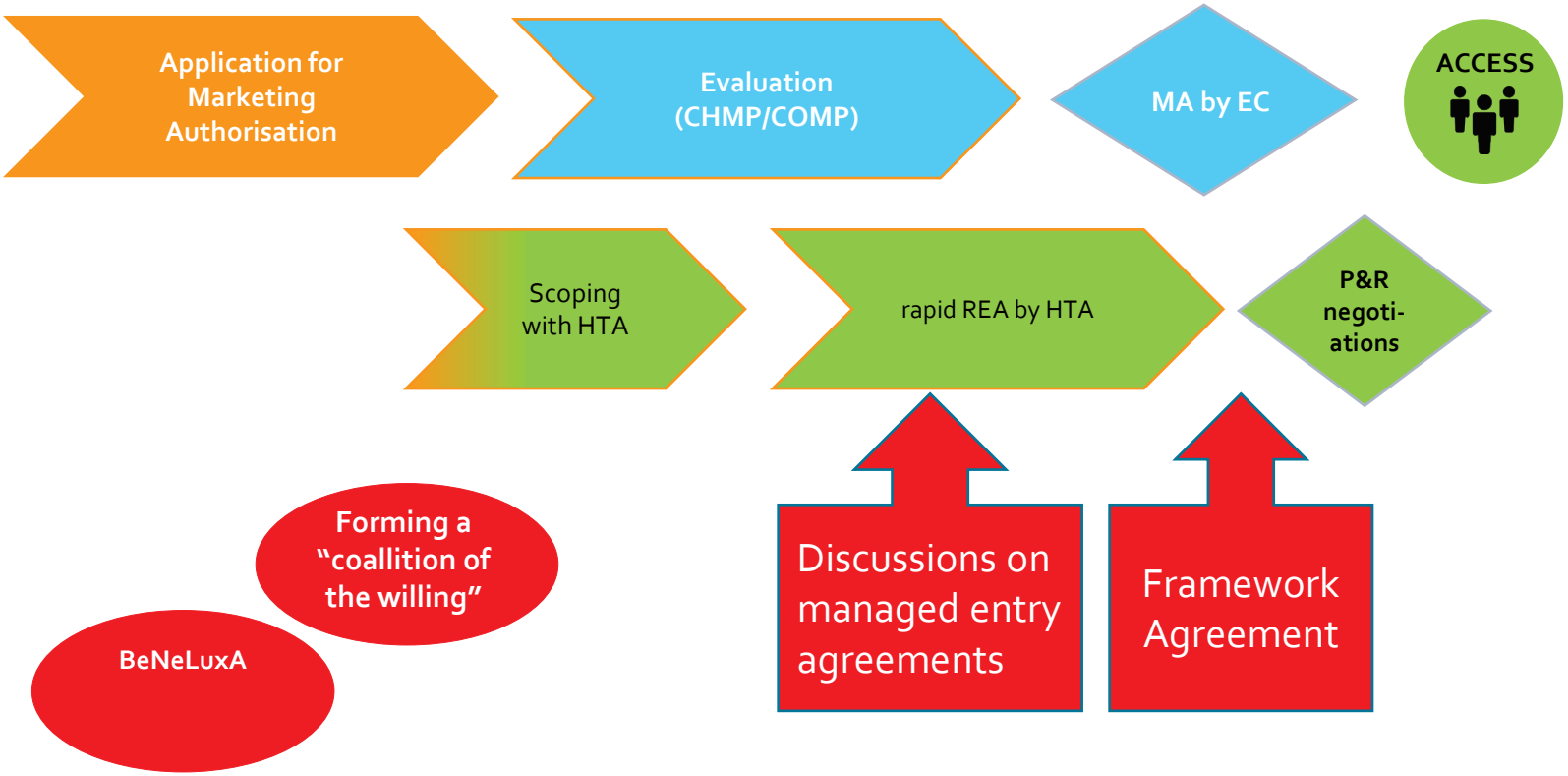
Participation is voluntary, confidential and non-binding!

PRE- APPROVAL



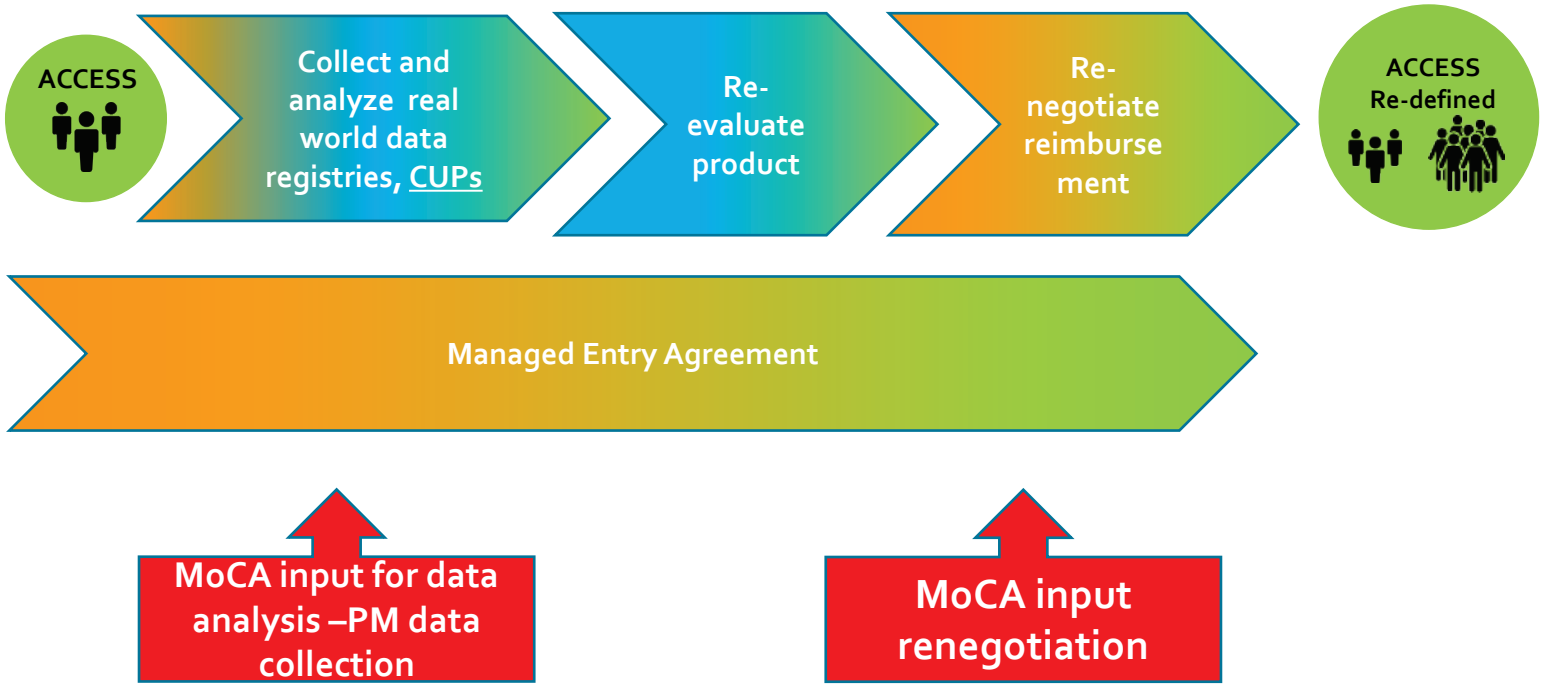
By participating in MoCA, companies can integrate **additional input from patients‘ and payers‘** perspectives at any stage of product development

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



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 f-up and collection of PROs and real-life experiences

Participants

Number of companies/consortia	11
No of payer-representing institutions (attended at least 1 meeting, estimate based on 2014 and 2016 records)	13
Other institutions (EMA, EUnetHTA, Academia)	3

Types of Products Discussed

Small Molecules	5
Biologicals	3
Advanced Therapies	4
Other	2

Status of product at first meeting

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

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

Survey on Improving MoCA

42 Responses:

- 5 Patient (participant) Responses
- 12 Payer Responses
- 9 Responses from companies which participated
- 16 Responses from companies that had not participated in a MoCA project

64% of responders „quite familiar" with MoCA

33% „have read or heard about it"

Products most interesting to payers: ultra-orphans, advanced therapies, hospital products

Relevant Responses 1/2

- **40% of respondents think MoCA is most useful in Stage 2-3 of development**
  - 17.5% think it's at Stage 1-2 and 15% think it's most useful just before and during the marketing authorization process; another 15% think it depends on the product, and 1 respondent thinks anytime during and after Phase 2-3

- **More than ¾ see key connections with other EU projects**
  - **80% with parallel scientific advice, adaptive licensing**
  - **77% with EUnetHTA**
  - 46% with PRIME
  - 44% with BeNeLuxA

Relevant Responses 2/2

- **51% of respondents see overlaps or redundancies** with other EU projects. Of these,
  - **74% of those who do, see it with EMA's scientific advice, parallel scientific advice, adaptive licensing EMA's COMP activities**
  - **63% with EUnetHTA**
  - 37% with BeNeLuxA
  - 21% with EMA's PRIME

- **75% of payers think better coordination with other efforts**, eg those by EMA, EUnetHTA, BeNeLuxA would make MoCA more useful

How to make MoCA more useful?

- MoCA can play a key role in coordinating the input from key stakeholders (EMA, HTA, patients, payers…)
- More resources, more stakeholders (hospitals, EMA, more payers) would make MoCA more effective
- Stronger mandates for payer participants
- Better alignment [with other processes] on key questions of evaluation and assessment
- Provide more guidance on when, where and how to approach MoCA
- …

Results of Brainstorming Session

Company Perspective

Feedback from „real" payers to make sure that the right evidence is generated

Companies choose fora to participate in

Balancing local requirements (more detail) vs EU level (may save resources vie harmonization)

Market access people may not be consulted by clinicians designing trials

MoCA is a forum for companies to be open about their own uncerntainies

During trial design, look at the evidentiary uncertainites remaining

Consider

- patient numbers
- adjudicating outcomes agreements
- where to place treatment centers – cross border healthcare

Interaction of patients with payers very helpful

EMA Perspective

- Values informal part of discussions at MoCA
- Payers are important players in registries, and were invited to EMA's Registries Workshop – will publish key principles for promoting registries
  - Not product registries
  - Liaise with holders of existing registries

Luca Pani, former AIFA / former CHMP / SAWP member EMA

"Start talking with the payers early – very early. When you start thinking about end-points. And I mean payers, not only HTAs. HTAs are not useful without the payers."

Payer Perspective

- Patients are actually more involved in advice and input than payers!
- Let‘s keep MoCA‘s Unique Selling Point:
  - Informal, brainstorming, patients‘ contributions always constructive
  - Formalization has the peril of confrontation
- Providing input into the development program at a time when it can be acted upon
- Opportunity to think about consequences of pricing at an early stage
- Maybe, hand off the product to another forum at some point in time?
- Will all these efforts make drugs more affordable?
- Talk also about pricing in MoCA
  - Can a company explain how the price was established
  - Can we all work on making drugs more affordable

Patient Perspective

- Patients are also concerned about affordabiliy and budget impact
- Connectivity, not overlap
- Value of MoCa is reducing uncertainty – on
  - Relative effectiveness
  - Cost effectiveness
  - Via registries which are enriched by input necessary for all stakeholders
  - Focus on ultra-orphan and advanced therapies

EUnetHTA Perspective

- EUnetHTA can provide tools for MoCA to work with
  - Parallel consultation with EMA
  - REA
  - Help in developing registries – these need to be no more complicated than necessary
- EUnetHTA would be interested in developing registries
- Present MoCA at EUnetHTA

Registries

- Who owns the data? Who pays for them? Must payers fund registries without access to data?
- Companies have concerns about ensuring company access to data, especially in the context of products from several companies
- However, having a separate registry for each product is inefficient and NOT the way forward
- Alignment on data elements is important
- It‘s unethical to deny access if a registry can provide more information about a product, and the alternative would be to make patients get treatment with less information
- If registries are just a mechanism to enhance market access, so payers should not finance them
- They have to be useful optimizing the outcomes of the healthcare system
- Can patient records substitute for registries? Can they be supplemented with data from a sentinel system

Final thoughts:

New medicines need to be affordable.
A new medicine has no benefit if a patient cannot get it, because the healthcare system cannot pay for it.