# PROSPECTIVE PLANNING OF EVIDENCE GENERATION FOR ORPHAN MEDICINAL PRODUCTS – OPPORTUNITIES FOR MULTI-STAKEHOLDER DIALOGUE & MoCA

## THE COMPANY PERSPECTIVE

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**-- On behalf of industry input from companies participating in the MoCA --**

## EMA-PAYER COMMUNITY MEETING
## 18 JUNE 2019
## ZORGINSTITUUT NEDERLAND (ZIN), DIEMEN, NETHERLANDS

# (UNIQUE) BENEFITS OF MoCA ENGAGEMENT (1)

## COMPANY & PAYERS – plus for all stakeholders with interest in real availability

- **Unique opportunity to get payers' input into early advice**
  - Integrating points of view of key decision-maker segment – i.e., payers – early enough to impact the development work-programme + decisions

  ➔ Not routinely possible / available in other offerings within the "ecosystem"
- **Build awareness of the condition, review + discuss "obscure" endpoints in Clinical Trials**
  - Payers dealing with multiple different conditions – non-rare as well as rare – cannot be expected to know all conditions in detail
  - Anchor the rationale for endpoints for the condition in question – why were they chosen – e.g., by Regulators – and why do they matter

  ➔ It might not be immediately clear why these chosen endpoints, why are they the relevant ones in this particular condition

# (UNIQUE) BENEFITS OF MoCA ENGAGEMENT (2)

**COMPANY & PAYERS – plus for all stakeholders with interest in real availability (cont.)**

- **Highlight, review and discuss disease specificities of relevance to healthcare systems, e.g., epidemiology**
  - Share what is known, identify gaps + seek feedback on implications
  - Sense-check is particularly critical in rare diseases where there is often a high level of heterogeneity – e.g., subsets of severity?
  - Willingness to treat, relevance to treat, desired / expected / reasonable treatment outcomes – maybe per subset
  - Review the Target Product Profile (TPP) – eventual (anticipated) label – this is critical to the use of a given product in national healthcare systems

  ➔ Implications on patient numbers, budgeting, forecasting – for both company and healthcare systems

  ➔ In totally uncharted rare diseases with no previously existing treatments, allowing sufficient time for clinical benefits to be explored and understood

# (UNIQUE) BENEFITS OF MoCA ENGAGEMENT (3)

**COMPANY & PAYERS – plus for all stakeholders with interest in real availability (cont.)**

- **Registries – post-launch data generation**
  - Feasibility of setting up a registry in a given rare condition
  - Understanding what is key from payers' point of view in post-launch data collection
  - ➔ Post-authorisation data collection may form part of the Marketing Authorisation – by Regulators
  - ➔ Healthcare systems might require similar data, with different elements
  - ➔ Registries often form key part of this – Regulators and HTAs / payers in-country
  - ➔ An integrated approach could provide better outcomes for all parties

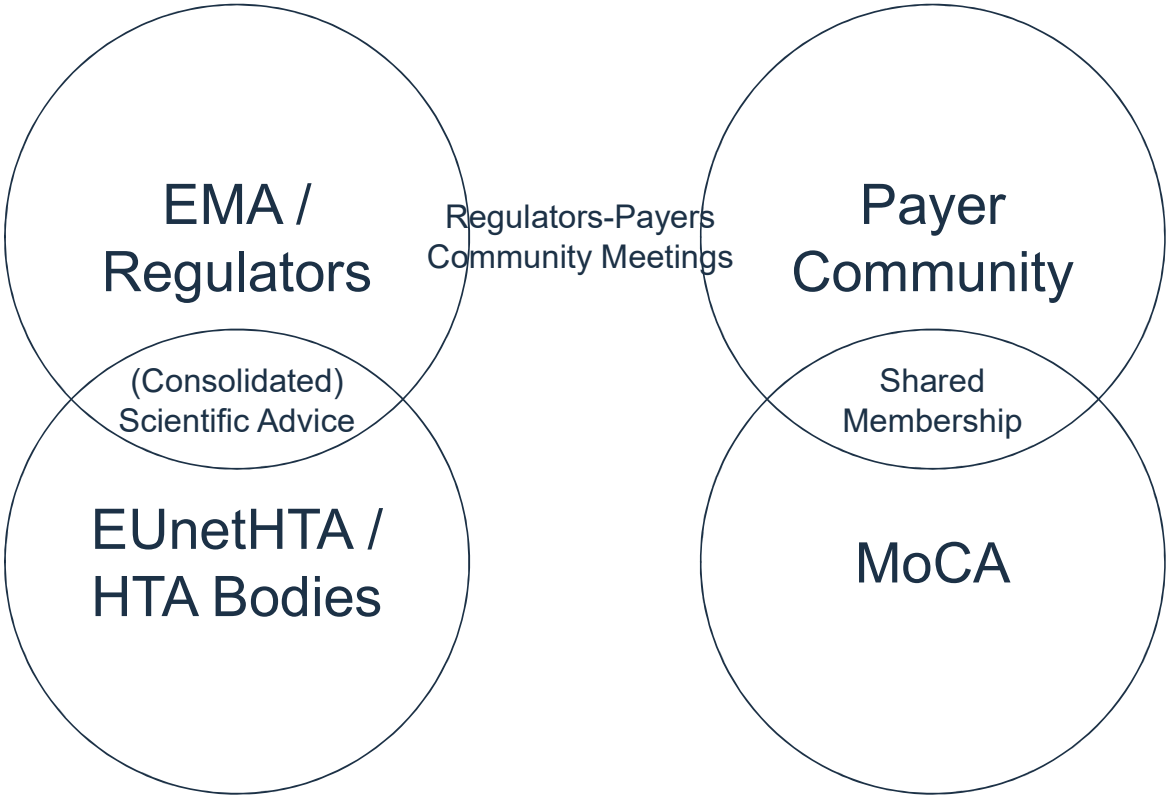
## OPPORTUNITIES FOR IMPACTFUL DIALOGUE BETWEEN REGULATORS & PAYERS

- **Post-authorisation, post-launch (-licensing) Evidence Generation Plans**
  - EMA / Committees requesting Post Authorisation Safety Studies (PASS) and Post-Authorisation Efficacy Studies (PAES)
  - In-country / individual country authorities may request similar information

  ➔ EMA-led: opportunity for more coordinated planning / awareness of contents of PAES / PASS by budget-holders + potential opportunity for MoCA to input?
- **Registries**
  - Contribution of Registries under review – EMA Patient Registry Initiative, consultation

  ➔ Registries often required at national level – collecting similar / same data – how to integrate?
- **Managed Entry Agreements**

# OPPORTUNITIES FOR POTENTIAL COLLABORATIVE (PILOT?) INTERACTIONS TO EXPLORE KEY QUESTIONS OF MUTUAL INTEREST




- EMA-suggested Pilot Project under PRIME to explore opportunities for payer-regulator dialogue
- How could we use opportunity to contribute to learnings?

POTENTIAL TOPICS?

1. Registry – content + link with national requirements
2. Post-Authorisation data generation plans – build on PAES and PASS
3. Biomarkers validation
4. Patient Reported Outcomes instruments

With grateful thanks to the companies
and company representatives
who agreed to participate in the MoCA Pilots

And particularly to those who have kindly agreed to
share their perspectives here – they have made this presentation possible.

Thank You!