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

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EMA-PAYER COMMUNITY MEETING
ZORGINSTITUUT NEDERLAND (ZIN), DIEMEN, NETHERLANDS

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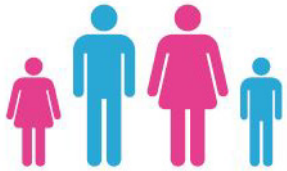
A reminder of what we are facing

OVER
6000
distinct rare diseases

Each one affects fewer than
1 IN
2000
PEOPLE



All together, an estimated
30
MILLION PEOPLE
are living with a rare disease in Europe and
300
MILLION
worldwide

Affects between
6 % AND
8%

of the population in the course of their lives

NO

CURE
for the vast majority of diseases and few treatments available

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RARE DISEASES EUROPE

The core of the matter

Rare Barometer Voices survey on access to treatment
(February 2017 – 1350 respondents)

24% of rare disease patients surveyed could not get the medical treatment they needed in 2016 because the treatment was not available where they live

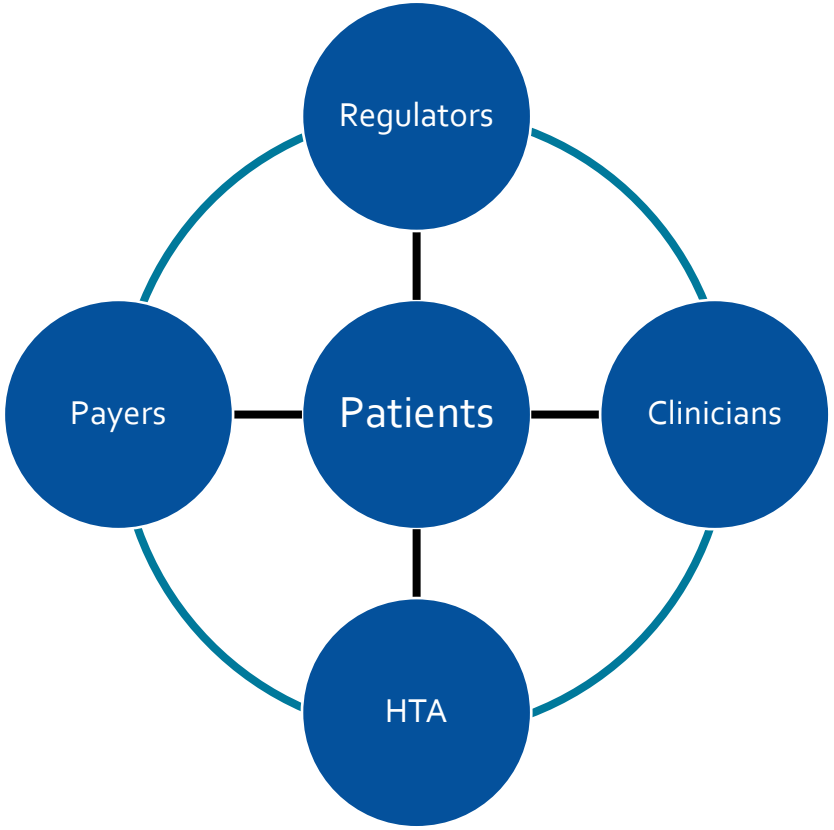
VS. 7% in the general population

(*Preliminary data 2019 – over 7,000 respondents = NO CHANGE*)

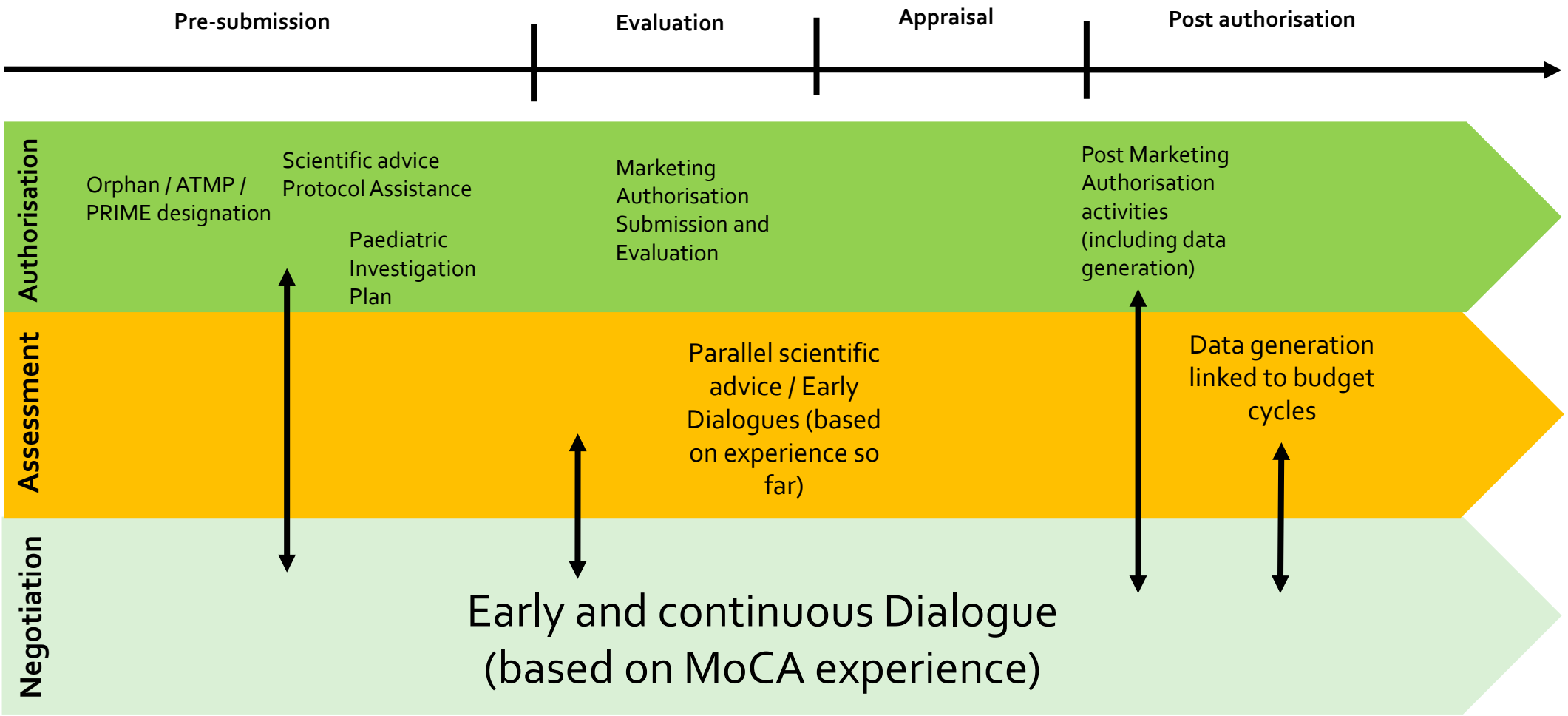
Patients’ perspective in evidence generation

The benefits of patients participation in MoCA for evidence generation planning

- Unique opportunity for patients to present what really brings value and improve health outcomes to payers and manufacturers
- Provide true insights into the real life impact of conditions that are (vastly) unknown
- Ability to point out the necessity of continuity of data gathering from clinical trials to post marketing authorisation commitment
- Contribute to the definition and validation of PCOMs / PROs / ¨PROMs
- Capability of connecting different parts of the system to foster better data gathering



The role of MoCA could be pivotal in determination of value in a multi-stakeholder context



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THANK YOU

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