PATIENT ENGAGEMENT

EMA Scientific Advice and MoCA

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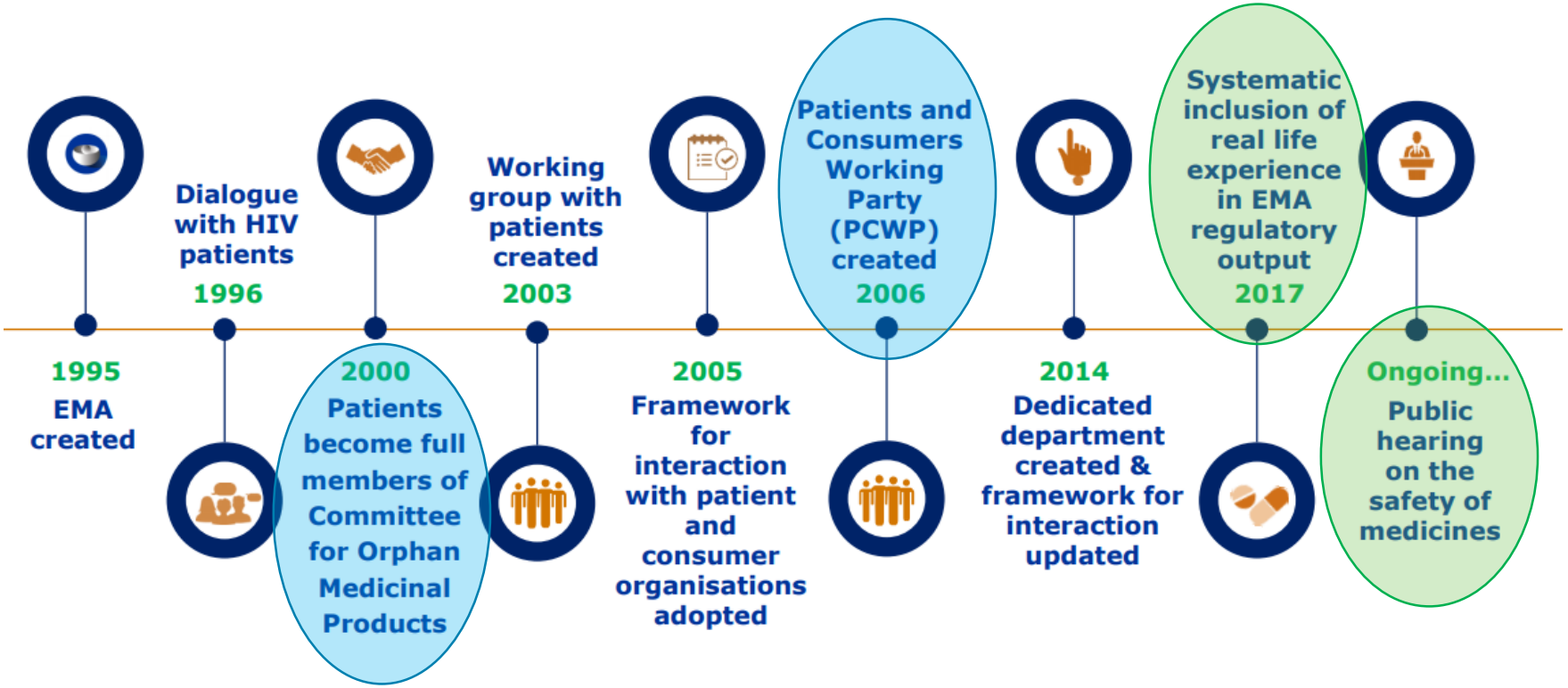
CNA & CEF joint meeting 26 Oct 2017

Interaction with patients at EMA
Key milestones



Representing patients in general

EMA management board
Scientific committees
COMP - CAT- PRAC
PDCO

Representing their own organisation

PCWP
Consultations
Meetings and workshops

Individual experts

Scientific advice
Consultations
Ad hoc expert meetings
Review of documents

Eligible patients and consumers organisations

- Non-profit umbrella organisations encompassing a number of smaller or national organisations
- **Legitimacy:** Statues registered in the EU/EEA
- Clear **mission and objectives**
- Activities: Specific interest in **medicinal products**
- **Representative** of patients and consumers throughout the EU/EEA
- Adequate **structure** and **consultation modalities**
- **Transparency:** Sources of funding should be disclosed to the EMA

Criteria to be fulfilled by patients' and consumers' organisations involved in EMA activities


EGAN
Patients Network for Medical Research and Health
EURORDIS
RARE DISEASES EUROPE
European Liver Patients Association
ELPA
ENFA
European Network of Fibromyalgia Associations
EUROPA UOMO
Pain Alliance Europe
SMA europe
Childhood Cancer International
debra
Working for a life free of pain
EFA
European Federation of Allergy and Airways Diseases Patients' Associations
epha
EPF
European Patients Forum
MPE
Myeloma Patients Europe
Alzheimer Europe
EFCNI
EGA
European Gaucher Alliance
THALASSAEMIA INTERNATIONAL FEDERATION
EIWH
International Diabetes Federation
International Alliance of Patients' Organizations
A global voice for patients
ELF
EUROPEAN LUNG FOUNDATION
emsp
EUROPEAN MULTIPLE SCLEROSIS PLATFORM
GAMIAIN
IPOPI
EATG
European AIDS Treatment Group
BEUC
The European Consumer Organisation
FABRY
International Network
EFNA
European Federation of Neurological Associations
EPDA
ehn
EUROPEAN CANCER PATIENT COALITION
EHC
european haemophilia consortium
IBE
AGE
Platform Europe
hai
EUROPE

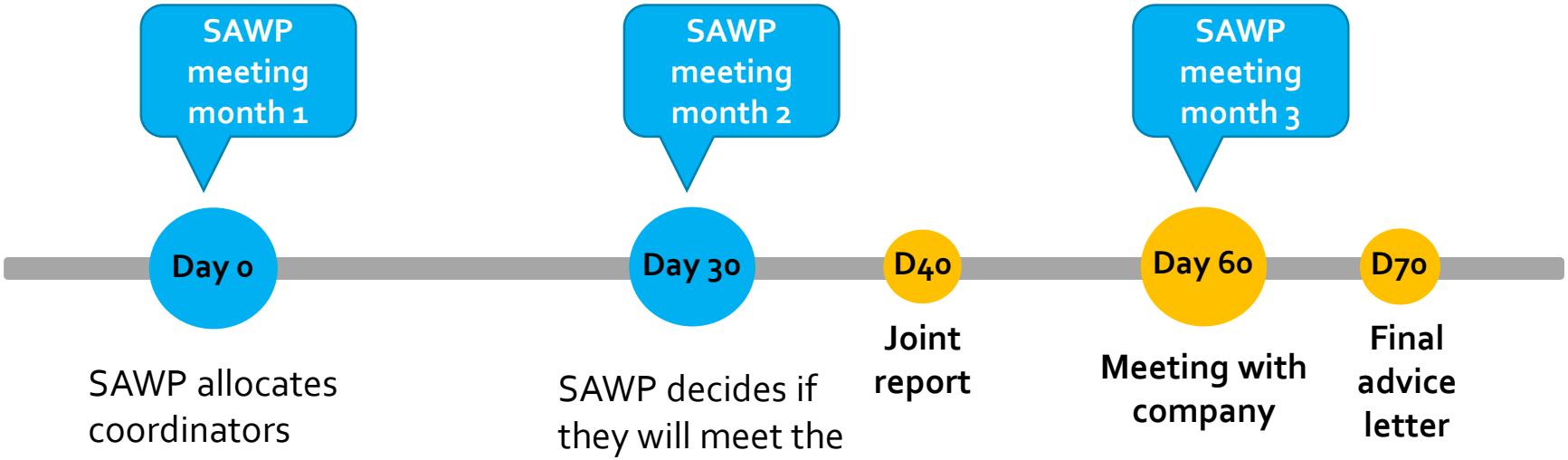
Scientific Advice – Protocol Assistance

- Medicine developers ask the EMA experts for advice on whether they are performing the **right tests and studies** in the development of a medicine
- At any stage of the development
- Scientific advice is **not binding**, but if followed, it is associated with higher success rates during the evaluation of the marketing authorisation application
- **Protocol assistance** is scientific advice related to **orphan medicinal products**

Scientific Advice Working Party

- Established by the CHMP
- Coordinates the **provision of scientific advice and protocol assistance**
- Ensures representation of **experts** in
  - non-clinical safety, pharmacokinetics, methodology and statistics and several therapeutic fields
- Provides advice on:
  - **quality** relating to the development of medicinal products
  - **non-clinical and clinical safety and efficacy**
  - the **significant benefit** of orphan medicinal products.

Timelines and workflow



SAWP allocates coordinators

EURORDIS contacted for dossiers needing patient input

SAWP decides if they will meet the company or will respond in writing

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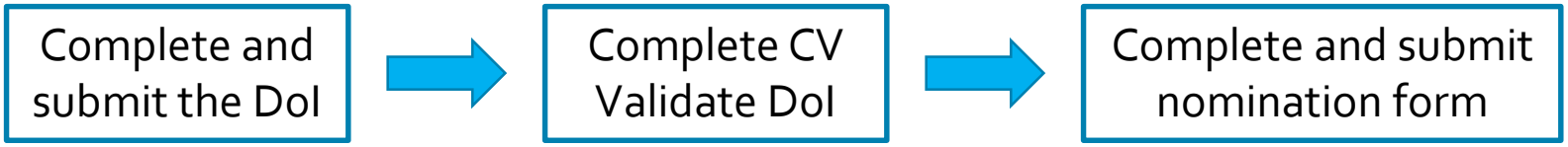
Patient experts

Practical aspects and process

- EURORDIS contacts you
- Your details are shared with the Scientific Advice Secretariat
- After validation of your DOI and CV you will receive the dossier
- A phone call with the product manager at EMA –the person that scientifically coordinates the dossier at the SAWP– can be scheduled to help you focus on the aspects where patient input is most needed
- Don’t hesitate to contact us!

Documents required and process

- Declaration of interest / Confidentiality form
- Curriculum vitae
- Nomination form



Declared interest	Time since declared interest ended (in years)	Scientific committee / Working party[1] expert
Employee (executive role)	Current interest	X
	0 to 3	XC
	> 3	XC
Employee (lead role in development of medicinal product)	Current interest	X
	0 to 3	XP
	> 3	XP
Employee (cross company role other than executive role)	Current interest	X
	0 to 3	XC
Employee (medicinal product involvement other than lead role in development of medicinal product)	Current interest	X
	0 to 3	XP
Consultancy to company (cross medicinal products/general)	Current interest	X
	0 to 3	XC
Consultancy to company (individual medicinal product)	Current interest	X
	0 to 3	XP
Strategic advisory role for company (cross medicinal products/general)	Current interest	X
	0 to 3	XC
Strategic advisory role for company (individual medicinal product)	Current interest	X
	0 to 3	XP
Financial interests	Current interest	X
	0 to 3	F
Principal investigator	Current interest	XP
	0 to 3	XP
Investigator	Current interest	DP
	0 to 3	DP
Grant/other funding to organisation/institution	Current interest	F
	0 to 3	F
Close family member	Current interest	F
	0 to 3	F

EMA POLICY ON HANDLING COMPETING INTERESTS

- **Consultancy – Any interaction with a pharmaceutical company,** excluding open public meetings
- **Strategic advisory role –** where you have voting rights or can influence company decisions
- **Financial interests –** shares, fees paid directly to you by the company
- **Grants or funding by a company**

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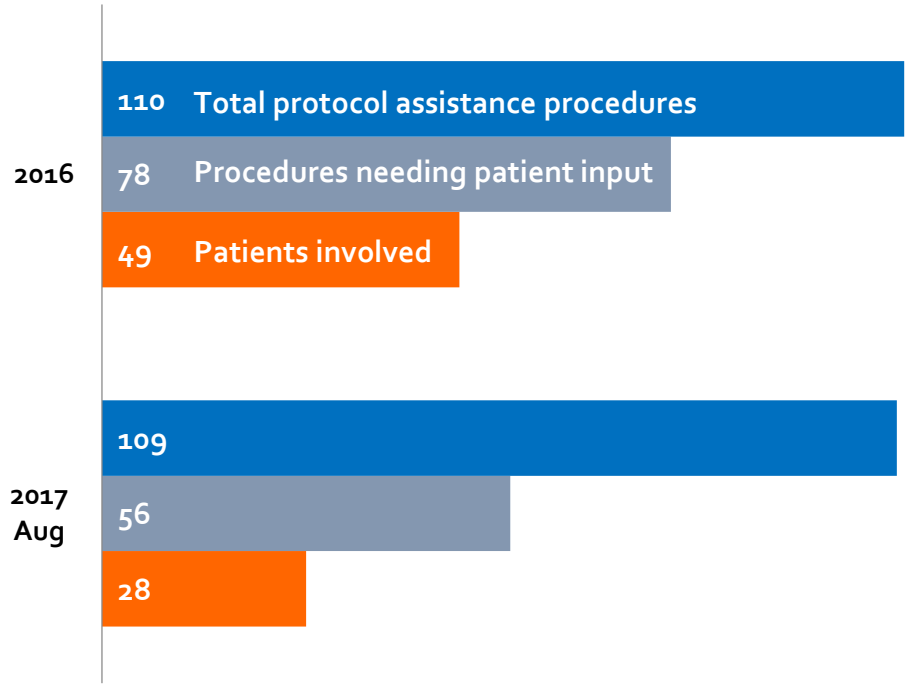
How to contribute

- In writing - shorter timeline (Day 40)
- Discussion meeting (Day 60)
  - Takes place at EMA
  - EMA staff, SAWP experts, company representatives and patient expert
  - You can join also remotely via TC, but higher impact if in person

Patient input on the scientific advice dossier

- Selection of appropriate endpoints
- Defining target population: inclusion/exclusion criteria
- Right comparator
- Study duration, treatment administration, formulation and dosage
- Clinical relevance vs statistical significance
- Identification and assessment of risk potential
- Significant benefit over existing treatments
- Ethical aspects

Patient involvement in figures



63%

50%

Impact of patient involvement

Dear ,

On behalf of EMA and the coordinators, I would like to thank you again for your testimony, which is very moving. This **gives us** additional **reassurance** that **treatment of BPDCN is a high unmet medical need,** and **urges us to make the drug available** as soon as possible to patients in Europe. The final advice letter reflects this spirit.

Kind Regards,

EMA Training resources

'EMA Basics' videos	Related documents
The European Medicines Agency	Presentation - The European Medicines Agency
The centralised procedure	Presentation - The centralised procedure
Involvement of patients	Presentation - Involvement of patients
The Patients' and Consumers' Working Party	Presentation - The Patients' and Consumers' Working Party
EMA video for patient representatives	Involvement of patient representatives in scientific advice procedures at EMA Involvement of patient representatives in scientific advisory groups at EMA
Pharmacovigilance	Presentation - Pharmacovigilance
How EMA works with healthcare professionals	Presentation - How EMA works with healthcare professionals
Scientific advice: what to expect and how to prepare	Presentation - Scientific advice: what to expect and how to prepare
Declarations of interests: a practical guide	Presentation - Declarations of interests: a practical guide
How patients are involved in the review of documents	Presentation - How patients are involved in the review of documents
What is a European safety referral	Presentation - What is a European safety referral

Patient engagement

MoCA

Mechanisms of Coordinated Access to Orphan Medicinal Products (MoCA)

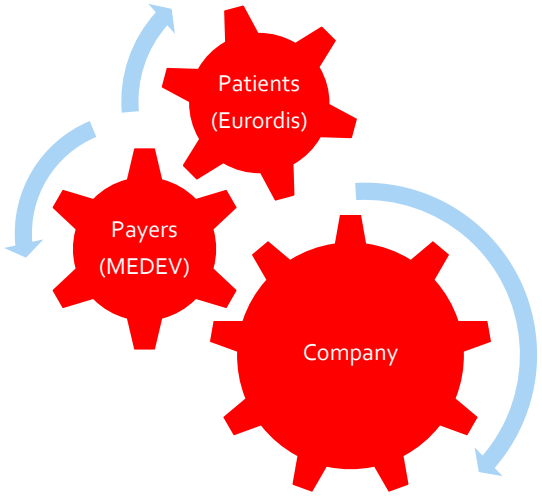
- High uncertainty around products for small populations
- Fear of high price and high budget impact
- Fragmented EU market – decisions on Pricing and Reimbursement at National level
- The solution – ***collaborative approach between different Member States***

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

- **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

REGULATORY TOOLS		OTHER TOOLS
TO ENHANCE DEVELOPMENT SUCCESS	**TO FACILITATE EARLY ACCESS**	**TO AVOID FAILURE AT PRICING & REIMBURSEMENT**
Scientific advice / protocol assistance	Conditional approval	**MoCA**
EMA-HTA parallel scientific advice	Accelerated assessment	EUNetHTA (methodology)
	Compassionate use	EMA-EunetHTA parallel consultation

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!

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Benefits of MoCA pilots

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

Example of a pilot

- Early dialogue on **a targeted gene therapy** for **a very small population** (~ 10,000 patients in Europe)
- **Very complex therapy** (80 days min for all treatment steps + 6 months of active follow-up)
- **Almost impossible to set up a Europe-wide network to serve all Member States** – treatment will be limited to **a few selected centers of excellence** across Europe
- If all European patients are to have access to treatment, huge implications in terms of:
    - enabling genuine **cross-border patient mobility,**
    - obtaining **administrative pre-authorisations** for treatment
    - securing **national payers' acceptance of need for treatment and its price**

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

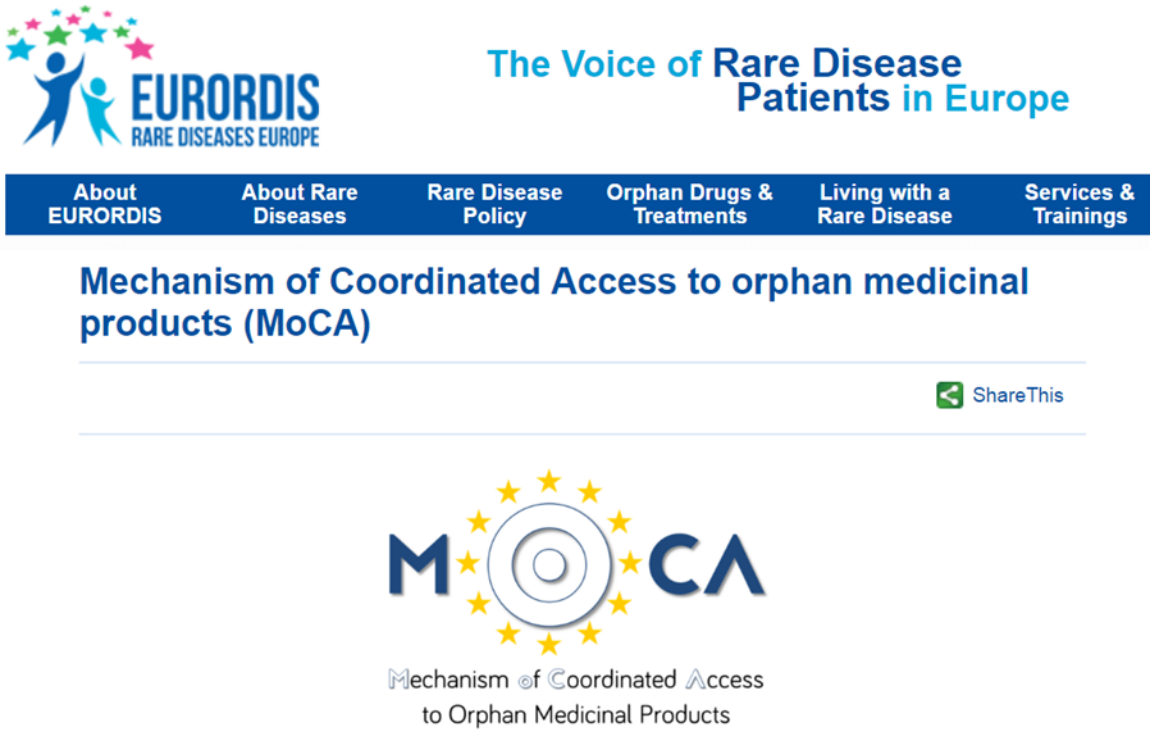
Patient contribution

- Patient involvement in MoCA is essential to bring the patients' voice to the table as legitimate experts on:
  - The disease they are suffering from
  - The disease's impact on their daily lives
  - The solutions offered by available medicines
  - The unmet needs that new treatments should aim to fill
  - The impact of the therapy in real-life
  - Patient-relevant outcome measures
  - How patients will be affected if the medicine is only accessible in some countries and not others

Practical aspects

- Invitation by EURORDIS approximately 1 month before the meeting
- Declaration of interest and confidentiality agreement need to be signed
- Pre-reads from company and agenda distributed
- Meetings usually take place in Brussels at the European Social Insurance Platform offices
- Meetings last **1** hour (exploring remote attendance)

For more information



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Thank you!

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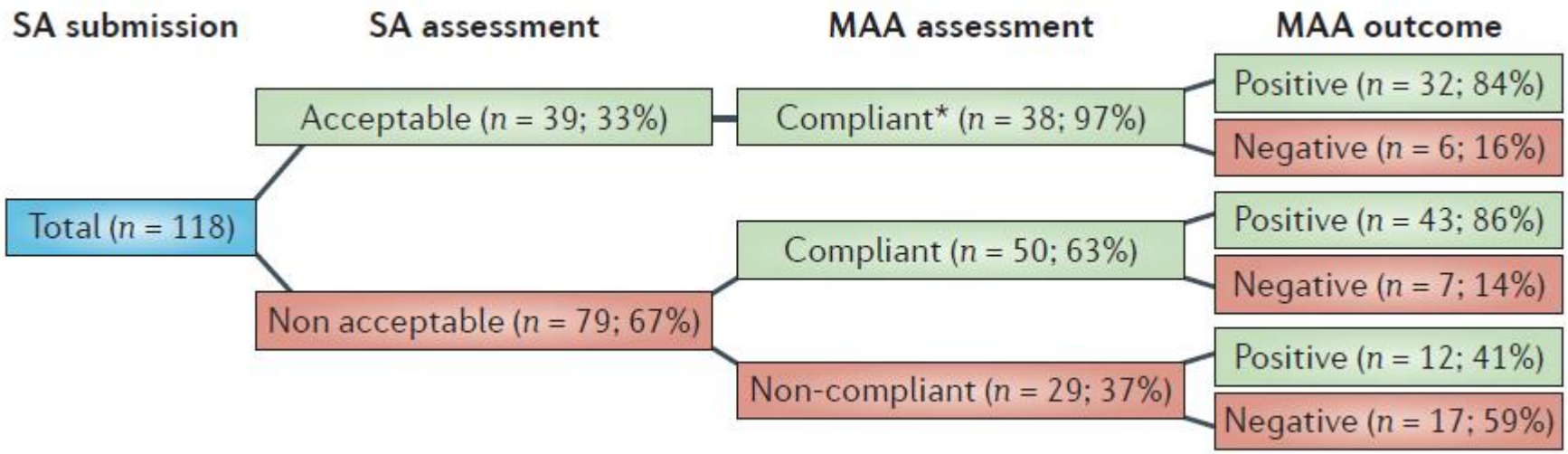
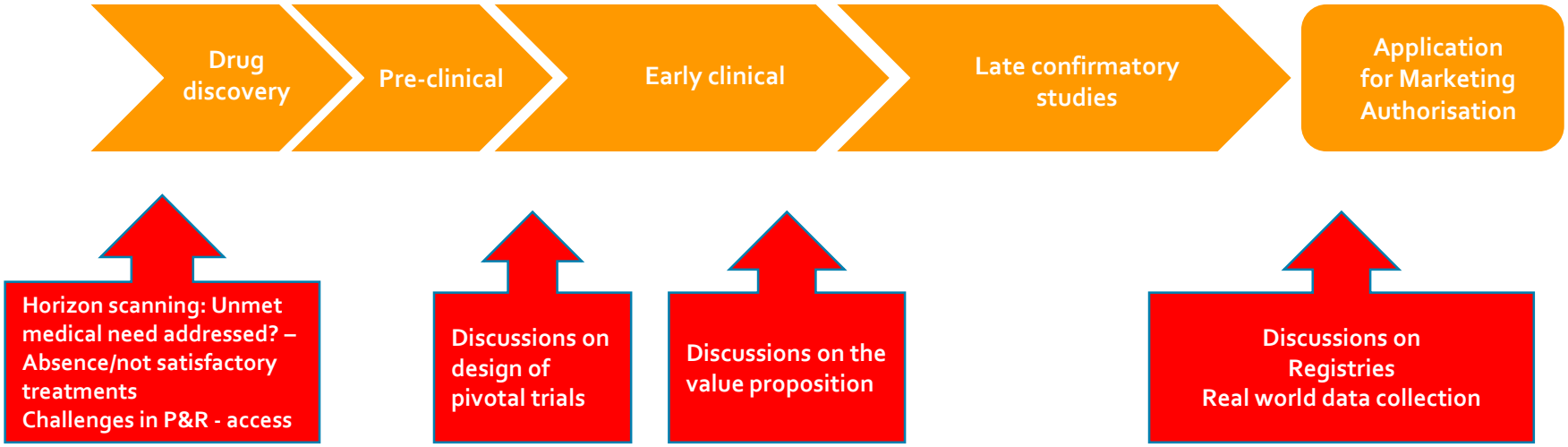
Back-up slides

Impact of scientific advice



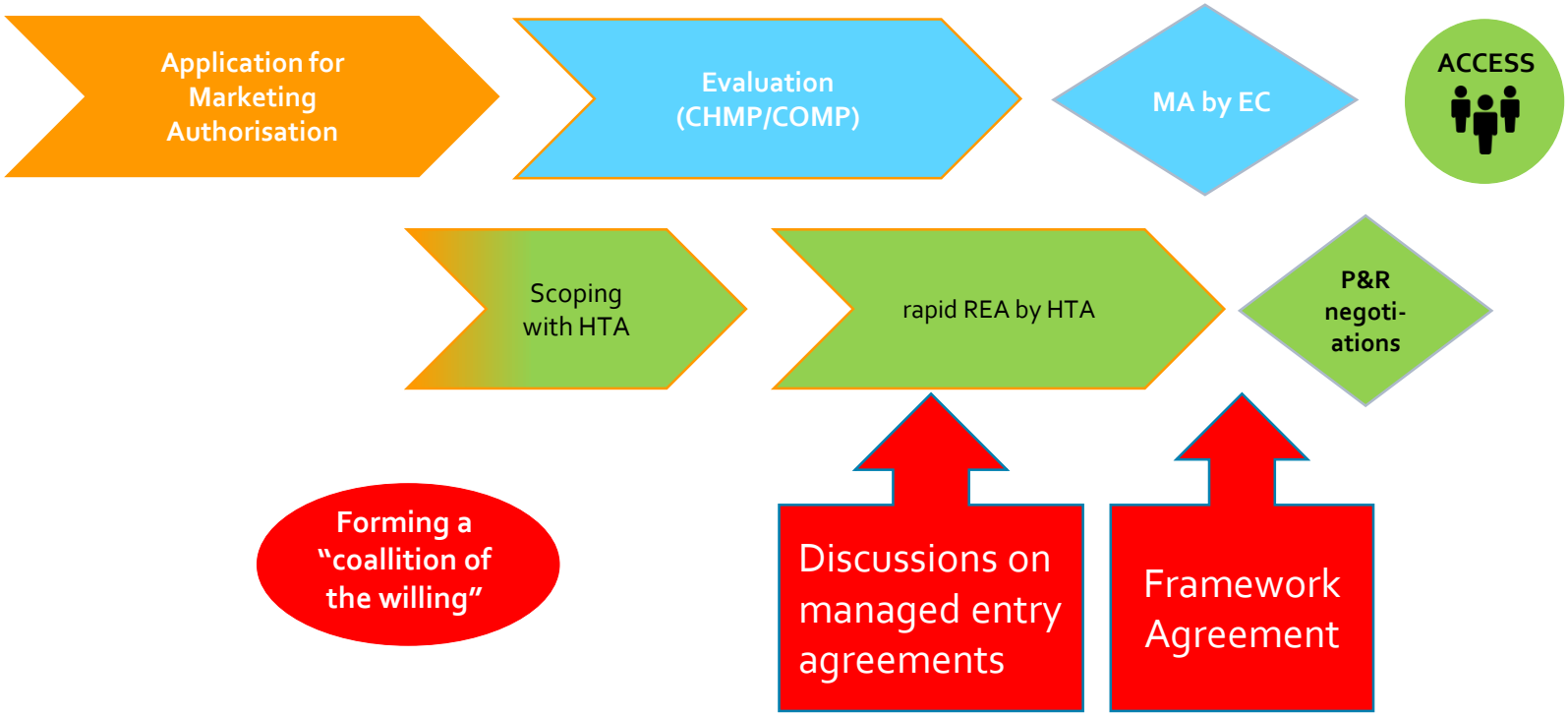
Clinical trial programmes compliant with SA recommendations were associated with higher chance of having a positive CHMP opinion

PRE- APPROVAL



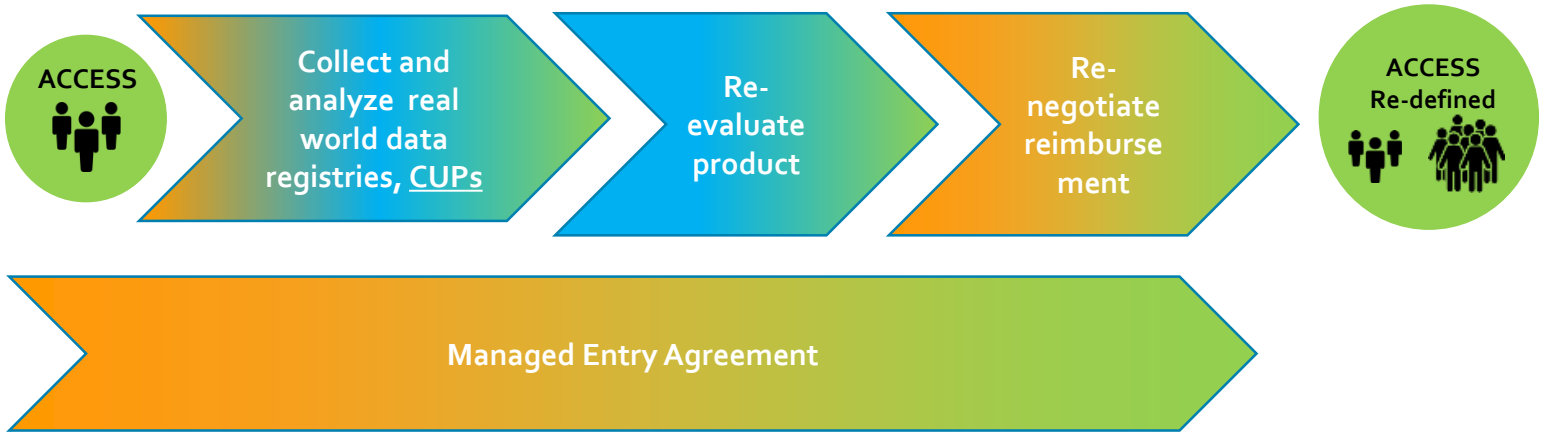
By participating in MoCA, companies can integrate **additional input from patients' and payers'** perspectives at any stage of product development

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



MoCA input for data analysis –PM data collection

MoCA input renegotiation

"Coalition of the willing" redefined