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# REGULATORY BENEFITS OF EARLY DIALOGUE WITH PATIENTS – PROTOCOL ASSISTANCE

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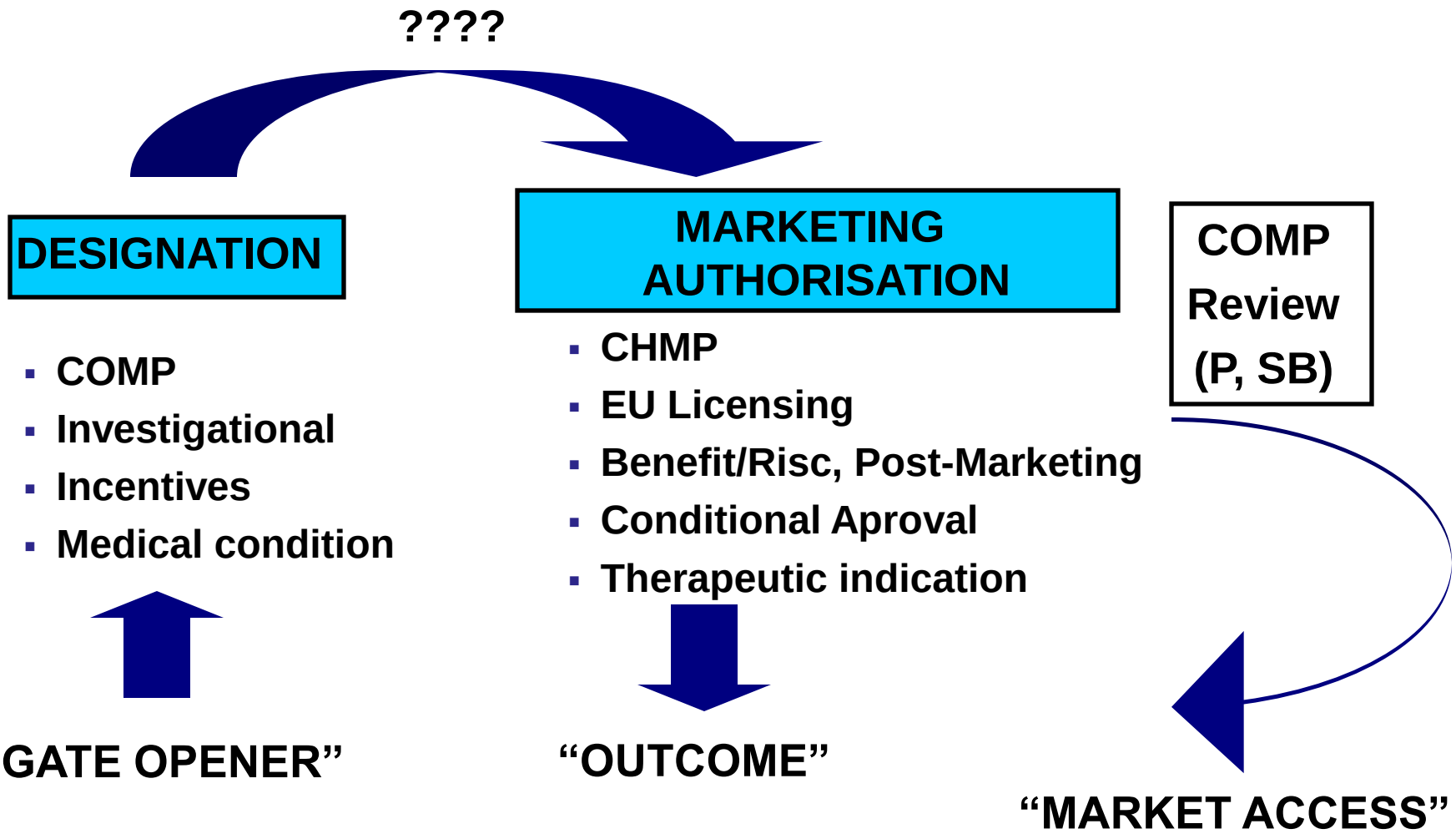
DIA
Drug Information Association

# DESIGNATION – MARKETING AUTHORISATION



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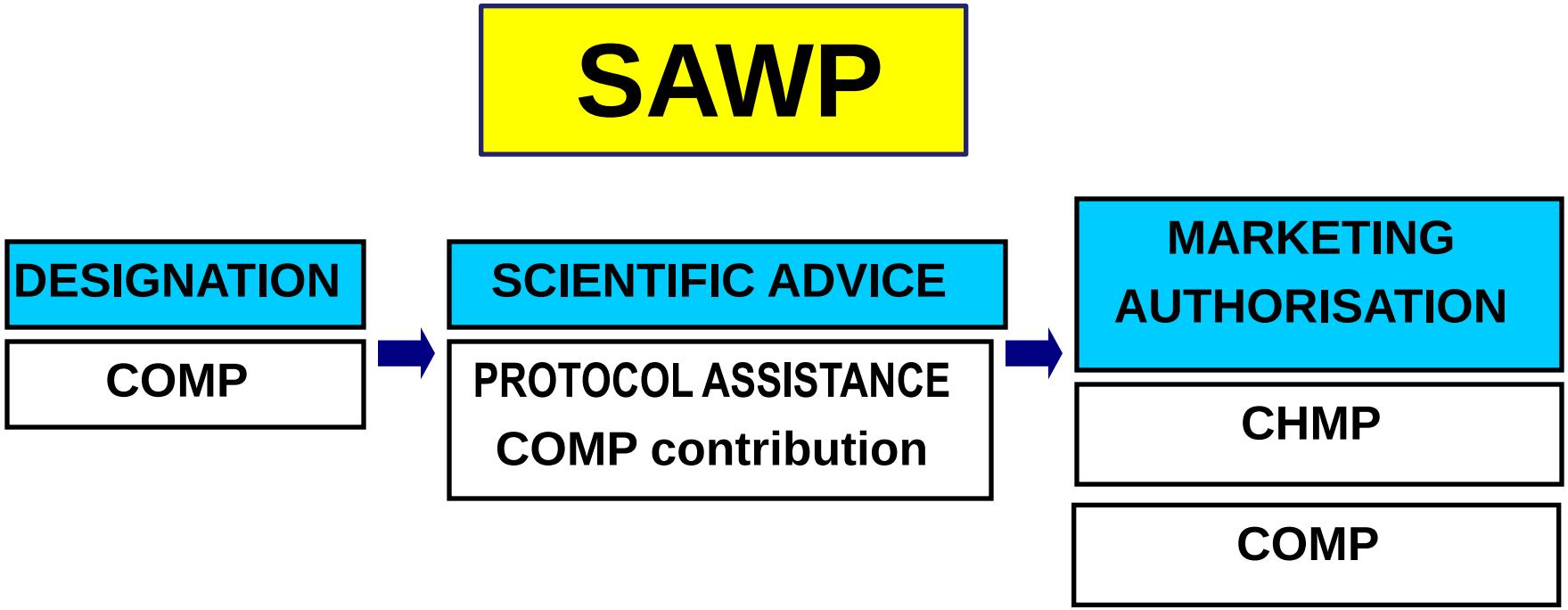
# BRIDGING DESIGNATION AND M.A.



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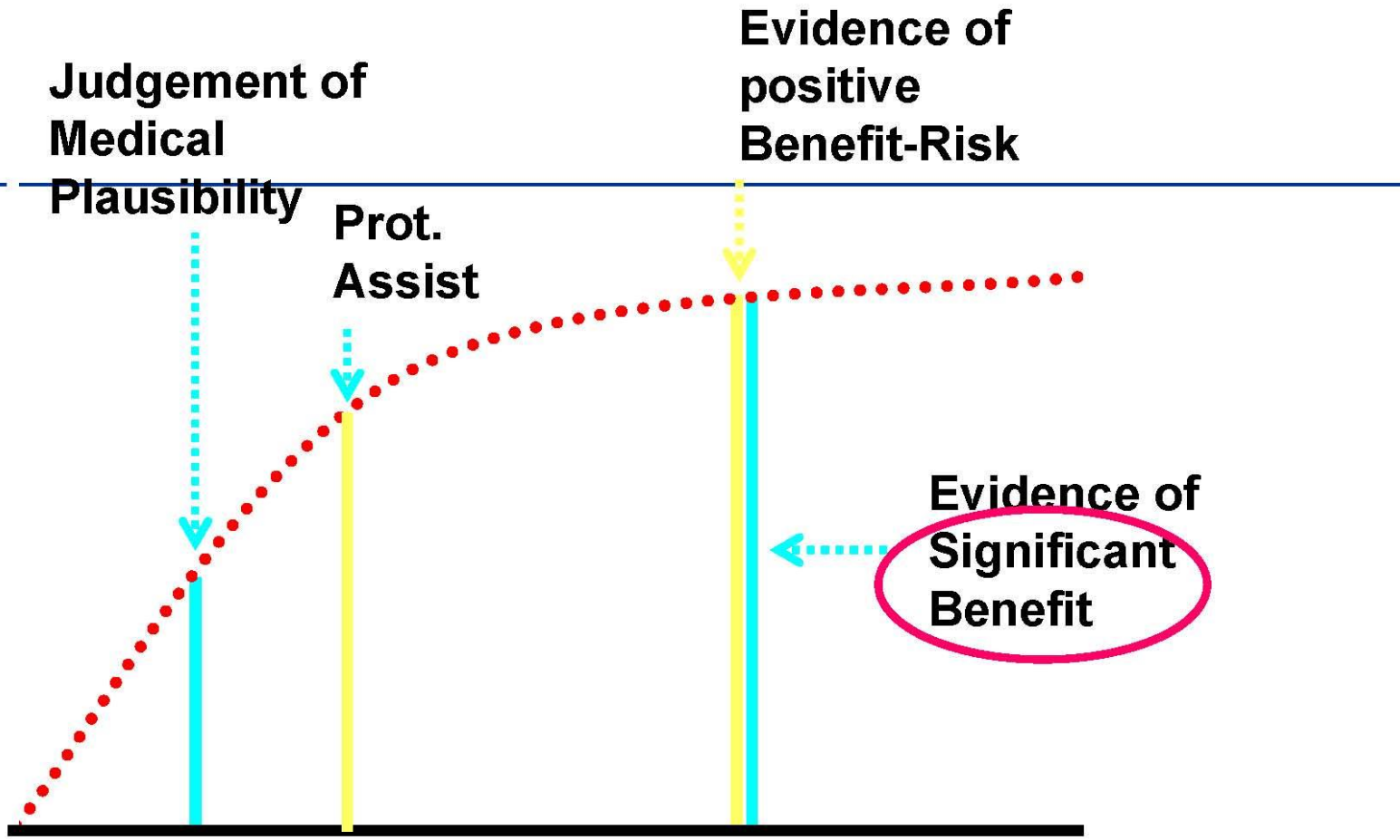
# COMP and CHMP roles



# SCIENTIFIC ADVICE / PROTOCOL ASSISTANCE

- **Based on current Pharmaceutical Legislation**
- **EMA gives a Pan – European advice for centralised applications and orphan medicines**
- **National competent authorisation (Networking with EMA)**

**Is a "tool" that brings together assessors, experts including voiced-patients to give the best recommendations to sponsors in optimizing drug development to meet the standards for marketing authorization**

**SAWP/EMA multidisciplinary , 90-day procedure**

**(2 coordinators appointed, Oral Hearing)**



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# WHY SCIENTIFIC ADVICE / P.A. IS NEEDED IN R.D.?

- **Limited public awareness (“Invisible diseases”)**
- **Scarcity of Clinical Experts and Reference Centres**
- **Delays on Diagnosis (Genetic Testing)**
- **Small sized population**
- **Geographic dispersion**
- **Life-threatening /chronic debilitating conditions**
- **Heterogeneous conditions**
- **Difficult to stratify by stage/severity**
- **Limited available treatments**

**Understanding the rarity paradigm**



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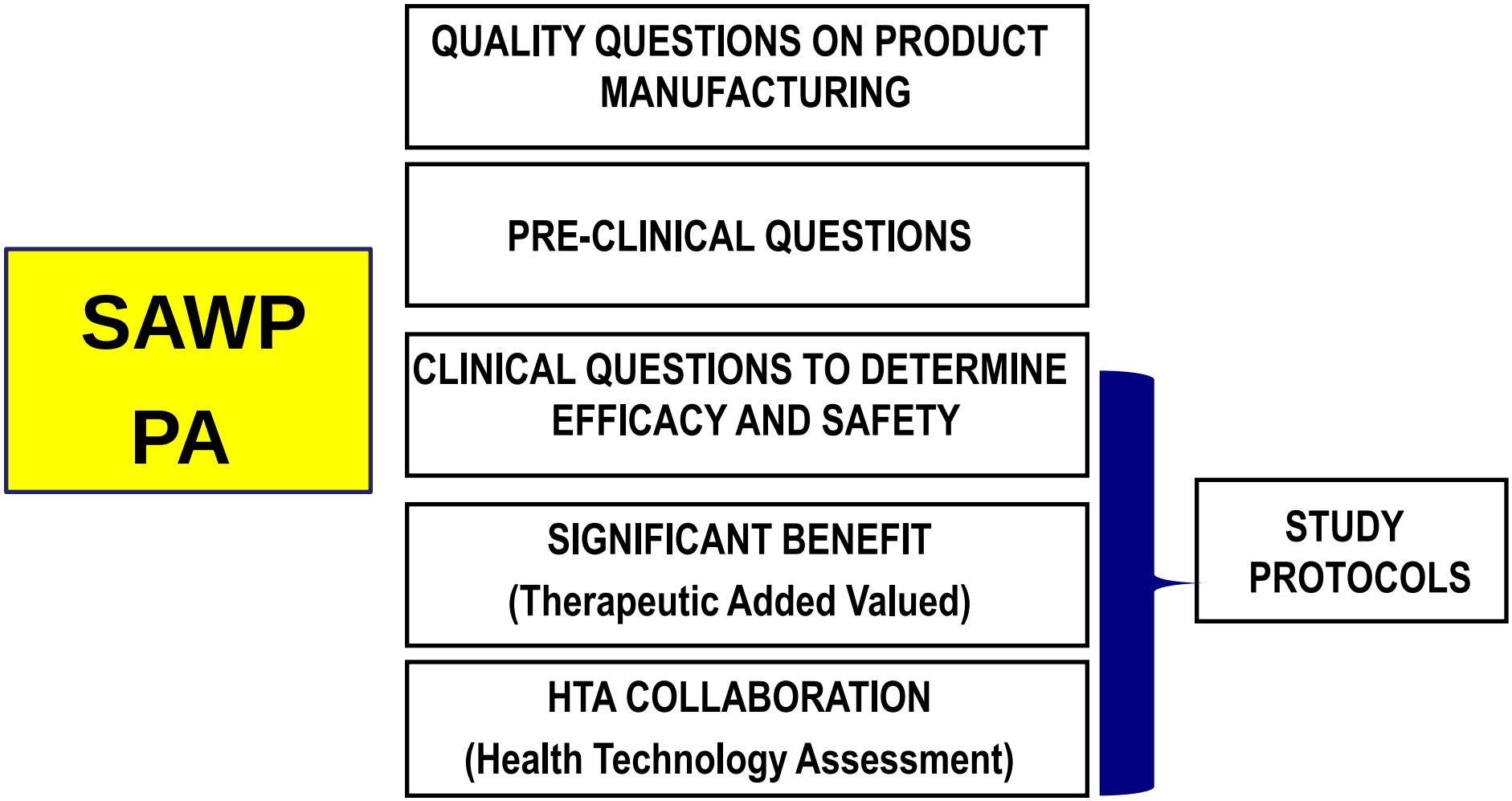
# WHY SCIENTIFIC ADVICE / P.A. IS NEEDED IN R.D.?

- **Limited validated biomarkers and surrogate endpoints**
- **Limited predictive/validated preclinical models**
- **Ethical concerns on the use of placebo and vulnerable population (e.g. emerging therapies)**
- **Off-label use (mostly medicines for children)**
- **More support to health professionals/investigators**
- **Limited information to “care-givers”/ relatives**
- **Excessive bureaucratic/administrative barriers**

**Participative role of patients to be increased**

# SCIENTIFIC ADVICE AND PROTOCOL ASSISTANCE



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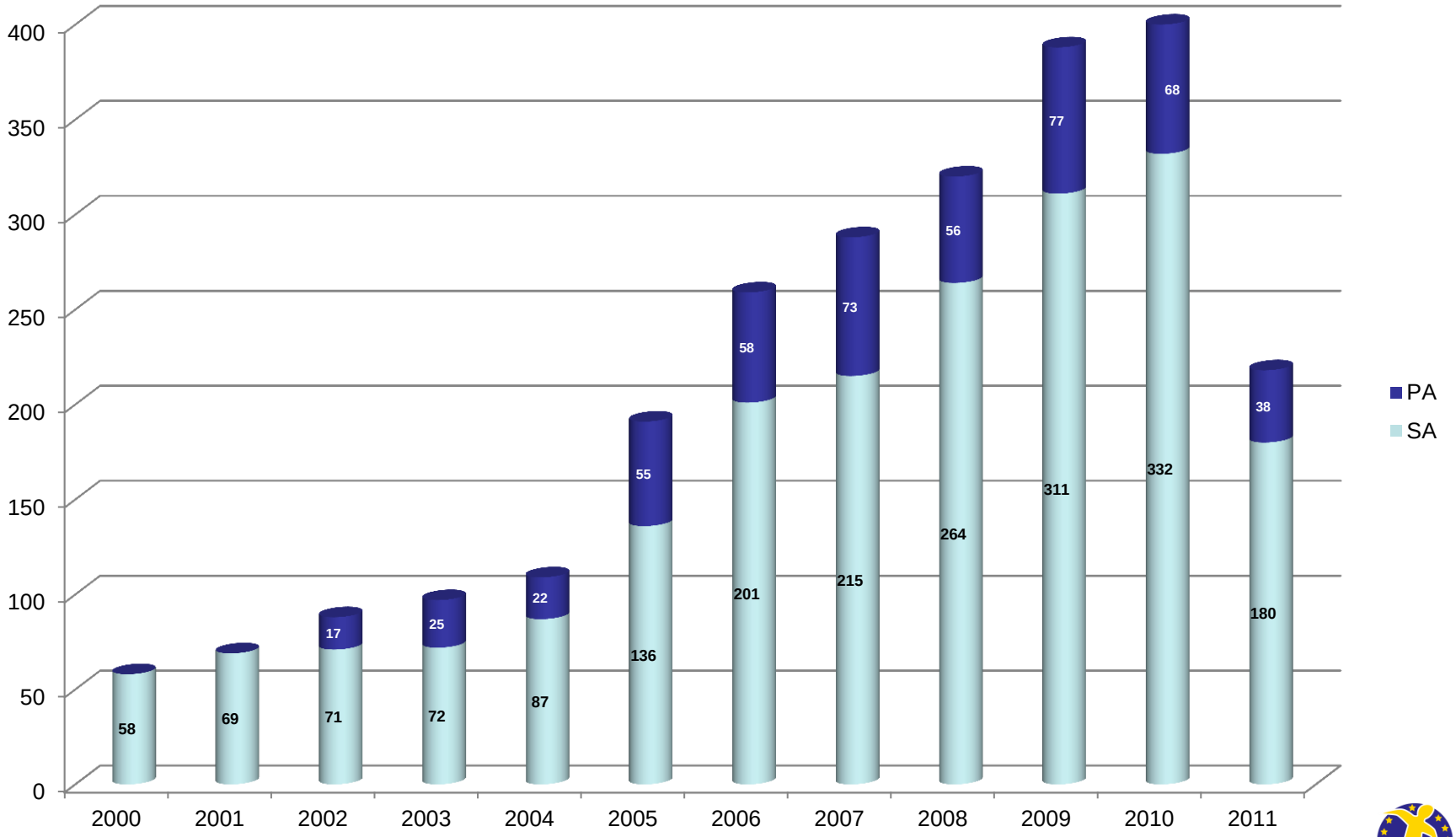
# CLINICAL QUESTIONS

- Methodological and study-design
- Selecting the appropriate end-points:
  - Hard /Soft end-points
  - Intermediate variables (subrogates)
  - Biomarkers for diagnostic, disease progression and therapeutic response
- Defining the target population: inclusion/ exclusion criteria
- Choosing the right comparator: placebo, standard of care and active treatment when available

# CLINICAL QUESTIONS II

- Study duration, treatment modalities and possology
- Clinical relevance versus statistical significance
- Identifying,  collecting and assessing risk potential
- Significant benefit (added-value) over existing therapies
- Logistics and managerial aspects of the trial
- Role of voiced-patients in following-up the study
- Ethical aspects: Informed consent, GCP compliance
- Study feasibility....

# NEW FRAMEWORK FOR SA& PA: the weight of success



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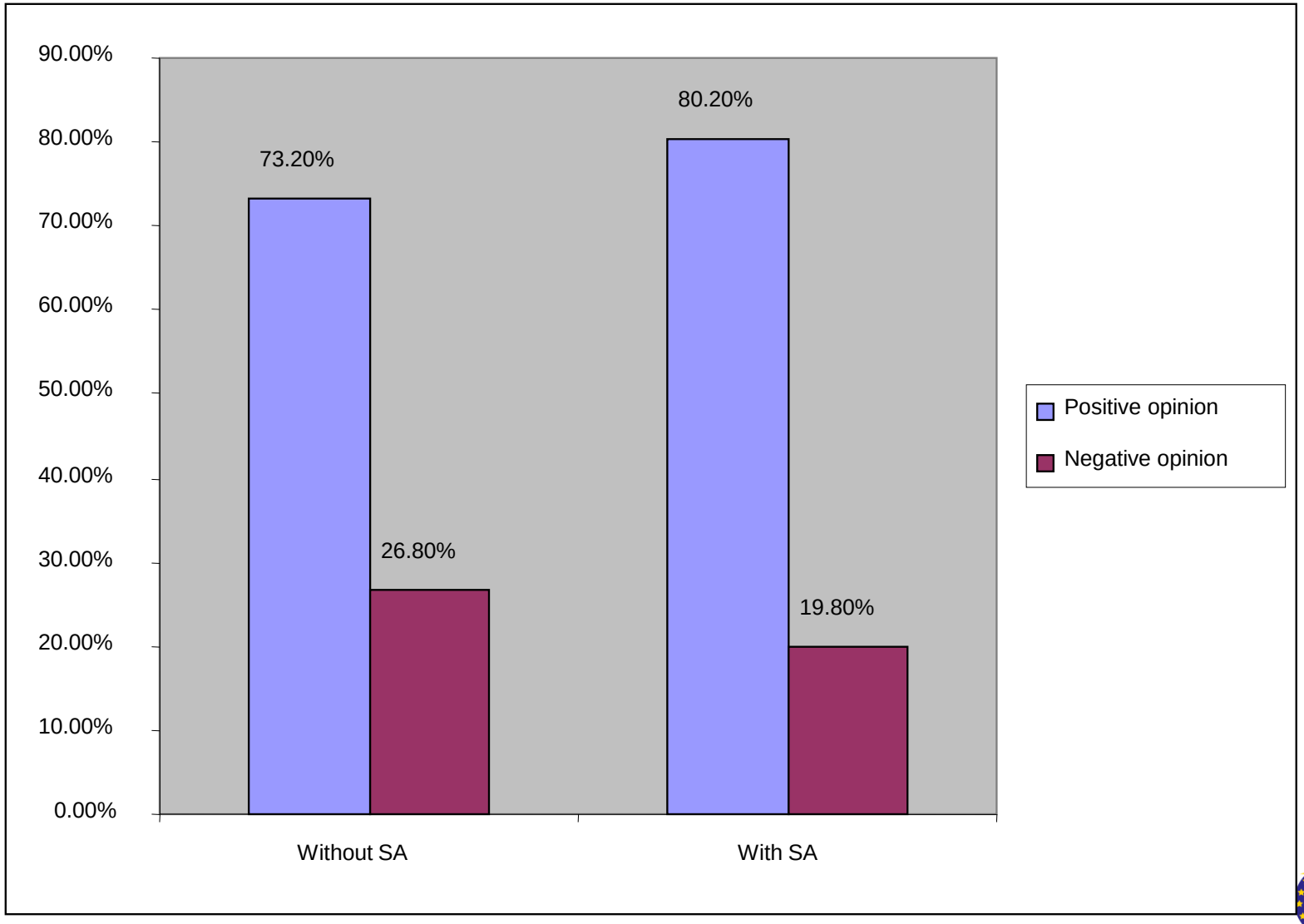
# IMPACT OF PRIOR SCIENTIFIC ADVICE IN ALL MAA WITH AN OUTCOME



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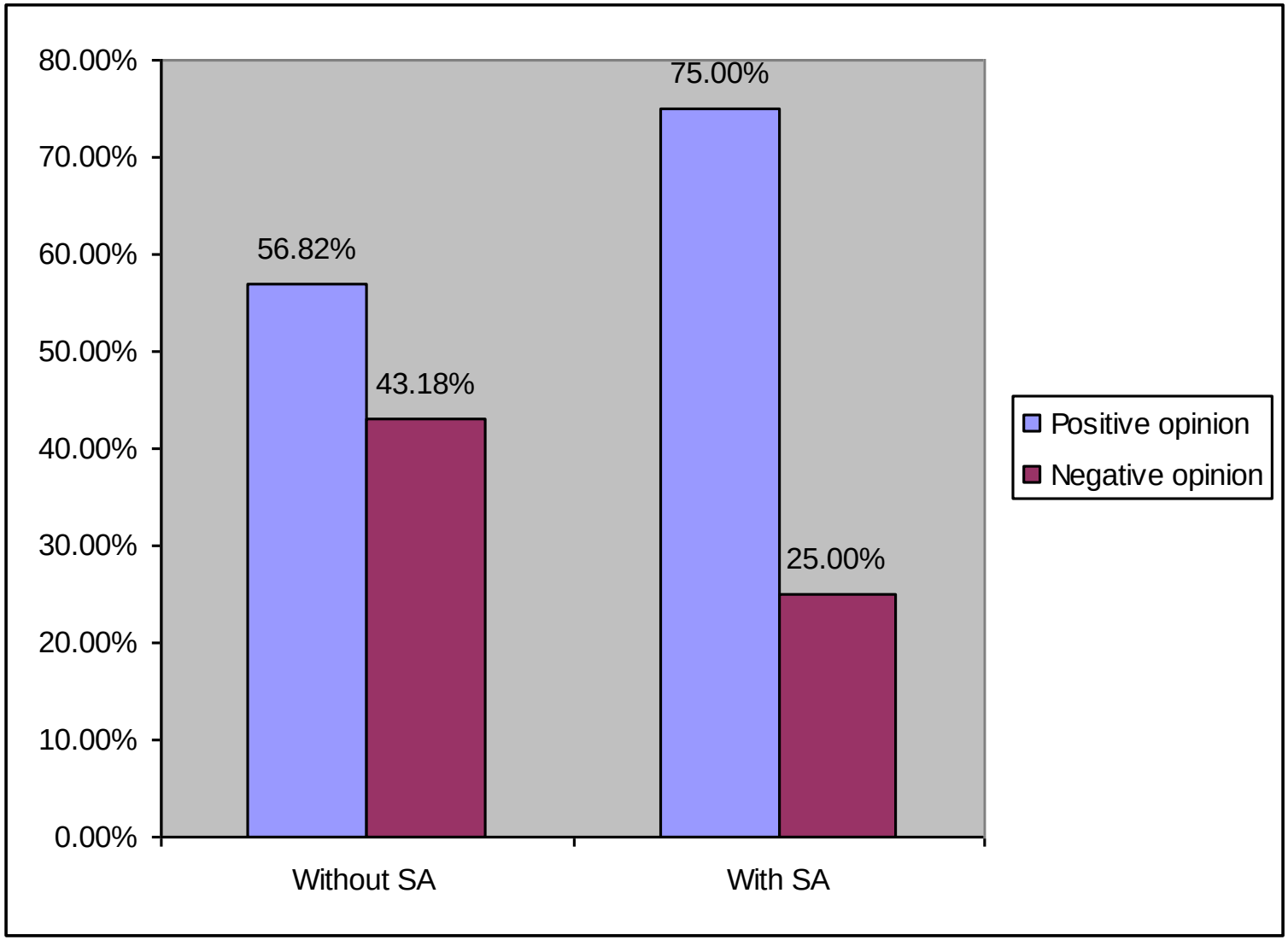
# IMPACT OF PRIOR SCIENTIFIC ADVICE ON ORPHAN MAA



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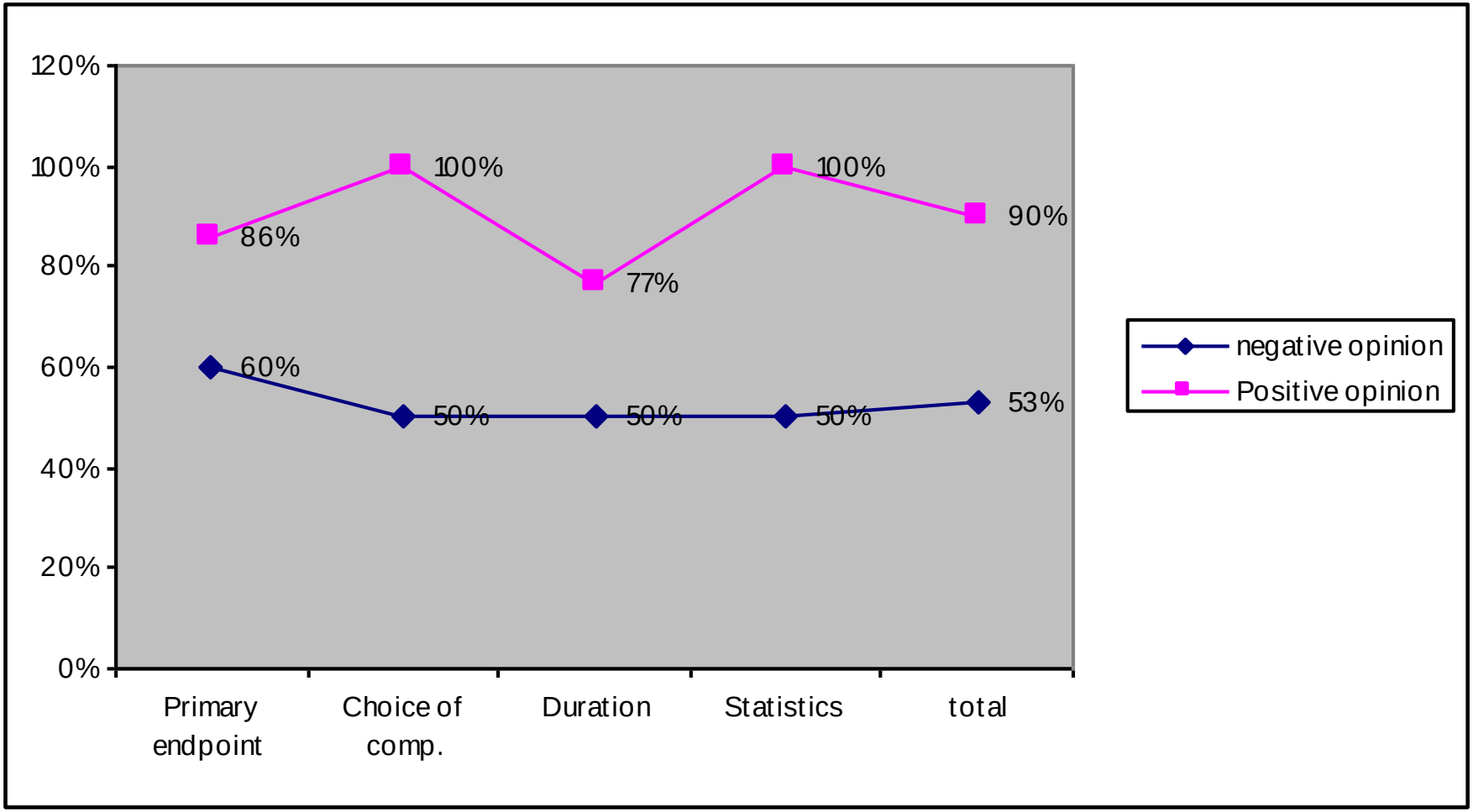
# ADHERENCE TO SCIENTIFIC ADVICE IN MAA WITH POSITIVE / NEGATIVE OUTCOME



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# Patients' Organisations involvement

- **According to EMA:**
  - 2008: patient representatives in 8/56 PA procedures (14%)
  - 2009: 13/77 (17%)
  - 2010: 18/62 (29%)
  - 2011: 5/33 (15%) (to date)

**Patient representative expert feedback highly regarded**

**Eurordis support and contribution to finding patient representatives commendable!**



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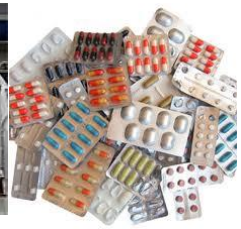
# A PATIENT-CENTERED DIALOGUE – COMMUNICATION AMONG RELEVANT PARTNERS

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

**Patient**





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