EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Breakout session 1

Principles and Policy

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Introduction/ start

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- All stakeholders have a primary interest in promoting public health. Rapid development and launch of safe and effective medicines that utilise the latest scientific knowledge is key to this.
- Notwithstanding the different remits for different stakeholders, a collaborative and harmonised approach (based on scientific dialogue/advice) should enable drug development to be as efficient as

Is this a common vision shared by all parties?

Agreed

BUT

Continuum of evidence. How much evidence is enough?

Questions and trade offs differ from the viewpoint

Value of predictability. We should aspire for added value in research, and for this to be rewarded financially.

Lifecycle management of innovation

Consensus and Compromise

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Sponsors will usually undertake a single (global) development programme.

The advice process should include discussion of options for this development, with pros and cons clearly articulated from each stakeholder involved.

For maximum impact the process should also enable a consensus to be reached, likely requiring compromise from some parties in the event of disagreement.

Experience suggests discussion is limited and compromise/changes of position are rare.

Do you agree? How should discussion be encouraged? Could agencies compromise to achieve consensus?

Consensus and Compromise

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Do you agree? How should discussion be encouraged? Could agencies compromise to achieve consensus?

Consensus would be ideal

Clarity on differences and reasons why:

- Legislative framework (eg for comparator)
- Acceptable level of compromise/methodology (eg indirect comparison)

Methods/best practices are expanding, but policy should be more explicit in addressing methods (clarity)

Development of guidelines is a good move in the right direction

More experience gained will be beneficial

Different formulas for different requirements (?) (orphan, SME, disease area….)

Who should give advice?

- Different models exist today (dictated by Law/Agency structure):
  1. Experts providing the advice are NOT the final decision makers and the advice may or may not be shared with the final decision maker
  2. Final decision makers give or approve the advice

- There are pros and cons:
  - Are there conflicts of interest for decision makers?
  - Continuation of the collaborative approach
  - Efficiency: knowledge and expertise of disease
  - Predictability of decisions, based on advice
  - The model influences extent to which advice could be binding

What are the advantages and disadvantages from your point of view? Which approach do you prefer?

Who should give advice?

What are the advantages and disadvantages from your point of view? Which approach do you prefer?

- There are multiple players at MS level, sometimes with a different level of relevance and decision making power
- Is the person giving advice the **final decision maker?**
- Could Industry make suggestions on who to involve? Could this help to gain **access** to decision-making parties that have participated to the process only remotely so far?
- Explicit, clear **methods and policies** ⇨ **transparency** and **knowledgeable**
- Hospitals, EU policymakers, WHO and other **proxies** involved
- Decentralised products: which route to take for a consultation?
- Size of HTA agency might prevent expert separation for advice/appraisal

"Morally Binding" Advice

- Whilst not legally binding, Regulatory SA is given in good faith and future assessments should reflect previous advice unless the advice is no longer relevant due to scientific or medical advance
- Regulatory SA is a detailed written report. HTA advice has been given in different formats: reports, corrected company minutes etc.

Can and should parallel advice be "Morally Binding" in a similar way? Is this feasible? What if there is no consensus? How should advice be recorded? How should it be used in assessments?

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The journey to regulatory SA to morally binding has taken time but has worked

We need to have a vision for longer term on how we want the system to look, including **patient/physician** input. Advice more linked to market access (?) + harmonised EU advice (?)

Given complexity of system and actors it seems very difficult. The **decision and policy makers** should be involved.

Empowerment is a necessary condition and not fulfilled at present

Individual advices should be clearly **recorded**.

Clear **understanding** of national **differences** is valuable too.