Closing Remarks

- AP is a scientific concept of medicine development and evidence generation intended for medicines that address unmet needs.

- It is <u>not</u> a new regulatory route for medicines approval. It aims to facilitate best use of existing tools through multistakeholder dialogue, including patients.

Closing Remarks

- Lifecycle management and further data acquisition is nothing new.

- Can we use all data sources better to increase the knowledge on the benefits and the risks in the shortest possible timeframe

Closing Remarks

- Focus is on evaluating the benefit/risk in a restricted population where unmet need is highest

- All regulatory decisions are based on evidence. Standards do not change.   A targeted subgroup based on high unmet need might permit use of Conditional MA with commitment for provision of additional data.

RCT/Observational/ real world data

- Sources of data exist, but are diverse and it is *key* to understand the strengths and limitations of them further

- This is not new. We have been using Real World Data in different aspects of regulatory decision making for some time, exceptionally to make decisions on efficacy and BR.

- Prospective planning is key, understanding how the approach will unfold over the life-cycle, and planning for an iterative decision making.

RCT/Observational/ real world data

- SLS: 8 years work, huge undertaking, a different way of looking at clinical practice. But SLS is a randomised trial in a very big population. Answers the question of most relevance for the clinician, HTA bodies also liked the design.

- Dialogue is not trivial. Needs competence, capacity and experience.

- Drug development evolves but is underpinned by RCTs. When moving away from the RCT paradigm, important to remember the benefits of each part of R, C and T