Session 2: Evidence generation to advance regulatory excellence, here and now

Changes in expectations associated with real-world evidence, as there is an increasing reliance on accelerated clinical development strategies and amplification of uncertainties regarding a product's effectiveness at the time of registration!

1. **Consider the generation of real-life data together with the clinical development plan.** Need for reinforcement of international collaborations on PLEG, including between regulators and HTA bodies e.g. joint scientific consultations introduced by the EU regulation on HTA will start in 2025.

2. **Use existing data sources.** Need for development of good quality disease registries / cohorts, which collect data on the efficacy and safety of treatments, including results of PROs.

3. **Provide comparative studies with a complex methodology**, to maximise the use of the results (e.g. effectiveness studies with external control arms or comparative studies using an observational dataset, use of causal inference methods and target-trial emulation approach are strongly recommended).

4. **Document representativity** of the results in terms of patients' and prescribers' characteristics.

5. **Share the results** to prescribers and patients and facilitate the reuse for research.