# Surrogate endpoints - can EMA and EU HTA align on common standards?

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8th EFSPI Workshop on Regulatory Statistics 2023
*Short topic presentation*

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# Background

- Surrogate endpoints are much more common in regulatory decision making than in HTA where there is a clear preference for final, patient-relevant endpoints (e.g. overall survival)
- Part of this is due to differences in decision context, but also due to HTA bodies' more conservative view on surrogate endpoint validity. E.g.
  - EUnetHTA21 D4.4: *'use of surrogate outcomes […] can be controversial since the validity of surrogate outcomes has rarely been fully established in a rigorous manner'*
- Once a surrogate endpoint is accepted by key regulators such as EMA and FDA, it is unlikely that pivotal trials will collect long-term comparative data on final endpoints
- Lack of alignment regarding evidence requirements for surrogate acceptance/validity may delay patient access to innovative therapies
- The issue is particularly relevant for EU HTA where oncology drugs and ATMPs (where surrogate endpoints are common) will be subject to Joint Clinical Assessment already in 2025

# Questions

1. What is needed to achieve pragmatic standards for the use of surrogate endpoints that are helpful for all stakeholders and viable in practice? (algorithmic rules not acknowledging context specific factors will not work)
2. Can uncertainty management/lifecycle HTA be a solution for HTAs? Is there a preferred approach to manage decision uncertainty while creating the right incentives for innovation?
3. What is the role of parallel Joint Scientific Consultation? Can parallel JSC solve the tension between regulatory and HTA views on acceptability of surrogate endpoints?