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- **European network for HTA**
Joint Action between **European Commission** and **EU Member States**
- 34 government-appointed organisations from 23 EU Member States, Norway and Croatia
- + relevant regional agencies
- + non-for-profit organisations that produce or contribute to HTA
- **EUnetHTA** Joint Action 2010–2012 | www.eunetha.eu



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EUnetHTA JA1 – WP5

10 methodological issues in Relative Effectiveness Assessment (REA) of pharmaceuticals:

- Comparators & comparisons
- **Criteria for choice of most appropriate comparator(s)**
- **Methods of comparison: direct and indirect comparisons**
 - Outcomes/Endpoints in the context of REA
- **Clinical endpoints**
- **Surrogate endpoints**
- **Composite endpoints**
- **Health-related quality of life**
- **Safety**
- Levels of evidence
- **Internal validity**
- **External Validity**



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Terminology (EunethHTA guidelines)

- **Clinical outcome = patient relevant outcome:** a valid measure of clinical benefit due to treatment; the impact of treatment on how a patient feels, functions and survives.
- **Patient-reported outcome (PRO):** an umbrella term used to describe any outcome evaluated directly by the patient himself/herself, without interpretation by clinicians or others, and based on patients' perception of a disease and its treatment(s).
- **HRQL** represents a specific type/subset of PROs, distinguished by its multi-dimensionality.



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HRQL and HTA

- Is there a place for HRQL in HTA?
 - In single HTA?
 - In full HTA?
 - When do we assess HRQL data?
 - What kind of HRQL data?
 - How to deal with HRQL data?
- Can a drug or a medical device be better reimbursed based on improvement in HRQL?



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HRQL assessment: purposes

- Regulatory: pre-MA setting
 - ➔ EMA and FDA guidelines apply
- HTA: REA of pharmaceuticals, MD, interventions and cost-utility assessment
 - ➔ EUnetHTA guideline (draft)
(REA of pharmaceuticals)



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When do we assess HRQL data ?

Relative Effectiveness Assessment (REA)

- In addition to efficacy assessment, to compare the impact on HRQL of a new drug with that of an active comparator
 - ➔ The same HRQL instruments as in regulatory (pre-MA) setting

When do we assess utility measures (cost utility assessment)?

- To estimate the ratio between the cost of an intervention and the benefit it produces in terms of the number of years lived in full health by the beneficiaries (QALYs: quality adjusted life year)
- Based on patient's preferences on his/her health state



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What kind of instruments?

➤ For REA:

- The same that those used in RCTs for drug approval
 - generic multidimensional HRQoL questionnaire
 - disease-specific multidimensional HRQoL questionnaire
 - both a generic and a disease-specific questionnaires
- But in a larger setting (full HTA): “real-life” studies
 - pragmatic trials, observational cohort studies
- For cost-utility assessment: short questionnaires (e.g. EQ-5D)



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Recommendation

- EUnetHTA guideline on HRQL (draft):
- Include both in the comparative phase 3 trial:
 - a generic or a disease-specific multidimensional HRQL questionnaire for REA
 - and**
 - a short generic questionnaire for cost-utility

Conclusion

- HRQL measures could have an impact on HTA and public health assessment
 - A drug or a medical device may be better reimbursed based on improvement in HRQL
- Recent guidelines (FDA 2009 + EMEA 2005) apply for regulatory context
- EUnetHTA guideline is being drafted to help HTA assessors to deal with HRQL data in the context of REA and cost-utility assessment of pharmaceuticals