

INTERVENTIONS FOR ULTRA-RARE DISORDERS (URDs) AND THE LOGIC OF COST EFFECTIVENESS

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Background: In many cases, medicines for ultra-rare disorders (URDs) have high acquisition costs and are associated with incremental cost per quality-adjusted life year (QALY) gained exceeding widely used benchmarks for cost effectiveness.

Objectives: To address the underlying reasons why interventions for URDs often fail to meet conventional benchmarks for cost effectiveness and deliberate implications for formal Health Technology Assessments (HTAs) including economic evaluation.

Methods: An international group of experts in health economics, medical ethics, evidence-based medicine (EBM), and HTA met in conjunction with the Annual European ISPOR Congresses in November 2012, 2013, and 2014.

Results: In contrast to the principles of EBM, the logic of cost effectiveness (including benchmarks for incremental cost per QALY gained, as applied by some HTA agencies as a measure of “value for money”) does not adequately capture well-established social norms and preferences regarding health care resource allocation. Such preferences include, but are not limited to, a priority for care for the worst off (related to initial health state), for those with more urgent conditions (the so called “rule of rescue”), and a relatively lower priority based upon capacity to benefit, as well as a dislike against “all or nothing” resource allocation decisions that might deprive certain groups of patients from any chance to access effective care.

Conclusions: The group concluded that there exists a strong need for an improved or new paradigm to assess value for money. Candidates include direct social value measurement using the relative social willingness-to-pay or person trade-off instruments, combined with a greater role for budget impact analysis. As a pragmatic interim alternative, multi-criteria decision analysis may prove useful. Further systematic research into social preferences, including their valid measurement, should be prioritized relative to the continued application of a descriptively flawed framework based on benchmarks for maximum incremental cost per QALY gained.

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