

Interventions for Ultra-Rare Disorders (URDs): How to Assess “Value for Money”?

Schlender M, Garattini S, Holm S, Kolominsky-Rabas P, Nord E, Persson U, Postma M, Richardson J, Simoens S, de Solà-Morales O, Tolley K, Toumi M

Background: Given the economics of biopharmaceutical research and development (R&D), characterized by substantial fixed and often low variable (volume-dependent) cost, many drugs for ultra-rare disorders (URDs) fail to meet widely used benchmarks for cost effectiveness.

Objectives: To identify key issues arising when interventions for URDs are subjected to formal Health Technology Assessments (HTAs), and to deliberate potential solutions.

Methods: An international group of clinical and health economic scholars met twice in conjunction with Annual European ISPOR Congresses in November 2012 and 2013.

Results: The group reached consensus that the complexities of R&D of new treatments for URDs may require conditional approval and reimbursement policies, but this should not be used as a justification for showing surrogate endpoint improvement only. Strong evidence of clinical effectiveness should be expected within reasonable timeframes. In contrast to well-established principles of evidence-based medicine, the logic of cost effectiveness (including benchmarks for incremental cost per quality-adjusted year, QALY, applied by some agencies as a measure of “value for money”) does not adequately capture prevailing 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: There is a strong need for an improved paradigm to determine value for money. Promising candidates include direct social value measurement using the relative social willingness-to-pay or person trade-off instruments, and a greater role for budget impact analysis. As a pragmatic alternative, multi-criteria decision analysis may be useful as a tool for assessment.