

## **Incremental Cost per Quality-Adjusted Life Year Gained?**

### **The Need for Alternative Methods to Evaluate Medical Interventions for Ultra-Rare Disorders**

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

**Objectives:** To critically appraise the problems posed by the systematic valuation of interventions for ultra-rare disorders using conventional health economic analysis methods.

**Methods:** An international group of clinical and health economic experts met in conjunction with the Annual European ISPOR Congress in Berlin/Germany, November 2012, to identify and deliberate underlying issues openly, adhering to the Chatham House rule.

**Results:** The group reached a broad consensus, including: The complexities of research and development of new treatments for ultra-rare disorders (URDs) may require conditional approval and reimbursement policies, such as coverage with evidence development agreements, but should not be used as a justification for showing surrogate endpoint improvement only. As a prerequisite for value assessment, demonstration of a minimum significant clinical benefit should be expected within a reasonable timeframe. Regarding the economic evaluation of interventions for URDs, the currently prevailing logic of cost effectiveness (using benchmarks for the maximum allowable incremental cost per quality-adjusted year, QALY, gained) was considered insufficient since it does not adequately capture well-established social preferences regarding health care resource allocation. Such social 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”), a relatively lower priority based upon capacity to benefit, and a dislike against “all or nothing” resource allocation decisions that might deprive certain groups of patients from any chance to access effective care.

**Conclusions:** Alternative paradigms to establish the “value for money” conferred by interventions for URDs should be developed with high priority. Such methods should capture and reflect prominent societal value judgments, beyond efficiency as conventionally defined by QALY maximization under a budget constraint.

Presented to the 16th Annual European ISPOR Congress, Dublin / Ireland, November 2-6, 2013.  
Full presentation will be made available for download at [www.innoval-hc.com](http://www.innoval-hc.com).

Abstract published in *Value in Health*, Vol. 16, No. 7, November 2013: A324.