

HLM⁴RARE

«Health without Postcodes»

Access to Rare and Complex Disease Therapies

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Are European HTA (Joint Clinical Assessments, JCAs) and Cost Effectiveness Analyses (CEAs) Up To Task?

▮ **EU Level JCAs: Comparative Clinical Effectiveness**

- ▮ Assessment of Clinical Evidence
 - ▮ noble objectives – but questions remain:
 - ▮ rigid standards or «best available evidence»? / room for judgment and appraisal?
 - ▮ the patients' experience / directly health-related impacts only? / broader ones?
- ▮ Appropriate Comparators (and «PICO») – Ambition vs. Feasibility
- ▮ Orphan Drug (OMP) Designation and Prioritization by Medical Need

▮ **EU Member States: Economic (Cost/) Value Assessments**

- ▮ Cost Effectiveness Analysis (CEA) Relying on Thresholds?
 - ▮ interface with JCAs? / «blindness» of CE ratios for prevalence and budget impact?
 - ▮ presumably «technical» assumptions vs. priority for patients in high need?
 - ▮ technocratic approach / evaluating (abstract) condition / treatment pairs
- ▮ Need to Consider Role of Health Care Systems as Social Institutions

Selected References

Cost Effectiveness Thresholds: More Harm Than Good?

- **M. Schlander, R. Schaefer, M. Postma, S. Holm:** *Cost Effectiveness Thresholds: Ethical Considerations and Practical Reality. Initial Findings from an Integrative Review.* Wiesbaden, July 2026: InnoVal^{HC} Discussion Paper No. 39. ISBN 978-3-941609-38-9.

The Patients' Perspective – Socioeconomic Impacts of Severe Disease

- **M. Schlander, W.H. van Harten, V.P. Retèl, et al.:** The socioeconomic impact of cancer on patients and their relatives: Organisation of European Cancer Institutes task force consensus recommendations on conceptual framework, taxonomy, and research directions. *The Lancet Oncology* 2024; 25 (4) e152-e163.

What Do We Really Know about the Cost of Developing a New Drug?

- **Schlander, K. Hernandez-Villafuerte, C-Y. Cheng, et al.:** How much does it cost to research and develop a new drug? A systematic review and assessment. *Pharmacoeconomics* 2021; 39 (11) 1243-1269.

Clinical and Economic Evaluation for Ultra-Rare Disorders:

- **M. Schlander, S. Garattini, S. Holm, et al.:** Incremental cost per quality-adjusted life year gained? The need for alternative methods to evaluate medical interventions for ultra-rare disorders. *Journal of Comparative Effectiveness Research* 2014; 3 (4) 399-422

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