

## **Drugs for Rare and Ultra-Rare Diseases in Europe: Analysis of Budget Impact and Cost Drivers**

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**Objectives:** The objective of the present study was (1) to review recent studies reporting health care expenditures for (or budgetary impact of) drugs for rare diseases in Europe, and (2) to contribute to our understanding of the cost drivers of drugs for non-oncological ultra-rare diseases (URDs) by means of an empirical analysis in Germany.

**Methods:** A systematic search for relevant studies was conducted in PubMed (1966 – December 2014) and in abstracts in congress proceedings. In addition, annual treatment costs of drugs for non-oncological URDs in Germany were analyzed with respect to five explanatory variables: availability of other treatment indications, availability of alternative treatments for the same indication, oral administration, prevalence of the disease, and evidence for a health benefit.

**Results:** A total of seven studies with specific estimates of the budget impact of drugs for rare diseases for a total of nine countries were identified. Annual per-capita spending for orphan drugs ranges from €0.48 in Russia to €16 in France. Only one study on URDs was identified. In Germany, annual treatment costs per patient for drugs for non-oncological URDs varies between €1,175 and €726,890. In all regression specifications, a significant inverse relationship between availability of alternative treatments for the same indication and annual treatment costs was found. In addition, log prevalence was found to have a significant inverse relationship with log annual treatment cost.

**Conclusions:** Despite annual treatment costs in the range of several hundreds of thousands of euros for some of the URD drugs, per-capita spending for URD drugs is relatively small. In this study, using German market data, an inverse relationship between prevalence and annual treatment costs was found specifically for drugs for non-oncological URDs.

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