# Budget Impact Analysis of Drugs for Ultra-Rare Non-Oncological Diseases in Europe

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**Objectives**: Ultra-rare disorders (URDs) have been defined by a prevalence of less than 1 per 50,000 persons. On a per patient basis, the annual acquisition costs of drugs for URDs can be very high, and there have been concerns that expenditures for these products might escalate in the future. The goal of this study was therefore to provide a budget impact analysis (BIA) of drugs for ultra-rare non-oncological diseases in Europe.

**Methods**: The BIA had a time horizon of 10 years (from 2012 to 2021) and adopted the perspective of all European payers in combination. The estimate was based on prevalence data for URDs for which patented drugs are currently available and for which drugs are in clinical development and hence may be expected to be launched in the foreseeable future. A power function was used to estimate the relation between (decreasing) prevalence and (increasing) cost per patient. For drugs in development, we applied phase duration data and attrition rates from the Tufts Center for the Study of Drug Development database.

**Results**: A total of 18 drugs under patent protection for non-oncological URDs were identified. Furthermore, 29 drugs for non-oncological URDs under development that have the potential of reaching the market by 2021 were found. Total budget impact over 10 years was estimated to be €14,112 and €4,965 million for approved and pipeline URD drugs, respectively (total: €19,077 million). Relative to total pharmaceutical expenditures in Europe, spending on drugs for URDs is estimated to rise from 0.7% at present to 1.6% in 2021. Univariate sensitivity analyses and extreme scenario analyses suggesting robustness of this projection will be presented.

**Conclusions**: Our analysis does not support concerns regarding an uncontrolled growth in expenditures for drugs for URDs. Nevertheless, continuous monitoring of the budget impact as an input to rational policy making is recommended.

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