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

Michael Schlander[1-3], Charles Christian Adarkwah[4], Afschin Gandjour[5]

[1]Institute for Innovation & Valuation in Health Care (INNOVAL[HC]), www.innoval-hc.com, An der Ringkirche 4, D-65197 Wiesbaden (Germany);
[2]University of Heidelberg (Germany), Mannheim Medical Faculty, Department of Public Health, Social and Preventive Medicine; [3]University of Applied Economic Sciences Ludwigshafen (Germany);
[4]University of Marburg (Germany), Department of General Practice; [5]Frankfurt School of Finance and Management (Germany)

Background/Aim:

Ultra-rare disorders (URDs) have been defined by a prevalence of less than 1 per 50,000 persons. Little is known about the current and future budget impact of ultra-orphan drugs, however. The goal of this study was therefore to conduct a budget impact analysis (BIA) of drugs for ultra-rare non-oncological diseases in Europe.

Methods:

For purposes of this analysis, 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.

Key Assumptions and Inputs:

Time horizon: 10 years (2012 to 2021)
Perspective: payer's perspective
Scope: European countries (EU and non-EU)
Cutoff prevalence rate for URDs: 1:50,000 (0.002%)
Source of **patent expiry** dates: Medtrack database
Market exclusivity periods: assumed to be 10 years
Source of **pipeline drugs**: Medtrack database

Future sales of pipeline URD drugs were estimated based on the relationship between annual per-patient drug costs and prevalence for approved drugs.

Sensitivity analysis: univariate sensitivity analyses and a worst-case and best-case scenario analysis, applying extreme values of the two most influential variables as identified in the sensitivity analysis.

Table 1: Base-case values and ranges used in the budget impact model and for sensitivity analysis.

Variable	Base Case (range)	Reference
Market penetration rate	22% (10%-30%)	Schey et al. 2011
Annual growth rate in sales volume	10% (5%-15%)	Adapted from EvaluatePharma (2013)
Savings one year after the first generic entry	0% (0%-20%)	EU Competition Commission
Savings two years after the first generic entry	0% (0%-25%)	EU Competition Commission
Clinical phase durations		Adapted from Tufts Center for the Study of Drug Development (REF)
Phase I trials	2 years (1.5-2.5)	
Phase II trials	1.5 years (1-2)	
Phase III trials	1.5 years (0-2)	
Approval	1.5 years (1-2)	
Transition probabilities		Adapted from Tufts Center for the Study of Drug Development
Phase I → phase II	70.6% (60%)	
Phase II → phase III	45.4% (40%)	
Phase III → New Drug Application	63.6% (50%-100%))	
New Drug Application → approval	93.2% (80%)	
Discount rate	3.5% (0%-5%)	Average of the discount rates recommended in England, Germany, and the Netherlands

For further information on the structure of the budget impact model, please contact the authors at michael.schlander@medma.uni-heidelberg.de or at michael.schlander@innoval-hc.com

Results:

A total of 18 drugs under patent protection or orphan drug designation for non-oncological URDs were identified. Furthermore, 29 ultra-orphan drugs for non-oncological diseases under development that have the potential of reaching the market by 2021 were found. Total budget impact over 10 years was estimated to be €15,660 and €4,965 million for approved and pipeline ultra-orphan drugs, respectively (total: €20,625 million). Relative to total pharmaceutical expenditures in Europe, spending on ultra-orphan drugs is estimated to be at 0.7% at present and expected to increase to 1.6% in 2021.

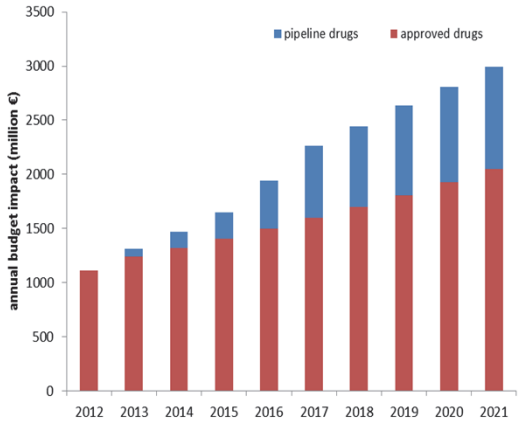

Figure 1: Annual budget impact of approved and pipeline drugs for ultra-rare diseases over 10 years (2012 to 2021) in Europe from a payer's perspective.

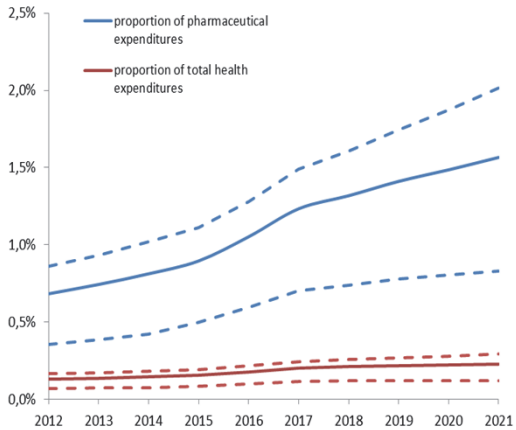

Figure 2: Proportion of pharmaceutical and total health expenditures in Europe spent on drugs for ultra-rare diseases (URDs). Dashed lines indicate ranges provided by the extreme-case scenario analyses.

Conclusions:

The analysis does not support concerns regarding an uncontrolled growth in expenditures for drugs for URDs. Continuous monitoring of the budget impact as an input to rational policy making is recommended.