

Global Market Access Solutions

Estimating the Budget Impact of Orphan Medicines In Europe: 2010 – 2020

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Poster 125

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Background

- Backdrop
 - Concerns voiced about escalating orphan drug costs ^(1,2)
 - Delayed uptake of newly marketed orphan drugs ⁽³⁾
 - Uneven access to orphan drugs in the Eurozone & UK ⁽⁴⁾
 - Limited studies published; primarily country-specific.
- Aim
 - To estimate the European budget impact of orphan drugs, as a percentage of the total pharmaceutical expenditure between 2010 and 2020.

1. McCabe C. *EJHP* 2010, 16(4):22-24

2. Nicholl D, *et al.* *BMJ* 2010, 341:c6466

3. Denis A, *et al.* *Health Policy*. 2010, 97:173-179

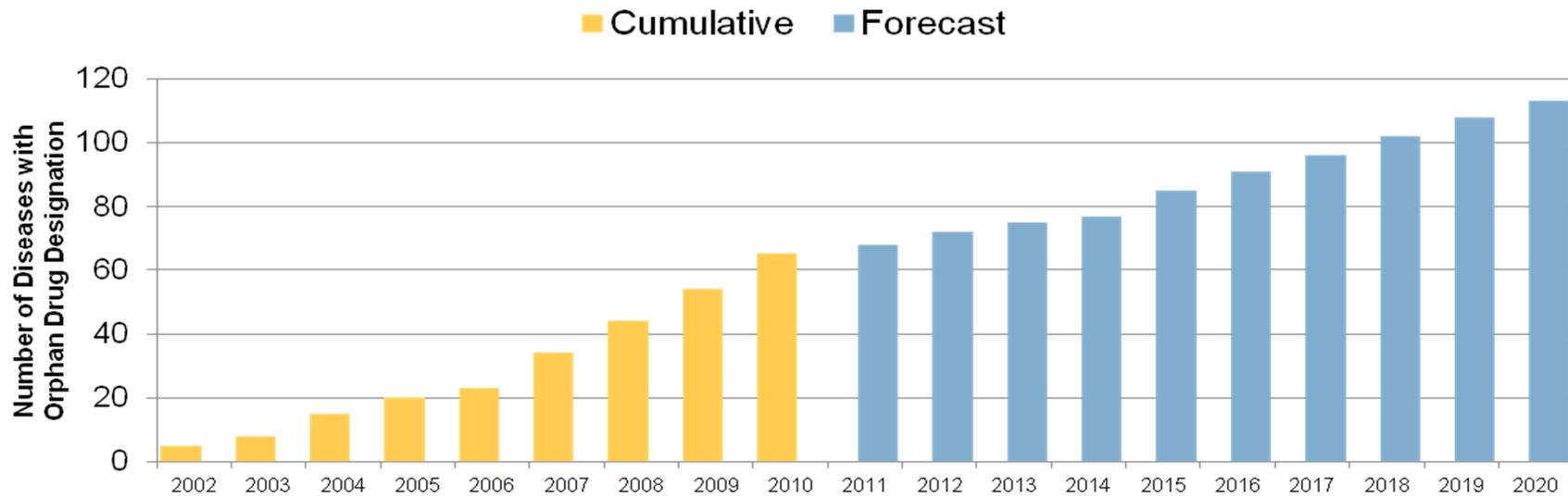
4. *Eurordis. Improving access to orphan drugs.* <http://www.eurordis.org/content/improving-access-orphan-drugs>

Methods

- The study was confined to the 17 Eurozone countries, plus the UK
 - Market homogeneity
- Only costs relating to designated orphan drugs
- Adopted a disease-based epidemiological modeling approach was used to predict the future budget impact of orphan medicines
- There were two stages to the analysis:
 - Predicting the number of orphan diseases for which new orphan medicines will be approved over the next decade
 - Estimating the average annual orphan medicine cost per orphan disease.

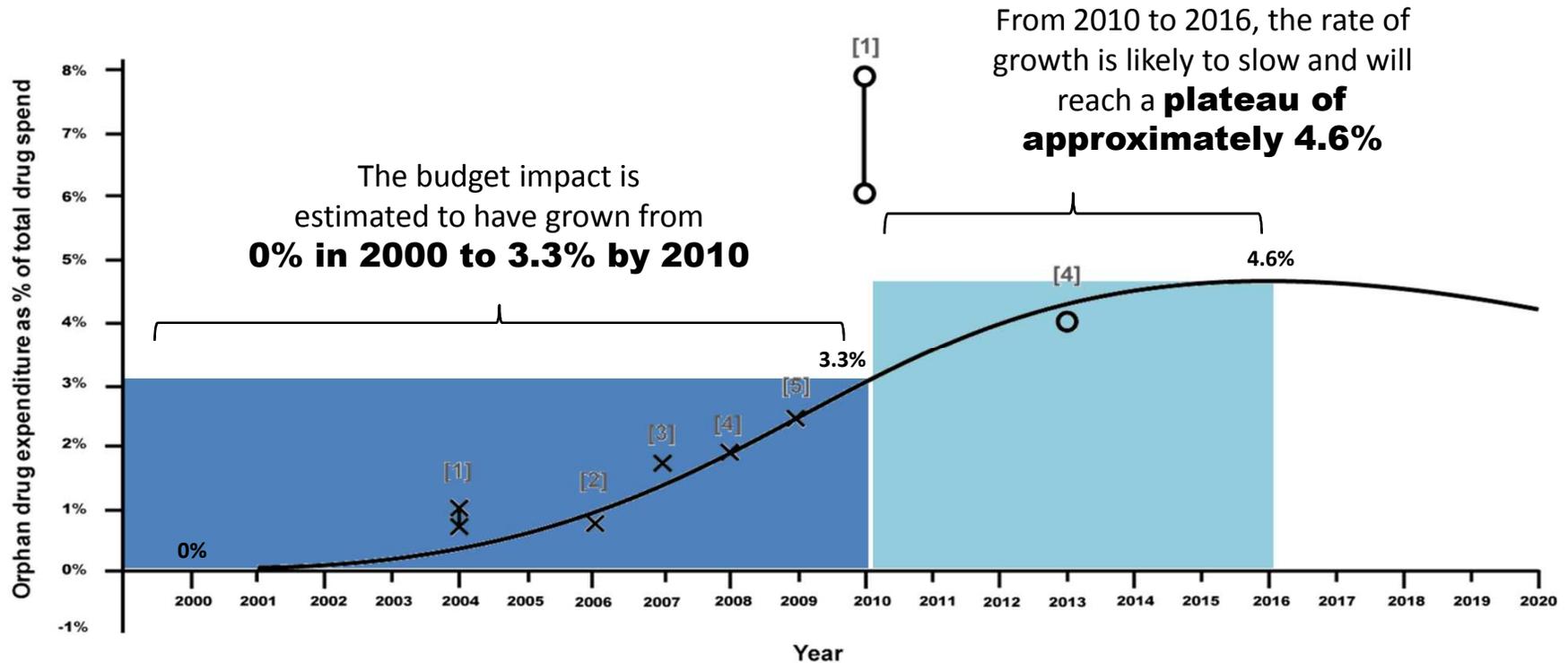
Results: Predicted number of Rare Diseases

- Trend is towards steady increase in the cumulative number of diseases for which an orphan medicine is designated
 - Averaging just over 5 new diseases per year (over the 20 years of the model)
- Rate of growth in diseases was more rapid between 2002 – 2010 than 2011 - 2014
- From 2015 forward, the additional increase in orphan diseases is forecast at approximately 10% per year.



Results: Orphan Drug Expenditure

- The annual per patient cost of existing orphan drugs ranged from €1,251 to €407,631 with a median per patient cost of €32,242
- The budget impact of orphan medicines in Europe has grown steadily over the 10 years.



[1] 2004 European Commission; 0.7 – 1.0% . Predicted to rise to 6 – 8% by 2010

[2] 2006 van Ekdom 0.75%

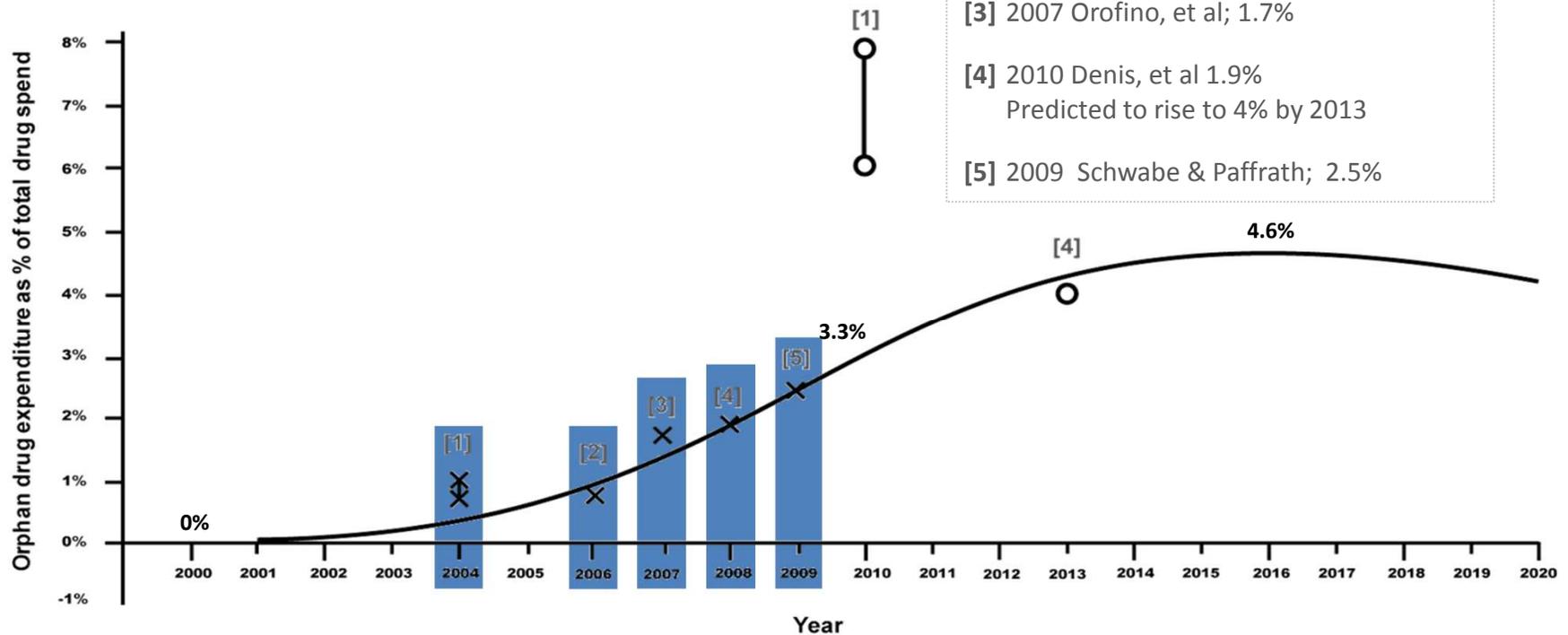
[3] 2007 Orofino, et al; 1.7%

[4] 2010 Denis, et al 1.9% Predicted to rise to 4% by 2013

[5] 2009 Schwabe & Paffrath; 2.5%

Results: Orphan Drug Expenditure

Historical estimates from the model (2001 - 2010) **fit closely with those produced from other sources**



Parameter Analysis

Parameter		Value		Peak year budget impact as % of total pharmaceutical spend (year)
1	Lag between orphan drug designation and marketing approval	Base	4 yr	4.6% (2016)
		Best	6 yr	4.2% (2016)
		Worst	2 yr	4.9% (2015)
2	Eurozone + UK Pharmaceutical market value in 2010 (ex-factory)	Base	€140bn	4.6% (2016)
		Best	€170bn	3.8% (2016)
		Worst	€110bn	5.8% (2016)
3	Total pharmaceutical market growth per year	Base	6.6%	4.6% (2016)
		Best	10%	4.0% (2014)
		Worst	3%	5.9% (2020)
4	% of products with orphan designation that achieve market authorisation	Base	10.92%	4.6% (2016)
		Best	7%	4.2% (2015)
		Worst	20%	6.6% (2016)
5	Growth in new designations per year after 2010	Base	10%	4.6% (2016)
		Best	5%	4.6% (2016)
		Worst	50%	4.7% (2020)
6	Average cost of orphan disease per year (Yr 1 – Yr 10)	Base	As per Figure 2	4.6% (2016)
		Best	-25% p.a	3.4% (2016)
		Worst	+25% p.a	5.7% (2016)
7	Drop in drug prices at the point that drugs lose patent protection or marketing exclusivity	Base	25%	4.6% (2016)
		Best	50%	4.2% (2014)
		Worst	0%	5.1% (2019)

Study Limitations

- Structural uncertainty
- Use of prevalence data to estimate patient populations
- The *uptake rate* of orphan drugs, as they come to market, was based on sales data of a smaller sample of drugs
- The average price per orphan drug
- Uncertainty of the impact of loss of marketing exclusivity on price erosion in the future.

Discussion

- Budget of orphan medicines in Europe has grown steadily since 2000
- Rate of budget impact is expected to grow slowly from 2010 to 2016, at which time it is expected to plateau at approximately 4.6% of total pharmaceutical market expenditure
- Estimates of budget impact in this analysis aligns well with the forecast for 2013 budget impact from the study performed by Denis et al
- Differences in methodology point to the need for further research to intelligently inform orphan drug policy moving forward
- Estimation of the cost of orphan medicines on a market-by-market basis should be investigated, to account for differences between countries.

References

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