

# Quantitative analysis of the influence of disease and product characteristics on orphan drug prices in Europe

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## INTRODUCTION

• With about 40 approved orphan drugs generating over \$200m each in yearly sales, including 10 blockbusters, rare diseases represent an important market. This, combined with regulation and incentives to develop new orphan drugs in US and Europe, has boosted the interest of pharmaceutical companies in this field.

• Pricing is a key success factor for manufacturers as sales volumes are very limited.

## OBJECTIVES

The objective was to investigate the determinants of orphan drugs prices in five European countries: France, Germany, Italy, Spain and United Kingdom, using regression analysis.

## METHODS

- Products designated as orphan drugs and approved by the European Medicines Agency were identified up to December 2009.
- Yearly prices per product were determined from public prices. We used defined daily doses (DDD, from WHO ATC) and average treatment duration was defined by guidelines or summary product characteristics.
- The analysis included disease-related and drug-related parameters.
  - Disease-related variables were: prevalence, prognosis, age of target population, seriousness, number of alternative treatments and course of illness.
  - Drug-related parameters were: year of approval, trial size, number of trials, ATC code, evidence of benefit.
- Price predictors for each of the 5 countries were studied using a Poisson model, with log link.

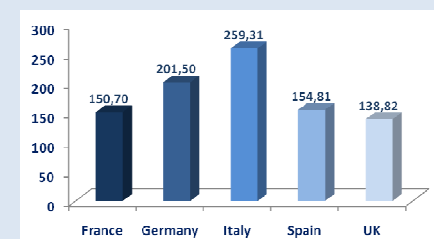
## RESULTS

### Identified drugs

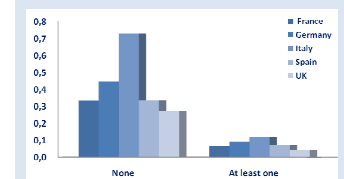
- 51 products were identified of which respectively 37, 35, 28, 36 and 39 were available in France, Germany, Italy, Spain and United Kingdom.
- 19 drugs were available in all the 5 countries.

### Comparison of average public prices of orphan drugs in Europe

5 European countries, 19 common drugs, K€

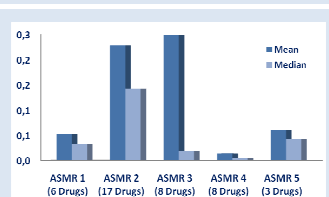


### Mean Prices of orphan drugs according to the number of therapeutics alternatives in Europe – 43 drugs, € millions



### Orphan drug prices according to ASMR

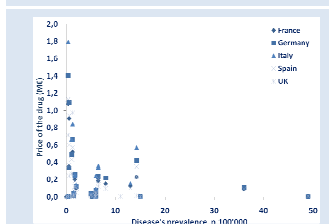
5 European countries, 42 drugs, € millions



- No significant correlation was found between any of the studied variables and price, possibly due to lack of power.
- Low disease prevalence and low number available therapeutic alternatives seemed however associated with increased yearly prices.

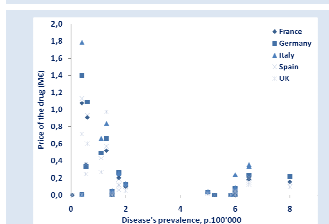
### Prices according to disease prevalence

5 European countries, 30 drugs, p. 100 000 population



### Prices according to disease prevalence

5 European countries, 23 drugs, p. 100 000 population



### Results of regression analyses on drug prices

Analyses performed for each variable independently

	France	Germany	Italy	Spain	United Kingdom
Prevalence	PS: 0.94, p-value: 0.95	PS: 0.92, p-value: 0.92	PS: 0.91, p-value: 0.91	PS: 0.91, p-value: 0.92	PS: 0.86, p-value: 0.13
Prognosis	1.00	1.00	1.00	1.00	1.00
- Non vital	1.00	1.00	1.00	1.00	1.00
- Vital	0.56, 0.26	0.62, 0.40	0.91, 0.37	0.56, 0.39	1.02, 0.97
Age	1.00	1.00	1.00	1.00	1.00
- Elderly	0.44, 0.28	0.43, 0.35	0.45, 0.38	0.35, 0.34	0.36, 0.35
- Adults	2.12, 0.29	2.48, 0.28	1.74, 0.52	2.32, 0.30	2.34, 0.39
- Children	0.58, 0.74	0.98, 0.98	1.42, 0.74	1.00, 1.00	1.15, 0.91
- Babies					
Course of illness	1.00	1.00	1.00	1.00	1.00
- Chronic	1.00	1.00	1.00	1.00	1.00
- Acute	0.96, 0.80	0.94, 0.78	2.93, 0.15	0.94, 0.77	0.99, 0.96
Severity	1.00	1.00	1.00	1.00	1.00
- Very severe	1.15, 0.85	0.89, 0.85	1.36, 0.69	1.01, 0.99	1.13, 0.87
- Severe					
Burden Of Taking Care	1.00	1.00	1.00	1.00	1.00
- Very Heavy	44.07, 0.60	44.71, 0.60	63.07, 0.56	49.93, 0.60	54.00, 0.68
- Heavy	1.12, 0.41	1.19, 0.34	1.07, 0.66	1.36, 0.13	1.02, 0.90
- Moderate	9.32, 0.76	12.02, 0.73	15.55, 0.68	8.48, 0.78	8.21, 0.83
- Number of Alternatives	0.64, 0.05	0.69, 0.11	0.70, 0.13	0.72, 0.12	0.60, 0.16
Place	1.00	1.00	1.00	1.00	1.00
- First Line	1.00	1.00	1.00	1.00	1.00
- Other	0.98, 0.84	1.01, 0.84	1.02, 0.68	1.03, 0.61	1.00, 0.97
Evidence of Benefit	0.83, 0.48	0.88, 0.66	0.82, 0.51	0.93, 0.79	0.73, 0.51
Year of Approval	1.12, 0.41	1.19, 0.34	1.07, 0.66	1.36, 0.13	1.02, 0.90
Trial Size (p. 100 more patients)	0.97, 0.05	0.95, 0.01	0.96, 0.02	0.96, 0.05	0.95, 0.01
Number of studies	1.07, 0.77	1.10, 0.70	1.13, 0.60	1.09, 0.74	1.03, 0.92
ATC Code	1.00	1.00	1.00	1.00	1.00
- Venous	39.66, 0.31	25.20, 0.40	19.89, 0.46	96.78, 0.31	36.86, 0.31
- Alimentary tract and metabolism	1.23, 0.97	0.80, 0.97	1.18, 0.98	5.03, 0.78	1.01, 1.00
- Cardiovascular system	3.72, 0.74	1.83, 0.91	2.29, 0.85	5.74, 0.72	1.87, 0.96
- Genito urinary system, sex hormones	0.87, 0.98	0.57, 0.93	-	1.86, 0.93	0.62, 0.95
- Hormonal preparations	3.76, 0.77	2.09, 0.87	1.97, 0.89	7.79, 0.70	2.47, 0.85
- Antineoplastic, immunomodulation	14.59, 0.46	7.07, 0.61	7.67, 0.61	30.66, 0.45	10.78, 0.50
- Nervous system	1.06, 0.99	0.25, 0.81	0.13, 0.82	1.00	1.09, 0.98

## CONCLUSIONS

- Pricing of orphan drugs is a complex process with no identifiable objective price determinant.
- Pro-active patient associations, low overall impact on health care expenditures, influence of opinion leaders and MAH's\* ability to shape perception of the unmet need in the target population might play an important role.
- This analysis generated hypotheses for further research into price settings of orphan drugs.

## REFERENCES

- Availability of medicines for rare diseases in EU countries, Trama A, et al., Pharm Pol Law 11 (2009) 101-9
- Orphan Drugs strategies. A route to unlocking new commercial opportunities, Datamonitor 2009
- WHO ATC, [http://www.who.no/atc\\_ddd\\_index/](http://www.who.no/atc_ddd_index/)

\* MAH : Marketing Authorization Holder