

BACKGROUND

Reimbursement of Orphan Drugs is a highly disputed issue. A challenge in pricing of Orphan Drugs is their very limited target population. Development costs are fix to a large extend. Hence reimbursement per patient should be higher with increased rarity of a disease.

In 2011 Germany introduced an early benefit assessment for all new drugs – including Orphans. The subsequent reimbursement negotiation gave the payers a say in the reimbursement. If manufacturers and payers do not agree on a reimbursement this will be set by arbitration board.

The negotiated reimbursement becomes effective one year after product launch in Germany, allowing for free pricing in the first year.

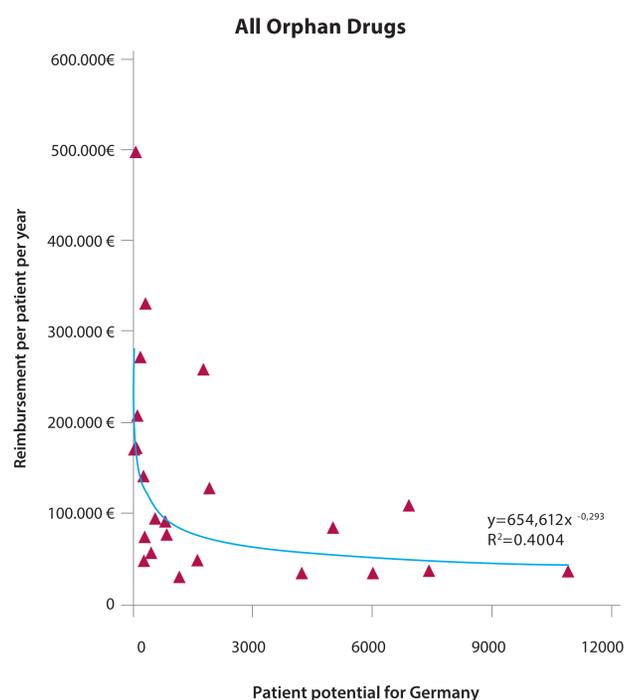
OBJECTIVES

The objective of the present study is to assess if it is still possible to achieve a premium in price to reflect the rarity of a disease, i.e. if negotiated reimbursement is inverse to epidemiology.

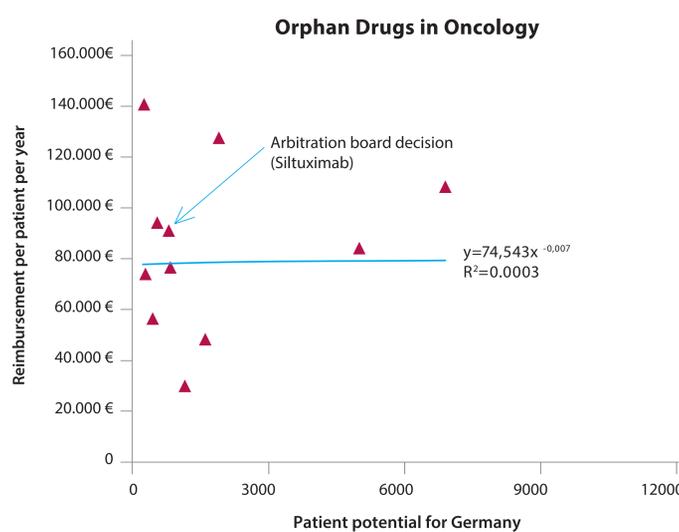
METHODS

This assessment is based on all first reimbursement negotiations completed until March 2016. For each drug the estimated population and the prices pre and post negotiation are captured. Prices are expressed on retail level. For sensitivity analysis a differentiation is made between oncological drugs and non-oncological drugs. As stated before, the reimbursement per patient is expected to be higher with increased rarity of a disease. To analyze this hypothesis we used an “inverse” regression approach of the type $y=ax^n$, where y is the reimbursement per patient per year, x the patient potential for Germany and a, n are real numbers.

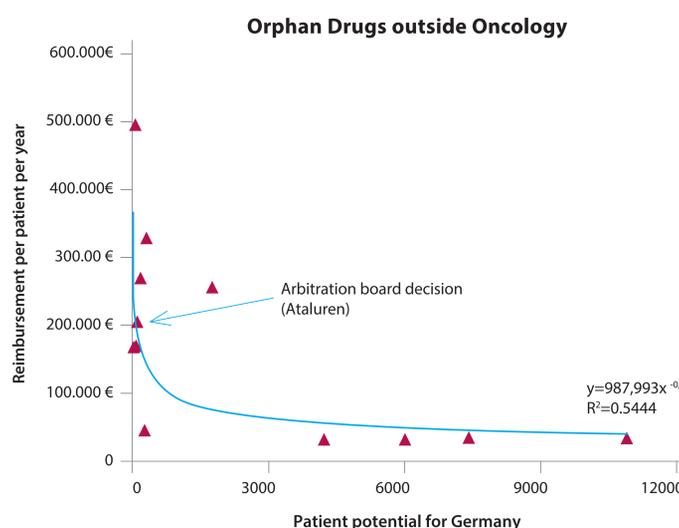
RESULTS



- 23 first reimbursement negotiations have been completed so far for Orphan Drugs in Germany.
- Negotiated reimbursements range between 30,160 € and 497,874 €.
- Regression analysis indicates that some relation between reimbursement and patient potential does exist – however there is still huge variation.
- After differentiation between oncological and non-oncological drugs the coefficient of determination increases considerable for Orphan Drugs outside oncology.



- 11 first reimbursement negotiations have been completed so far for Orphan Drugs in oncology.
- Negotiated reimbursements range between 30,160 € and 140,991 €.
- Negotiated reimbursements are nearly flat (mean 84,922.18 €), i.e. reimbursement per patient does not depend on the rarity of the disease.
- $R^2 = 0.0003$, i.e. there is no relevant statistical inverse relation between patient potential in Germany and reimbursement per patient per year.
- Arbitration board decision for Siltuximab is slightly above the average outcome of negotiations for Orphan Drugs in oncology: 84,922 € (mean) vs. 91,292 € (actual).



- 12 first reimbursement negotiations have been completed so far for Orphan Drugs outside oncology.
- Negotiated reimbursements range between 34,343 € and 497,874 €.
- Negotiated reimbursements are inverse to epidemiology, i.e. reimbursement per patient increases with the rarity of the disease. This is confirmed by a coefficient of determination of $R^2 = 0.5444$.
- Arbitration board decision for Ataluren is very much consistent with outcomes of negotiations: 204,595 € (expected) vs. 207,736 € (actual).

CONCLUSIONS

- Reimbursement for pharmaceuticals is drastically modified by the new laws in Germany.
- For Orphan Drugs in the field of oncology patient potential seems to have no impact on the level of the negotiated reimbursement.
- However for Orphan Drugs outside oncology the negative inverse relationship between patient potential and cost could be maintained, i.e. allowing for higher reimbursement when diseases are rarer.
- Both arbitration board decisions are consistent with these observations.
- Note that the data set is still limited.

REFERENCES

- Final decisions on early benefit assessment were taken from the G-BA website: www.g-ba.de/informationen/nutzenbewertung/ (English version [less recent]: www.english.g-ba.de/benefitassessment/resolutions/)
- Initial prices and negotiated final prices were taken from: ABDA Pharma-Daten-Service der Werbe- & Vertriebsgesellschaft Deutscher Apotheker (WuV), ABDA-Artikelstamm, www.pharmazie.com/dacon32/global/infoseiten_eng/abdaartikelstamm.htm

