

BACKGROUND

Reimbursement of Orphan Drugs is a highly disputed issue. A challenge in pricing of Orphan Drugs is their very limited target population. Development cost are given; 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 Orphan Drugs. 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.

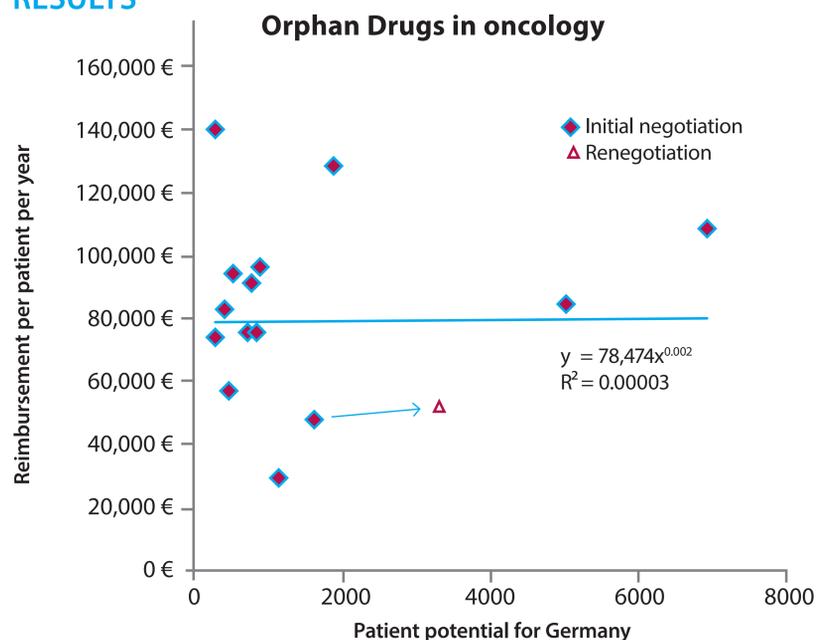
OBJECTIVES

Based on limited data it has been observed that reimbursement for Orphan Drugs in Germany seems to be inverse to epidemiology, i.e. reimbursement per patient increases with the rarity of the disease. The objective of the present study is to verify if this relationship is still true, especially if renegotiations due to reassessments or assessments of new indications still follow this general observation.

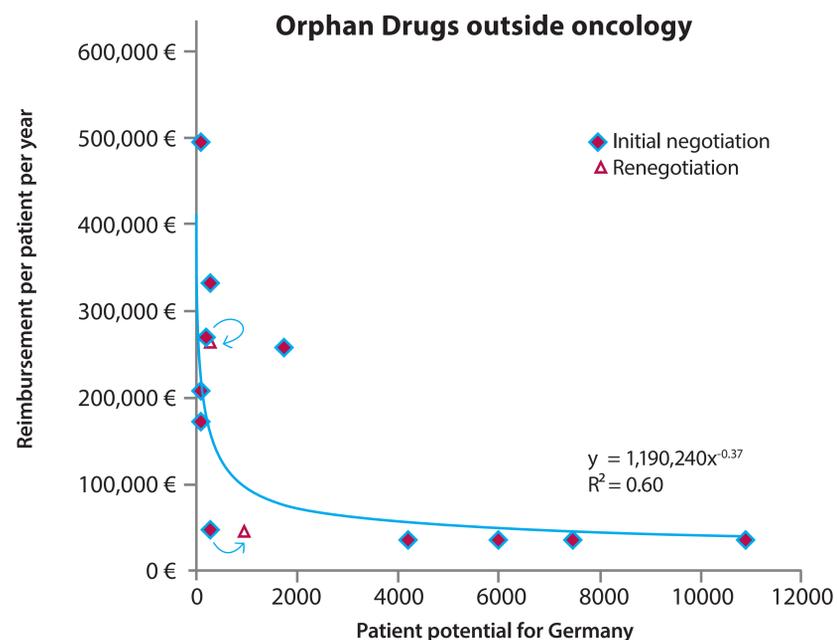
METHODS

Our analysis is based on all price negotiations completed and prices published until September 2016. For each drug the estimated population and the retail price post negotiation are captured. 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 (only first negotiation, no renegotiation), x the patient potential for Germany and a, n are real numbers. Based on previous observations separate regressions have been performed for Orphan Drugs in oncology and outside oncology. For sensitivity analysis all renegotiations due to reassessments or assessments of new indications are checked if they follow the general hypothesis.

RESULTS



- 15 first reimbursement negotiations have been completed so far for Orphan Drugs in oncology.
- Negotiated reimbursements per patient and year range between 30,160 € and 140,991 €.
- Negotiated reimbursements are nearly flat (mean 85,235 €), i.e. reimbursement per patient does not depend on the rarity of the disease.
- $R^2 = 0.00003$, i.e. there is no relevant statistical relation between patient potential in Germany and reimbursement per patient per year.
- Renegotiation for ruxolitinib after reaching 50 Mio. € sales led to increased patient potential and higher negotiated reimbursement, based on a higher degree of additional benefit during reassessment!



- 14 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 $R^2 = 0.60$.
- Renegotiation for ivacaftor after approval of a second indication increased patient population by 50% with reduction of reimbursement per patient and year by 1%. Renegotiation for pasireotide nearly quadrupled patient population with a reduction in reimbursement of around 7%.

CONCLUSIONS

- Reimbursement is drastically modified by the new laws in Germany.
- 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.
- However for Orphan Drugs in the field of oncology patient potential seems to have no impact on the level of the negotiated reimbursement.
- Renegotiations after assessment of additional indications or reassessments are consistent with this observation for Orphan Drugs outside oncology, i.e. increased patient population leads to a (small) reduction in reimbursement per patient and year.

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

