Outcomes of Benefit Assessments of Orphan Drugs Without Orphan Designation in Germany

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Objectives

Orphan drugs with orphan designation (OD) are granted an added benefit by law in the German HTA process. However, some drugs indicated for rare diseases do not obtain or lose orphan designation, leading to regular benefit assessments without orphan privilege. The objective of the current study is to investigate the outcomes of benefit assessments of orphan drugs without OD in Germany compared to non-orphan drugs and orphan drugs with OD with respect to the frequency of a granted added benefit as well as the resulting negotiated rebates and annual therapy costs.

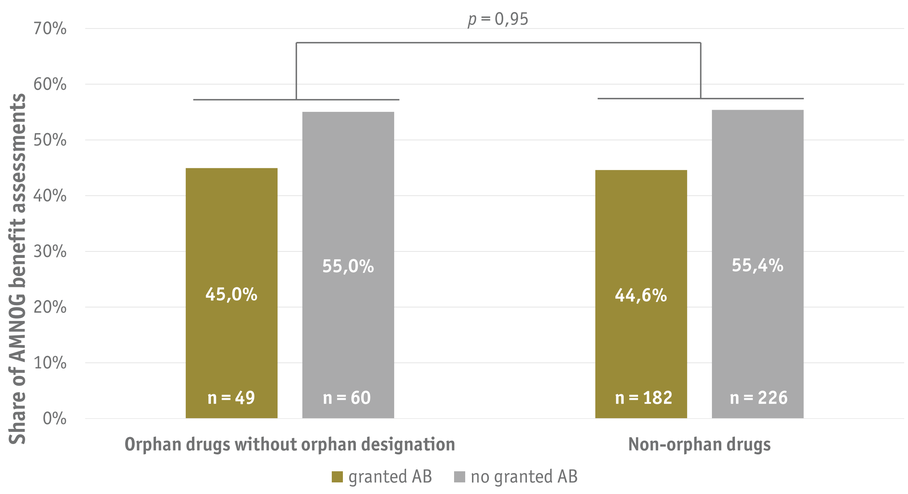
Methods

Outcomes of 729 benefit assessments performed until March 2022 in Germany were analyzed regarding the granted added benefit, negotiated net rebate, and net annual therapy costs for orphan drugs without orphan designation (n = 114), non-orphan drugs (n = 450), and orphan drugs with orphan designation (n = 165), respectively. Pair-wise comparisons were conducted to compare benefit assessment outcomes between orphan drugs without orphan designation and non-orphan drugs as well as orphan drugs with orphan designation, respectively.

Results

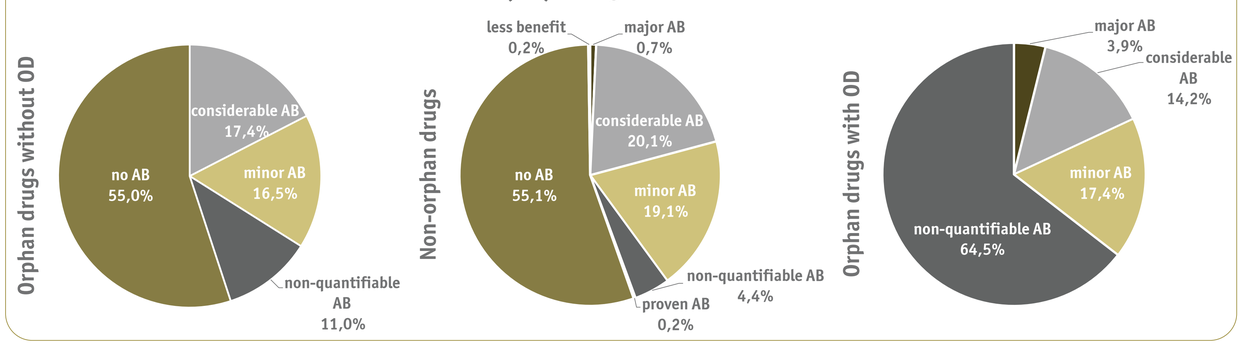
Orphan drugs without orphan designation were granted an added benefit equally frequently as non-orphan drugs. The distribution of the added benefit categories was similar between orphan drugs without orphan designation and non-orphan drugs.

Frequency of a granted added benefit for orphan drugs without OD compared to non-orphan drugs



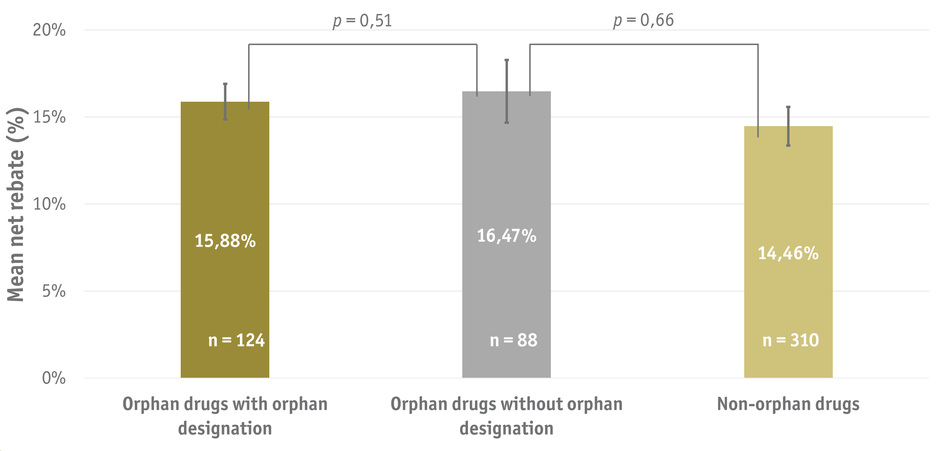
Differences in the frequencies of a granted added benefit were tested using a chi-square test. 109/114 AMNOG assessments for orphan drugs without OD and 408/450 assessments for non-orphan drugs were included in the analysis. Reasons for exclusion of certain assessments were: ongoing AMNOG assessment and procedure discontinued or exempted.

Distribution of the added benefit (AB) categories in German AMNOG benefit assessments



Average negotiated net rebates did not differ significantly between orphan drugs without orphan designation and non-orphan drugs or orphan drugs with orphan designation.

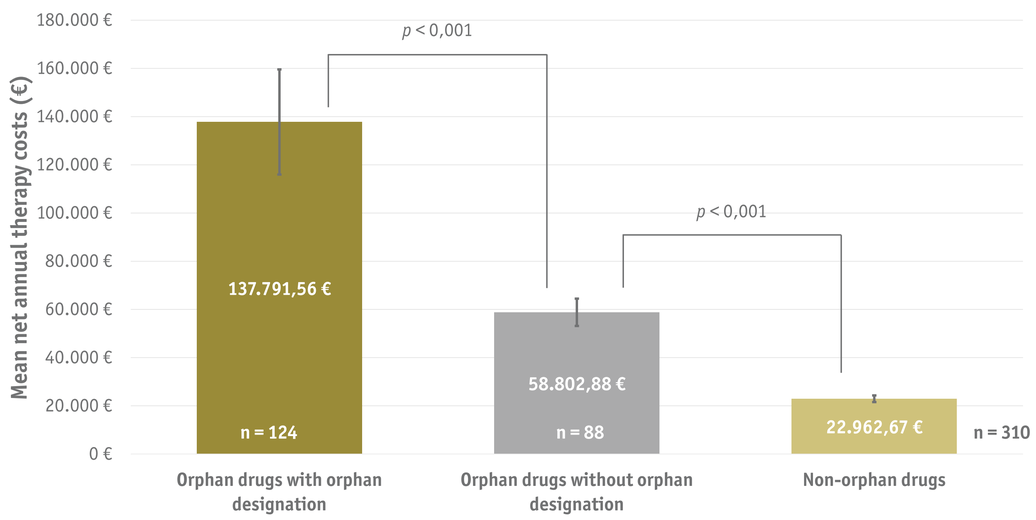
Negotiated net rebates for orphan drugs without orphan designation compared to orphan drugs with orphan designation and non-orphan drugs, respectively



Differences in negotiated net rebates were tested using the non-parametric Mann-Whitney U-test. Error bars show standard errors of mean. See analysis of net annual therapy costs for more details on the numbers of assessments included in the analysis.

Resulting average net annual therapy costs were significantly higher for orphan drugs without orphan designation than for non-orphan drugs and dramatically higher for orphan drugs with orphan designation than for orphan drugs without orphan designation.

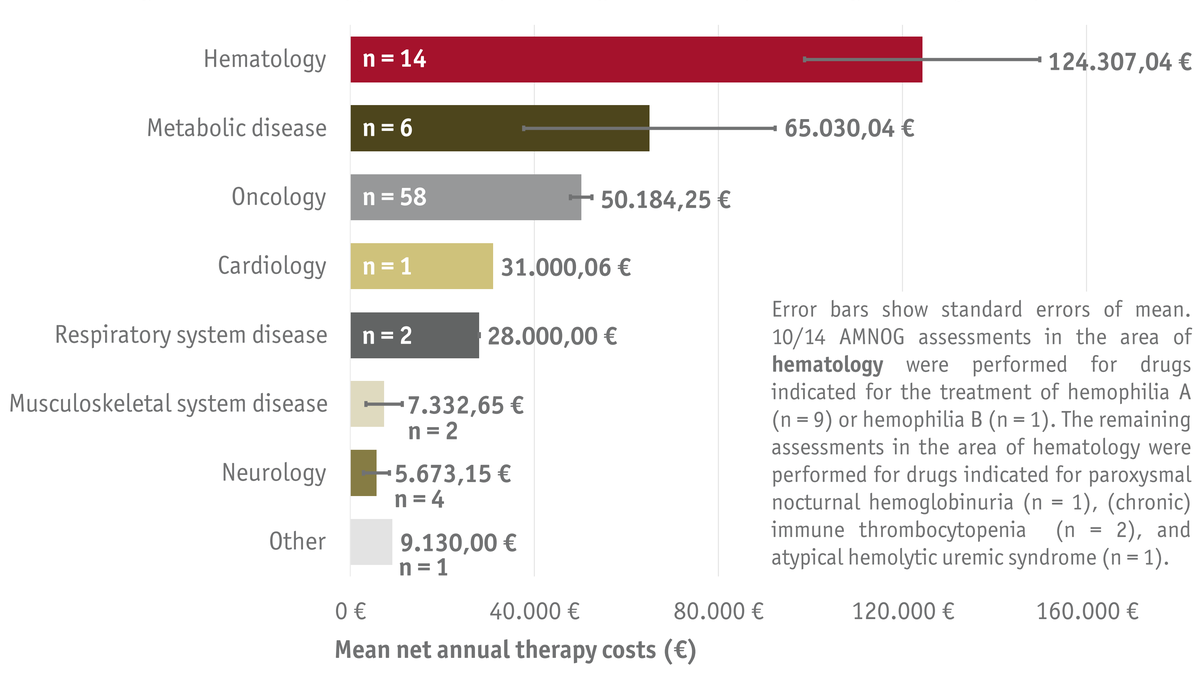
Resulting net annual therapy costs for orphan drugs without orphan designation compared to orphan drugs with orphan designation and non-orphan drugs, respectively



Differences in net annual therapy costs were tested using the non-parametric Mann-Whitney U-test. Error bars show standard errors of mean. 124/165 AMNOG assessments for orphan drugs with OD, 88/114 assessments for orphan drugs without OD and 310/450 assessments for non-orphan drugs were included in the analysis. Reasons for exclusion of certain assessments were: ongoing assessment, procedure discontinued or exempted, drug withdrawn or opt-out, no submitted dossier, ongoing price negotiations, renewed benefit assessment, reference price class inclusion.

Resulting average net annual therapy costs for orphan drugs without orphan designation were highest in the therapeutic area of hematology.

Resulting net annual therapy costs for orphan drugs without orphan designation by therapeutic area



Error bars show standard errors of mean. 10/14 AMNOG assessments in the area of **hematology** were performed for drugs indicated for the treatment of hemophilia A (n = 9) or hemophilia B (n = 1). The remaining assessments in the area of hematology were performed for drugs indicated for paroxysmal nocturnal hemoglobinuria (n = 1), (chronic) immune thrombocytopenia (n = 2), and atypical hemolytic uremic syndrome (n = 1).

Conclusion

- Based on the frequency of a granted added benefit, the added benefit categories, and the negotiated net rebates, no disadvantages in the AMNOG benefit assessment in Germany were identified for orphan drugs without OD compared to non-orphan drugs despite the usually higher difficulties in generating high-quality clinical evidence including the comparison against an appropriate comparator therapy.
- The higher net annual therapy costs for orphan drugs without OD compared to non-orphan drugs may reflect a higher willingness to pay for conditions with a higher unmet medical need and risk in clinical development even if not recognized as such by the European Medicines Agency. This willingness to pay could be particularly demonstrated in the therapeutic area of hematology.

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All analyses have been conducted with our own comprehensive MAIS database that contains and links AMNOG information of all completed and ongoing benefit assessment procedures according to §35a SGB V of the German Federal Joint Committee (G-BA).