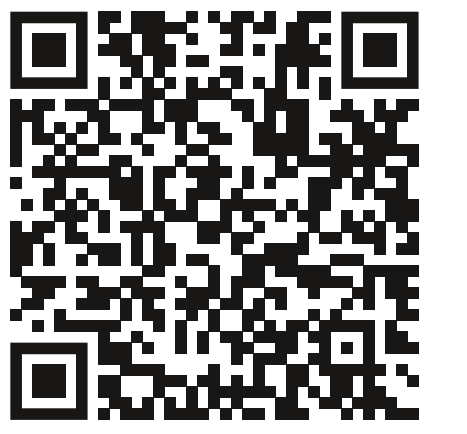


Szczesny, J., Tasler, J., Leismann, J., Ecker, T.
Ecker + Ecker GmbH, Hamburg, Germany

Re-assessment of orphan drugs in Germany after exceeding the annual sales limit of € 30 million: Impact on the reimbursement price and key influencing factors



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Background & Objectives

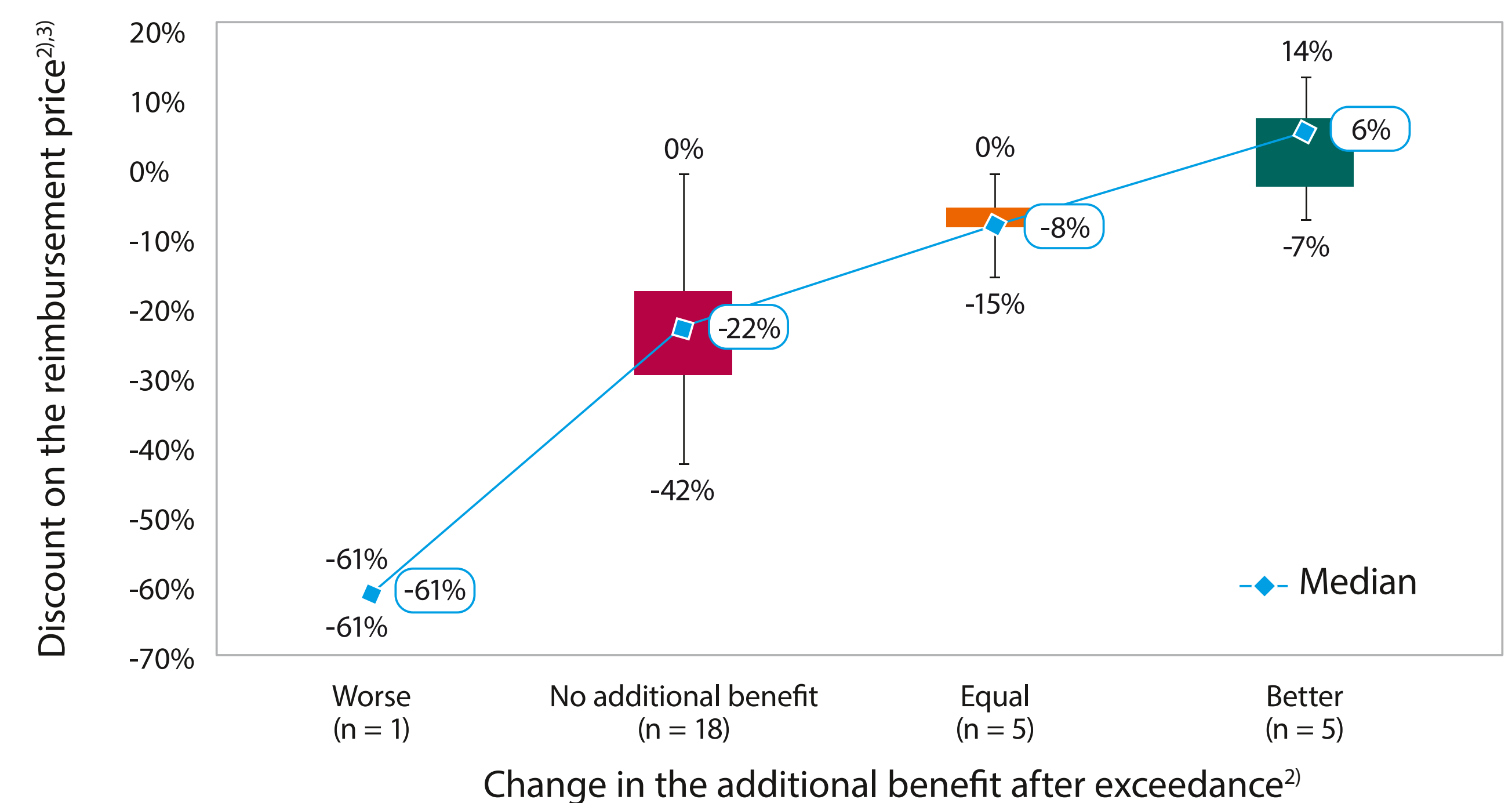
- In Germany, orphan drugs (OD) are exempt from the obligation to show an additional benefit over the appropriate comparator (AC) if annual sales are below € 30 million.
- Initially, an additional benefit is considered as proven with marketing authorization and at least categorized as “non-quantifiable”.
- If the sales limit is exceeded, re-assessment against an AC defined by the G-BA follows and the reimbursement price is re-negotiated.
- This research aims to analyze the impact of a re-assessment on the price and to identify influencing factors.

Methods

- Publicly available G-BA documents on the initial and re-assessment of OD are systematically reviewed considering extent of the additional benefit (worse, no additional benefit, equal, better), costs of the AC and population size.
- The extracted findings are compared to the initial and re-negotiated price published in the Lauer-Taxe.
- Based on this pre-post-comparison, price changes are analyzed and influencing factors are identified.

Results

Figure 1: Discount on the reimbursement price after re-assessment¹⁾

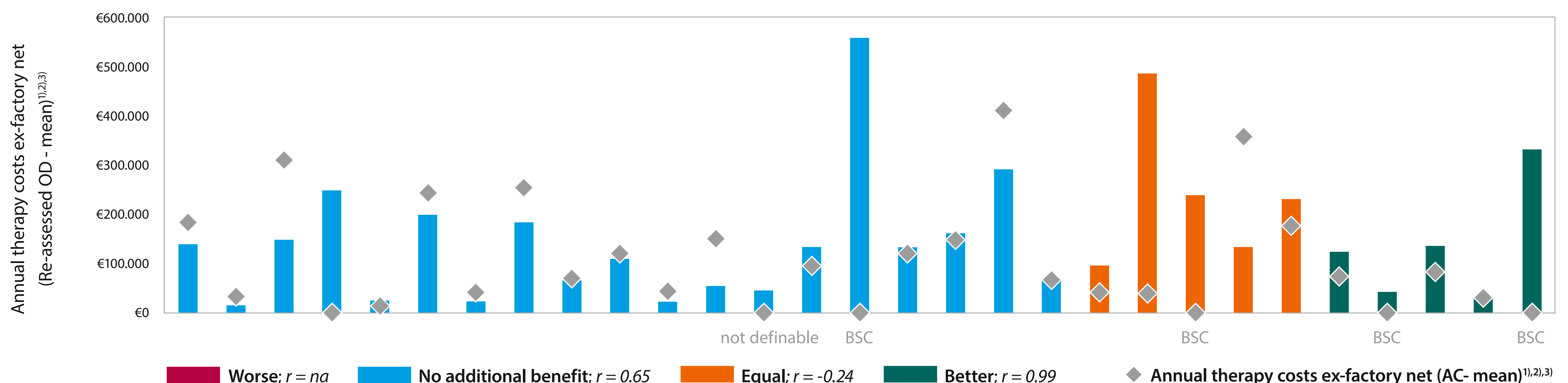


¹⁾ Simultaneously negotiated procedures after exceedance are included.

²⁾ For multiple subpopulations, the benefit extent of the largest group is shown.

³⁾ Analysis is based on ex-factory price adjusted for VAT (19%) and manufacturer discounts per §130a German Social Code Book. Temporary VAT reduction was applied for comparability.

Figure 2: Annual therapy costs: Comparator vs. Re-assessed orphan drug



- By September 15, 2025, 29 OD had undergone re-assessment:
 - In 1 case, the additional benefit worsened, resulting in a 61% price discount.
 - In 18 cases, no additional benefit was confirmed, with discounts ranging from 0–42%.
 - In 5 cases, the additional benefit was maintained, still resulting in discounts of up to 15%.
 - In 5 cases, the additional benefit improved, with price changes ranging from a 7% discount to a 14% markup.

- Analysis of the costs between re-assessed OD and its AC shows:
 - A moderate to strong correlation of the annual therapy costs when no additional benefit could be shown ($r = 0.65$)
 - A weak negative correlation between unchanged additional benefit and annual therapy costs ($r = -0.24$)
 - A very strong correlation between improved benefit and annual therapy costs ($r = 0.99$), supporting the link between clinical value and pricing.
- However, a correlation between the increase in patients and the amount of the discount could not be established ($r = 0.11$).

^{1),(2),(3)}: See footnotes Figure 1. BSC: Best Supportive Care

Conclusions & Recommendations

- Re-assessments under AMNOG can significantly impact OD pricing. If the required AC is not met in the clinical trials, re-assessment represents a risk, at least at the subpopulation level, and results in non-proven additional benefit.
- In contrast, if the AC is reflected in the clinical trials, re-assessment offers the opportunity to present further data and thus to maintain or even improve the initial assessment.
- Ultimately, changes in reimbursement prices closely follow the re-assessment outcome, reinforcing the need for early alignment with comparator requirements to avoid financial risks.

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2. The Federal Joint Committee (“Gemeinsamer Bundesausschuss/G-BA”) [Internet]: Overview of resolutions to benefit assessments [only German version up-to-date, last accessed: Sep 30th, 2025]: <https://www.g-ba.de/bewertungsverfahren/nutzenbewertung/>
3. Initial prices and reimbursement prices were taken from: “ABDATA Pharma-Daten Service der Werbe- & Vertriebsgesellschaft Deutscher Apotheker (WuV), ABDA-Artikelstamm”: <https://www.pharmazie.com/#/>