Evaluating the impact of benefit reassessment outcomes for orphan drugs surpassing the revenue limit in Germany

HTA188

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SUMMARY

OBJECTIVES

- To investigate the time to reassessment for orphan drugs in Germany.
- To analyse the impact of reassessment on the benefit assessment outcomes of these drugs.
- To examine trends in the ratings of reassessed orphan drugs and consider the potential implications of these trends.

METHODS

- A comprehensive analysis of Gemeinsamer Bundesausschuss (G-BA) benefit assessment decisions was conducted.
- The time to reassessment for orphan drugs and the impact on their benefit assessment outcomes were examined.
- Trends in the ratings of reassessed orphan drugs were analysed, and potential implications considered.

FINDINGS

- A total of 20 orphan drugs were reassessed by the G-BA after exceeding the annual turnover limit between June 2019 and May 2024.
- 65% of these drugs lost their added benefit rating, with 61.9% receiving a 'no additional benefit' rating and 4.4% receiving a 'lesser benefit' rating.
- The average time to reassessment was 49.9 months.
- There was a wide range of price reductions (range: 0-61.8%), with an average of 23.2%.

BACKGROUND & AIMS

- The GKV Financial Stabilization Act (GKV-FinStG), which came into effect in November 2022, aims to stabilise the financial health of Germany's statutory health insurance system (GKV) by implementing several key changes to the pricing and reimbursement of pharmaceuticals, including orphan drugs [1].
- Previously, all EMA-designated orphan drugs enjoyed a privileged status under the AMNOG (Arzneimittelmarkt-Neuordnungsgesetz) framework. They were exempt from the full benefit assessment required for other drugs, provided their annual revenue did not exceed €50 million [1].
- The new GKV-FinStG law has reduced this revenue threshold to €30 million, meaning that once an orphan drug's revenue exceeds €30 million, the privilege is revoked. This triggers a reassessment of its added benefit rating and a subsequent price renegotiation [1].
- This research aimed to explore the time to reassessment for orphan drugs, analyse reassessment outcomes, and examine trends in added benefit ratings and their potential impact on pricing in Germany.

METHODS

- A targeted search of G-BA benefit assessment decisions was conducted, focusing on orphan drugs that underwent reassessment after exceeding the specified annual turnover limit of €30 million (previously €50 million).
- Data was collected on 20 orphan drugs, covering 38 indications, that received reassessment decisions from the G-BA between June 2019 and May 2024.
- Key data points included the time taken for reassessment, any changes in the outcomes of benefit assessments, and subsequent adjustments in pricing. This approach allowed for a detailed understanding of the relationship between turnover thresholds, benefit reassessment outcomes, and price changes in the German market for orphan drugs.

RESULTS

- A total of 20 orphan drugs across 38 indications underwent reassessment by the G-BA after exceeding the annual turnover limit of €30 or €50 million between June 2019 and May 2024.
- Among these, 13 (65%) lost their added benefit rating, that is, they were found to have no proven benefit (13 [61.9%]), or a lesser benefit rating (1 [4.4%]). Notably, 11 (55%) of these drugs had multiple indications, which likely contributed to the increase in revenue required to exceed the turnover limit, as demonstrated in Figure 1.

Figure 1. Benefit reassessment outcome for orphan drugs between June 2019 and May 2024

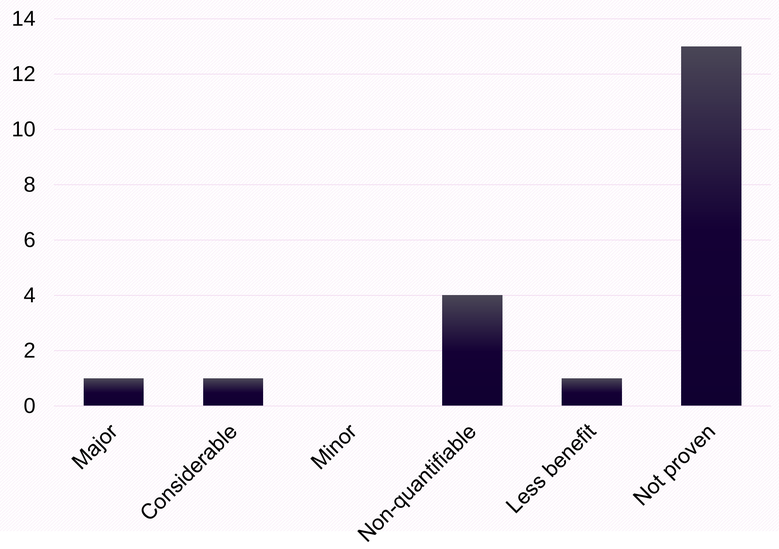


Figure 2. An illustrative sample of the variation in time to drug reassessment in Germany

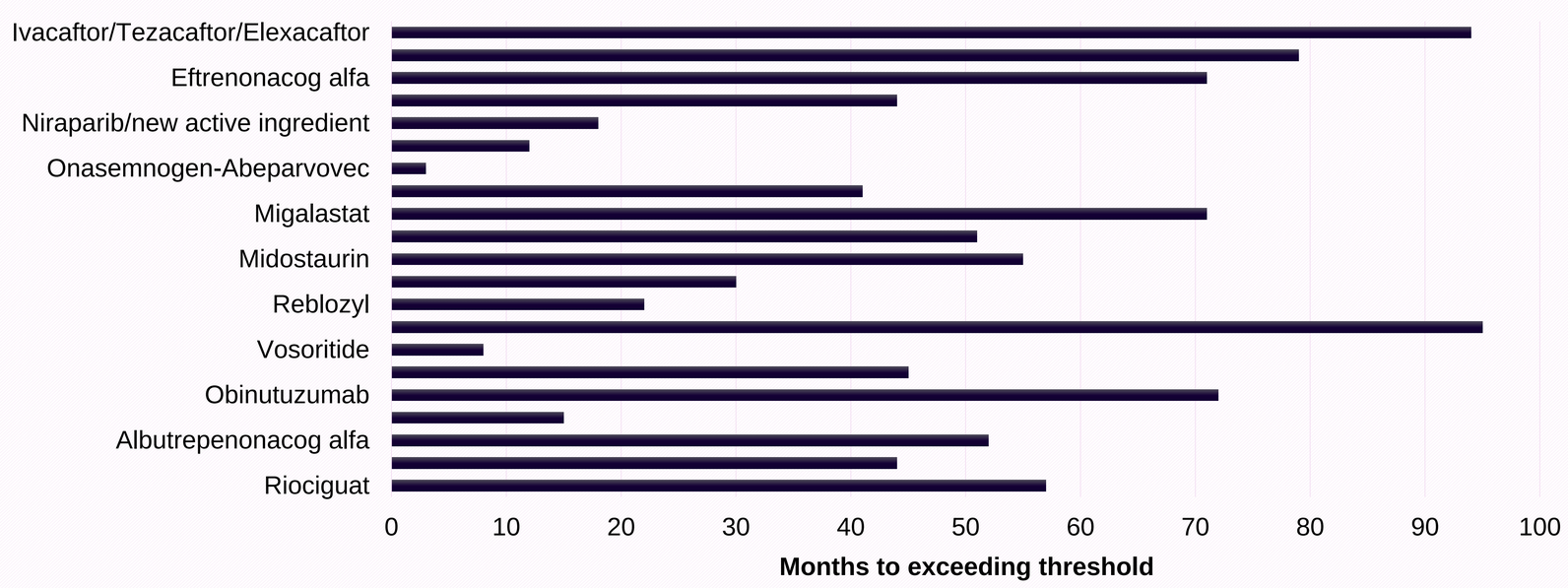
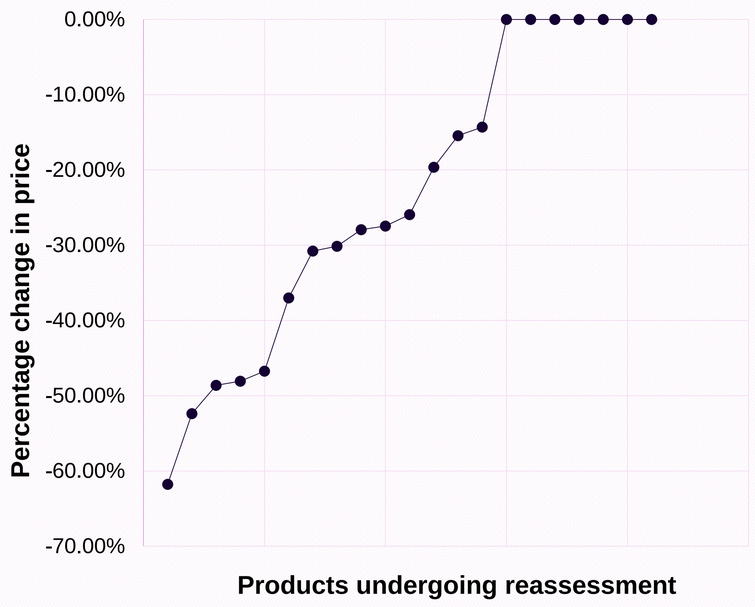


Figure 3. Percentage change in drug price post negotiation after benefit reassessment for drugs between June 2019 and May 2024



- The average time to reassessment was approximately 49.9 months.
- Price changes across the sample varied, with an average price reduction of 23.2% (range: 0-61.8%), as demonstrated in Figure 2.
- Figure 3 demonstrates the percentage change from the price pre- and post-negotiation of orphan drugs that underwent a benefit reassessment in Germany.

CONCLUSIONS

- The reassessment of orphan drugs that exceed the annual turnover limit has significant economic implications for pharmaceutical manufacturers. When an orphan drug loses its added benefit rating, it can often lead to substantial price reductions. Pharmaceutical companies must strategically plan for the possibility of reassessment.
- The potential for reassessment and subsequent price reductions might influence the research and development strategies of pharmaceutical companies. Companies may become more hesitant to invest in orphan drugs if the financial risks are perceived to be high. This could impact the overall innovation landscape for rare disease treatments.
- Balancing the need for innovation with cost containment and patient access remains a key challenge in the evolving landscape of orphan drug regulation.

References

1. *G-BA. Zum Entwurf eines Gesetzes zur finanziellen Stabilisierung der gesetzlichen Krankenversicherung. (GKV-Finanzstabilisierungsgesetz – GKV-FinStG). https://www.g-ba.de/downloads/17-98-5329/2022-07-12-PA-BMG_G-BA_Stellungnahme_GKV-FinStG.pdf; 2022 (accessed November 2024).*

Presented at ISPOR Europe, 17th-20th November 2024
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