REASSESSMENT OF ORPHAN DRUGS IN GERMANY AFTER EXCEEDING 50 M EURO SALES: EFFECTS ON ADDITIONAL BENEFIT AND REIMBURSEMENT PRICE

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BACKGROUND

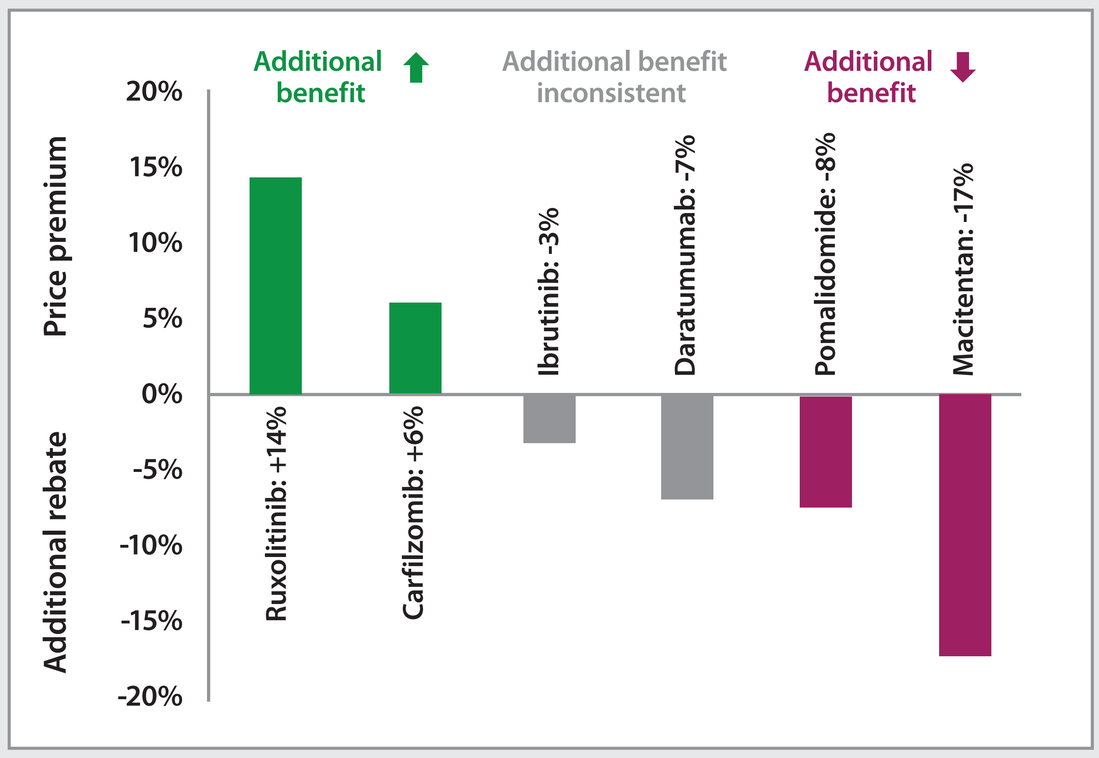
In the context of early benefit assessment of orphan drugs, additional benefit is considered as proven with marketing authorization and at least categorized as non-quantifiable.
However, if the orphan drug has generated expenses over 50 M € for statutory health insurance in the last 12 months, the manufacturer is obliged to prove the additional benefit against an appropriate comparative therapy. As a consequence of this reassessment, the reimbursement price will be renegotiated.
We analyzed the impact of this specific reassessment on additional benefit and on the resulting renegotiated reimbursement price.

METHODS

- We included all orphan drugs where sales had exceeded 50 M € (n=6) and benefit reassessment and price renegotiation had already been completed as of Oct 1st, 2019.
- We analyzed the price development of these medicinal products and included the negotiated reimbursement price before and after the 50 M € reassessment.
- Analysis is based on the resolutions of the Federal Joint Committee and the officially published pharmaceutical prices.
- Basis for the calculation are ex-factory prices minus mandatory rebates (ex-factory net).

RESULTS

Medicinal product	Additional benefit 1st assessment	Additional benefit after exceeding 50 M € sales
Ruxolitinib (Jakavi®)	Minor	**Considerable:** Advantage overall survival, reduction of symptoms, improvement quality of life
Carfilzomib (Kyprolis®)	Non-quantifiable	**Considerable:** Moderate prolongation of survival
Ibrutinib (Imbruvica®)	Non-quantifiable over all subpopulations	**Not proven:** Data not appropriate **Non-quantifiable:** Statistical advantage overall survival **Non-quantifiable:** Statistical advantage overall survival **Considerable:** Significant advantages adverse events and quality of life **Not proven:** Data not appropriate **Not proven:** Data not appropriate
Daratumumab (Darzalex®)	Non-quantifiable	**Considerable (new indication):** Moderate prolongation of survival **Not proven (50 M €):** Data not appropriate
Pomalidomide (Imnovid®)	Considerable	**Considerable:** Moderate prolongation of survival, positive effects for quality of life **Not proven:** Data not appropriate
Macitentan (Opsumit®)	Minor	**Not proven:** Data not appropriate



- Improved or substantiated additional benefit for ruxolitinib and carfilzomib resulted in price premiums of 14% and 6%
- Partially improved and partially degraded additional benefit over the subpopulations for ibrutinib resulted in an additional rebate of 3%
- Degraded additional benefit for the indication relevant to the 50 M € reassessment for daratumumab (a new indication was assessed and negotiated concurrently to the original indication) resulted in an additional rebate of 7%
- Partial degradation of additional benefit for pomalidomide results in an additional rebate of 8%
- The degradation of additional benefit for macitentan leads to the highest additional rebate of 17%

CONCLUSIONS

- If the requirements regarding the appropriate comparative therapy have been fulfilled in the clinical studies, reassessment offers the possibility of submitting further data and thus improving and/or substantiating additional benefit.
- Non-fulfillment concerning the appropriate comparative therapy is an actual risk, at least on subpopulation level, and results in non-proven additional benefit.
- The change of the reimbursement price largely corresponds to the reassessment outcome: Additional rebate in case of a worse rating, price premium in case of a better outcome.

REFERENCES

Prices taken from „ABDATA Pharma-Daten-Service der Werbe- & Vertriebsgesellschaft Deutscher Apotheker (WuV), ABDA-Artikelstamm“ – Current commercial data on pharmacy products.

Resolutions and decision rationale of Federal Joint Committee resolutions taken from the Federal Joint Committee website.

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