Citation

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Details on the benefit assessment of Balversa (erdafitinib)

No added benefit for formal reason

"Far removed from the reality of care and without taking into account the expert opinions of leading professional societies", is Johnson & Johnson's conclusion on the decision of the Federal Joint Committee (G-BA), which on 18 June 2025 did not certify any additional benefit for Balversa (erdafitinib) for the treatment of metastatic bladder cancer for formal reasons.

>> According to the pharmaceutical company Johnson & Johnson in a recent statement, there was no adequate targeted therapy option for patients with unresectable or metastatic urothelial carcinoma with fibroblast growth factor receptor-3 before the introduction of Balversa. The provision of a safe, targeted therapy is therefore "a significant advance for this small but vulnerable group of patients who are in palliative treatment situations". The THOR phase III trial could also have demonstrated the efficacy of Balversa: with a median overall survival of 12.1 months in the intervention arm compared to 7.8 months in the control group.

However, this does not count for the G-BA: in its assessment (1), it considers the extent and probability of an additional benefit compared to the appropriate comparator therapy (cisplatin in combination with gemcitabine) to be unproven. In its "supporting reasons" (2), the G-BA describes exactly why it came to its negative decision.

Firstly: According to the G-BA, the data from the THOR study are not suitable for the assessment of added benefit. The reason: According to the Federal Committee, the patients in the comparator arm of cohort 1 were treated with chemotherapy (vinflunine or docetaxel), which would not correspond to the appropriate comparator therapies for two patient groups. Therefore, no suitable data were available for an assessment of added benefit. At the time of the start of the phase III THOR study in 2018, the comparator therapy corresponded to the current standard of care. However, shortly before the start of the benefit assessment procedure, the G-BA redefined the appropriate comparator therapy (ACT) in May 2024.

In the commenting procedure, the professional associations DGHO and DGU involved in this process have therefore expressed their opposition both in their written comments submitted to the G-BA and in the oral hearing on 5 May 2025. hearing on 5 May 2025 that the G-BA's definition of zVT does not correspond to the reality of care and called for a reorientation. The G- BA can certainly adjust the definition of zVT in the ongoing procedure if it becomes apparent that it is not correct. In Johnson & Johnson's view, the fact that the G-BA is sticking to its own definition, contrary to expert opinion, and ignoring the reality of healthcare provision is not expedient and contradicts the principles of benefit assessment.

This raises the question of why the G-BA does not follow the findings on the standard of care from the commenting procedure. Secondly, although the active substances vinflunine and docetaxel used in the comparator arm of cohort 1 - in addition to paclitaxel - would represent the appropriate comparator therapy for the patient population, not all patients in cohort 1 represented the patient population a2. In order to form a population relevant for the benefit assessment, the company would have presented data for a subpopulation in its dossier, which it referred to as the analysis population. For this purpose, the company would have excluded 15 patients in the intervention arm and 79 in the comparator arm: As a result, of the 136 vs. 130 patients (erdafitinib vs. chemotherapy) in cohort 1, 121 vs. 51 patients would be considered in the analysis, meaning that significantly more were excluded from the analysis in the comparator arm than in the intervention arm.

The G-BA considers the procedure for forming the analysis population to be inappropriate because it breaks the structural equality of the study arms to be compared. At this point, Johnson & Johnson cannot understand why the G-BA did not relate the 5% rule introduced by the Medical Research Act (Section 35a (1) sentence 3 no. 7 in conjunction with (3) sentences 5 and 6 SGB V) to the authorised and therefore also assessment- relevant therapeutic indication (AWG).

In the G-BA's view, the calculation of the proportion of study participants who took part in trial sites within the scope of SGB V should therefore also include studies that did not take place in the AWG to be assessed. In the company's view, this would not be appropriate. This would have been one of the first cases in which the 5% rule would have been relevant at all and would have been up for decision. <<

by: MVF-Editor in Chief Peter Stegmaier

Literature:

1: https://t1p.de/19141 2: https://t1p.de/03ucx

EMEA approval status in Europe of Balversa (erdafitinib)

Land	Status der Erstattung
Deutschland	Erstattung erfolgt: GBA-Beschluss veröffentlicht
Österreich	Erstattung im Einzelfall
Irland	Erstattet
Niederlande	Erstattet
Norwegen	Erstattung: Empfohlen von NoMA
Schweden	Erstattung: Empfohlen von TLV
Schweiz	Erstattet
Spanien	Erstattet
Großbritannien	Erstattet: NICE und SMC werden veröffentlicht
Dänemark	Erstattung: Empfohlen von DMC
Polen	Erstattet
Kroatien	Erstattung im Einzelfall
Russland	Erstattung im Einzelfall
Tschechische Republik	Erstattung im Einzelfall
Königr. Saudi-Arabien	Erstattet
Israel	Erstattet

***Tab. 1:** EMEA approval status in Europe of Balversa (erdafitinib)*