Pomalidomide in multiple myeloma: No hints of added benefit

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Pomalidomide (trade name: Imnovid) has been approved since 2013 for the treatment of multiple myeloma that has returned and is difficult to treat. The drug is an option for adults who have received two or more prior treatment regimens, including treatment with the drugs lenalidomide and bortezomib, and in whom the last treatment had no sufficient effect. Pomalidomide is used in combination with the drug dexamethasone.

The German Institute for Quality and Efficiency in Health Care (IQWiG) examined in a dossier assessment whether this drug offers an added benefit over the appropriate comparator therapy. No such added benefit could be derived, however, because the dossier contained no suitable data.

As tumour inhibitor after unsuccessful pretreatments

Multiple myeloma is a rare life-threatening cancer disease where plasma cells become abnormal and multiply uncontrollably in the bone marrow. This leads to bone damage, impaired blood formation and a weakened immune system.

In combination with the glucocorticoid dexamethasone, pomalidomide is used to inhibit tumour growth in recurrent and refractory multiple myeloma: It is approved for the treatment of adults who have received at least two prior treatment regimens (including lenalidomide and

bortezomib), and in whom tumour growth has progressed under the last treatment.

Orphan drug exceeds turnover threshold

In drugs for rare diseases (orphan drugs), such as pomalidomide, the added benefit is formally regarded as proven by their approval. However, this only applies for as long as the annual turnover in the statutory health insurance does not exceed 50 million euros.

The annual turnover of pomalidomide has now exceeded this formal threshold, and the manufacturer had to present evidence on the medical added benefit of the drug in comparison with the appropriate comparator therapy in its dossier.

The G-BA distinguished between two treatment situations

The Federal Joint Committee (G-BA) distinguished between two patient groups and specified different appropriate comparator therapies for them: Patients for whom targeted therapy is suitable should receive individual treatment specified by the physician in compliance with the approval. Among other factors, this therapy depends on which treatments have already been tried.

If targeted therapy is no longer an option, therapy that provides the patient with the best possible, individually optimized, supportive treatment to alleviate symptoms and improve quality of life is to be used (best supportive care, BSC).

Targeted therapy: implementation and dosage inadequate

The dossier provided no relevant data for any of the two patient groups, however: The only study (MM003) with patients for whom targeted therapy is an option is inadequate for the derivation of an added benefit.

In the study, administration of pomalidomide in combination with low-dose dexamethasone was compared with uniform administration of high-dose dexamethasone to all study participants in the control arm. It was not explained in the dossier in how far individual factors such as prior therapies and response were considered. Treatment adjustments were not envisaged in the course of the study. Furthermore, the high dosage of dexamethasone does not comply with the approval. Hence the appropriate comparator therapy, i.e. individual targeted therapy specified by the physician, was not implemented adequately.

Treatment without targeted therapy: data are missing

The manufacturer presented no study data for the second treatment situation, i.e. patients without targeted therapy. Hence overall the dossier provided no hints of an added benefit of pomalidomide.

G-BA decides on the extent of added benefit

This dossier assessment is part of the early benefit assessment according to the Act on the Reform of the Market for Medicinal Products (AMNOG) supervised by the G-BA. After publication of the dossier assessment, the G-BA conducts a commenting procedure and makes a final decision on the extent of the added benefit.

More information: www.iqwig.de/download/A15-42_P … ertung-35a-SGB-V.pdf