Medical Xpress

Vandetanib in thyroid cancer: No proof of added benefit

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Vandetanib (trade name Caprelsa) has been approved in Germany since February 2012 for adult patients suffering from a particular form of aggressive thyroid cancer. The German Institute for Quality and Efficiency in Health Care (IQWiG) has now examined the added benefit of the drug pursuant to the Act on the Reform of the Market for Medicinal Products (AMNOG). According to the findings, there is no proof of added benefit, because no data on patient-relevant outcomes for those patients for whom the drug is approved were presented by the drug manufacturer in its dossier. In view of these deficiencies, IQWiG considered itself compelled to declare the contents of the dossier as "incomplete".

"Best supportive care" as the appropriate comparator therapy

As specified by the Federal Joint Committee (G-BA), vandetanib should be compared with "best supportive care". This means the best possible supportive therapy, optimized for the individual patient for alleviation of symptoms and improvement in the quality of life.

The only study relevant for this benefit assessment compared the administration of vandetanib in combination with "best supportive care" with a treatment consisting of "best supportive care" alone.

Study population differs from approval population

Vandetanib is approved for patients with an "aggressive and symptomatic" medullary thyroid cancer (MTC) in whom surgery is no longer possible, the cancer is already very large or from which secondaries (metastases) have already formed in other regions of the body. The European regulatory authority - the European Medicines Agency (EMA) - had restricted the therapeutic indication in such a way in order to produce an overall positive benefit-risk balance. This is because treatment with vandetanib also involves major risks and can, for example, lead to severe heart rhythm disturbances.

However, in the study presented by the manufacturer, patients in whom the course of the disease was not "aggressive and symptomatic" were also enrolled. This applied to a total of about 44% of the study participants. This means that the study population - in contrast to the statements made by the manufacturer - was much wider than just the approval population. A benefit assessment pursuant to AMNOG should, however, only be undertaken for patients for whom the drug is actually approved. But the analysis of the study presented by the manufacturer did not allow for this.

Separate analysis would have been possible

The pharmaceutical company did not provide a separate analysis of the data with regard to patient-relevant outcomes (mortality, morbidity, quality of life) for patients treated in accordance with the approval status. However, comments of the EMA from the approval process prove that this group of patients is clearly distinguishable within the study and therefore the company could have presented a separate analysis for the benefit assessment.

The incompleteness of the dossier does not allow a reliable assessment of the new drug. An added benefit of vandetanib compared with "best

supportive care" is therefore not proven.

G-BA decides on the extent of added benefit

The dossier assessment is part of the overall procedure for early benefit assessment conducted by the G-BA. After publication of the manufacturer's dossier and its assessment by IQWiG, the G-BA initiates a formal commenting procedure, which provides further information and can result in a change to the benefit assessment. The G-BA then decides on the extent of the added benefit, thus completing the early benefit assessment.

More information: www.g-ba.de/informationen/nutzenbewertung/31/

Provided by Institute for Quality and Efficiency in Health Care