Ruxolitinib in polycythaemia vera: Hint of non-quantifiable added benefit

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Ruxolitinib (trade name: Jakavi) has been approved since March 2015 for the treatment of adults with polycythaemia vera, a rare disease of the bone marrow. It can be used when the drug hydroxyurea is ineffective or not tolerated. 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.

According to the findings, ruxolitinib offers better relief of individual symptoms and improves quality of life. Dyspnoea and muscle cramps are more frequent, however. The study design generally limits the informative value of the data, which is why IQWiG can derive a hint of a non-quantifiable added benefit from them.

One quarter of the patients in the control arm not treated according to approval

The drug manufacturer used a multicentre, open-label, randomized controlled parallel group trial in its dossier (RESPONSE). It compared ruxolitinib with the best available therapy (BAT). The study design had two features that are important for the interpretation of the results. On the one hand, the BAT was individually specified only after the patients had been randomly assigned to the control group. On the other, only 75% of the participants in the control group were treated in compliance with the recommendations in the approval.

Since the BAT was specified only after randomization, no analyses can be conducted on patients with approval-compliant treatment in whom randomization was maintained. However, the results of the total population of the RESPONSE study were largely in line with the ones of the population with approval-compliant treatment, which is why also IQWiG used the data of the RESPONSE study for the benefit assessment. Overall, the data only have limited informative value, however.

Patients complain of fatigue less frequently

The results of the RESPONSE study showed that patients in the ruxolitinib arm complained of fatigue less frequently and considered their health status to be better. The participants who received ruxolitinib assessed their quality of life to be better at least with regard to physical function. However, there were also results to the disadvantage of the new drug: Dyspnoea and muscle cramps were more frequent.

Group differences were sufficiently large

The informative value of the results is limited because of the study design. However, the effects were so large that they cannot be caused by the study design alone. Overall, IQWiG derived a hint of a non-quantifiable added benefit from the dossier.

IQWiG had conducted the first assessment of ruxolitinib according to the Act on the Reform of the Market for Medicinal Products (AMNOG) in August 2014, but in a different therapeutic indication (myelofibrosis).

G-BA decides on the extent of added benefit

This dossier assessment is part of the early benefit assessment according

to AMNOG supervised by the Federal Joint Committee (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/A15-13_R … ertung-35a-SGB-V.pdf

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