Fingolimod: Advantages for some children and adolescents with highly active RRMS

June 24 2019

Credit: CC0 Public Domain

Since 2018, the immunosuppressant drug fingolimod has also been approved for children and adolescents aged 10 years and older whose relapsing remitting multiple sclerosis (RRMS) is highly active or severe and rapidly evolving. In an early benefit assessment conducted in the beginning of 2019, the German Institute for Quality and Efficiency in

Health Care (IQWiG) had investigated whether the drug has an added benefit for these patients. No added benefit was proven for three of four patient groups; a hint of a non-quantifiable added benefit was found for the fourth group.

Added benefit in health-related quality of life

In the course of the hearing at the Federal Joint Committee (G-BA), the manufacturer of the drug subsequently submitted data from the study on which its dossier was based. The G-BA therefore commissioned IQWiG to analyse these data in an addendum. Now it was also possible to analyse the data on the outcome "health-related quality of life" for young patients with highly active RRMS who need to switch their basic therapy: Whereas children and adolescents in the comparator arm reported a worsening in their quality of life at the end of the study, there was clear improvement in the fingolimod arm. This resulted in a hint of an added benefit.

No relevant differences in cognitive function

The original dossier additionally lacked detailed results on cognitive function testing in the subjects. The study used five tests, which together covered all core areas of cognitive functioning. However, only two of them were used in all study centres: In the Symbol Digit Modality Test, subjects were asked to use a simple code table to match digits to specific symbols; in the test on so-called visual-motor integration, children traced simple shapes such as crosses or circles. Hence, not all core domains of cognition are covered anymore and the results are therefore not informative. Regardless of this, there were no relevant differences between the study arms anyhow.

"The addendum has not changed the overall conclusion on added

benefit", says Stefan Lange, Deputy Director of IQWiG. "But we are pleased that we have been able to see informative data on health-related quality of life here. This is an extremely important treatment effect particularly for patients so young."

G-BA decides on the extent of added benefit

The 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 manufacturer's dossier and the IQWiG dossier assessment, the manufacturer submitted additional information in the commenting procedure. The G-BA subsequently commissioned IQWiG to assess the data subsequently submitted. IQWiG now presents this assessment in the form of an addendum. The G-BA makes a final decision on the extent of added benefit.

Provided by Institute for Quality and Efficiency in Health Care