Efmoroctocog alfa for hemophilia A: Added benefit not proven

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Efmoroctocog alfa (trade name: Elocta) has been approved since November 2015 for people with type A haemophilia. This is an inherited disorder that impairs blood clotting. The German Institute for Quality and Efficiency in Health Care (IQWiG) examined in a dossier assessment whether this new drug offers an added benefit over the appropriate comparator therapy both in prevention and in on-demand treatment. Such an added benefit cannot be derived from the dossier, however, because it contained no study data adequate for the research question.

Historical comparison: study pool incomplete

According to the Federal Joint Committee (G-BA), the new drug was to be compared with a recombinant or human plasma-derived coagulation factor VIII drug as appropriate comparator therapy. The drug manufacturer presented no data for such a direct comparison, however.

Instead, the dossier contained an unadjusted historical comparison on prophylactic treatment only. Data from a study on efmoroctocog alfa were compared with data from seven studies on the comparator therapy. A simplified search conducted by IQWiG showed that the study pool was incomplete. This was caused by an erroneous and incomplete search by the manufacturer for studies on the comparator therapy; no search at all was conducted in trial registries.

Information inadequate for benefit assessment

In addition, the data were inadequate. On the one hand, the manufacturer did not implement its own research question for the appropriate comparator therapy: It narrowed the inclusion criteria regarding the study population and the comparator therapy in such a way that patients under twelve years of age and studies on human plasma preparations were not included, for example. On the other, it only considered two outcomes: the number of annual bleeding events and the use of factor VIII preparations. However, all available results on patient-relevant outcomes from the four categories "mortality", "morbidity", "health-related quality of life" and "adverse events" are to be considered in a benefit assessment.

Hence an added benefit of efmoroctocog alfa in comparison with the appropriate comparator therapy is not proven.

More information: www.iqwig.de/download/A15-54_Efmoroctocog%20alfa_Kurzfassung_Nutzenbewertung-35a-SGB-V.pdf

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