

Turoctocog alfa in patients with hemophilia

A: Added benefit not proven

April 22 2014

Turoctocog alfa (trade name: NovoEight) has been approved since November 2013 for the prevention and treatment of bleeding in patients with haemophilia A. In an early benefit assessment pursuant to the "Act on the Reform of the Market for Medicinal Products" (AMNOG), the German Institute for Quality and Efficiency in Health Care (IQWiG) examined whether this new active ingredient offers an added benefit over the appropriate comparator therapy.

According to the findings, an added benefit of turoctocog alfa is not proven. As no relevant study is available for comparison with the appropriate comparator therapy, the benefit assessment of the drug manufacturer is based on its own deliberations and assumptions, for which, however, a valid data basis is lacking.

Other blood clotting factors VIII as comparator therapy

The deficiency in [blood clotting](#) factor VIII is inherited and linked to the X chromosome. This is why haemophilia A is almost exclusively seen in men, as they only have one X chromosome in their DNA. The defective blood clotting leads to strong spontaneous bleeding (or bleeding after injuries) in joints, muscles, and internal organs.

Turoctocog alfa is produced biotechnologically (recombinant) and is supposed to compensate the deficiency in blood clotting factor, i.e. the

tendency to bleed. As prophylactic treatment it has to be injected intravenously every 2 to 3 days.

The Federal Joint Committee (G-BA) specified blood clotting factor VIII agents produced biotechnologically or gained from human plasma as the appropriate comparator therapy.

Market entry does not prove added benefit

Out of various options, the manufacturer chose blood clotting factor VIII octocog alfa as a comparator, but limited its selection to a single third-generation agent of octocog alfa (trade name: Advate). This restriction does not correspond to the comparator therapy specified by the G-BA, as according to the G-BA's requirements, for a complete comparison all agents containing the [active ingredient](#) octocog alfa must be considered.

But even for the comparison chosen by the manufacturer, no relevant studies are presented. Instead the manufacturer bases its benefit assessment on fundamental deliberations and assumptions, which are inferred from different publications and statistics.

For instance, from the manufacturer's point of view, an "improvement in the safety of [health care](#)" and a "better integration of patients with haemophilia into a normal social life" already arise through market entry. However, an added benefit cannot be inferred from market entry alone.

Medical relevance for patients unclear

In comparison with octocog alfa the manufacturer claims an advantage for turoctocog alfa, as the new active ingredient can be stored at higher temperatures (up to 30 °C) than octocog alfa (25°C). Particularly during

the summer months and during travels to warmer countries, this is supposed to be beneficial and thus enable a "better integration of patients with haemophilia into a normal social life".

However, whether this difference in storage temperatures is medically relevant for patients needs to be investigated and proven in studies. The manufacturer's dossier provides no such data.

Therefore, the manufacturer's deliberations are overall not suited to assess the patient-relevant beneficial and harmful effects of turoctocog alfa versus the appropriate comparator therapy. The added benefit of the new active ingredient 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 assessments supervised by the G-BA. After publication of the [manufacturer's](#) dossier and IQWiG's assessment, the G-BA conducts a commenting procedure, which may provide further information and 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.

Provided by Institute for Quality and Efficiency in Health Care

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