Simoctocog alfa for haemophilia A: No suitable data

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Simoctocog alfa (trade name Nuwiq) has been approved since July 2014 for people with type A haemophilia, 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. Such an added benefit cannot be derived from the dossier, however, because the drug manufacturer did not submit any suitable data.

Studies only compared single-dose administration of the drugs

The manufacturer presented two randomized controlled trials (RCTs) with crossover design for the direct comparison with octocog alfa. Their primary outcome criterion was pharmacokinetics such as ingestion and excretion of the drug. In these two studies, the patients received a single dose of simoctocog alfa or octocog alfa and - after a wash-out phase - a second single dose of the respective other drug.

However, a single dose is insufficient to assess the added benefit. Patients with haemophilia require longterm treatment and prevention of bleeding. European Medicines Agency (EMA) guidelines therefore recommend a minimum duration of six months for studies that aim to test the advantages and disadvantages of haemophilia drugs used for prophylaxis. Incidentally, the manufacturer itself also derived no

advantage of its drug from these two studies.

No systematic search on one-arm octocog alfa studies

The pharmaceutical company claimed an added benefit of simoctocog alfa based on data from one-arm studies on the two drugs. Regarding octocog alfa, however, in its dossier it only referred to the Summaries of Product Characteristics and publicly available documents of the regulatory authorities.

It conducted no systematic search for studies as is required for the dossier. IQWiG researchers already found one further study on octocog alfa in a first orientation search. Since it was unclear whether the manufacturer considered all studies on octocog alfa, the data were unsuitable to derive an added benefit. On the basis of one-arm studies, this would only be possible anyway if the differences between the treatment groups were very high and hence so-called dramatic effects were present. The reason is that the certainty of results of studies without control group is low.

G-BA decides on the extent of added benefit

This 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 dossier assessment, the G-BA conducts a commenting procedure and makes a final decision on the extent of the added benefit.

An overview of the results of IQWiG's benefit assessment is given by a German-language executive summary. In addition, the website http://www.gesundheitsinformation.de, published by IQWiG, provides easily understandable German-language information on simoctocog alfa.

More English-language information will be available soon (Sections 2.1 to 2.6 of the dossier assessment as well as subsequently published health information on http://www.informedhealthonline.org).

More information: www.iqwig.de/download/A14-41_S … ertung-35a-SGB-V.pdf

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