IQWiG Reports – Commission No. A18-32

Rurioctocog alfa pegol (haemophilia A) –

Benefit assessment according to §35a Social Code Book V[1]

Extract

[1] Translation of the executive summary of the dossier assessment *Rurioctocog alfa pegol (Hämophilie A) – Nutzenbewertung gemäß § 35a SGB V* (Version 1.0; Status: 13 August 2018). Please note: This document was translated by an external translator and is provided as a service by IQWiG to English-language readers. However, solely the German original text is absolutely authoritative and legally binding.

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IQWiG thanks the medical and scientific advisor for his contribution to the dossier assessment. However, the advisor was not involved in the actual preparation of the dossier assessment. The responsibility for the contents of the dossier assessment lies solely with IQWiG.

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Executive summary of the benefit assessment

Background

In accordance with §35a Social Code Book (SBG) V, the Federal Joint Committee (G-BA) commissioned the Institute for Quality and Efficiency in Health Care (IQWiG) to assess the benefit of the drug rurioctocog alfa pegol. The assessment is based on a dossier compiled by the pharmaceutical company (hereinafter referred to as the "company"). The dossier was sent to IQWiG on 16 May 2018.

Research question

The aim of this report is to assess the added benefit of rurioctocog alfa pegol in comparison with the appropriate comparator therapy (ACT) in the treatment and prophylaxis of bleeding episodes in patients aged 12 years or older with haemophilia A (congenital factor VIII deficiency).

The ACT specified by the G-BA served as the basis for the research question presented in Table 2 for this benefit assessment.

Table 2[2]: Research question for the benefit assessment of rurioctocog alfa pegol

Research question	Indication	ACT[a]
1	Treatment and prevention of bleeding in patients from age 12 years with haemophilia A (congenital factor VIII deficiency).	**Recombinant** or human plasma-derived **coagulation factor VIII products**[b]
a: Presentation of the respective ACT specified by the G-BA. In cases where the ACT specified by the G-BA allows the company to choose a comparator therapy from several options, the respective choice by the company is printed in **bold**. b: Company's choice: Efmoroctocog alfa ACT: appropriate comparator therapy; G-BA: Federal Joint Committee		

The company followed the G-BA's specification and selected the recombinant coagulation factor efmoroctocog alfa from among the presented options.

The assessment was conducted by means of patient-relevant outcomes on the basis of the data provided by the company in the dossier. For prophylactic treatment, a minimum study duration of 6 months applies. The assessment of event-based treatment requires a study duration of at least 50 exposure days.

Results

The company did not identify any randomized controlled trial for the comparison of rurioctocog alfa pegol with the ACT either for prophylaxis or episodic treatment. Searching for non-randomized direct comparative studies as well as further studies with rurioctocog alfa pegol,

[2] Table numbers start with "2" as numbering follows that of the full dossier assessment.

the company identified 2 studies (study 261201 and study 261204), but it did not use them to prove added benefit. Both studies are unsuitable for deriving an added benefit since they do not allow a comparison with the ACT.

In summary, there is no hint of an added benefit of rurioctocog alfa pegol in comparison with the ACT; an added benefit is therefore not proven.

Probability and extent of added benefit, patient groups with therapeutically important added benefit[3]

On the basis of the results presented, the probability and extent of the added benefit of the drug rurioctocog alfa pegol in comparison with the ACT is assessed as follows:

Table 3: Rurioctocog alfa pegol – probability and extent of added benefit

Indication	**ACT[a]**	**Probability and extent of added benefit**
Treatment and prevention of bleeding in patients from age 12 years with haemophilia A (congenital factor VIII deficiency).	**Recombinant** or human plasma-derived **coagulation factor VIII products**[b]	Added benefit not proven
a: Presentation of the respective ACT specified by the G-BA. In cases where the ACT specified by the G-BA allows the company to choose a comparator therapy from several options, the respective choice by the company is printed in **bold**. b: Company's choice: Efmoroctocog alfa ACT: appropriate comparator therapy; G-BA: Federal Joint Committee		

The G-BA decides on the added benefit.

[3] On the basis of the scientific data analysed, IQWiG draws conclusions on the (added) benefit or harm of an intervention for each patient-relevant outcome. Depending on the number of studies analysed, the certainty of their results, and the direction and statistical significance of treatment effects, conclusions on the probability of (added) benefit or harm are graded into 4 categories: (1) "proof", (2) "indication", (3) "hint", or (4) none of the first 3 categories applies (i.e., no data available or conclusions 1 to 3 cannot be drawn from the available data). The extent of added benefit or harm is graded into 3 categories: (1) major, (2) considerable, (3) minor (in addition, 3 further categories may apply: non-quantifiable extent of added benefit, added benefit not proven, or less benefit). For further details see [1,2].

References for English extract

Please see full dossier assessment for full reference list.

1. Institute for Quality and Efficiency in Health Care. General methods: version 5.0 [online]. 10 July 2017 [Accessed: 04 June 2018]. URL: https://www.iqwig.de/download/General-Methods_Version-5-0.pdf.

2. Skipka G, Wieseler B, Kaiser T, Thomas S, Bender R, Windeler J et al. Methodological approach to determine minor, considerable, and major treatment effects in the early benefit assessment of new drugs. Biom J 2015; 58(1): 43-58

The full report (German version) is published under https://www.iqwig.de/en/projects-results/projects/drug-assessment/a18-32-rurioctocog-alfa-pegol-haemophilia-a-benefit-assessment-according-to-35a-social-code-book-v.9649.html.