IQWiG Reports – Commission No. A18-21

Patiromer (hyperkalaemia) –

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

Extract

[1] Translation of the executive summary of the dossier assessment *Patiromer (Hyperkaliämie) – Nutzenbewertung gemäß § 35a SGB V* (Version 1.0; Status: 27 June 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 (SGB) V, the G-BA commissioned IQWiG to assess the benefit of the drug patiromer. The assessment was based on a dossier compiled by the pharmaceutical company (hereinafter referred to as "the company"). The dossier was sent to IQWiG on 28 March 2018.

Research question

The aim of this report was to assess the added benefit of patiromer compared with individualized therapy specified by the physician as the appropriate comparator therapy (ACT) in adult patients with hyperkalaemia.

The benefit assessment is to answer 1 resulting research question, for which the G-BA has specified the ACT shown in Table 2.

Table 2 [2]: Research question for the benefit assessment of patiromer

Indication	Appropriate comparator therapy[a]
Hyperkalaemia in adults[b]	Individualized therapy specified by the physician, taking into account aetiology, severity, and symptoms. Optimization of the treatment of underlying diseases and comorbidities, particularly adjustment of the drug regimen, as well as any dietary change are measures taken as part of individualized therapy and deemed as standard therapy for hyperkalaemia. The respective drug approvals must be taken into account.
a: Appropriate comparator therapy specified by the G-BA b: It is assumed that the patients in this therapeutic indication do not suffer from potentially life-threatening hyperkalaemia, which would require emergency treatment. Other therapeutic interventions are available for emergency treatment. G-BA: Federal Joint Committee	

According to the G-BA, measures involved in individualized therapy, which represent the standard treatment of hyperkalaemia, include the optimization of the treatment of underlying diseases and comorbidities, particularly an adjustment of the drug regimen (and a change in diet, if applicable). According to the G-BA, the comparator arm of a study must allow for an adjustment to be made to this standard treatment. In the consultation, the G-BA again explicitly mentioned the treatment of hyperkalaemia itself as a potential part of this standard treatment; drugs approved for the indication hyperkalaemia are sodium and calcium polystyrene sulphonates.

The company followed the G-BA's specification in its choice of ACT.

The assessment was conducted based on patient-relevant outcomes from the data provided by

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

the company in the dossier. RCTs with a minimum length of 24 weeks were used to derive conclusions on added benefit.

Results

In its dossier, the company uses the placebo-controlled approval study OPAL-HK as the best available evidence for assessing added benefit, although the company states that this study fails to meet the inclusion criteria for the benefit assessment.

The study OPAL-HK is not suitable for deriving an added benefit of patiromer when compared to the ACT since the study's comparison arm fails to meet G-BA specifications concerning the ACT. Furthermore, the 8-week duration of the randomized part of the study is far too short.

For patients with hyperkalaemia, no relevant data are therefore available for assessing the added benefit of patiromer in comparison with the ACT. Therefore, there is no proof of added benefit.

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

Table 3 shows a summary of the probability and extent of added benefit of patiromer.

Table 3: Patiromer – probability and extent of added benefit

Indication	Appropriate comparator therapy[a]	Probability and extent of added benefit
Hyperkalaemia in adults[b]	Individualized therapy specified by the physician, taking into account aetiology, severity, and symptoms. Optimization of the treatment of underlying diseases and comorbidities, particularly adjustment of the drug regimen, as well as any dietary change are measures taken as part of individualized therapy and deemed the standard treatment of hyperkalaemia. The respective marketing authorizations must be taken into account.	Added benefit not proven

a: In each case, the comparator therapy defined by the G-BA is shown.
b: It is assumed that the patients in this therapeutic indication do not suffer from potentially life-threatening hyperkalaemia, which would require emergency treatment. Other therapeutic measures are available for emergency treatment.
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/a18-21-patiromer-hyperkalaemia-benefit-assessment-according-to-35a-social-code-book-v.9394.html.