Justification

Gemeinsamer Bundesausschuss

to the Resolution of the Federal Joint Committee (G-BA) on an Amendment of the Pharmaceuticals Directive (AM-RL): Annex XII – Benefit Assessment of Medicinal Products with New Active Ingredients According to Section 35a SGB V Emicizumab (haemophilia A with inhibitors, quality-assured application)

of 17 October 2019

Contents

1. Legal basis

According to Section 35a, paragraph 1 German Social Code, Book Five (SGB V), the Federal Joint Committee (G-BA) assesses the benefit of reimbursable medicinal products with new active ingredients. According to Section 35a, paragraph 6 SGB V, the G-BA may also arrange for a benefit assessment according to Section 35a, paragraph 1 SGB V for reimbursable medicinal products containing an active ingredient that is not a new active ingredient within the meaning of Section 35a, paragraph 1 SGB V if a new marketing authorisation with new data protection is granted for the medicinal product. This includes in particular the assessment of the additional benefit and its therapeutic significance. The benefit assessment is carried out on the basis of evidence provided by the pharmaceutical company, which must be submitted to the G-BA electronically, including all clinical trials the pharmaceutical company has conducted or commissioned, at the latest at the time of the first placing on the market as well as the marketing authorisation of new therapeutic indications of the medicinal product, and which must contain the following information in particular:

1. Approved therapeutic indications,
2. Medical benefit,
3. Additional medical benefit in relation to the appropriate comparator therapy,
4. Number of patients and patient groups for whom there is a therapeutically significant additional benefit,
5. Treatment costs for statutory health insurance funds,
6. Requirements for a quality-assured application.

The G-BA may commission the Institute for Quality and Efficiency in Health Care (IQWiG) to carry out the benefit assessment. According to Section 35a, paragraph 2 SGB V, the assessment must be completed within three months of the relevant date for submission of the evidence and published on the internet.

According to Section 35a, paragraph 3 SGB V, the G-BA shall pass a resolution on the benefit assessment within three months of its publication. The resolution is to be published on the internet and forms part of the Pharmaceuticals Directive.

According to Chapter 5, Section 20, paragraph 4 of the VerfO, the sub-committee on Medicinal Products may, in the event of a need for change in the sense of a factual and mathematical correction with regard to the information according to Chapter 5, Section 20, paragraph 3, no. 2 (Number of patients or demarcation of patient groups eligible for treatment) or no. 4 (Treatment costs) of the VerfO, make the corresponding changes by mutual consent.

2. Key points of the resolution

At its session on 20 September 2018, the G-BA passed a resolution on the benefit assessment of emicizumab in accordance with Section 35a SGB V. Following publication of the resolution on the website of the G-BA, the G-BA concluded that there is a need to adapt the information on the quality-assured application described in the resolution.

In the resolution of 20 September 2018, the section “Requirements for a quality-assured application” in sentence 4 of the training material refers to “specific information on the handling of thrombotic microangiopathy and thromboembolism, on the use of bypassing products, and on the influence of emicizumab on coagulation tests”. To clarify the facts, at the end of the sentence after “on the influence of emicizumab on coagulation tests”, the parenthetical words “(risk of misinterpretation)” shall be inserted.

3. Written statement procedure according to Section 92, paragraph 3a SGB V

The Pharmaceuticals Directive does not require the submission a written statement procedure according to Section 92, paragraph 3a SGB V. Pharmaceutical companies will not be adversely affected by the adjustment of the information in the quality-assured application of the active ingredient emicizumab; the amendment merely provides a factual specification of the quality-assured application.

4. Bureaucratic costs

The proposed resolution does not create any new or amended information obligations for care providers within the meaning of Annex II to Chapter 1 VerfO and, accordingly, no bureaucratic costs.

5. Process sequence

Following the adoption of the resolution, the necessity of the adjustment in the resolution with regard to the factual specification in the quality-assured application in the resolution of 20 September 2018 on an amendment of the Pharmaceuticals Directive Annex XII – Resolutions on the benefit assessment of medicinal products with new active ingredients according to Section 35a SGB V – emicizumab has become apparent.

The matter was discussed in the Working Group Section 35a as well as the medicinal products sub-committee.

At its session on 17 October 2019, the plenum unanimously adopted the amendment to the AM-RL with regard to a factual correction of the data for the quality-assured application in the resolution of 20 September 2018.

Chronological course of consultation

Session	Date	Subject of consultation
Working group Section 35a	3 September 2019	Consultation on the facts of the case
Subcommittee Medicinal product	10 September 2019	Consultation on an amendment decision regarding the quality assured-application of the Resolution of 20 September 2018.
Plenum	17 October 2019	Resolution on an amendment decision regarding the quality assured-application of the Resolution of 20 September 2018.

Berlin, 17 October 2019

Federal Joint Committee (G-BA)
in accordance with Section 91 SGB V
The chair

Prof Hecken