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

Tisagenlecleucel (acute lymphoblastic B-cell leukaemia, quality-assured application)

of 1 August 2019

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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.

For medicinal products for the treatment of a rare disease (orphan drugs) that are approved according to Regulation (EC) No. 141/2000 of the European Parliament and of the Council of 16 December 1999, according to Section 35a, paragraph 1, sentence 11, 1st half of the sentence SGB V, the additional medical benefit is deemed to be proven through the grant of the marketing authorisation. Evidence of the medical benefit and the additional medicinal benefit in relation to the appropriate comparator therapy need not be submitted (Section 35a, paragraph 1, sentence 11, 2nd half of the sentence SGB V). Section 35a, paragraph 1, sentence 11 1st half of the sentence SGB V thus guarantees an additional benefit for an approved orphan drug, although an evaluation of the orphan drug in accordance with the principles laid down in Section 35a, paragraph 1, sentence 3, numbers 2 and 3 SGB V in conjunction with the Chapter 5, Sections 5 et seq. of the Rules of Procedure, G-BA (VerfO) has not been carried out. Only the extent of the additional benefit has to be demonstrated.

However, the restrictions on the benefit assessment of orphan drugs resulting from the statutory obligation to the marketing authorisation do not apply if the turnover of the medicinal product with the SHI at pharmacy retail prices including VAT exceeds €50 million in the last 12 calendar months According to Section 35a, paragraph 1, sentence 12 SGB V, the pharmaceutical company must then, within three months of being requested to do so by the G-BA, submit evidence according to Chapter 5, Section 5, subsection 1–6 VerfO, in particular regarding the additional medicinal benefit in relation to the appropriate comparator therapy as defined by the G-BA according to Chapter 5, Section 6 VerfOand prove the additional benefit in comparison with the appropriate comparator therapy.

In accordance with Section 35a, paragraph 2 SGB V, the G-BA decides whether to carry out the benefit assessment itself or to commission the Institute for Quality and Efficiency in Health Care (IQWiG). On the basis of the statutory requirement in Section 35a, paragraph 1, sentence 11 SGB V that the additional benefit of an orphan drug is deemed to have been proven through the grant of marketing authorisation, the G-BA modified the procedure for the benefit assessment of orphan drugs at its session on 15 March 2012 to the effect that, in the case of orphan drugs, the G-BA initially no longer independently determines an appropriate comparator therapy as the basis for the legally permissible assessment of the extent of an additional benefit to be assumed by law. Rather, the extent of the additional benefit provided by the G-BA is evaluated exclusively on the basis of the approval studies.

Accordingly, at its session on 15 March 2012, the G-BA amended the mandate given to the IQWiG in its resolution of 1 August 2011 for the benefit assessment of medicinal products with new active ingredients in accordance with Section 35a, paragraph 2 SGB V in such a way that, in the case of orphan drugs, IQWiG is only commissioned to carry out a benefit assessment in case of a previously defined comparator therapy when the sales volume of the drug concerned has exceeded the legal limit of €50 million and is therefore subject to an unrestricted benefit assessment (*cf* Section 35a, paragraph 1, sentence 12 SGB V. According to Section 35a paragraph 2 SGB V, the assessment by the G-BA 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.

2. Key points of the resolution

At its session on 7 March 2019, the G-BA decided on the benefit assessment of tisagenlecleucel (acute lymphoblastic B-cell leukaemia) 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 requirements for a quality-assured application described in the resolution.

In the resolution of 7 March 2019, section 1.3.3 “Requirements for the qualification of the care service” refers only to the term “caregiver” in relation to the training of the ward management or its replacement and shift management. Contrary to the fact that it was not explicitly mentioned, this term also referred to caregivers specially trained in paediatric care when the resolution was adopted. In order to clarify this situation, in paragraphs 1.3.3.1 and 1.3.3.2 the term “caregiver” is followed by “or paediatric caregiver”.

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

The amendment of the Pharmaceuticals Directive does not require the submission a written statement procedure according to Section 92, paragraph 3a of the German Social Code, Book V. Pharmaceutical companies are not adversely affected by the adjustment of the information in the quality-assured application of the active ingredient tisagenlecleucel; 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 7 March 2019 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 – Tisagenlecleucel 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 1 August 2019, the plenum unanimously adopted the amendment to the Pharmaceuticals Directive with regard to an objective specification in the quality-assured application in the resolution.

Chronological course of consultation

Session	Date	Subject of consultation
Working group Section 35a	17 July 2019	Consultation on the facts of the case
Subcommittee Medicinal products	23 July 2019	Consultation on an amendment decision regarding the quality assured-application of the Resolution of 7 March 2019.
Plenum	1 August 2019	Resolution on an amendment decision regarding the quality assured-application of the Resolution of 7 March 2019.

Berlin, 1 August 2019

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

Prof Hecken