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 Betibeglogene Autotemcel (β-Thalassaemia) (Treatment Costs)

of 3 December 2020

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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, the additional medical benefit is considered to be proven through the grant of the marketing authorisation in accordance with Section 35a, paragraph 1, sentence 11, 1st half of the sentence SGB V. Evidence of the medical benefit and the additional medical 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 assessment of the orphan drug in accordance with the principles laid down in Section 35a, paragraph 1, sentence 3, Nos. 2 and 3 SGB V in conjunction with Chapter 5, Sections 5 et seq. of the Rules of Procedure (VerfO) of the G-BA has not been carried out. In accordance with Section 5, paragraph 8 AM-NutzenV, only the extent of the additional benefit is to be quantified indicating the significance of the evidence.

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 selling prices and outside the scope of SHI-accredited medical care, including VAT, exceeds € 50 million during the last 12 calendar months. In accordance with 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 in accordance with Chapter 5, Section 5, paragraphs 1–6 VerfO, in particular regarding the additional medical benefit in relation to the appropriate comparator therapy as defined by the G-BA according to Chapter 5, Section 6 VerfO and 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 solely legally permissible assessment of the extent of an additional benefit to be assumed by law. Rather, the extent of the additional benefit provided is assessed exclusively on the basis of the pivotal studies by the G-BA indicating the significance of the evidence.

Accordingly, at its session on 15 March 2012, the G-BA amended the mandate issued to the IQWiG by 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 to that effect that, in the case of orphan drugs, the IQWiG is only commissioned to carry out a benefit assessment in the case of a previously defined comparator therapy when the sales volume of the medicinal product 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 of 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 decides 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 Subcommittee 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 resolution insofar as this does not affect the core content of the directive.

2. Key points of the resolution

At its session on 14 May 2020, the G-BA passed a resolution on the benefit assessment of betibeglogene autotemcel 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 treatment costs described in the resolution.

Complete myeloablative conditioning must be performed before infusion of betibeglogene autotemcel. In the resolution of 14 May 2020, the costs for myeloablative conditioning were presented on the basis of the pharmacy sales price less statutory rebates for the active ingredient busulfan used in the clinical studies. However, because myeloablative conditioning takes place exclusively in the context of inpatient care, the information on the additional costs of myeloablative conditioning incurred by the SHI system is adjusted and indicated on the basis of inpatient costs.

According to the information in the justification of the resolution of 14 May 2020 and the product information, the calculation of the additionally required SHI services for the annual treatment costs is derived as follows.

"Costs for additionally required SHI services:

Only costs directly related to the use of the medicinal product are taken into account. If there are regular differences in the necessary use of medical treatment or in the prescription of other services in the use of the medicinal product to be assessed in accordance with the product information, the costs incurred for this must be taken into account as costs for additionally required SHI services.

Medical treatment costs, medical fee services, and costs incurred for routine examinations (e.g. regular laboratory services such as blood count tests) that do not exceed standard expenditure in the course of the treatment are not shown.

Betibeglogene autotemcel is a cell product produced from autologous CD34+ stem cells. To obtain the cell material, HSC mobilisation and stem cell apheresis is therefore regularly necessary. Because HSC mobilisation and stem cell apheresis are part of the production of the medicinal product in accordance with Section 4, paragraph 14 AMG, no further costs are incurred in this respect for the medicinal product to be assessed.

Complete myeloablative conditioning must be performed before infusion of betibeglogene autotemcel. However, because this takes place exclusively in the context of inpatient care, the additional costs incurred by the SHI system in the inpatient sector are presented in the resolution. The product information of betibeglogene autotemcel does not contain any explicit guidelines on the type and duration of the medicinal products to be used for myeloablative conditioning. In addition, the costs of the active ingredients used for this purpose (e.g. for the

active ingredient busulfan used in the clinical studies) may be incurred in the form of additional hospital-specific charges. The additionally required SHI services can therefore not be quantified".

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

The amendment of the Pharmaceuticals Directive does not require the implementation of a written statement procedure according to Section 92, paragraph 3a SGB V. The pharmaceutical company is not adversely affected by the correction of the information on the costs of the active ingredient betibeglogene autotemcel; the amendment merely provides a factual correction of the cost representation suggested by the pharmaceutical company.

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 calculation of the annual treatment costs in the resolution of 14 May 2020 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 – betibeglogene autotemcel has become apparent.

The matter was discussed in the Working Group Section 35a as well as in the Subcommittee on Medicinal Products.

At its session on 3 December 2020, the plenum unanimously adopted the amendment to the AM-RL with regard to a factual correction in the resolution of 14 May 2020.

Chronological course of consultation

Session	Date	Subject of consultation
Working group Section 35a	4 November 2020 18 November 2020	Consultation on the facts of the case
Subcommittee on Medicinal Products	24 November 2020	Consultation on an amendment resolution regarding the information on costs in the resolution of 14 May 2020
Plenum	3 December 2020	Adoption of the resolutions on an amendment resolution regarding the information on costs in the resolution of 14 May 2020

Berlin, 3 December 2020

Federal Joint Committee
in accordance with Section 91 SGB V
The Chair

Prof. Hecken