Gemeinsamer Bundesausschuss

Justification

of 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) Exagamglogene autotemcel (sickle cell disease); requirement of routine practice data collection and evaluations – Amendment

of 18 September 2025

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1. Legal basis

According to Section 35a, paragraph 3b, sentence 1 SGB V, the Federal Joint Committee (G-BA) can demand the pharmaceutical company to submit routine practice data collections and evaluations for the purpose of the benefit assessment within a reasonable period of time for the following medicinal products:

1. in the case of medicinal products authorised to be placed on the market in accordance with the procedure laid down in Article 14, paragraph 8 of Regulation (EC) No. 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency (OJ L 136, 30.4.2004, p. 1), as last amended by Regulation 162 Rules of Procedure last revised: 16 December 2020 (EU) 2019/5 (OJ L 4, 7.1.2019, p. 24), or for which a marketing authorisation has been granted in accordance with Article 14-a of Regulation (EC) No. 726/2004; and
2. for medicinal products approved for the treatment of rare diseases under Regulation No. 141/2000.

2. Key points of the resolution

At their session on 21 December 2023, the G-BA decided on the requirement of routine practice data collection and evaluations for the active ingredient exagamglogene autotemcel in accordance with Section 35a SGB V and amended by resolution of 7 November 2024.

The marketing authorisation of exagamglogene autotemcel was granted by the European Commission on 9 February 2024.

Due to the marketing authorisation and in the course of the review of the study documents for the RPDC, the G-BA have made changes with regard to the requirement of routine practice data collection and evaluations for exagamglogene autotemcel (sickle cell disease).

On the changes in detail

The planned therapeutic indication of exagamglogene autotemcel for sickle cell disease was as follows during the marketing authorisation procedure: "Exagamglogene autotemcel is indicated for the treatment of severe sickle cell disease in patients 12 years of age and older with recurrent vaso-occlusive crises who have the genotype ßS/ßS, ßS/ß0 or ßS/ß+, for whom haematopoietic stem cell transplantation is an option and for whom no HLA-compatible, related haematopoietic stem cell donor is available."

The resolution on the requirement of routine practice data collection and evaluations was adopted on 21 December 2023.

On 9 February 2024, the European Commission issued the marketing authorisation for exagamglogene autotemcel. According to the marketing authorisation, the therapeutic indication for sickle cell disease is as follows:

"Casgevy is indicated for the treatment of severe sickle cell disease (SCD) in patients 12 years of age and older with recurrent vaso-occlusive crises (VOCs) for whom haematopoietic stem cell (HSC) transplantation is appropriate and a human leukocyte antigen (HLA)-matched related HSC donor is not available".

The following changes arise:

The information on specific genotypes is omitted in the therapeutic indication according to the marketing authorisation. In addition, editorial adjustments to the wording of the authorisation text are required.

The marketing authorisation must be taken into account in the routine practice data collection. Pursuant to the marketing authorisation, the therapeutic indication and the patient population in the PICO scheme are accordingly adjusted in the resolution on the requirement of routine practice data collection and evaluations.

The adjustment of the patient population and the therapeutic indication has no influence on the indicative sample size estimate for the required routine practice data collection, as the changes to the authorisation text have no or no relevant influence on the assumptions on which the sample size estimate is based. Irrespective of this, it cannot be assumed that the omission of the genotype definition in the authorisation text will result in a reduction in the patient numbers.

3. Submission according to Section 35a, paragraph 3b, sentences 7 and 8 SGB V

A new submission procedure need not be carried out as the therapeutic indication and the patient population was already the subject of the submission procedure - also involving the higher federal authorities - as part of the adoption of the resolution on the requirement of routine practice data collection and evaluations of 21 December 2023 and the adjustment does not lead to any significant change in the factual basis that would directly affect those entitled to make a statement, thus triggering a new right to make a statement, see Chapter 1 Section 14, paragraph 1 of the G-BA's Rules of Procedure.

4. Bureaucratic costs calculation

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

Subsequent to the adoption of the resolution of 21 December 2023 on an amendment to the Pharmaceuticals Directive (AM-RL) Annex XII – Resolutions on the benefit assessment of medicinal products with new active ingredients according to Section 35a SGB V – exagamglogene autotemcel, an amendment to the resolution is necessary due to the granting of the marketing authorisation.

The issue was discussed in the working group WG RPDC and in the Subcommittee on Medicinal Products.

At their session on 18 September 2025, the plenum adopted by consensus a resolution to amend the AM-RL.

Chronological course of consultation

Session	Date	Subject of consultation
WG RPDC	18 August 2025 4 September	Consultation on the issue
Subcommittee on Medicinal Products	9 September 2025	Consultation on the amendment to the resolution of 21 December 2023
Plenum	18 September 2025	Resolution on the amendment to the resolution of 21 December 2023

Berlin, 18 September 2025

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

Prof. Hecken