Gemeinsamer Bundesausschuss

Justification

of the Resolution of the Federal Joint Committee (G-BA) on the 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); restriction of the authority to supply care

of 21 December 2023

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1. Legal basis

According to Section 35a, paragraph 3b, sentence 2 SGB V, the Federal Joint Committee may decide for a medicinal product that is the subject of a resolution according Section 35a, paragraph 3b, sentence 1 SGB V that the authority to supply insured persons such a medicinal product at the expense of the statutory health insurance is restricted to those care providers who participate in the required routine practice data collection according to Section 35a, paragraph 3b SGB V (restriction of the care providers' authority to supply care). The resolution is to be published online and is part of the Pharmaceuticals Directive (AM-RL).

2. Key points of the resolution

At its session on 21 December 2023, the G-BA decided on the restriction of the authority to supply care according to Section 35a, paragraph 3b, sentence 2 SGB V for application in the case of sickle cell disease. The active ingredient exagamglogene autotemcel in sickle cell disease is the subject of a resolution on the requirement of routine practice data collection according to Section 35a, paragraph 3b, sentence 1 SGB V.

The restriction of the authority to supply care to those care providers who participate in the required routine practice data collection according to Section 35a, paragraph 3b SGB V aims to obtain complete and valid data from the care of insured persons with the medicinal product and to prevent only fragmentary data collection in order to obtain reliable, suitable data for the purposes of the benefit assessment.

The need for information for a benefit assessment of exagamglogene autotemcel has led to the research question of a (long-term) additional benefit compared to the appropriate comparator therapy for the approved patient population. IQWiG's corresponding data collection activities as part of drawing up of the concept for routine practice data collection showed that the ongoing and planned studies, including the extension studies, are unsuitable for addressing existing gaps in the evidence as no comparison is performed in those studies. Moreover, the Post Authorisation Safety Study (PASS) required by the regulatory authority only covers part of the population of the approved therapeutic indication of exagamglogene autotemcel. The research question of routine practice data collection requires the collection of comparator data.

The expected eligible number of patients who can be treated with exagamglogene autotemcel is low because sickle cell disease is a rare genetic disease, treatment with exagamglogene autotemcel is not considered for all subjects with sickle cell disease and other therapy options are available in the present therapeutic indication.

In order to ensure a data stock that is sufficient for the routine practice data collection, it is necessary that the data collection is as complete as possible, at least from the care context of insured persons who are administered exagamglogene autotemcel.

Care providers within the meaning of Chapter 5, Section 66 of the G-BA's Rules of Procedure (VerfO) are physicians participating in SHI-accredited medical care, medical care centres and

facilities according to Section 95 SGB V, as well as hospitals approved for care provision according to Section 108 SGB V.

Participation in the required routine practice data collection is ensured by the proper (proven in writing) participation of the (approved) healthcare provider in the data collection for the required routine practice data collection on the basis of the confirmed study protocol of the pharmaceutical company. In this context, efforts must also be made to ensure that the data transmission is as complete as possible.

3. 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.

4. Process sequence

In order to hold consultations and prepare a recommendation for a resolution on the initiation of a written statement procedure for the restriction of the authority to supply care according to Section 35a, paragraph 3b, sentence 2 SGB V, the Subcommittee on Medicinal Products commissioned a working group (WG routine practice data collection (RPDC)) consisting of the members nominated by the leading organisations of the care providers, the members nominated by the National Association of Statutory Health Insurance Funds, and the representative(s) of the patient organisations. Representatives of the IQWiG also participate in the sessions. The working group discussed the amendment of the AM-RL in its session on 5 October 2023.

The draft resolution to initiate a written statement procedure was discussed at the session of the Subcommittee on Medicinal Products on 10 October 2023 and the draft resolution was consented to.

At its session on 10 October 2023, the Subcommittee unanimously decided to initiate the written statement procedure according to Chapter 1 Section 10, paragraph 1 of the G-BA's Rules of Procedure.

The written statement procedure was carried out. After submitting their written statement, the assessment experts waived their right to an oral hearing.

The evaluation of the written statements received was discussed at the session of the subcommittee on 12 December 2023, and the draft resolution was approved.

At its session on 21 December 2023, the plenum adopted a resolution to amend the Pharmaceuticals Directive.

Chronological course of consultation

Session	Date	Subject of consultation
WG RPDC	5 October 2023	Consultation on the amendment of the AM-RL
Subcommittee Medicinal products	10 October 2023	Discussion and consensus on the draft resolution Resolution to initiate the written statement procedure on the amendment of the AM-RL Scheduling the oral hearing
WG RPDC	20 November 2023	Consultation on the statements received
WG RPDC	7 December 2023	Consultation on the draft resolution and evaluation of the written statement procedure
Subcommittee Medicinal products	12 December 2023	Concluding discussion of the draft resolution
Plenum	21 December 2023	Adoption of the resolution on the amendment of Annex XII AM-RL

Berlin, 21 December 2023

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

Prof. Hecken