Gemeinsamer Bundesausschuss

Justification

of the Resolution of the Federal Joint Committee (G-BA) on an Amendment of the Pharmaceuticals Directive
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 7 November 2024

Contents

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 its session on 21 December 2023, the G-BA decided on the requirement of routine practice data collection (RPDC) and evaluations for the active ingredient exagamglogene autotemcel in accordance with Section 35a SGB V.

Following adoption of the resolution, the European Commission decided to temporarily suspend the marketing authorisation of voxelotor.

This results in changes regarding the requirement of routine practice data collection and evaluations for exagamglogene autotemcel (sickle cell disease) by the G-BA.

On the changes in detail

The active ingredient voxelotor was approved in the European Union on 14 February 2022. On 4 October 2024, the European Commission decided to suspend the approval for placing on the (German) market of voxelotor until a final decision is taken as part of the ongoing procedure under Article 20 of Regulation (EC) No. 726/2004. The background to this is a recommendation by the Committee for Medicinal Products for Human Use (CHMP) dated 26 September 2024 based on new findings on the safety of voxelotor. Therapy with voxelotor was characterised by an increase in vaso-occlusive crises and an increased incidence of deaths, which, according to the CHMP's assessment, lead to the conclusion that the benefit-risk ratio of voxelotor is currently not considered positive. Based on CHMP’s recommendation, the European Commission decided by implementing decision of 4 October 2024 to suspend the placing on the market and supply of the medicinal product Oxbryta with the active ingredient

voxelotor in all affected EU Member States and to recall all batches on the EU market, including pharmacies and hospitals.

Due to the safety concerns that led to the suspension of the marketing authorisation, the requirements for the designation of voxelotor as an RPDC comparator are not met. Furthermore, it can be assumed that voxelotor will no longer be used in medical treatment practice in Germany.

For this reason, voxelotor will be deleted from the list of comparators in the context of patient-individual therapy for the routine practice data collection of exagamglogene autotemcel.

The deletion of voxelotor from the patient-individual therapy of the comparator arm has no influence on the indicative sample size estimate for the required routine practice data collection, since the sample size estimate is primarily based on effect sizes of hydroxycarbamide and voxelotor was not taken into account here.

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

A new submission procedure is not need to be carried out as the inclusion of the comparator voxelotor was already the subject of the submission procedure as part of the adoption of the resolution on the requirement of routine practice data collection and evaluations of 21 December 2023 and the deletion of the comparator 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 suspension of the marketing authorisation of voxelotor.

The issue was consulted in the working group routine practice data collection (WG RPDC) and in the Subcommittee on Medicinal Products.

At its session on 7 November 2024, the plenum adopted by consensus a resolution to amend the AM-RL.

Chronological course of consultation

Session	Date	Subject of consultation
WG RPDC	21 October 2024	Consultation on the issue
Subcommittee Medicinal products	29 October 2024	Consultation on the amendment to the resolution of 21 December 2023
Plenum	7 November 2024	Resolution on the amendment to the resolution of 21 December 2023

Berlin, 7 November 2024

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

Prof. Hecken