Gemeinsamer Bundesausschuss

Justification

on the Resolution of the Federal Joint Committee (G-BA) on the Suspension of a Consultation Procedure under Section 35a paragraph 3b SGB V Marstacimab (haemophilia A and B); requirement of routine practice data collection and evaluations

of 5 December 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

The centralised marketing authorisation procedure of the European Medicines Agency (EMA) for the active ingredient marstacimab started in October 2023.

By resolution of 4 April 2024, the G-BA initiated a procedure for the requirement of a routine practice data collection according to Section 35a, paragraph 3b, sentence 1 SGB V for the active ingredient marstacimab in the therapeutic indication haemophilia A and B.

The initiation of the procedure was based on the EMA's orphan designation - available at the time of the resolution - for the active ingredient marstacimab of 14 October 2016 for haemophilia A (EU/3/16/1752) and of 13 December 2023 for haemophilia B (EU/3/23/2866).

A concept was drawn up in preparation for the resolution on the requirement of routine practice data collection and evaluations. The concept contains in particular requirements for:

1. the type, duration and scope of data collection,
2. the research question (PICO framework: patient/population, intervention, comparison, outcomes) that is to be the subject of the data collection and evaluations, including the patient-relevant endpoints to be recorded,
3. the data collection methods,
4. the evaluations by the pharmaceutical company according to Section 50, paragraph 2 of the VerfO.

The G-BA decides whether to prepare the concept itself or to commission the Institute for Quality and Efficiency in Health Care (IQWiG) to do so. In the present case, the G-BA

commissioned IQWiG to prepare the concept. The expert bodies according to Section 35a, paragraph 3b, sentences 7 and 8 SGB V made a written submission in drawing up the concept. The submission took place in such a way that the expert bodies were given the opportunity in writing to comment on the requirements of routine practice data collection and evaluations in accordance with the concept that had been drawn up. In addition, expert consultation was held.

The requirement of routine practice data collection and evaluations is essentially determined by the prerequisites according to Section 35a, paragraph 3b, sentence 1, numbers 1 and 2 SGB V that the medicinal product in question is a medicinal product, whose placing on the market has been authorised in accordance with the procedure set out 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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency (OJ L 136 of 30.04.2004, p. 1), as last amended by Regulation (EU) 5/2019 (OJ L 4, 07.01.2019, p. 24).or for which a marketing authorisation has been granted in accordance with Article 14-a of Regulation (EC) No 726/2004, or it is a medicinal product approved for the treatment of rare diseases under Regulation (EC) No 141/2000.

By letter dated 17 October 2024, the pharmaceutical company informed the G-BA that the orphan designation for the treatment of haemophilia A (EU/3/16/1752) and the orphan designation for the treatment of haemophilia B (EU/3/23/2866) for the active ingredient marstacimab had been withdrawn. The revocation of the active ingredient marstacimab for the treatment of haemophilia A and B from the Community Register of orphan drugs was published on the European Commission's website ("Community Register of not active orphan medicinal products") on 11 October 2024. This ruled out marketing authorisation as an orphan drug.

On 18 November 2024, a marketing authorisation was granted for marstacimab for routine prophylaxis of bleeding episodes in patients 12 years of age and older, weighing at least 35 kg, with severe haemophilia A (congenital factor VIII deficiency, FVIII < 1%) without factor VIII inhibitors, or severe haemophilia B (congenital factor IX deficiency, FIX < 1%) without factor IX inhibitors. This was neither a conditional marketing authorisation nor a marketing authorisation under exceptional circumstances.

The legal requirements for the requirement of a routine practice data collection in accordance with Section 35a, paragraph 3b, sentence 1, numbers 1 and 2 SGB V for the active ingredient marstacimab are therefore not fulfilled at the time of this resolution.

The G-BA is therefore suspending the consultation on the requirement of routine practice data collection and evaluations for the active ingredient marstacimab in the treatment of "Adults and adolescents 12 years and older with severe haemophilia A without factor VIII inhibitors or with moderate-to-severe haemophilia B without factor IX inhibitors".

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 prepare a recommendation for a resolution on the initiation of a procedure for the requirement of a routine practice data collection (amendment of Annex XII of AM-RL (Pharmaceuticals Directive)) according to Section 35a, paragraph 3b 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 SHI umbrella organisation, and the representatives of the patient organisations. Representatives of the IQWiG also participate in the sessions. In addition, the competent higher federal authority, the Paul Ehrlich Institute, was involved in the consultation to assess the requirement of routine practice data collection according to Section 35a, paragraph 3b, sentence 1 SGB V.

The recommended resolution on the initiation of a procedure for the requirement of a routine practice data collection was discussed on 26 March 2024 at the subcommittee session and the draft resolution was approved.

At its session on 4 April 2024, the plenum resolved to initiate a procedure for the requirement of a routine practice data collection.

In conjunction with the resolution of 4 April 2024 regarding the initiation of a procedure for the requirement of a routine practice data collection, the G-BA commissioned IQWiG to scientifically develop a concept for routine practice data collection and evaluations for the purpose of preparing a resolution.

IQWiG's concept was submitted to the G-BA on 31 July 2024. On 2 August 2024, the written submission of the expert bodies according to Section 35a, paragraph 3b, sentences 7 and 8 SGB V was initiated. The deadline for making the written submission was 30 August 2024.

The expert consultation within the framework of the submission by the expert bodies took place on 23 September 2024.

The suspension of the procedure was discussed at the session of the subcommittee on 26 November 2024, and the draft resolution was approved.

At its session on 5 December 2024, the plenary decided on the suspension of consultations on the requirement of routine practice data collection and evaluations.

Chronological course of consultation

Session	Date	Subject of consultation
WG RPDC	5 January 2024 1 February 2024 7 March 2024	Consultation on the initiation of a procedure for the requirement of a routine practice data collection (amendment of Annex XII of the AM-RL), involvement of the higher federal authority
Subcommittee Medicinal products	26 March 2024	Concluding discussion of the draft resolution
Plenum	4 April 2024	Resolution on the initiation of a procedure for the requirement of a routine practice data collection (amendment of Annex XII of the AM-RL)
WG RPDC	5 September 2024	Information on written submissions received, preparation of the expert consultation
Subcommittee on Medicinal Products	23 September 2024	Implementation of the expert consultation
WG RPDC	2 October 2024 21 October 2024 18 November 2024	Consultation on IQWiG's concept and on the specifications for the review of the obligation to conduct and submit evaluations, evaluation of the submission procedure and on the suspension of the procedure
Subcommittee on Medicinal Products	26 November 2024	Consultation on the draft resolution on suspension of the procedure
Plenum	5 December 2024	Resolution on the suspension of the consultation procedure on the requirement of a routine practice data collection

Berlin, 5 December 2024

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

Prof. Hecken