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 Valoctocogen roxaparvovec (severe haemophilia A); Restriction of the Authority to Supply Care

of 2 February 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 2 February 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 the treatment of severe haemophilia A. The active ingredient valoctocogen roxaparvovec for the treatment of severe haemophilia A 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 valoctocogen roxaparvovec has led to the question of an additional benefit compared to the appropriate comparator therapy for the approved patient population. A search by IQWiG for ongoing and planned data collection for the active ingredient valoctocogen roxaparvovec as part of the concept development for routine practice data collection showed that the ongoing and planned studies, including the extension studies, are not suitable for addressing the existing gaps in the evidence. The observational or registry studies commissioned by the regulatory authorities refer exclusively to a single-arm data collection for the active ingredient valoctocogen roxaparvovec and not to a comparative data collection. The pivotal 270-301 study is not comparative in design and thus cannot remedy the lack of comparison with existing appropriate therapeutic alternatives. The question of routine practice data collection requires the collection of comparator data.

The expected eligible number of subjects who can be treated with valoctocogen roxaparvovec is low because severe haemophilia A is a rare disease, treatment with valoctocogen roxaparvovec is not considered for all people with severe haemophilia A according to the marketing authorisation and, in addition, other treatment options are available in the present therapeutic indication.

In order to ensure a sufficient data stock 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 with valoctocogen roxaparvovec.

Care providers within the meaning of Chapter 5, Section 60 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.

The requirement of routine data collection and evaluations according to Section 35a paragraph 1, sentence 1 SGB V (resolution of 2 February 2023) refers to adult patients with severe haemophilia A (congenital factor VIII deficiency) without a history of factor VIII inhibitors. A negative AAV5 antibody status is not seen as an inclusion criterion. The AAV5 antibody status is a relevant criterion in the treatment decision for or against gene therapy with valoctocogen roxaparvovec, but is currently not regularly collected in patients with haemophilia A. It is assumed that the AAV5 antibody status has no connection with the severity grade of haemophilia and therefore does not influence the course of the disease. Therefore, in the requirement of routine data collection and evaluations, the patient population with reference to the control group is not restricted to the lack of detectability of AAV5 antibodies.

However, the present restriction of the authority to supply care refers specifically to the active ingredient valoctocogen roxaparvovec, which, according to the marketing authorisation, may only be used in patients with negative AAV5 antibody status. Therefore, the restriction of supply concerns the patient population according to the approved therapeutic indication of valoctocogen roxaparvovec.

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 SHI umbrella organisation, 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 3 November 2022.

The draft resolution to initiate a written statement procedure was discussed at the session of the Subcommittee on Medicinal Products on 8 November 2022 and the draft resolution was consented to.

At its session on 8 November 2022, 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 statements, 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 24 January 2023, and the proposed resolution was approved.

At its session on 2 February 2023, the plenum adopted a resolution to amend the Pharmaceuticals Directive.

Chronological course of consultation

Session	Date	Subject of consultation
WG RPDC	3 November 2022	Consultation on the amendment of the AM-RL
Subcommittee Medicinal products	8 November 2022	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	5 January 2023	Consultation on the statements received
WG RPDC	5 January 2023 16 January 2023	Consultation on the draft resolution and evaluation of the written statement procedure
Subcommittee Medicinal products	24 January 2023	Concluding discussion of the draft resolution
Plenum	2 February 2023	Adoption of the resolution on the amendment of Annex XII AM-RL

Berlin, 2 February 2023

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

Prof. Hecken