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
Risdiplam (spinal muscular atrophy);
Restriction of the Authority to Supply Care

of 21 July 2022

Contents

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 or will be the subject of a resolution according Section 35a, paragraph 3b, sentence 1 SGB V that the authority to supply insured individuals 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 July 2022, 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 use of risdiplam. The active ingredient risdiplam in spinal muscular atrophy 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 serves 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 risdiplam has led to the 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 not suitable for addressing existing gaps in the evidence. No comparison is made in the three interventional studies identified. They also cover only part of the population of the approved therapeutic indication of risdiplam. The three assigned extension studies for the follow-up of patients do not include any further patients, thus sharing the shortcoming of the respectively associated intervention studies. The question of routine practice data collection requires the collection of comparator data.

The expected eligible number of patients who can be treated with risdiplam is small because spinal muscular atrophy is a rare genetic disease, risdiplam is not approved for all patients with spinal muscular atrophy, and an approved therapeutic alternative exists.

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

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.

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 (Section 35a) 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 22.03.2022.

The draft resolution to initiate a written statement procedure was discussed at the session of the Subcommittee on Medicinal Products on 29.03.2022 and the draft resolution was consented to.

At its session on 29.03.2022, 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.

Statements were received during the written statement procedure. The oral hearing was held on 24.05.2022.

The evaluation of the written statements received and the oral hearing was discussed at the session of the subcommittee on 12.07.2022, and the proposed resolution was approved.

At its session on 21 July 2022, the plenum adopted a resolution to amend the Pharmaceuticals Directive.

Chronological course of consultation

Session	Date	Subject of consultation
Working group Section 35a	22 March 2022	Consultation on the amendment of the AM-RL
Subcommittee Medicinal products	29 March 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
Working group Section 35a	18 May 2022	Consultation on the statements received
Subcommittee Medicinal products	24 May 2022	Conduct of the oral hearing
Working group Section 35a	1 June 2022 15 June 2022 6 July 2022	Consultation on the draft resolution and evaluation of the written statement procedure
Subcommittee Medicinal products	12 July 2022	Concluding discussion of the draft resolution
Plenum	21 July 2022	Adoption of the resolution on the amendment of Annex XII AM-RL

Berlin, 21 July 2022

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

Prof. Hecken