Justification

Gemeinsamer Bundesausschuss

to 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 Onasemnogene Abeparvovec (Spinal Muscular Atrophy); Restriction of the Authority to Supply Care

of 4 February 2021

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1. Legal basis

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

2. Key points of the resolution

At its session on 4 February 2021, the G-BA passed a resolution on the restriction of the authority to supply care according to Section 35a, paragraph 3b, sentence 2 SGB V for the use of onasemnogene abeparvovec for spinal muscular atrophy (SMA). The active ingredient onasemnogene abeparvovec for SMA is the subject of a resolution on the requirement of a routine data collection according to Section 35a, paragraph 3b, sentence 1 SGB V.

The restriction of the authority to supply care of such care providers who participate in the required routine 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 thus obtain significant, suitable data for the purposes of the benefit assessment.

The need for information for a benefit assessment of onasemnogene abeparvovec has led to the question of a (long-term) additional benefit compared with the appropriate comparator therapy for the approved patient population. The corresponding data collection of the IQWiG within the framework of the conceptual design for a routine data collection showed that the ongoing and planned studies, including the extension studies, are not suitable for addressing existing evidence gaps. No comparison is made in the three interventional studies identified. They also cover only part of the population of the approved therapeutic indication of use of onasemnogene abeparvovec. The three assigned extension studies for the follow-up of patients do not include any further patients and thus share the shortcoming of the respective associated intervention studies. The question of routine data collection requires the collection of comparative data.

The expected eligible number of patients who can be treated with onasemnogene abeparvovec is small because spinal muscular atrophy is a rare genetic disease, there is no marketing authorisation for treatment with onasemnogene abeparvovec for all patients with spinal muscular atrophy, and an approved alternative therapy exists.

In order to ensure a sufficient database for the routine data collection, it is necessary that a complete data collection takes place at least from the area of the care of insured persons with onasemnogene abeparvovec.

Care providers within the meaning of Chapter 5, Section 60 of Rules of Procedure of the G-BA (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 to provide care according to Section 108 SGB V.

A decision on an amendment to Section 1 of the Pharmaceuticals Directive (AM-RL) regarding the clarification of the scope of the AM-RL will be made in a separate resolution. However, the scope of application of the AM-RL with regard to resolutions according to Section 35a SGB V also extends to the supply of medicinal products by hospitals in addition to the supply by SHI-

accredited physicians, even without a corresponding clarification. This results directly from the scope of application of Section 35a SGB V, according to which generally reimbursable medicinal products (and not only those that can be prescribed in the SHI-accredited sector) are the subject of the benefit assessment according to Section 35a SGB V. Resolutions on the pharmaceutical supply must also be taken across sectors in accordance with Section 91, paragraph 7, sentence 2 SGB V and therefore claim corresponding validity. Consequently, there is no doubt about the validity of the present restriction of the authority to supply care also for approved hospitals and other care providers participating in inpatient care.

3. Bureaucratic costs

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 discuss and prepare a recommendation for a resolution on the initiation of a written statement procedure on the restriction of the authority to supply care according Section 35a, paragraph 3b, sentence 2 SGB V, the Subcommittee on Medicinal Products commissioned a working group (AG Section 35a) consisting of the members nominated by the leading organisations of the care providers, the members nominated by the SHI umbrella organisation, and representatives 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 4 November 2020.

The draft resolution to initiate a written statement procedure was discussed at the session of the Subcommittee on Medicinal Products on 10 November 2020 and the proposed resolution was approved.

At its session on 10 November 2020, the Subcommittee unanimously passed a resolution to initiate the written statement procedure according to Chapter 1, Section 10, paragraph 1 of the Rules of Procedure of the G-BA.

Written statements were received during the written statement procedure. The oral hearing was held on 11 January 2021.

The evaluation of the written statements received and the oral hearing were discussed at the session of the subcommittee on 26 January 2021, and the proposed resolution was approved.

At its session on 4 February 2021, the plenum adopted a resolution to amend the Pharmaceuticals Directive.

Chronological course of consultation

Session	Date	Subject of consultation
Working group Section 35a	4 November 2020	Consultation on the amendment of the AM-RL
Subcommittee on Medicinal Products	10 November 2020	Consultation and consensus on the draft resolution Resolution to initiate the written statement procedure on the amendment of the AM-RL

Subcommittee on Medicinal Products	8 December 2020	Scheduling of the oral hearing
Working group Section 35a	6 January 2021	Consultation on the written statements received
Subcommittee on Medicinal Products	11 January 2021	Conduct of the oral hearing
Working group Section 35a	20 January 2021	Consultation on the draft resolution as well as evaluation of the written statement procedure
Subcommittee on Medicinal Products	26 January 2021	Concluding discussion of the draft resolution
Plenum	4 February 2021	Adoption of the resolution on the amendment of Annex XII AM-RL

Berlin, 4 February 2021

Federal Joint Committee
in accordance with Section 91 SGB V
The Chair

Prof. Hecken