Gemeinsamer Bundesausschuss

Justification

of the Resolution of the Federal Joint Committee on an Amendment of the Pharmaceuticals Directive:
Annex XII – Benefit Assessment of Medicinal Products with New Active Ingredients according to Section 35a SGB V and Annex IX (Definition of Reference Price Groups)
– Coagulation Factors VIII, Recombinant, Group 1, in Tier 2

of 2 October 2024

Contents

1. Legal basis

According to Section 35a, paragraph 1, sentences 4 and 5 SGB V, the additional medical benefit according to sentence 3 number 3 (additional medical benefit in relation to the appropriate comparator therapy) must be proven as a therapeutic improvement according to Section 35, paragraph 1b, sentences 1 to 5 SGB V in the case of medicinal products that are pharmacologically and therapeutically comparable to reference price medicinal products. If the pharmaceutical company does not submit the required evidence in time or in full, despite a request by the Federal Joint Committee, an additional benefit is deemed not to be proven.

If no therapeutic improvement has been established for a medicinal product in accordance with Section 35a, paragraph 1, sentence 4 SGB V, it is to be classified in the resolution in accordance with Section 35a paragraph 3 SGB V into the reference price group in accordance with Section 35 paragraph 1 SGB V with pharmacologically and therapeutically comparable medicinal products (Section 35a, paragraph 4, sentence 1 SGB V). A separate written statement procedure pursuant to Section 35, paragraph 1b, sentence 7 and paragraph 2 SGB V needs not be carried out (Section 35a, paragraph 4, sentence 3 SGB V). The resolution is part of the guideline according to Section 92, paragraph 1, sentence 2, number 6 SGB V, Section 94 paragraph 1 SGB V does not apply (Section 35a, paragraph 3, sentence 7 SGB V).

Pursuant to Section 35 paragraph 1 SGB V, the Federal Joint Committee determines in the guidelines pursuant to Section 92, paragraph 1, sentence 2, number 6 SGB V the groups of medicinal products for which reference prices may be fixed. The groups should include medicinal products with

1. the same active ingredients,
2. pharmacologically and therapeutically comparable active ingredients, in particular with chemically related substances,
3. therapeutically comparable effect, in particular medicinal product combinations.

The Federal Joint Committee also determines the arithmetical mean daily or single doses required in accordance with Section 35 paragraph 3 SGB V, or other suitable comparative figures.

2. Key points of the resolution

The marketing authorisation holder, Swedish Orphan Biovitrum AB, was requested by the Federal Joint Committee to submit a dossier to the Federal Joint Committee in due time, i.e. no later than the date of inclusion of the medicinal product Altuvoct® with the active ingredient efanesoctocog alfa in the "LAUER-TAXE®", the extensive German registry of available drugs and their prices. The tender date was 15 July 2024.

The legal consequence of the company's decision not to submit a dossier at the relevant time is the finding of an unproven additional benefit. The pharmaceutical company did not claim a therapeutic improvement, partly due to fewer side effects, or pharmacological and therapeutic incomparability.

As a starting point for finding whether a medicinal product is pharmacologically and therapeutically comparable to medicinal products in an existing reference price group, the official ATC classification pursuant to Section 73, paragraph 8, sentence 5 SGB V is to be used, with level 1 reflecting the anatomical classification, levels 2 to 4 the therapeutic classification and level 5 the chemical classification. The active ingredient efanesoctocog alfa has the ATC code B02BD02.

The active ingredients already grouped have the following ATC codes:

Damoctocog alfa pegol	B02BD39
Efmoroctocog alfa	B02BD32
Lonoctocog alfa	B02BD35
Moroctocog alfa	B02BD31
Octocog alfa	B02BD28
Rurioctocog alfa pegol	B02BD38
Simoctocog alfa	B02BD17
Turoctocog alfa	B02BD43
Turoctocog alfa pegol	B02BD41

Therefore, the same ATC code at level 4 is assigned to all active ingredients concerned.

Efanesoctocog alfa is a recombinant coagulation factor. All active ingredients included in the reference price group belong to the substance class of coagulation factors. All of them are coagulation factors VIII synthesised by recombinant production. The active ingredients replace the coagulation factor VIII that is missing in patients with haemophilia A, thereby provisionally correcting the deficiency and the bleeding tendency. The active ingredients thus share an identical mode of action that is crucial for pharmacological comparability.

In addition, all recombinant coagulation factor VIII preparations included in the reference price group have a common point of reference - leading to therapeutic comparability - due to the marketing authorisation of the preparations in the therapeutic indications "prevention and therapy of bleeding in haemophilia A (congenital deficiency of factor VIII)".

In the present definition of reference price groups of tier 2 in accordance with Section 35, paragraph 1, sentence 2, number 2 SGB V, a pharmacological and therapeutic comparability of the active ingredients to be grouped therefore occurs, as required by Section 35a paragraph 4 SGB V.

Therapy options are not restricted and medically required alternative prescriptions are available. The marketing authorisation under the Medicinal Products Act does not allow the conclusion that one of the included proprietary medicinal products has a singular therapeutic indication.

In its consultations on the finding of an additional benefit of efanesoctocog alfa and on the update of the reference price group "coagulation factors VIII, recombinant, group 1" in tier 2, the Subcommittee on Medicinal Products came to the conclusion that an additional benefit of efanesoctocog alfa according to Section 35a, paragraph 1, sentence 5 SGB V is considered not proven, the requirements according to Section 35a, paragraph 4, sentence 1 SGB V are fulfilled and, accordingly, efanesoctocog alfa is assigned to the reference price group "coagulation factors VIII, recombinant, group 1" in tier 2 according to Section 35a, paragraph 4, sentence 1 in conjunction with Section 35, paragraph 1, sentence 2, number 2 SGB V (definition of reference price groups).

The present resolution therefore updates the existing reference price group "coagulation factors VIII, recombinant, group 1" in tier 2 as follows:

- Classification of a new active ingredient "efanesoctocog alfa".

The documents on which the update of the present reference price group is based are attached to the Justification as an Annex.

In deviation from the determination of the comparative figure according to Annex I to Chapter 4 of the Rules of Procedure of the Federal Joint Committee, the calculated average single dose in relation to the average weekly application is used for the present reference price group in order to make the active ingredients comparable with each other.

It is not necessary to conduct a written statement procedure according to Section 35a, paragraph 3, sentence 2 in conjunction with Section 92 paragraph 3a SGB V to arrive at the finding that an additional benefit of efanesoctocog alfa is not proven. This follows from the purpose of the written statement procedure regulated in Section 92 paragraph 3a SGB V.

The procedure primarily serves the public interest of involving the expertise of third parties in addition to the expertise of the members of the Federal Joint Committee for determining the decision-making facts underlying the standardisation and facilitating the weighing processes to be undertaken (see Superior State Social Court Berlin-Brandenburg, resolution of 27.02.2008, file ref.: L 7 B 112/07 KA ER).

However, the present resolution is not based on a substantive assessment of the benefit of efanesoctocog alfa according to the generally recognised state of medical knowledge, which could justify the need to conduct a written statement procedure. However, with its decision, the Federal Joint Committee merely implements the legal consequence of the failure to submit a dossier stipulated in Section 35a, paragraph 1, sentence 5 SGB V, according to which in this case an additional benefit is deemed to be not proven.

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

Chronological course of consultation:

Session	Date	Subject of consultation
Working group Section 35a	31.07.2024	Information that no dossier has been received at the relevant time, advice on classification into the relevant reference price group
Subcommittee on Medicinal Products	06.08.2024	Information that no dossier has been received at the relevant time, advice on classification into the relevant reference price group
Subcommittee on Medicinal Products	10.09.2024	Discussion and consensus on the draft resolution
Plenum	02.10.2024	Resolution

Berlin, 2 October 2024

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

Prof. Hecken

5. Annex