Gemeinsamer Bundesausschuss

Justification

to the Resolution of the Federal Joint Committee (G-BA) on an Amendment of the Pharmaceuticals Directive: Annex XII – Benefit Assessment of Medicinal Products with New Active Ingredients according to Section 35a SGB V Ataluren (repeal of the resolution of 1 December 2016)

of 20 November 2025

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1. Legal basis

According to Section 35a paragraph 1 German Social Code, Book Five (SGB V), the Federal Joint Committee (G-BA) assess the benefit of all reimbursable medicinal products with new active ingredients.

For medicinal products for the treatment of rare diseases (orphan drugs) that are approved according to Regulation (EC) No. 141/2000 of the European Parliament and the Council of 16 December 1999, the additional medical benefit is considered to be proven through the grant of the marketing authorisation according to Section 35a, paragraph 1, sentence 11, 1st half of the sentence German Social Code, Book Five (SGB V). Evidence of the medical benefit and the additional medical benefit in relation to the appropriate comparator therapy do not have to be submitted (Section 35a, paragraph 1, sentence 11, 2nd half of the sentence SGB V). Section 35a, paragraph 1, sentence 11, 1st half of the sentence SGB V thus guarantees an additional benefit for an approved orphan drug, although an assessment of the orphan drug in accordance with the principles laid down in Section 35a, paragraph 1, sentence 3, No. 2 and 3 SGB V in conjunction with Chapter 5 Sections 5 et seq. of the Rules of Procedure (VerfO) of the G-BA has not been carried out. In accordance with Section 5, paragraph 8 AM-NutzenV, only the extent of the additional benefit is to be quantified indicating the significance of the evidence.

However, the restrictions on the benefit assessment of orphan drugs resulting from the statutory obligation to the marketing authorisation do not apply if the turnover of the medicinal product with the SHI at pharmacy sales prices and outside the scope of SHI-accredited medical care, including VAT exceeds € 30 million in the last 12 calendar months. According to Section 35a, paragraph 1, sentence 12 SGB V, the pharmaceutical company must then, within three months of being requested to do so by the G-BA, submit evidence according to Chapter 5 Section 5, paragraphs 1–6 VerfO, in particular regarding the additional medical benefit in relation to the appropriate comparator therapy as defined by the G-BA according to Chapter 5 Section 6 VerfO and prove the additional benefit in comparison with the appropriate comparator therapy.

In accordance with Section 35a, paragraph 2 SGB V, the G-BA decide whether to carry out the benefit assessment itself or to commission the Institute for Quality and Efficiency in Health Care (IQWiG). Based on the legal requirement in Section 35a, paragraph 1, sentence 11 SGB V that the additional benefit of an orphan drug is considered to be proven through the grant of the marketing authorisation, the G-BA modified the procedure for the benefit assessment of orphan drugs at their session on 15 March 2012 to the effect that, for orphan drugs, the G-BA initially no longer independently determine an appropriate comparator therapy as the basis for the solely legally permissible assessment of the extent of an additional benefit to be assumed by law. Rather, the extent of the additional benefit is assessed exclusively on the basis of the approval studies by the G-BA indicating the significance of the evidence.

Accordingly, at their session on 15 March 2012, the G-BA amended the mandate issued to the IQWiG by the resolution of 1 August 2011 for the benefit assessment of medicinal products with new active ingredients in accordance with Section 35a, paragraph 2 SGB V to that effect that, in the case of orphan drugs, the IQWiG is only commissioned to carry out a benefit assessment in the case of a previously defined comparator therapy when the sales volume of the medicinal product concerned has exceeded the turnover threshold according to Section 35a, paragraph 1, sentence 12 SGB V and is therefore subject to an unrestricted benefit assessment. According to Section 35a, paragraph 2 SGB V, the assessment by the G-BA must be completed within three months of the relevant date for submission of the evidence and published on the internet.

According to Section 35a paragraph 3 SGB V, the G-BA decide on the benefit assessment within three months of its publication. The resolution is to be published on the internet and is part of the Pharmaceuticals Directive.

2. Key points of the resolution

The active ingredient ataluren was first approved as a medicinal product on 31 July 2014 (Translarna). The marketing authorisation was granted for the therapeutic indication: "Translarna is indicated for the treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 5 years and older (see section 5.1). No efficacy has been demonstrated in non-ambulatory patients. The presence of a nonsense mutation in the dystrophin gene should be determined by genetic testing (see section 4.4)." This marketing authorisation was a conditional marketing authorisation for a medicinal product for the treatment of a rare disease.

After the active ingredient ataluren was placed on the market for the first time on 1 December 2014, the G-BA conducted a benefit assessment according to Section 35a SGB V and supplemented Annex XII of the Pharmaceuticals Directive with the active ingredient ataluren by resolution of 21 May 2015.

The benefit assessment was based on the results of the PTC124-GD-007-DMD study – a randomised, placebo-controlled, blinded phase 2b dose-finding study. Against the background that the medicinal product Translarna was granted conditional marketing authorisation, the European Medicines Agency EMA required that the results of the previously ongoing phase III study in the therapeutic indication (PTC124-GD-020-DMD study) be submitted with regard to the evidence to be provided by the pharmaceutical company.

The G-BA had limited the period of validity of their resolution on the benefit assessment of ataluren to 1 June 2016, and made it subject to the condition that the expected results from the PTC124-GD-020-DMD study must be submitted for the new benefit assessment after the expiry of the deadline.

On 1 June 2016, the pharmaceutical company submitted a dossier for the benefit assessment of ataluren to the G-BA in due time in accordance with Chapter 5 Section 8 Number 5 VerfO, according to which the required evidence must be submitted no later than the day on which the deadline expires. The G-BA conducted a new benefit assessment after the expiry of the deadline in accordance with Section 35a SGB V and, by resolution of 1 December 2016, deleted the information on ataluren in the version of the resolution of 21 May 2015 and supplemented the Pharmaceuticals Directive with the active ingredient ataluren.

In September 2023, the EMA recommended for the first time that the conditional marketing authorisation for ataluren should not be prolonged because the efficacy of Translarna could not be confirmed on the basis of a complete reassessment of the medicinal product as part of the prolongation of the marketing authorisation, including the results of a new study.

The pharmaceutical company applied for a review of the EMA's scientific assessment, which resulted in a new EMA recommendation in January 2024 not to prolong the existing conditional marketing authorisation for ataluren. At the request of the European Commission, the statement was reviewed again, taking into account additional data from the medical treatment practice, which led to another EMA recommendation in June 2024 not to prolong the existing conditional marketing authorisation. Following a renewed application by the pharmaceutical company, the EMA issued a further recommendation in October 2024 not to prolong the existing conditional marketing authorisation for ataluren.

On 23 March 2025, the extension of the conditional marketing authorisation for ataluren was refused by the European Commission due to the circumstances described above, as a positive benefit-risk ratio could not be confirmed. With the failure of the prolongation of the marketing authorisation and the resulting expiry of the marketing authorisation, the basis for the benefit assessment according to Section 35a, paragraph 1 SGB V by the G-BA no longer applies. Consequently, the resolution on ataluren dated 1 December 2016 (BAnz AT 22.12.2016 B4) must be repealed.

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

Session	Date	Subject of consultation
Working group Section 35a	4 November 2025	Consultation on the draft resolution
Subcommittee on Medicinal Products	11 November 2025	Consultation and consensus on the draft resolution on the repeal of the resolution
Plenum	20 November 2025	Adoption of the repeal of the resolution

Berlin, 20 November 2025

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

Prof. Hecken