Gemeinsamer Bundesausschuss

Resolution

of the Federal Joint Committee on an Amendment of the Pharmaceuticals Directive (AM-RL): Annex XII – Benefit Assessment of Medicinal Products with New Active Ingredients according to Section 35a SGB V: etranacogene dezaparvovec (haemophilia B); requirement of routine practice data collection and evaluations - Revision

From 2 July 2026

At their session on 2 July 2026, the Federal Joint Committee (G-BA) resolved to amend the Pharmaceuticals Directive (AM-RL) in the version dated 18 December 2008/ 22 January 2009 (Federal Gazette, BAnz. No. 49a of 31 March 2009), as last amended by the publication of the resolution of D Month YYYY (Federal Gazette, BAnz AT DD.MM.YYYY BX), as follows:

I. The information on the requirement of routine practice data collection and evaluations according to Section 35a, paragraph 3b, sentence 1 SGB V on the active ingredient etranacogene dezaparvovec in the version of the resolution of 12 May 2023 (Federal Gazette, BAnz AT 09.06.2023 B3) is amended as follows:

1. Number 1.1. "Research question according to PICO scheme" is amended as follows:

   In the table, the information "Recombinant or human plasma-derived coagulation factor IX products[a]" in the "Comparator" row is replaced by the information "Individualised therapy with the selection of recombinant or human plasma-derived coagulation factor IX products[a] and marstacimab and concizumab".

2. Number 1.4 "Evaluations of the data for the purpose of the benefit assessment" is amended as follows:

   In the information "For the first interim analysis:", the word "first" is replaced by the word "second".

3. Number 1.5 "Requirements for the preparation of the study protocol and statistical analysis plan" is amended as follows:

   The information "and marstacimab and concizumab" is inserted after the information "Pre-specification of a sensitivity analysis for the separate evaluation of data on etranacogene dezaparvovec compared with data on recombinant or human plasma-derived coagulation factor IX products".

4. Number 2.3 "Submission of interim analyses" is amended as follows:
   a) The information "54 months after the date of resolution" is deleted.
   b) The information "36" is replaced by the information "44".
   c) The information "Date of resolution" is replaced by the information "Start of routine practice data collection".
5. Number 3 "Deadline for the submission of evaluations of the data collected as part of the routine practice data collection" is amended as follows: In the information "For carrying out a renewed benefit assessment, the analyses of the data collected as part of the routine practice data collection must be submitted by 2 November 2029 at the latest", the date "2 November 2029" is replaced by the date "1 July 2030".

II. The revised study protocol and the revised SAP are to be submitted to the G-BA by 30 April 2028.

III. The resolution will enter into force on the day of its publication on the G-BA website on 2 July 2026.

The justification for this resolution will be published on the G-BA website at www.g-ba.de.

Berlin, 2 July 2026

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

Dr Optendrenk