

Justification

for the Resolution of the Federal Joint Committee (G-BA) on
the Finding in the Procedure of Routine Practice Data
Collection and Evaluations according to Section 35a
paragraph 3b SGB V:

Valoctocogene roxaparvovec (severe haemophilia A) – Review
of the study protocol, statistical analysis plan and interim
analyses

of 2 April 2026

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1. Legal basis

According to Section 35a, paragraph 3b, sentence 1 SGB V, the Federal Joint Committee (G-BA) can demand the pharmaceutical company to submit routine practice data collections and evaluations for the purpose of the benefit assessment within a reasonable period of time for the following medicinal products:

1. in the case of medicinal products authorised to be placed on the market in accordance with the procedure laid down in Article 14, paragraph 8 of Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency (OJ L 136, 30.4.2004, p. 1), as last amended by Regulation 162 Rules of Procedure last revised: 16 December 2020 (EU) 2019/5 (OJ L 4 from 07.01.2019, p. 24), or for which a marketing authorisation has been granted in accordance with Article 14-a of Regulation (EC) No 726/2004; and
2. for medicinal products approved for the treatment of rare diseases under Regulation No. 141/2000.

According to Section 35a, paragraph 3b, sentence 10 SGB V in conjunction with Chapter 5 Section 60 Rules of Procedure of the G-BA (VerfO), the G-BA review the data obtained and the obligation to collect data at regular intervals, at least every eighteen months.

2. Key points of the resolution

At their session on 2 February 2023, the G-BA decided on the requirement of routine practice data collection and evaluations for the active ingredient valoctocogene roxaparvovec in accordance with Section 35a, paragraph 3b, sentence 1 SGB V. This resolution required the pharmaceutical company to submit details of the progress of the data collection and interim analyses to the G-BA 18 months after the start of the routine practice data collection.

By declaratory resolution of 18 July 2024, the start of the routine practice data collection was fixed on 30 August 2024. Moreover, it was found by this resolution that the requirements for routine practice data collection and evaluations were considered implemented - only subject to specific conditions - in the study protocol and statistical analysis plan prepared by the pharmaceutical company and submitted to the G-BA for review. The pharmaceutical company was therefore required to make further amendments deemed necessary to the study protocol and the statistical analysis plan and to submit these to the G-BA by 2 March 2026 at the latest, at the time of the review 18 months after the start of routine practice data collection.

As of 2 March 2026, the pharmaceutical company had not submitted any revised versions of the study protocol and the statistical analysis plan to the G-BA, nor had they provided any details of the progress of data collection and any interim analyses.

This result shall be communicated to the National Association of Health Insurance Funds for the purpose of a decision pursuant to Section 130b, paragraph 3, sentence 9 SGB V.

3. Process sequence

In accordance with the resolutions of 2 February 2023 and 18 July 2024 on the requirement and assessment in the procedure of routine practice data collection and evaluations for the active ingredient valoctocogene roxaparvovec, the pharmaceutical company must submit

revised versions of the study protocol and the statistical analysis plan, as well as details of the progress of data collection and interim analyses to the G-BA by 2 March 2026 at the latest, at the time of the review 18 months after the start of routine practice data collection.

The pharmaceutical company had not submitted any revised versions of the study protocol and the statistical analysis plan to the G-BA, nor had they provided any details of the progress of data collection and any interim analyses.

The issue was discussed in the working group WG RPDC and in the Subcommittee on Medicinal Products.

At their session on 2 April 2026, the plenum decided that the routine practice data collection, which has been ongoing for 18 months, will not be continued.

Chronological course of consultation

Session	Date	Subject of consultation
WG RPDC	16 March 2026	Consultation on the review of the pharmaceutical company's obligation to revise the study protocol and SAP, as well as to provide details of the progress of data collection and interim analyses
Subcommittee on Medicinal Products	24 March 2026	Consultation on the result of the review of the pharmaceutical company's obligation to revise the study protocol and SAP, as well as to provide details of the progress of data collection and interim analyses
Plenum	2 April 2026	Adoption of the resolution on the review of the pharmaceutical company's obligation to revise the study protocol and SAP, as well as to provide details of the progress of data collection and interim analyses

Berlin, 2 April 2026

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

Prof. Hecken