

Resolution

of the Federal Joint Committee on a Finding in the Procedure of Routine Practice Data Collection and Evaluations according to Section 35a, paragraph 3b SGB V:

Exagamglogene autotemcel (sickle cell disease) – Submission of the study protocol and statistical analysis plan

From 3 September 2026

At their session on 3 September 2026, the Federal Joint Committee (G-BA) decided the following in the procedure of routine practice data collection and evaluations according to Section 35a, paragraph 3b SGB V concerning the active ingredient exagamglogene autotemcel (sickle cell disease):

- I. It is stated that the requirements for routine practice data collection and evaluations have been inadequately implemented in the study protocol (SP) and statistical analysis plan (SAP) prepared by the pharmaceutical company and submitted to the G-BA for review, meaning that the routine practice data collection will not be carried out.

The following adjustments to the study documents, identified as mandatory in the declaratory resolution of 18 September 2025, were not implemented by the pharmaceutical company in the revised study documents submitted on 21 May 2026 (SP and SAP, version 3.0):

- a) Question according to PICO: patient population

The study documents (version 3.0) still do not define any clear criteria to be used to assess the suitability or unsuitability for treatment with exagamglogene autotemcel or stem cell transplantation (see Section a of the declaratory resolution of 18 September 2025).

- b) Study design: baseline assessment

The study documents do not include any information to clarify how the assessment of continued suitability of patients in the comparator arm for treatment with exagamglogene autotemcel will be carried out on the index date (see Section n of the declaratory resolution of 18 September 2025).

- c) Study design: confounders

The study documents merely include the specification "yes/ no" as the operationalisation for the confounders – with the exception of the characteristics of age, HbF percentage, sex and number of vaso-occlusive crises. Consequently, for the

majority of confounders in the study documents, there is still no clear and appropriate definition to enable their consistent assessment (see Section q of the declaratory resolution of 18 September 2025).

d) Study design: baseline assessment of the confounders

The study documents still do not provide for a new assessment of the confounders on the index date, nor is there any documentation of the time interval between the baseline assessment and the index date. Furthermore, the pharmaceutical company did not provide any clear, substantive justification to suggest that the confounders will remain stable during the 2-year baseline period (see Section n of the declaratory resolution of 18 September 2025).

- II. The continuing deficiencies in the study documents listed under I. relate to mandatory adjustments that are considered indispensable for the start of routine practice data collection. As these deficiencies have not been rectified by the pharmaceutical company, the routine practice data collection will not be carried out.
- III. The resolution will enter into force on the day of its publication on the G-BA website on 3 September 2026.

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

Berlin, 3 September 2026

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

Dr Optendrenk