Gemeinsamer Bundesausschuss

Justification

of 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: Onasemnogen abeparvovec (spinal muscular atrophy); requirement of routine practice data collection and evaluations

of 20 January 2022

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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, 7.1.2019, 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 authorised for the treatment of rare diseases under Regulation No 141/2000.

2. Key points of the resolution

In its session on 4 February 2021, the G-BA decided on the requirement of routine practice data collection and evaluations for the active ingredient onasemnogene abeparvovec in accordance with Section 35a SGB V. Subsequent to the publication of the resolution on the websites of the G-BA, an adjustment of the time points for the interim analyses and an adjustment of the scope of the reporting 18 months after the resolution became necessary due to the extensive consultation of the study protocol as well as the statistical analysis plan (SAP) with the pharmaceutical company.

In a letter dated 11 August 2021, received on 13 August 2021, the pharmaceutical company submitted drafts for a study protocol and a SAP to the G-BA in due time. With the involvement of the Institute for Quality and Efficiency in Health Care (IQWiG), a review of these documents was carried out by the G- BA.

Due to the extensive need for adaptation, the pharmaceutical company was requested by the G-BA to revise the study protocol and the SAP accordingly in a letter dated 28 September 2021. The pharmaceutical company then submitted the revised study documents to the G-BA by letter dated 21 November, received on 23 November 2021, within the deadline set. With the involvement of IQWiG, a new review of the documents was subsequently carried out by the G BA.

The start of the routine practice data collection accompanying the application has been delayed due to the extensive need for adaptation and the necessary 2nd examination of the documents by the G-BA. Between the start time of the routine practice data collection for the active ingredient onasemnogene abeparvovec with entry into force of the resolution on a determination in the procedure of routine practice data collection and evaluations pursuant to Section 35a (3b) SGB V: onasemnogene abeparvovec (spinal muscular atrophy) – study protocol and statistical analysis plan submission dated 20 January 2022 (hereafter: determination resolution) on the 1 February 2022 and the time point for a interim analysis

according to the G-BA resolution of 4 February 2021 (August 2022) there are now only a few months. It can therefore be assumed that the first interim analysis with a reporting date of August 2022 (18 months after the resolution) will not contain any significant results.

The G-BA therefore considers it appropriate to adjust the dates for the submission of interim analyses and corresponding evaluations in Annex XII of the Pharmaceuticals Directive (AM-RL) as specified in the resolution.

In addition, the remaining second interim analysis will be brought forward by six months and thus merged with the third status report.

Since the revised study documents could only be confirmed under the condition that the pharmaceutical company makes any remaining adjustments to the study protocol and SAP that are still considered necessary, the G-BA considers it necessary for the pharmaceutical company to submit the adjusted final study documents to the G-BA. The pharmaceutical company shall implement the conditions in the determination resolution of 20 January 2022. The submission of the final study documents is to take place during the information on the course of data collection 18 months after the resolution of the G-BA of 4 February 2021.

3. Written statement procedure according to Section 92, paragraph 3a SGB V

A new written statement procedure does not have to be carried out, as the adjustment of the timelines does not represent a significant change compared to the draft resolution on the requirement of routine practice data collection and evaluations, cf. 1st Chapter, Section 14 Rules of Procedure of the G-BA.

4. 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.

5. Process sequence

Subsequent to the resolution of 4 February 2021 on an amendment to the Pharmaceuticals Directive (AM-RL) Annex XII – Resolutions on the Benefit Assessment of Medicinal Products with New Active Ingredients according to Section 35a SGB V - onasemnogene abeparvovec, an adjustment of the dates for the interim analyses and an adjustment of the scope of the reporting 18 months after the resolution in the AM-RL became necessary due to the extensive coordination of the study protocol as well as the statistical analysis plan (SAP) with the pharmaceutical company.

The issue was discussed in the working group Section 35a and in the Subcommittee on Medicinal Products.

At its session on 20 January 2022, the plenum adopted by consensus the amendment of the Pharmaceuticals Directive with regard to an adjustment of the dates for the interim analyses and an adjustment of the scope of reporting 18 months after the resolution.

Chronological course of consultation

Session	Date	Subject of consultation
Working group Section 35a	4 January 2022	Consultation on the issue
Subcommittee Medicinal product	11 January 2022	Consultation on an amending resolution regarding an adjustment of the dates for the interim analyses and an adjustment of the scope of reporting 18 months after the version of resolution of 4 February 2021.
Plenum	20 January 2022	Resolution on the amendment of the dates for the interim analyses and the scope of reporting 18 months after the version of the resolution of 4 February 2021

Berlin, 20 January 2022

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

Prof. Hecken