Justification

Gemeinsamer Bundesausschuss

to the Resolution of the Federal Joint Committee on an amendment to the Pharmaceuticals Directive (AM-RL): Annex XII – Amendment of information on the period of validity of a resolution on the benefit assessment of medicinal products with new active ingredients according to Section 35a SGB V Nusinersen

From 16. May 2019

Contents

1. Legal basis

According to Section 35a paragraph 1 SGB V, the Federal Joint Committee (G-BA) assesses the benefit of reimbursable medicinal products with new active ingredients. This includes in particular the assessment of the additional benefit and its therapeutic significance. The benefit assessment shall be carried out on the basis of evidence provided by the pharmaceutical manufacturer. This must be submitted to the G-BA electronically (including all clinical trials carried out or commissioned) at the latest at the time of the first placing on the market and the marketing authorisation of new therapeutic indication for the medicinal product. It must contain the following information in particular:

1st Approved therapeutic indication

2nd medicinal benefits

3rd additional medical benefits in relation to appropriate comparator therapy

4th Number of patients and patient groups for whom there is a therapeutically significant additional benefit

5th Therapy costs for statutory health insurance

6th Requirement for quality-assured application

The G-BA may commission the Institute for Quality and Efficiency in Health Care (IQWiG) with the benefit assessment. According to Section 35a paragraph 2 SGB V, the assessment 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 shall decide on the benefit assessment within three months of its publication. The resolution is to be published on the Internet and forms part of the Pharmaceuticals Directive.

2. Key points of the decision

At its meeting on 21 December 2017, the G-BA decided on the benefit assessment of Nusinersen in accordance with Section 35a SGB V. The period of validity of the resolution is limited to 1 January 2020.

In accordance with the justification to this resolution, the limitation was set because further data on patient-relevant endpoints for patients with 5q-SMA, in particular on the later forms of 5q-SMA and on the long-term use of nusinersen, are considered necessary for the assessment of the additional benefit. Various data are expected from the EMA: In April 2019, from the EMBRACE phase II study regarding the safety, efficacy, and pharmacokinetics of nusinersen; safety data from prospective registry entries for patients not yet investigated (e.g. type 0 and type 4 SMA, adults); results of the NURTURE open-label phase II study to evaluate the long-term efficacy and safety of nusinersen in presymptomatic patients; results of the SHINE open-label extension study in 2023.

The pharmaceutical manufacturer has informed the G-BA that because of the low number of patients and the high heterogeneity of the patients, the results of the completed EMBRACE study do not allow any statements to be made that go beyond the statements made in the resolution on nusinersen of 21 December 2017. Results on the long-term safety and efficacy of nusinersen in these patients will only be available after further observation in the SHINE

study. In addition, only interim data from the NURTURE and SHINE studies with little data on a treatment duration of at least two years with nusinersen will be available at the time of the deadline. Final data for both studies are expected to be available in 2023. In addition, data collection in the recently launched prospective registry for patients with SMA (SMArtCARE) has only been possible since the beginning of 2019; there is also no long-term data available.

In order to allow the inclusion of longer-term efficacy and safety data in the benefit assessment of Nusinersen after the deadline, the period of validity of the Resolution, originally limited until 1 January 2020, is extended. An extension of the deadline until 1 July 2024 is considered appropriate for this purpose.

In accordance with Section 3 number 5 AM-NutzenV in conjunction with Chapter 5 Section 1 paragraph 2 number 7 VerfO, the procedure for the benefit assessment of the active ingredient Nusinersen begins again when the deadline has expired. For this purpose, the pharmaceutical manufacturer must submit a dossier to the G-BA at the latest on the day of expiry of the deadline for the benefit assessment of Nusinersen (Section 4, paragraph 3, number 5 AM-NutzenV in conjunction with Chapter 5, Section 8, number 5 VerfO).

The questions underlying the limitation with regard to the assessment of the additional benefit remain unaffected by this.

The possibility that a benefit assessment for Nusinersen can be carried out at an earlier point in time for other reasons (cf. Chapter 5, Section 1, paragraph 2, Nos. 2, 3, 5, and 6 VerfO) remains unaffected by this.

3. Bureaucratic costs

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

The matter was discussed in the medicinal products sub-committee, and an amendment resolution was passed.

At its meeting on16. May 2019, the plenary decided to change the limitation of the period of validity of the resolution.

Berlin, 16. May 2019

Federal Joint Committee
in accordance with Section 91 SGB V
Chair

Prof Hecken