Gemeinsamer Bundesausschuss

Resolution

of the Federal Joint Committee on an Amendment of the Pharmaceuticals Directive:
Annex XII – Benefit Assessment of Medicinal Products with New Active Ingredients according to Section 35a SGB V
Onasemnogene abeparvovec (exceeding € 50 million turnover limit: Spinal muscular atrophy)

of 4 November 2021

At its session on 4 November 2021, 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 amended by the publication of the resolution of D. month YYYY (BAnz AT TT.MM.JJJJ BX), as follows:

I. Annex XII is amended as follows:

1. The information on onasemnogene abeparvovec in the version of the resolution of 3 December 2020 (Federal Gazette, BAnz AT 02.02.2021 B2) is repealed.

2. Annex XII shall be amended in alphabetical order to include the active ingredient onasemnogene abeparvovec as follows:

Onasemnogene abeparvovec

Resolution of: 4 November 2021
Entry into force on: 4 November 2021
BAnz AT DD. MM YYYY Bx

Therapeutic indication (according to the marketing authorisation of 18 May 2020):

Zolgensma is indicated for the treatment of:

- patients with 5q spinal muscular atrophy (SMA) with a biallelic mutation in the SMN1 gene and a clinical diagnosis of SMA Type 1, or
- patients with 5q SMA with a biallelic mutation in the SMN1 gene and up to 3 copies of the SMN2 gene

Therapeutic indication of the resolution (resolution of 4 November 2021):

- see therapeutic indication according to marketing authorisation

1. Additional benefit of the medicinal product in relation to the appropriate comparator therapy

a) Patients with 5q spinal muscular atrophy (5q SMA) type 1

Appropriate comparator therapy:

Nusinersen

Extent and probability of the additional benefit of onasemnogene abeparvovec compared to the appropriate comparator therapy:

An additional benefit is not proven

b) Patients with 5q SMA type 2 and up to 3 copies of the SMN2 gene

Appropriate comparator therapy:

Nusinersen

Extent and probability of the additional benefit of onasemnogene abeparvovec compared to the appropriate comparator therapy

An additional benefit is not proven

c) Patients with 5q SMA type 3 and up to 3 copies of the SMN2 gene

Appropriate comparator therapy:

Treatment according to the doctor's instructions with the selection of nusinersen or BSC

Extent and probability of the additional benefit of onasemnogene abeparvovec compared to the appropriate comparator therapy:

An additional benefit is not proven

d) Pre-symptomatic patients with 5q SMA and up to 3 copies of the SMN2 gene

Appropriate comparator therapy:

Nusinersen

Extent and probability of the additional benefit of onasemnogene abeparvovec compared to the appropriate comparator therapy:

An additional benefit is not proven

Study results according to endpoints:

a) Patients with 5q SMA type 1

There are no assessable data.

Summary of results for relevant clinical endpoints

Endpoint category	Direction of effect/ Risk of bias	Summary
Mortality	n.a.	There are no assessable data.
Morbidity	n.a.	There are no assessable data.
Health-related quality of life	∅	No data available.
Side effects	n.a.	There are no assessable data.
Explanations: ↑: statistically significant and relevant positive effect with low/unclear reliability of data ↓: statistically significant and relevant negative effect with low/unclear reliability of data ↑↑: statistically significant and relevant positive effect with high reliability of data ↓↓: statistically significant and relevant negative effect with high reliability of data ↔: no statistically significant or relevant difference ∅: There are no usable data for the benefit assessment. n.a.: not assessable		

b) Patients with 5q SMA type 2 and up to 3 copies of the SMN2 gene

No data available.

Summary of results for relevant clinical endpoints

Endpoint category	Direction of effect/ Risk of bias	Summary
Mortality	∅	No data available.
Morbidity	∅	No data available.
Health-related quality of life	∅	No data available.
Side effects	∅	No data available.
Explanations: ↑: statistically significant and relevant positive effect with low/unclear reliability of data ↓: statistically significant and relevant negative effect with low/unclear reliability of data ↑↑: statistically significant and relevant positive effect with high reliability of data ↓↓: statistically significant and relevant negative effect with high reliability of data ↔: no statistically significant or relevant difference ∅: There are no usable data for the benefit assessment. n.a.: not assessable		

c) Patients with 5q SMA type 3 and up to 3 copies of the SMN2 gene

No data available.

Summary of results for relevant clinical endpoints

Endpoint category	Direction of effect/ Risk of bias	Summary
Mortality	∅	No data available.
Morbidity	∅	No data available.
Health-related quality of life	∅	No data available.
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d) Pre-symptomatic patients with 5q SMA and up to 3 copies of the SMN2 gene

No data available.

Summary of results for relevant clinical endpoints

Endpoint category	Direction of effect/ Risk of bias	Summary
Mortality	∅	No data available.
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2. Number of patients or demarcation of patient groups eligible for treatment

a) Patients with 5q SMA type 1

b) Patients with 5q SMA type 2 and up to 3 copies of the SMN2 gene

c) Patients with 5q SMA type 3 and up to 3 copies of the SMN2 gene

approx. 45 to 65 patients

d) Pre-symptomatic patients with 5q SMA and up to 3 copies of the SMN2 gene

No specification possible

3. Requirements for a quality-assured application

The requirements in the product information are to be taken into account. The European Medicines Agency (EMA) provides the contents of the product information (summary of product characteristics, SmPC) for Zolgensma (active ingredient: onasemnogene abeparvovec) at the following publicly accessible link (last access: 11 August 2021):

https://www.ema.europa.eu/en/documents/product-information/zolgensma-epar-product-information_en.pdf

The resolution of 20 November 2020 on requirements for a quality-assured application for the use of onasemnogene abeparvovec in spinal muscular atrophy provides further details.

This medicinal product was approved under "special conditions". This means that further evidence of the benefit of the medicinal product is anticipated. The European Medicines Agency will evaluate new information on this medicinal product at a minimum once per year and update the product information where necessary.

4. Treatment costs

Treatment costs / Annual treatment costs:

Patient groups a, b and d

Designation of the therapy	Treatment costs or annual treatment costs/patient
Medicinal product to be assessed:	
Onasemnogene abeparvovec	€ 2,314,550[1]
Additionally required SHI services	different from patient to patient
Appropriate comparator therapy:	
Nusinersen 1st year	€ 566,745.01
additionally required SHI services:	non-quantifiable
Nusinersen subsequent years	€ 261,574.62
additionally required SHI services:	non-quantifiable

Costs after deduction of statutory rebates (LAUER-TAXE®) as last revised: 15 October 2021)

[1] Single dose

Patient group c

Designation of the therapy	Treatment costs or annual treatment costs/patient
Medicinal product to be assessed:	
Onasemnogene abeparvovec	€ 2,314,550[1]
Additionally required SHI services	different from patient to patient
Best supportive care	different from patient to patient
Appropriate comparator therapy:	
Nusinersen 1st year	€ 566,745.01
additionally required SHI services:	non-quantifiable
Nusinersen subsequent years	€ 261,574.62
additionally required SHI services:	non-quantifiable
Best supportive care	different from patient to patient

Costs after deduction of statutory rebates (LAUER-TAXE®) as last revised: 15 October 2021)

II. The resolution will enter into force on the day of its publication on the website of the G-BA on 4 November 2021.

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

Berlin, 4 November 2021

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

Prof. Hecken