Evinacumab (new therapeutic indication: homozygous familial hypercholesterolaemia, ≥ 5 to < 12 years)

Resolution of: 4 July 2024 Valid until: unlimited
Entry into force on: 4 July 2024
Federal Gazette, BAnz AT 16 08 2024 B2

New therapeutic indication (according to the marketing authorisation of 11 December 2023):

Evkeeza is indicated as an adjunct to diet and other low-density lipoprotein-cholesterol (LDL-C) lowering therapies for the treatment of adults and paediatric patients aged 5 years and older with homozygous familial hypercholesterolaemia (HoFH).

Therapeutic indication of the resolution (resolution of 4 July 2024):

Evkeeza is indicated as an adjunct to diet and other low-density lipoprotein-cholesterol (LDL-C) lowering therapies for the treatment of **children and adolescents aged 5 to < 12 years** with homozygous familial hypercholesterolaemia (HoFH).

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

Children and adolescents aged 5 to < 12 years with homozygous familial hypercholesterolaemia for whom dietary and medicinal lipid-lowering options have been exhausted

Appropriate comparator therapy:

- Evolocumab (10 years and older), possibly with concomitant lipid-lowering medicinal therapy, *or*
- LDL apheresis (as an "ultima ratio" for therapy-refractory courses), if necessary with concomitant lipid-lowering medicinal therapy, *or*
- Evolocumab (10 years and older) and LDL apheresis (as an "ultima ratio" for therapy-refractory courses), if necessary with concomitant lipid-lowering medicinal therapy

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

An additional benefit is not proven.

Study results according to endpoints:[1]

Children and adolescents aged 5 to < 12 years with homozygous familial hypercholesterolaemia for whom dietary and medicinal lipid-lowering options have been exhausted

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 ∅: No data available. n.a.: not assessable		

2. Number of patients or demarcation of patient groups eligible for treatment

Children and adolescents aged 5 to < 12 years with homozygous familial hypercholesterolaemia for whom dietary and medicinal lipid-lowering options have been exhausted

approx. 5-6 patients

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 Evkeeza (active ingredient: evinacumab) agreed upon in the context of the marketing authorisation at the following publicly accessible link (last access: 27 May 2024):

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

[1] Data from the dossier assessment of the Institute for Quality and Efficiency in Health Care (IQWiG) (A24-06) unless otherwise indicated.

This medicinal product was approved under “exceptional circumstances”. This means that due to the rarity of the disease, it was not possible to obtain complete information on this medicinal product. The EMA will assess any new information that becomes available on an annual basis, and, if necessary, the summary of product characteristics will be updated.

4. Treatment costs

Annual treatment costs:

Children and adolescents aged 5 to < 12 years with homozygous familial hypercholesterolaemia for whom dietary and medicinal lipid-lowering options have been exhausted

Designation of the therapy	Annual treatment costs/ patient
Medicinal product to be assessed:	
Evinacumab	€ 105,278.16- € 210,556.32
Cholestyramine	€ 87.69 - € 876.91
Ezetimibe	€ 96.14
Rosuvastatin	€ 45.63 - € 73.26
Evolocumab[2]	€ 5,336.44 - € 11,606.76
LDL apheresis	€ 23,150.18 - € 67,522.12
Evinacumab in combination with other lipid-lowering therapies	
Evinacumab + rosuvastatin + ezetimibe + cholestyramine	€ 105,507.62 - € 211,602.63
Evinacumab + rosuvastatin + evolocumab[2]	€ 110,660.23 - € 222,236.33
Evinacumab + rosuvastatin + ezetimibe + evolocumab[2]	€ 110,756.37 - € 222,332.47
Evinacumab + rosuvastatin + ezetimibe + cholestyramine + evolocumab[2]	€ 110,887.90 - € 223,209.39
Evinacumab + rosuvastatin + ezetimibe + cholestyramine + evolocumab[2] + LDL apheresis	€ 134,038.08 - € 290,731.51
Evinacumab + rosuvastatin + LDL apheresis	€ 128,473.96- € 278,151.70
Evinacumab + rosuvastatin + ezetimibe + LDL apheresis	€ 128,570.10 - € 278,247.84
Evinacumab + rosuvastatin + ezetimibe + cholestyramine + LDL apheresis	€ 128,657.80 - € 279,124.75
Appropriate comparator therapy:	
• Evolocumab[2] possibly with concomitant lipid-lowering medicinal therapy, *or* • LDL apheresis (as an "ultima ratio" for therapy-refractory courses), if necessary with concomitant lipid-lowering medicinal therapy, *or* • evolocumab[2] and LDL apheresis (as an "ultima ratio" for therapy-refractory courses), if necessary with concomitant lipid-lowering medicinal therapy	
Evolocumab[2]	€ 5,336.44 - € 11,606.76

[2] 10 years and older.

Designation of the therapy	Annual treatment costs/ patient
LDL apheresis	€ 23,150.18 - € 67,522.12
Cholestyramine	€ 87.69 - € 876.91
Ezetimibe	€ 96.14
Rosuvastatin	€ 45.63 - € 73.26
Evolocumab[2], if necessary + concomitant lipid-lowering medicinal therapy	
Evolocumab[2], if necessary + rosuvastatin	€ 5,382.07 - € 11,680.01
Evolocumab[2], if necessary + rosuvastatin + ezetimibe	€ 5,478.21 - € 11,776.15
Evolocumab[2], if necessary + rosuvastatin + ezetimibe + cholestyramine	€ 5,609.74 - € 12,653.07
LDL apheresis, if necessary + concomitant lipid-lowering medicinal therapy	
LDL apheresis, if necessary + rosuvastatin	€ 23,195.80 - € 67,595.38
LDL apheresis, if necessary + rosuvastatin + ezetimibe	€ 23,291.94 - € 67,691.52
LDL apheresis, if necessary + rosuvastatin + ezetimibe + cholestyramine	€ 23,379.64 - € 68,568.43
Evolocumab[2] and LDL apheresis, if necessary + concomitant lipid-lowering medicinal therapy	
Evolocumab[2] + LDL apheresis	€ 28,486.62 - € 79,128.88
Evolocumab[2] + LDL apheresis, if necessary + rosuvastatin	€ 28,532.24 - € 79,202.13
Evolocumab[2] + LDL apheresis, if necessary + rosuvastatin + ezetimibe	€ 28,628.38 - € 79,298.27
Evolocumab[2] + LDL apheresis, if necessary + rosuvastatin + ezetimibe + cholestyramine	€ 28,759.92 - € 80,175.19

Costs after deduction of statutory rebates (LAUER-TAXE®) as last revised: 15 June 2024)

Costs for additionally required SHI services: not applicable

Other SHI services:

Designation of the therapy	Type of service	Costs/ unit	Number/ cycle	Number/ patient year	Costs/ patient year
Medicinal product to be assessed:					
Evinacumab	Surcharge for the preparation of a parenteral solution containing monoclonal antibodies	€ 100	1	13.0	€ 1,300

5. Designation of medicinal products with new active ingredients according to Section 35a, paragraph 3, sentence 4 SGB V that can be used in a combination therapy with the assessed medicinal product

In the context of the designation of medicinal products with new active ingredients pursuant to Section 35a, paragraph 3, sentence 4 SGB V, the following findings are made:

Children and adolescents aged 5 to < 12 years with homozygous familial hypercholesterolaemia for whom dietary and medicinal lipid-lowering options have been exhausted

The following medicinal products with new active ingredients that can be used in a combination therapy with evinacumab in the therapeutic indication of the resolution on the basis of the marketing authorisation under Medicinal Products Act are named (active ingredients and invented names) in accordance with Section 35a, paragraph 3, sentence 4 SGB V:

evolocumab (Repatha) [10 years and older]

The designation of combinations exclusively serves the implementation of the combination discount according to Section 130e SGB V between health insurance funds and pharmaceutical companies. The findings made neither restrict the scope of treatment required to fulfil the medical treatment mandate, nor do they make statements about expediency or economic feasibility.

II. In Annex XIIa of the Pharmaceuticals Directive, the following information shall be added in alphabetical order:

"Active ingredient of the assessed medicinal product

Evinacumab

Resolution according to Section 35a paragraph 3 SGB V from

4 July 2024

Therapeutic indication of the resolution

Evkeeza is indicated as an adjunct to diet and other low-density lipoprotein-cholesterol (LDL-C) lowering therapies for the treatment of children and adolescents aged 5 to < 12 years with homozygous familial hypercholesterolaemia (HoFH).

Patient group

Children and adolescents aged 5 to < 12 years with homozygous familial hypercholesterolaemia for whom dietary and medicinal lipid-lowering options have been exhausted

Naming of medicinal products with new active ingredients according to Section 35a, paragraph 3, sentence 4 SGB V (active ingredients and invented names[2])

evolocumab (Repatha) [10 years and older]

Period of validity of the designation (since... or from... to)

Since 4 July 2024

The designation of combinations exclusively serves the implementation of the combination discount according to Section 130e SGB V between health insurance funds and pharmaceutical companies. The findings made neither restrict the scope of treatment required to fulfil the medical treatment mandate, nor do they make statements about expediency or economic feasibility.