SUMMARY

evinacumab

EVKEEZA 150 mg/ml

concentrate for solution for infusion

Reassessment

Original French opinion adopted by the Transparency Committee on 24 April 2024

The legally binding text is the original French opinion version

Summary of opinion

Favourable opinion for maintenance of reimbursement for the treatment of adults and adolescents aged 12 years and older with homozygous familial hypercholesterolaemia (HoFH) as an adjunct to diet and other low-density lipoprotein-cholesterol (LDL-C) lowering therapies.

Transparency Committee's conclusions

Clinical Benefit

- Homozygous familial hypercholesterolaemia is a very rare and severe disease, characterised by the presence of extravascular cholesterol deposits, high LDL levels (>3.30 g/L) and cardiovascular diseases, from childhood onwards. Such diseases are favoured by these dyslipidaemias and can be prematurely life-threatening due to complications.
- The proprietary medicinal product EVKEEZA (evinacumab) is a preventive treatment.
- The efficacy of EVKEEZA (evinacumab) in combination with other lipid-lowering therapies (statins + other lipid-lowering medications +/- LDL-apheresis) has been demonstrated on reduction of LDL-c levels. The efficacy in terms of morbidity and mortality has therefore only been partially demonstrated to date. However, the efficacy/adverse effects ratio remains high.
- There is an alternative currently only available to treat adults: LOJUXTA (lomitapide), which is not widely used in France and which has known serious adverse effects, particularly hepatic.

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➔ It is a last-line treatment that should be reserved for adult patients and adolescents over 12 years of age with uncontrolled HoFH, as an adjunct to a low-fat diet and in combination with an optimised oral lipid-lowering treatment, including at least one statin, ezetimibe and a PCSK9 inhibitor (evolocumab), with or without LDL apheresis.

➔ Public health benefit

Considering:

- the seriousness of the cardiovascular diseases promoted by HoFH,
- the rarity of this disease with a current prevalence of about 1 case/300,000,
- the medical need to have access to effective and well-tolerated medicines in children, adolescents and adults with HoFH,
- the partial response of EVKEEZA (evinacumab) to the medical need, due to:
  - the demonstrated impact of EVKEEZA on LDL-c reduction but in the absence of a fully demonstrated impact on morbidity and mortality,
  - the lack of evidence of an impact of EVKEEZA (evinacumab) on quality of life and the organisation of care,

EVKEEZA (evinacumab) is unlikely to have a public health benefit.

Considering all these elements, the Committee deems that the clinical benefit of EVKEEZA (evinacumab) remains substantial in the MA indication.

The Committee issues a favourable opinion for maintenance of inclusion of EVKEEZA (evinacumab) in the list of covered drugs for inpatients approved for use in the indication and at the MA dosages.

Clinical Added Value

Therapeutic improvement in the care pathway.

Considering:

- the final results of the open-label extension study (OLE) with longer follow-up of evinacumab treatment in 116 patients (median follow-up of 2 years with around 25% of patients having a treatment follow-up period of ≥ 2.5 years), which demonstrate maintenance of the efficacy of evinacumab on LDL-c reduction and support the safety profile judged to be favourable for evinacumab with no new safety signals observed;
- the inadequately met medical need to have access to effective and well-tolerated medicines reducing the need for LDL apheresis, in patients not controlled despite optimised lipid-lowering treatment, in particular those with a biallelic mutation on the LDLR gene devoid of functional LDLR receptors;

the Transparency Committee deems that EVKEEZA (evinacumab) provides a moderate clinical added value (CAV III) in the care pathway for the treatment of adults and adolescents aged 12 years and older with homozygous familial hypercholesterolaemia (HoFH) as an adjunct to diet and other low-density lipoprotein-cholesterol (LDL-C) lowering therapies.

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