SUMMARY

lanadelumab

TAKHZYRO 150 mg

solution for injection in pre-filled syringe

First listing

Original French opinion adopted by the Transparency Committee on 14 February 2024

Summary of opinion

Favourable opinion for reimbursement in “routine prevention of recurrent attacks of hereditary angioedema (HAE) in patients aged from 2 years to less than 12 years.”

Transparency Committee’s conclusions

Clinical Benefit

- Hereditary angioedema (HAE) is characterised by episodes of subcutaneous and/or submucosal oedema, which are transient and recurrent and affect variable locations (cutaneous, gastrointestinal, pharyngeal, etc.). HAE is a rare and incapacitating, chronic genetic disease, with a major impact on quality of life and which can be life-threatening in the event of laryngeal location.
- This is a preventive medicinal product.
- The efficacy/adverse effects ratio is high.
- This is a first-line treatment in view of the therapies available.

Public health benefit

Considering:

- the rarity and seriousness of the disease, which can be life-threatening in the event of laryngeal location,
- the identified medical need to have access to an effective, well-tolerated alternative that is more convenient than the IV route in the context of long-term prevention for patients aged 6 years and older and the unmet medical need for patients aged from 2 years to less than 6 years,
- the partial response to the identified need given the available data based on a non-comparative study, but the absence of a therapeutic alternative in patients aged from 2 years to less than 6 years, and the absence of comparison with a clinically relevant comparator in patients aged from 6 years to less than 12 years, suggesting:
  - an expected, but not demonstrated, additional impact on morbidity, mortality and on quality of life,

- an expected additional impact on the patient's care and life pathway given the administration conditions of the medicinal product, with a subcutaneous injection every 2 or 4 weeks, despite the absence of data quantifying this impact,

TAKHZYRO (lanadelumab) is likely to have an impact on public health.

Considering all these elements, the Committee deems that the clinical benefit of TAKHZYRO (lanadelumab) 150 mg solution for injection in pre-filled syringe is substantial in the prevention of recurrent attacks of hereditary angioedema (HAE) in patients aged from 2 years to less than 12 years.

The Committee issues a favourable opinion for inclusion of TAKHZYRO (lanadelumab) 150 mg solution for injection in pre-filled syringe in both the list of covered drugs for inpatients and the list of covered drugs for outpatients approved for use in the routine prevention of recurrent attacks of hereditary angioedema (HAE) in patients aged from 2 years to less than 12 years and at the MA dosages.

➔ **Recommended reimbursement rate for inclusion in the list of covered drugs for outpatients: 65%**

Clinical Added Value

In children aged from 2 years to less than 6 years

Considering:

- the suggested efficacy of TAKHZYRO (lanadelumab) in a non-comparative phase 3 study in 21 patients with a median age of 8.7 years (min 3.5 – max 10.9 years) over a significant period of 52 weeks, with a marked reduction in the number of attacks per month, decreasing from 1.8 to 0,08, i.e. a 94.8% reduction, and the number of severe attacks, decreasing from 1.27 to 0.08, i.e. a reduction of 96.8%,
- a safety profile marked primarily by pain at injection site, skin abrasions and headaches, and which appears to be similar to that observed in older patients,
- the convenience of TAKHZYRO (lanadelumab), with subcutaneous administration every 2 to 4 weeks,
- the unmet medical need in the absence of a therapeutic alternative in these patients and the uncertain efficacy of tranexamic acid used off-label,

the Committee deems that TAKHZYRO (lanadelumab) 150 mg provides a moderate clinical added value (CAV III) in the prevention of recurrent attacks of hereditary angioedema in patients aged 2 years to less than 6 years.

In children aged from 6 years to 11 years

Considering:

- the suggested efficacy of TAKHZYRO (lanadelumab) in a non-comparative phase 3 study in 21 patients with a median age of 8.7 years (with the majority of patients (81%) aged from 6 years to under 12 years) over a significant period of 52 weeks, with a marked reduction in the number

of attacks per month, decreasing from 1.8 to 0,08, i.e. a 94.8 % reduction, and the number of severe attacks, decreasing from 1.27 to 0.08, i.e. a reduction of 96.8%,

- a safety profile marked primarily by pain at injection site, skin abrasions and headaches, and which appears to be similar to that observed in older patients,
- the convenience of TAKHZYRO (lanadelumab), with subcutaneous administration every 2 to 4 weeks, compared with the alternative, CINRYZE (C1 esterase inhibitor), which is administered by the IV route every 3 to 4 days,
- but the absence of comparison versus the clinically relevant comparator CINRYZE (C1 esterase inhibitor), despite the fact that such a comparison would have been possible,

the Committee deems that TAKHZYRO (lanadelumab) 150 mg provides a minor clinical added value (CAV IV) in the prevention of recurrent attacks of hereditary angioedema in patients aged 6 years to 11 years.