SUMMARY

vamorolone

AGAMREE 40 mg/mL

oral suspension

First listing

Original French opinion adopted by the Transparency Committee on 12 June 2024

The legally binding text is the original French opinion version

Summary of opinion

Favourable opinion for reimbursement in the "the treatment of Duchenne muscular dystrophy (DMD) in patients aged 4 years and older".

Transparency Committee's conclusions

Clinical Benefit

- Duchenne muscular dystrophy is a rare disease, with a prevalence of 1/3,500 to 1/9,300 male births, an unfavourable prognosis with cardiomyopathy and respiratory failure that can lead to death during adolescence and have a significant impact on patients' quality of life.
- This is a symptomatic medicinal product.
- The efficacy/adverse effects ratio is moderate given the observed effect size, which cannot be robustly estimated compared to prednisone, and its safety profile does not appear to be more favourable than that of prednisone in a study versus placebo and versus prednisone.
- This is a first-line treatment with regard to the available therapies.

Public health benefit

Considering:

- the seriousness of the disease and its prevalence,
- the partially met medical need,
- the partial response to the identified need given:
  - the lack of evidence of an additional impact on morbidity and mortality and on quality of life in the comparative placebo-controlled study versus prednisone, which did not enable a robust comparison between vamorolone and prednisone,

- the lack of evidence of an additional impact on the organisation of care, and the care or life pathway for patients or their families,

AGAMREE (vamorolone) is unlikely to have an additional impact on public health.

Considering all these elements, the Committee deems that the clinical benefit of AGAMREE 40 mg/mL (vamorolone) oral suspension is moderate in the MA indication.

The Committee issues a favourable opinion for inclusion of AGAMREE 40 mg/mL (vamorolone) oral suspension in both the list of covered drugs for outpatients and the list of covered drugs for inpatients approved for use in the MA indication and at the MA dosages.

➔ **Recommended reimbursement rate for inclusion in the list of covered drugs for outpatients: 30%**

Clinical Added Value

Considering:

- the non-analysable results for the comparison between vamorolone and prednisone, scheduled in the hierarchical sequence of secondary endpoints in the comparative study versus placebo and versus prednisone,
- the results relative to the superiority of vamorolone versus placebo for the primary endpoint, i.e. the change from baseline in Time to Stand Test (TTSTAND) velocity in patients treated with vamorolone 6 mg/kg versus placebo at week 24,
- the lack of analysable results in this study for quality of life criteria, since these were the subject of a descriptive evaluation only,
- the safety profile of vamorolone in the study versus placebo and versus prednisone, which does not appear to be more favourable than that of prednisone; in particular, in the same way as with glucocorticoid therapy, weight gain was observed during treatment with vamorolone,
- uncertainties with respect to the long-term safety of vamorolone; in particular, analysis over a limited time of 30 months does not make it possible to confirm the expected positive effect on children's growth, and there are no data available on a potential effect of vamorolone on puberty,

the Committee deems that AGAMREE 40 mg/mL (vamorolone) oral suspension provides no clinical added value (CAV V) compared to the prednisone-based drugs used off-label, but recommended in the treatment of Duchenne muscular dystrophy.