SUMMARY

ataluren

TRANSLARNA 125 mg, 250 mg, 1,000 mg

granules for oral suspension

Reassessment at the request of the CT

Adopted by the Transparency Committee on 23 October 2024

Summary of opinion

Unfavourable opinion for reimbursement in "the treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 2 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 of TRANSLARNA (ataluren) has not been established given the absence of evidence of a superiority of ataluren on the 6-minute walk test compared to placebo in three studies; its safety profile otherwise being favourable.
- TRANSLARNA (ataluren) has no role in the care pathway for Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 2 years and older.

Public health benefit

Considering:

- the seriousness of the disease and its prevalence,
- the partially met medical need,
- the lack of response to the identified need given:
  - the lack of evidence of an additional impact on morbidity and mortality or on quality of life compared to placebo in three clinical trials,

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

TRANSLARNA (ataluren) is unlikely to have an additional impact on public health.

Considering all these elements, the Committee deems that the clinical benefit of TRANSLARNA 125 mg, 250 mg, 1,000 mg (ataluren) granules for oral suspension is insufficient in the MA indication to justify public funding.

The Committee issues a favourable opinion for inclusion of TRANSLARNA 125 mg, 250 mg, 1,000 mg (ataluren) granules for 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.