

Original French opinion adopted by the Transparency Committee.

The legally binding text is the original French opinion version.

SUMMARY

tiratricol

EMCITATE 350 µg

dispersible tablets

Inclusion on list

Adopted by the Transparency Committee on 10 September 2025

Summary of opinion

Unfavourable opinion for reimbursement in “the treatment of peripheral thyrotoxicosis in patients with monocarboxylate transporter 8 (MCT8) deficiency (Allan-Herndon-Dudley Syndrome), from birth”.

Transparency Committee’s conclusions

Clinical Benefit

- ➔ Monocarboxylate transporter 8 (MCT8) deficiency is a rare and disabling disease that can lead to potentially fatal serious complications, including lung infections and choking on food due to hypotonia of the neck.
- ➔ This is a curative and symptomatic medicinal product.
- ➔ The efficacy/adverse effects ratio has not been adequately established given the efficacy demonstrated for biological endpoints only, in the absence of evidence of an improvement for clinically relevant endpoints, during a non-comparative study.
- ➔ EMCITATE (tiratricol) has no role in the care pathway.

➔ Public health benefit

Considering:

- the seriousness of the disease and its low prevalence,
- the currently unmet medical need,
- the lack of response to the identified need due to:
 - the lack of evidence of an additional impact on morbidity and mortality and on quality of life,
 - the lack of evidence of an additional impact on the organisation of care and the patient’s life pathway,

EMCITATE (tiratricol) is unlikely to have an additional impact on public health.

Considering all of these elements, the Committee deems that the clinical benefit of EMCITATE (tiratricol) dispersible tablets is insufficient in the MA indication to justify public funding.

The Committee issues an unfavourable opinion for inclusion of EMCITATE (tiratricol) dispersible tablets in both the list of covered drugs for outpatients and the list of covered drugs for inpatients approved for use in the MA indication.