SUMMARY

pegzilarginase

LOARGYS 5 mg/ml

solution for injection/infusion

First listing

Original French opinion adopted by the Transparency Committee on
29 May 2024

The legally binding text is the original French opinion version

Summary of opinion

Favourable opinion for reimbursement in the “treatment of arginase 1 deficiency (ARG1-D), also known as hyperargininemia, in adults, adolescents and children aged 2 years and older”.

Transparency Committee’s conclusions**Clinical Benefit**

- ➔ Arginase 1 deficiency is a serious disease generally starting in childhood and causing a gradual loss of cognitive and motor functions, associated with spasticity and epilepsy, with an impact on the functional performance and quality of life of patients.
- ➔ This is a curative medicinal product.
- ➔ The efficacy/adverse effects ratio is high.
- ➔ This is a first-line treatment in the absence of available therapies.

➔ Public health benefit

Considering:

- the seriousness of the disease and its prevalence,
- the unmet medical need,
- the partial response to the identified need given:
- evidence of an additional impact on change in plasma arginine at 24 weeks, but with no demonstrated impact to date on morbidity and mortality and on quality of life,
- the lack of evidence of an impact on the organisation of care, and the care or life pathway for patients or their families,

LOARGYS (pegzilarginase) is unlikely to have an additional impact on public health.

Considering all these elements, the Committee deems that the clinical benefit of LOARGYS (pegzilarginase) is substantial in the treatment of arginase 1 deficiency (ARG1-D), also known as hyperargininemia, in adults, adolescents and children aged 2 years and older.

The Committee issues a favourable opinion for inclusion of LOARGYS (pegzilarginase) in both the list of covered drugs for outpatients and the list of covered drugs for inpatients approved for use in the indication and at the MA dosages.

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

Clinical Added Value

Considering:

- demonstration of the superiority of LOARGYS (pegzilarginase) for the primary endpoint, which was a major biological endpoint for treatment follow-up, i.e. change in plasma arginine at 24 weeks compared to placebo, both combined with individualised disease management (dietary protein restriction $\pm$ nitrogen scavengers) in a phase 3, randomised, double-blind trial conducted in 32 patients with arginase 1 deficiency, aged from 2 to 29 years (mean age 10.7 years),
- the safety profile of LOARGYS (pegzilarginase), which seems to be favourable with limited follow-up,
- the absence of evidence of a benefit of LOARGYS (pegzilarginase) on neurocognitive or motor impairment,
- exploratory results concerning simplification of background therapy, which is particularly restrictive, in parallel with plasma arginine levels,
- exploratory results concerning quality of life in this disease with a significant impact on quality of life,

the Committee deems that LOARGYS (pegzilarginase) 5 mg/ml solution for injection/infusion provides a minor clinical added value (CAV IV) in the current care pathway.