SUMMARY

odevixibat

KAYFANDA 200, 400, 600, 1,200 micrograms

hard capsules

Inclusion on list

Adopted by the Transparency Committee on 15 January 2025

Summary of opinion

Favourable opinion for reimbursement in "the treatment of cholestatic pruritus in Alagille syndrome (ALGS) in patients aged 6 months or older."

Transparency Committee's conclusions

Clinical Benefit

- KAYFANDA (odevixibat) is intended for the treatment of cholestatic pruritus in Alagille syndrome (ALGS), a rare, serious and disabling disease. The associated itching, which is common, can be very severe and highly incapacitating, and may result in recourse to biliary diversion surgery and/or liver transplantation.
- This is a symptomatic medicinal product.
- The efficacy/adverse effects ratio of odevixibat in the treatment of cholestatic pruritus in Alagille syndrome in patients aged 6 months or older is high.
- This is a first-line treatment in view of the therapies available.
- There is a medicinal alternative: maralixibat (LIVMARMI) indicated from the age of 2 months.

Public health benefit

Considering:

- **the seriousness of pruritus in Alagille syndrome (rare disease), which can be incapacitating**,
- **its low prevalence** in **patients who can be very young**,
- the **inadequately met medical need** with, in particular, a lower level of evidence of efficacy on pruritus with LIVMARLI (maralixibat) in this indication,
- a **partial response to the identified need**, given **an established effect on pruritus**, **with this symptom having a major impact on quality of life**;

but also considering the fact that:

- this treatment could reduce the risk of progression to cirrhosis secondary to cholestasis, bearing in mind that it is not an aetiological treatment since it has no action on bile duct paucity;
- **there are no data on morbidity and mortality (insufficient follow-up in the pivotal study), particularly concerning recourse to liver transplantation** (impact on the organisation of care, with reduced use of resources, remaining to be determined)

KAYFANDA (odevixibat) is unlikely to have an additional impact on public health.

Considering all these elements, the Committee deems that the clinical benefit of KAYFANDA (odevixibat) is substantial in the MA indication.

The Committee issues a favourable opinion for inclusion of KAYFANDA (odevixibat) 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: 100%.**

Clinical Added Value

Considering:

- **the results of a randomised, placebo-controlled, phase 3 trial conducted in children with a rare disease, demonstrating a clinically relevant effect in terms of reducing pruritus (reduction in scratching severity score) in 52 patients;**
- **the safety profile of odevixibat;**
- **but also the absence of data on cholestasis complications and recourse to transplantation due to refractory pruritus,**

the Committee deems that KAYFANDA (odevixibat) provides a moderate clinical added value (CAV III) in the current care pathway.