Original French opinion adopted by the Transparency Committee on [Date]

The legally binding text is the original French opinion version

SUMMARY

elafibranor

IQIRVO 80 mg

film-coated tablets

Inclusion on list

Adopted by the Transparency Committee on 29 January 2025

Summary of opinion

Favourable opinion for reimbursement in the "treatment of primary biliary cholangitis (PBC) in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA, or as monotherapy in adults unable to tolerate UDCA."

Transparency Committee's Conclusions

Clinical Benefit

- Primary biliary cholangitis is a rare, chronic autoimmune inflammatory disease characterised by the progressive destruction of the bile ducts within the liver, causing chronic cholestasis and the gradual development of fibrosis.
- This is a curative medicinal product.
- The efficacy/adverse effects ratio is moderate.
- There is a therapeutic alternative (bezafibrate, used off-label).
- This is a second-line treatment in view of the therapies available.

Public health benefit

Considering:

- the seriousness of the disease, ultimately leading to chronic cholestasis and the progressive development of hepatic fibrosis,
- its low prevalence,
- the partially met medical need in patients with an inadequate response or unable to tolerate UDCA,
- the partial response to the identified need given:
  - the lack of evidence of an impact on morbidity and mortality, quality of life, pruritus and fatigue,
  - uncertainties concerning the clinical benefit in patients with PBC, and concerning the safety profile,

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

IQIRVO (elafibranor) is unlikely to have an additional impact on public health.

Considering all of these elements, the Committee deems the clinical benefit of IQIRVO (elafibranor) is moderate in the MA indication.

The Committee issues a favourable opinion for inclusion of IQIRVO (elafibranor) 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. The Committee makes maintenance of the moderate clinical benefit conditional on its reassessment based on the results of the phase 3 confirmatory study (ELFIDENCE CLIN-60190-454 study) required by the EMA in the context of the conditional MA.

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

Clinical Added Value

Considering:

- an adapted development plan in primary biliary cholangitis for elafibranor, with two randomised controlled studies (phase 3 pivotal study and phase 3 confirmatory study ongoing), with efficacy results in favour of elafibranor, but in comparison with placebo, concerning an interim endpoint (alkaline phosphatase and bilirubin levels),
- a reassuring safety profile based on the available data,

but also:

- a low effect size for normalisation of alkaline phosphatase (more relevant biochemical endpoint) and the absence of an established effect on bilirubin levels (relevant endpoint) of elafibranor in comparison with placebo,
- the lack of evidence of efficacy on pruritus (highly incapacitating symptom) in comparison with placebo,
- the absence of established efficacy on disease progression endpoints, in particular clinical events or transplantation-free survival (relevant assessment endpoints),
- the lack of comparative data versus bezafibrate (used off-label, but recommended by experts), which is a clinically relevant comparator in second-line treatment,

the Committee considers that, on the basis of currently available data, IQRVO 80 mg film-coated tablets (elafibranor) provides no clinical added value (CAV V) in the current care pathway, in combination with ursodeoxycholic acid (UDCA) in adults with primary biliary cholangitis with an inadequate response to UDCA, or as monotherapy in adults unable to tolerate UDCA.