HAUTE AUTORITÉ DE SANTÉ

HEPATOLOGY
New medicinal product
June 2017

BRIEF SUMMARY OF THE TRANSPARENCY COMMITTEE OPINION

OCALIVA (obeticholic acid), bile acid

No clinical benefit demonstrated in the treatment of primary biliary cholangitis in combination with ursodeoxycholic acid in adults with insufficient response to UDCA, or as monotherapy in adults who do not tolerate UDCA.

Main points

- OCALIVA has marketing authorisation in the treatment of primary biliary cholangitis in combination with ursodeoxycholic acid (UDCA) in adults with insufficient response to UDCA, or as monotherapy in adults who do not tolerate UDCA.
- Its efficacy has been demonstrated versus placebo only on an interim composite biochemistry endpoint but with no impact on morbidity and mortality.
- This is a second-line treatment.

Therapeutic strategy

- In patients with primary biliary cholangitis, the first-line treatment is based on the prescription of UDCA, regardless of the stage of the disease. The effectiveness of UDCA is better when treatment is initiated at an early stage of the disease.
- At a more advanced stage of the disease, which has become very rare with UDCA, liver transplantation is often necessary.
- **Role of the proprietary medicinal product in the therapeutic strategy**
  OCALIVA can be offered as second-line treatment only to patients who do not respond to or who do not tolerate UDCA.

Clinical data

- A randomised, double-blind study evaluated the efficacy of OCALIVA 10 mg/day, with or without titration (5 mg/day for 6 months, then 10 mg/day) versus placebo, on a composite biochemistry endpoint in 216 patients with primary biliary cholangitis with insufficient response or intolerance to UDCA.
- After 12 months of treatment, the percentage of responders simultaneously achieving ALP activity < 1.67 ULN, a reduction of ALP activity $\geq$ 15% and a total bilirubin < ULN was greater in the groups treated with OCALIVA than in the placebo group (-47% and -46% versus -10%).
- The most common and most severe adverse effect was pruritus, occurring primarily in the month following initiation of treatment and most commonly observed when there was no titration.

Benefit of the medicinal product

- The actual clinical benefit* of OCALIVA is substantial.
- OCALIVA does not provide any clinical added value** (CAV V) in patients with primary biliary cholangitis when there is failure or intolerance of UDCA.
- Recommends inclusion on the list of reimbursable products for supply by pharmacists and for hospital use.

HAUTE AUTORITÉ DE SANTÉ

This document was created on the basis of the Transparency Committee Opinion of 07 June 2017 (CT-15943) and is available at **www.has-sante.fr**

* The actual clinical benefit (ACB) of a medicinal product describes its benefit primarily in terms of its clinical efficacy and the seriousness of the condition being treated. The HAS Transparency Committee assesses the ACB, which can be substantial, moderate, low or insufficient for reimbursement of the medicinal product for hospital use.

** The clinical added value (CAV) describes the improvement in treatment provided by a medicinal product compared with existing treatments. The HAS Transparency Committee assesses the degree of CAV on a scale from I (major) to IV (minor). A level V CAV (equivalent to “no CAV”) means “no clinical added value”.