EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

13 October 2016
EMA/CHMP/651446/2016
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Ocaliva

obeticholic acid

On 13 October 2016, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a conditional marketing authorisation for the medicinal product Ocaliva, intended for the treatment of primary biliary cholangitis (also known as primary biliary cirrhosis). Ocaliva was designated as an orphan medicinal product on 27 July 2010. The applicant for this medicinal product is Intercept Pharma Ltd.

Ocaliva will be available as 5 mg and 10 mg tablets. The active substance of Ocaliva is obeticholic acid, a bile acid preparation (ATC code: A05AA04). By activating the farnesoid X receptor (FXR), Ocaliva is expected to reduce the production of bile in the liver, thus reducing the exposure of the liver to toxic levels of bile acids.

The benefits with Ocaliva are its ability to reduce alkaline phosphatase and bilirubin levels in adults with primary biliary cholangitis. This is likely to lead to clinical benefits for the patient such as delayed development of liver fibrosis, cirrhosis liver transplant and death. However, this remains to be formally demonstrated by means of the post-authorisation follow up within this conditional marketing authorisation.

The most common side effects are itching and feeling tired which are also common symptoms of the disease. Elevations in alanine amino transferase (ALT) and aspartate aminotransferase (AST) have been observed in patients taking obeticholic acid. Infrequently clinical signs and symptoms of hepatic decompensation have also been observed in clinical studies even though primarily at doses higher than the maximum recommended dose. These events have occurred as early as within the first month of treatment. Liver-related adverse events have primarily been observed at doses higher than the maximum recommended dose of 10 mg once daily.

The full indication is: "OCALIVA is indicated for the treatment of primary biliary cholangitis (also known as primary biliary cirrhosis) in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA or as monotherapy in adults unable to tolerate UDCA."

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion

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