



EUROPEAN MEDICINES AGENCY  
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## Ocaliva (*obeticholic acid*)

An overview of Ocaliva and why it is authorised in the EU

### What is Ocaliva and what is it used for?

Ocaliva is used to treat adults with a liver disease known as primary biliary cholangitis.

Primary biliary cholangitis is an autoimmune condition in which there is gradual destruction of the small bile ducts in the liver. These ducts transport fluid called bile from the liver to the intestines, where it is used to help digest fats. As a result of the damage to the ducts, bile builds up in the liver causing damage to the liver tissue. This may lead to scarring and liver failure, and may increase the risk of liver cancer.

Ocaliva contains the active substance obeticholic acid. It is used together with another medicine, ursodeoxycholic acid (UDCA), in patients who do not respond sufficiently to UDCA alone, and on its own in patients who cannot take UDCA.

Primary biliary cholangitis is rare, and Ocaliva was designated an 'orphan medicine' (a medicine used in rare diseases) on 27 July 2010. Further information on the orphan designation can be found here: [ema.europa.eu/Find\\_medicine/Human\\_medicines/Rare\\_disease\\_designation](http://ema.europa.eu/Find_medicine/Human_medicines/Rare_disease_designation).

### How is Ocaliva used?

Ocaliva is available as tablets (5 and 10 mg) and can only be obtained with a prescription. The recommended starting dose is 5 mg once a day or 5 mg once a week, depending on the patient's degree of liver impairment (which should be determined before starting treatment with Ocaliva). After a few months, if Ocaliva is well tolerated the dose can be increased. Doses can be reduced or treatment may need to be stopped in patients who experience intolerable itching (a possible side effect with Ocaliva).

For more information about using Ocaliva, see the package leaflet or contact your doctor or pharmacist.



## **How does Ocaliva work?**

The active substance in Ocaliva, obeticholic acid, is a modified form of a bile acid (the main components of bile). It works mainly by attaching to receptors in the liver and gut (farnesoid X receptors) which control the production of bile. By attaching to these receptors, Ocaliva reduces the production of bile in the liver, preventing it from building up and damaging the liver tissue.

## **What benefits of Ocaliva have been shown in studies?**

Ocaliva was compared with placebo (a dummy treatment) in a main study involving 217 adults with primary biliary cholangitis who either had been taking UDCA for at least 1 year, or who could not take UDCA. The measure of effectiveness was based on the number of patients whose blood levels of the substances bilirubin and ALP (markers of liver damage) decreased by at least 15% (for ALP) and below a certain value considered normal (for bilirubin) after 1 year of treatment.

The study showed that Ocaliva was more effective than placebo at reducing the blood levels of bilirubin and ALP: levels decreased by the required amount in 47% (34 out of 73) of patients treated with Ocaliva 10 mg and in 46% (32 out of 70) of patients treated with increasing doses of Ocaliva (from 5 mg up to 10 mg), compared with 10% (7 out of 73) of patients on placebo.

## **What are the risks associated with Ocaliva?**

The most common side effects with Ocaliva are itching (which may affect more than 6 in 10 people) and tiredness (which may affect more than 2 in 10 people). Itching is also the most common side effect that can lead to discontinuation of treatment. In the majority of cases seen, itching occurred within the first month of treatment and tended to resolve over time while continuing treatment. For the list of all side effects with Ocaliva, see the package leaflet.

Ocaliva must not be used in patients whose bile ducts are completely blocked. For the full list of restrictions, see the package leaflet.

## **Why is Ocaliva authorised in the EU?**

Patients with primary biliary cholangitis have limited treatment options. Ocaliva has been shown to reduce the blood levels of bilirubin and ALP in patients with primary biliary cholangitis, including those who could not be treated with UDCA. Reductions in bilirubin and ALP were to an extent which is indicative of an improvement in the condition of the liver. However, the clinical benefits of Ocaliva need to be demonstrated in further studies. The safety profile of the medicine was considered to be favourable, with side effects that were tolerable and manageable with supportive treatment (e.g. for itching) and dose adjustments. The European Medicines Agency therefore decided that Ocaliva's benefits are greater than its risks and it can be authorised for use in the EU.

Ocaliva has been given 'conditional authorisation'. This means that there is more evidence to come about the medicine, which the company is required to provide. Every year, the Agency will review any new information that becomes available and this overview will be updated as necessary.

## **What information is still awaited for Ocaliva?**

Since Ocaliva has been granted a conditional authorisation, the company that markets Ocaliva will provide additional data from two studies to confirm the effectiveness and safety of the medicine. The first study is designed to demonstrate the clinical benefit of Ocaliva, while the second study will investigate the benefits of Ocaliva in patients with moderate to severe liver disease.

## **What measures are being taken to ensure the safe and effective use of Ocaliva?**

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Ocaliva have been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Ocaliva is continuously monitored. Side effects reported with Ocaliva are carefully evaluated and any necessary action taken to protect patients.

## **Other information about Ocaliva**

Ocaliva received a marketing authorisation valid throughout the EU on 12 December 2016.

Further information on Ocaliva can be found on the Agency's website: [ema.europa.eu/Find/medicine/Human medicines/European public assessment reports](http://ema.europa.eu/Find/medicine/Human%20medicines/European%20public%20assessment%20reports).

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