

(English version)

**Question for written answer E-001881/25
to the Commission
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(12 May 2025)**

Subject: Withdrawal of authorisation of the drug Ocaliva for treatment of primary biliary cholangitis

On 30 August 2024, the European Commission removed the marketing authorisation for the drug Ocaliva (obeticholic acid), used to treat primary biliary cholangitis, a rare, chronic and progressive liver disease.

As a result of this decision, Advanz Pharma Specialty Medicine Italia voluntarily withdrew the drug from sale in Italy and it was taken off the list maintained under Law No 648/96 of drugs paid for by the national health service.

Ocaliva was the only approved second-line treatment for patients who do not respond to or cannot tolerate ursodeoxycholic acid treatment, and its withdrawal has left many patients without any viable treatment options.

Although the European Medicines Agency has suggested compassionate use or programmes for designated patients, access to such measures remains fragmented at European level, giving rise to inequalities in access to treatment.

Can the Commission therefore say:

1. Whether, in view of the provisions of EU law, it believes measures such as the purchase of Ocaliva from abroad should be taken to enable Italian patients to continue their treatment?
2. How it intends to ensure continuity of treatment for patients without any valid therapeutic alternatives, even given the emergence of new drugs?

Submitted: 12.5.2025

**Answer given by Mr Várhelyi on behalf of the European Commission
(3 July 2025)**

In 2016, the medicinal product Ocaliva was granted a conditional marketing authorisation in the EU to treat adults with primary biliary cholangitis.

The authorisation was based on a less complete dataset but was conditional on the submission of the results of a post-authorisation study to confirm that observed biochemical changes translated into positive clinical outcomes.

However, the completed study results did not show the expected improvement in clinical outcomes over placebo and highlighted related safety issues.

Following a request from the Commission, the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) evaluated all available evidence about the efficacy and safety of Ocaliva, including feedback from physicians' associations and patient groups.

The CHMP concluded that the benefits of Ocaliva no longer outweighed its risks⁽¹⁾. Consequently, on 27 June 2024, the CHMP recommended revoking the conditional marketing authorisation for Ocaliva. Based on the CHMP's recommendation, the Commission adopted a decision on 30 August 2024, revoking the conditional marketing authorisation for this product⁽²⁾.

As far as alternative treatment options, there are other medicines currently authorised in the EU for the treatment of the same condition.

In addition, in exceptional circumstances, Member States can also allow at national level the supply of an unauthorised medicine to patients already under treatment⁽³⁾, or to an individual patient under the direct personal responsibility of the healthcare professional⁽⁴⁾.

More generally, patients' treatment decisions should be made by their consulting medical doctor in the patient's best interest, and thus the Commission cannot provide any advice in this regard.

⁽¹⁾ Ocaliva — referral, European Medicines Agency: <https://www.ema.europa.eu/en/medicines/human/referrals/ocaliva>.

⁽²⁾ Union Register of medicinal products, European Commission <https://ec.europa.eu/health/documents/community-register/html/h1139.htm>.

⁽³⁾ Article 117(3) of Directive 2001/83/EC.

⁽⁴⁾ Article 5(1) of Directive 2001/83/EC.