FDA Approves Ocaliva, New Drug for Primary Biliary Cholangitis (PBC)

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The U.S. Food and Drug Administration (FDA) has approved Intercept Pharmaceuticals' Ocaliva (obeticholic acid) to treat primary biliary cholangitis, previously known as primary biliary cirrhosis (PBC), Fierce Biotech reports. The drug is approved for use in combination with ursodeoxycholic acid (UDCA) in adults who have responded inadequately to that drug or as a monotherapy in adults who cannot tolerate UDCA.

PBC is a chronic nonviral disease that causes inflammation in and damage to the small bile ducts in the liver and ultimately can destroy them.

The drug, which will be available in about a week and which costs $69,350 per year, was approved based not on evidence that it increases survival rates or improvements in liver disease–related symptoms but on its capacity to reduce levels of the liver enzyme alkaline phosphatase (ALP).

The approval indicated that those starting on Ocaliva should begin at a 5 milligram dose and then titrate up to 10 mg in order to maximize the response to the drug while minimizing side effects.

Itching was the most common side effect associated with Ocaliva in the placebo-controlled, Phase III POISE trial of the drug. One participant (1 percent) who was treated with the titration method dropped out of the study because of itching. Other side effects included fatigue, abdominal pain and discomfort, rash, oropharyngeal pain, dizziness, constipation, arthralgia, thyroid function abnormality and eczema.

There is a possibility that the drug may eventually receive approval to treat non-alcoholic steatohepatitis (NASH).

To read the Fierce Biotech article, click here.

To read a press release about the accelerated approval, click here.