Intercept Seeks Approval of Ocaliva for NASH

The application is based on study results showing that up to 23% of treated patients saw an improvement in liver fibrosis.

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Intercept Pharmaceuticals has submitted a new drug application to the Food and Drug Administration (FDA) for Ocaliva for the treatment of fibrosis related to non-alcoholic steatohepatitis (NASH), according to a recent company announcement.

Non-alcoholic fatty liver disease (NAFLD) and its more severe form, NASH, are responsible for a growing proportion of advanced liver disease. The buildup of fat in the liver triggers inflammation, which over time can lead to the development of fibrosis, cirrhosis (severe scarring) and liver cancer. With no effective approved medical therapies, management relies on lifestyle changes such as weight loss and exercise.

Ocaliva (obeticholic acid) is a farnesoid X receptor agonist that activates receptors that regulate glucose and lipid metabolism and inflammation. It is currently FDA-approved for primary biliary cholangitis, a disease of the bile ducts.

The request for approval is based on interim results from the Phase III REGENERATE trial, which enrolled participants with diagnosed NASH and mild to advanced fibrosis (Stage F1 to F3). They were randomly assigned to receive 10 or 25 milligrams of Ocaliva or a placebo once daily. Liver biopsies were done at the start of the study and 18 months later.

As reported at the 2019 International Liver Congress in April, this interim analysis included 931 people with Stage F2 or F3 fibrosis who received at least one dose of treatment. Nearly 60% were women, around 90% were white and about 18% were Latino; the average age was 55.

At 18 months, 23% of participants in the 25 mg Ocaliva group, 18% in the 10 mg group and 12% in the placebo group experienced fibrosis improvement without worsening of NASH. The differences between both Ocaliva groups and the placebo group were statistically significant, meaning they were probably not driven by chance. However, the NASH resolution endpoint was not achieved significantly more often in the Ocaliva groups compared with the placebo group (12%, 11% and 8%, respectively).

Treatment was described as generally safe and well tolerated. The most common side effect was itching (pruritus), reported by 51% in the Ocaliva 25 mg group, 28% in the 10 mg group and 19% in the placebo group. This was usually mild to moderate, but 9% of patients in the higher-dose Ocaliva group stopped treatment for this reason. The frequency of serious adverse events was similar across treatment groups (14%, 11% and 11%, respectively).

“There is an urgent need for effective treatment regimens for NASH, a common liver disease which can lead to cirrhosis, liver failure and need for transplant,” lead study author Zobair Younossi, MD, PhD, of Inova Fairfax Medical Campus in Falls Church, Virginia, said in a conference press release. “These first results from the REGENERATE study give us hope that a new targeted approach to NASH treatment may soon become available and potentially reverse some of the liver damage associated with this important liver disease.”

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