Intercept Resubmits New Drug Application to U.S. FDA for Obeticholic Acid in Patients with Liver Fibrosis due to NASH

December 23, 2022

NDA supported by robust NASH clinical development program, including two positive interim analyses from the Phase 3 REGENERATE study

NDA includes detailed safety analysis of 2,477 patients with nearly 1,000 patients on study drug for 4 years

MORRISTOWN, N.J., Dec. 23, 2022 (GLOBE NEWSWIRE) -- Intercept Pharmaceuticals, Inc. (Nasdaq: ICPT), a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat progressive non-viral liver diseases, today announced that it has resubmitted a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for obeticholic acid (OCA) for the treatment of patients with pre-cirrhotic liver fibrosis due to nonalcoholic steatohepatitis (NASH).

The resubmission is supported by a robust body of evidence from the OCA NASH clinical development program, including two positive interim 18-month analyses from the pivotal Phase 3 REGENERATE study in patients with pre-cirrhotic liver fibrosis due to NASH. In both REGENERATE analyses, treatment with OCA 25 mg demonstrated a statistically significant improvement in liver fibrosis by at least 1 stage without worsening of NASH—an improvement that was more pronounced in individuals with more advanced disease at baseline. Other measures of liver disease, including blood levels of liver enzymes and noninvasive measures of liver stiffness, demonstrated dose-dependent improvements after 18 and 48 months of therapy. Further, a detailed analysis of the largest safety database in NASH demonstrated a monitorable and manageable safety and tolerability profile that supports the potential chronic administration of OCA.

“We are pleased to resubmit our NDA for the treatment of fibrosis due to NASH – an achievement made possible by years of hard work and dedication from patients, physicians, study personnel and Intercept employees, and a major milestone for the NASH community,” said Jerry Durso, President and Chief Executive Officer of Intercept. “NASH is the most rapidly growing cause of liver transplantation in the U.S., and people living with fibrosis due to NASH urgently need an approved therapy. OCA has demonstrated consistent, statistically significant antifibrotic efficacy across multiple analyses, and if approved, we believe it has the potential to become a foundational therapy. We look forward to continuing our work with FDA throughout this NDA review process.”

The company anticipates that the FDA will classify this application as a Class 2 resubmission with a PDUFA target review time of 6 months.

OCA has not been approved for the treatment of NASH by any regulatory authority in any geography and is considered an investigational treatment for this indication.

About the REGENERATE Study

REGENERATE (Randomized Global Phase 3 Study to Evaluate the Impact on NASH with Fibrosis of Obeticholic Acid Treatment) is an ongoing Phase 3, randomized, double-blind, placebo-controlled, multicenter, international study assessing the safety and efficacy of obeticholic acid (OCA) on clinical outcomes in patients with liver fibrosis due to NASH. A pre-specified interim analysis was conducted in 931 subjects who had a liver biopsy at Month 18 to assess the effect of OCA on liver histology as compared to baseline biopsies. REGENERATE is fully enrolled with 2,480 randomized participants and is expected to continue while collecting data on the incidence of clinical outcomes for verification and description of clinical benefit. The end-of-study primary endpoint will compare the impact of treatment group (placebo, OCA 10 mg or OCA 25 mg daily) on all-cause mortality and liver-related clinical outcomes, as well as on long-term safety.

About Liver Fibrosis due to NASH

Nonalcoholic steatohepatitis (NASH) is a serious progressive liver disease caused by excessive fat accumulation in the liver that induces chronic inflammation, resulting in progressive fibrosis (scarring) that can lead to cirrhosis, eventual liver failure, cancer and death. Advanced fibrosis is associated with a substantially higher risk of liver-related morbidity and mortality in patients with NASH. There are currently no medications approved for the treatment of NASH.

About Intercept

Intercept is a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat progressive non-viral liver diseases, including primary biliary cholangitis (PBC), nonalcoholic steatohepatitis (NASH) and severe alcohol-associated hepatitis (sAH). For more information, please visit www.interceptpharma.com or connect with the Company on Twitter and LinkedIn.

Forward-Looking Statements

This press release contains forward-looking statements (“FLS”), including regarding the classification by the FDA of our resubmitted NDA; the timing of FDA review of our NDA; the prospects for FDA approval of our NDA; the results of our clinical studies; drug efficacy, safety, and tolerability; the commercial opportunity for our product candidate; and the prospects of our product candidate compared to potential competitors. Important factors could cause actual results to differ materially from the FLS. For example, the FDA could take longer than expected to review our NDA; our product candidate could not receive FDA approval in a timely manner or at all; the FDA could require us to provide additional information that is not timely or economical to provide; we could be unable to address to the satisfaction of the FDA the issues raised in its complete response letter of June 2020 responding to our earlier submission; there could be efficacy, safety, or tolerability concerns about our product candidate; our clinical studies could have problems; and our product candidate could have less commercial potential than anticipated or could be superseded by a competing product.

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