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Intercept Presents New Data at AASLD Examining the Effects of Ocaliva® (Obeticholic Acid) on Non-Invasive Assessments of Liver Fibrosis in Patients with PBC

Poster presentations explore changes in biochemical markers of disease progression in PBC patients with cirrhosis and renal impairment

NEW YORK, Nov. 11, 2016 (GLOBE NEWSWIRE) -- Intercept Pharmaceuticals, Inc. (Nasdaq:ICPT) (Intercept), a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat progressive non-viral liver diseases, today announced results from three new exploratory analyses of the Phase 3 POISE trial of Ocaliva® (obeticholic acid) in patients with primary biliary cholangitis (PBC). The analyses will be presented at the American Academy for the Study of Liver Diseases (AASLD) Annual Meeting (The Liver Meeting®), taking place in Boston, MA from November 11-15.

The POISE trial evaluated the safety and efficacy of once-daily treatment with Ocaliva in PBC patients with an inadequate therapeutic response to, or who are unable to tolerate, ursodeoxycholic acid (UDCA). Of 216 patients randomized to three treatment arms—placebo, Ocaliva 5 mg titrated to 10 mg or Ocaliva 10 mg—93% continued receiving UDCA.

The first POISE presentation (abstract #209) evaluated the effects of Ocaliva on non-invasive assessments of liver fibrosis using both transient elastography (Fibroscan™) and the AST to Platelet Ratio (APRI). These tests have been shown to be effective in predicting clinical outcomes in PBC, and Ocaliva-treated patients experienced improvements in both compared with those receiving placebo. In patients with transient elastography assessments at baseline and month 12 (approximately 43% of the study population), only Ocaliva-treated patients experienced a reduction in liver stiffness below 16.9 kPa, a threshold associated with the presence of cirrhosis. Mean liver stiffness reduction was observed in the 10 mg Ocaliva group compared to placebo. In patients with a baseline APRI score above 0.54 (a threshold associated with increased risk of adverse clinical outcomes in PBC patients), 35% of Ocaliva-treated patients compared to 13% of placebo-treated patients experienced an improvement to below 0.54 at the end of the 12 month double-blind phase.

"Because liver biopsies are not routinely used for staging patients with PBC, it is important that we explore non-invasive strategies to evaluate the effects of new therapies like Ocaliva on liver fibrosis," said Gideon Hirschfield, M.D., Professor and Honorary Consultant Hepatologist, Centre for Liver Research at the University of Birmingham, UK, who presented the data. "These results are very promising, and the ongoing Phase 4 COBALT trial of Ocaliva will provide us with a more definitive understanding of the drug's ability to improve non-invasive measures of liver fibrosis and reduce the risk of clinical outcomes in our patients with PBC."

The second POISE presentation (abstract #366) evaluated the efficacy and safety of Ocaliva in the subset (17%) of patients with cirrhosis who were at the greatest risk of progression to liver-related adverse outcomes or death. At month 12, more Ocaliva-treated patients with cirrhosis achieved the primary composite study endpoint compared to placebo. Ocaliva treatment improved markers of both cholestasis (alkaline phosphatase) and hepatic impairment (bilirubin) relative to placebo in patients with cirrhosis. Consistent with previous study results, pruritus (itch) was the most common adverse event associated with Ocaliva treatment. Additional side effects observed during the trial included fatigue, abdominal pain and discomfort, rash, oropharyngeal pain, dizziness, constipation, arthralgia, thyroid function abnormality, and eczema.

"This exploratory analysis suggests that high-risk PBC patients with compensated cirrhosis benefited from treatment with Ocaliva, and the results were comparable to what we observed in earlier stage non-cirrhotic patients," said John Vierling, M.D., F.A.C.P., F.A.A.S.L.D., Professor of Medicine and Surgery at Baylor College of Medicine, Past President of AASLD, and lead author of the abstract. "The bilirubin improvements in these patients are particularly meaningful because increasing bilirubin levels — even within the normal range — are one of the hallmarks of progressive disease and strongly associated with clinical outcomes."

The third POISE presentation (abstract #401) examined the effects of Ocaliva in PBC patients with mild and moderate renal impairment. In this exploratory analysis, Ocaliva demonstrated comparable efficacy regardless of renal status and enabled patients with renal impairment to achieve significant improvements in markers of cholestasis and hepatic damage. Ocaliva had no apparent effect on renal safety, with mild to moderate pruritus the most commonly occurring adverse event in all renal function groups.

About Primary Biliary Cholangitis
Primary biliary cholangitis (PBC) is a rare, autoimmune cholestatic liver disease that puts patients at risk for life-threatening complications. PBC is primarily a disease of women, afflicting approximately one in 1,000 women over the age of 40. If left untreated, survival of PBC patients is significantly worse than the general population.

About Ocaliva® (obeticholic acid)
Ocaliva (obeticholic acid) is a potent and highly selective agonist of the farnesoid X receptor (FXR), a nuclear receptor expressed in the liver and intestine. FXR is a key regulator of bile acid, inflammatory, fibrotic and metabolic pathways.

In May 2016, the U.S. Food and Drug Administration (FDA) granted accelerated approval to obeticholic acid for the treatment of PBC under the brand name Ocaliva based on a reduction in ALP. An improvement in survival or disease-related symptoms has not been established. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

In October 2016, the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) adopted a positive opinion recommending the conditional marketing authorization of Ocaliva in PBC. Based on the CHMP's positive recommendation, the final decision of the European Commission on the conditional marketing authorization of Ocaliva in PBC is expected by the end of 2016. The brand name Ocaliva has been provisionally approved by the EMA.

U.S. IMPORTANT SAFETY INFORMATION
Contraindications
Ocaliva is contraindicated in patients with complete biliary obstruction.

Warnings and Precautions
Liver-Related Adverse Reactions
In two 3-month, placebo-controlled clinical trials, a dose-response relationship was observed for the occurrence of liver-related adverse reactions including jaundice, ascites and primary biliary cholangitis flare with dosages of Ocaliva of 10 mg once daily to 50 mg once daily (up to 5-times the highest recommended dosage), as early as one month after starting treatment with Ocaliva.

In a pooled analysis of three placebo-controlled trials in patients with PBC, the exposure-adjusted incidence rates for all serious and otherwise clinically significant liver-related adverse reactions, and isolated elevations in liver biochemical tests, per 100 patient exposure years (PEY) were: 5.2 in the Ocaliva 10 mg group (highest recommended dosage), 19.8 in the Ocaliva 25 mg group (2.5 times the highest recommended dosage) and 54.5 in the Ocaliva 50 mg group (5 times the highest recommended dosage) compared to 2.4 in the placebo group.

Monitor patients during treatment with Ocaliva for elevations in liver biochemical tests and for the development of liver-related adverse reactions. Weigh the potential risks against the benefits of continuing treatment with Ocaliva in patients who have experienced clinically significant liver-related adverse reactions. The maximum recommended dosage of Ocaliva is 10 mg once daily. Adjust the dosage for patients with moderate or severe hepatic impairment.

Discontinue Ocaliva in patients who develop complete biliary obstruction.

Severe Pruritus
Severe pruritus was reported in 23% of patients in the Ocaliva 10 mg arm, 19% of patients in the Ocaliva titration arm and 7% of patients in the placebo arm in the POISE trial, a 12-month double-blind randomized controlled trial of 216 patients. Severe pruritus was defined as intense or widespread itching, interfering with activities of daily living, or causing severe sleep disturbance, or intolerable discomfort, and typically requiring medical interventions. In the subgroup of patients in the Ocaliva titration arm who increased their dosage from 5 mg once daily to 10 mg once daily after 6 months of treatment (n=33), the incidence of severe pruritus was 0% from months 0 to 6 and 15% from months 6 to 12. The median time to onset of severe pruritus was 11, 158 and 75 days for patients in the Ocaliva 10 mg, Ocaliva titration and placebo arms, respectively.

Management strategies include the addition of bile acid resins or antihistamines, Ocaliva dosage reduction and/or temporary interruption of Ocaliva dosing.

Reduction in HDL-C
Patients with PBC generally exhibit hyperlipidemia characterized by a significant elevation in total cholesterol primarily due to increased levels of high density lipoprotein-cholesterol (HDL-C). In the POISE trial, dose-dependent reductions from baseline in mean HDL-C levels were observed at 2 weeks in Ocaliva-treated patients, 20% and 9% in the 10 mg and titration arms, respectively, compared to 2% in the placebo arm. At month 12, the reduction from baseline in mean HDL-C level was 19% in the Ocaliva 10 mg arm, 12% in the Ocaliva titration arm and 2% in the placebo arm. Nine patients in the Ocaliva 10

mg arm and six patients in the Ocaliva titration arm, versus three patients in the placebo arm, had reductions in HDL-C to less than 40 mg/dL.

Monitor patients for changes in serum lipid levels during treatment. For patients who do not respond to Ocaliva after one year at the highest recommended dosage that can be tolerated (maximum of 10 mg once daily), and who experience a reduction in HDL-C, weigh the potential risks against the benefits of continuing treatment.

Adverse Reactions
The most common adverse reactions from subjects taking Ocaliva (≥5%) were pruritus, fatigue, abdominal pain and discomfort, rash, oropharyngeal pain, dizziness, constipation, arthralgia, thyroid function abnormality and eczema.

Drug Interaction
Bile Acid Binding Resins
Bile acid binding resins such as cholestyramine, colestipol or colesevelam absorb and reduce bile acid absorption and may reduce the absorption, systemic exposure and efficacy of Ocaliva. If taking bile acid binding resins, take Ocaliva at least 4 hours before or 4 hours after (or at as great an interval as possible) taking a bile acid binding resin.

Please see **Full Prescribing Information** for Ocaliva (obeticholic acid) 5 mg and 10 mg tablets.

To report SUSPECTED ADVERSE REACTIONS, contact Intercept Pharmaceuticals, Inc. at 1-844-782-ICPT or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

About the POISE Trial
The POISE trial studied the safety and efficacy of once-daily treatment with Ocaliva in PBC patients with an inadequate therapeutic response to, or who are unable to tolerate, UDCA, the current standard of care. Of 216 patients randomized to three treatment arms—placebo, Ocaliva 5 mg titrated to 10 mg or Ocaliva 10 mg—93% continued receiving UDCA. The Ocaliva 5-10 mg titration group received Ocaliva 5 mg for six months, after which dosing was increased to 10 mg based on tolerability and biochemical response. The trial's primary endpoint was a reduction in ALP to below a threshold of 1.67 times the upper limit of normal, with a minimum of 15% reduction in ALP level from baseline, and a normal bilirubin level after 12 months of therapy.

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), primary sclerosing cholangitis (PSC) and biliary atresia. Founded in 2002 in New York, Intercept now has operations in the United States, Europe and Canada. For more information about Intercept, please visit www.interceptpharma.com.

Safe Harbor Statements
This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including, but not limited to, statements regarding the clinical relevance and utility of ALP and the surrogate endpoint used in the Phase 3 POISE trial to predict clinical outcomes, the acceptance of Ocaliva® (obeticholic acid) as a treatment for PBC by healthcare providers, patients and payors, the potential approval of OCA in PBC by the European Commission and other regulatory bodies and the timelines related thereto, the availability of OCA for the treatment of PBC in Europe and other jurisdictions outside the United States and timelines related thereto, the anticipated prevalence of and other epidemiological estimates and market data related to PBC, the continued development of OCA and Intercept's other product candidates, and our strategic directives under the caption "About Intercept." These "forward-looking statements" are based on management's current expectations of future events and are subject to a number of important risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to: Intercept's ability to successfully commercialize Ocaliva in PBC, and Intercept's ability to maintain its regulatory approval of Ocaliva in the United States for Ocaliva in PBC; the initiation, cost, timing, progress and results of Intercept's development activities, preclinical studies and clinical trials, including Intercept's development program in NASH; the timing of and Intercept's ability to obtain and maintain regulatory approval of OCA in PBC in countries outside the United States and in indications other than PBC and any other product candidates it may develop such as INT-767; conditions that may be imposed by regulatory authorities on Intercept's marketing approvals for its product candidates such as the need for clinical outcomes data (and not just results based on achievement of a surrogate endpoint), and any related restrictions, limitations, and/or warnings in the label of any approved product candidates; Intercept's plans to research, develop and commercialize its product candidates; Intercept's ability to obtain and maintain intellectual property protection for its product candidates; Intercept's ability to successfully commercialize OCA in indications other than PBC and its other product candidates; the size and growth of the markets for Intercept's product candidates and its ability to serve those markets; the rate and degree of market acceptance of any of Intercept's products, which may be affected by the reimbursement that it may receive for its products from payors; the success of competing drugs that are or become available; the election by Intercept's collaborators to pursue research, development and commercialization activities; Intercept's ability to attract collaborators with development, regulatory and

commercialization expertise; regulatory developments in the United States and other countries; the performance of third-party suppliers and manufacturers; Intercept's need for and ability to obtain additional financing; Intercept's estimates regarding expenses, future revenues and capital requirements and the accuracy thereof; Intercept's use of cash, short-term investments and the proceeds from the offering; Intercept's ability to attract and retain key scientific or management personnel; and other factors discussed under the heading "Risk Factors" contained in our annual report on Form 10-K for the year ended December 31, 2015 filed on February 29, 2016 as well as any updates to these risk factors filed from time to time in our other filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and Intercept undertakes no duty to update this information unless required by law.

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