October 14, 2016

Intercept Pharmaceuticals Receives Positive CHMP Opinion for Ocaliva® (Obeticholic Acid) for the Treatment of Primary Biliary Cholangitis in the European Union

NEW YORK, Oct. 14, 2016 (GLOBE NEWSWIRE) -- Intercept Pharmaceuticals, Inc. (Nasdaq:ICPT) (Intercept), a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat non-viral, progressive liver diseases, today announced that the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion recommending marketing authorization of the Company's Marketing Authorization Application (MAA) for obeticholic acid (OCA), an FXR agonist, for the treatment of primary biliary cholangitis (PBC) conditional to the company providing further data post-approval to confirm benefit.

Ursodeoxycholic acid (UDCA) is currently the only approved medication for the treatment of PBC in Europe and is the standard of care for all PBC patients. However, a substantial percentage of patients treated with UDCA continue to experience persistent elevations above the upper limit of normal in the serum marker alkaline phosphatase (ALP), which has been shown to correspond with increased risk of liver failure, need for liver transplant and death. Patients with PBC also face a risk of experiencing adverse outcomes when bilirubin levels are elevated. Total bilirubin levels, even within the normal range, have been shown to predict clinical outcomes in PBC.

"Although it is a rare disease, PBC remains one of the most common indications for liver transplant among women in Europe," said David Jones, M.D., Ph.D., Professor of Liver Immunology at Newcastle University and Consultant Hepatologist at Newcastle upon Tyne Hospitals Trust, which hosts one of Europe's leading clinical services in the disease. "There is substantial unmet need in this disease and a real urgency around the need for new therapies to help the many PBC patients who are either intolerant of the single existing approved therapy ursodeoxycholic acid or don't respond to it sufficiently to protect their livers and prevent the development of cirrhosis and the need for transplant."

The MAA submission included data from more than 1,500 subjects exposed to at least a single dose of OCA. The positive opinion of the CHMP was based on efficacy and safety data derived from three randomized double-blind, placebo-controlled clinical trials in patients with PBC evaluating the effect of OCA on ALP and bilirubin. The MAA submission was also supported by two clinical databases that include more than 10,000 patients from the Global PBC Study Group and UK-PBC Group, both independently confirming that achieving lower ALP and/or bilirubin levels is significantly correlated with increased transplant-free survival.

The CHMP opinion will form the basis for a European Commission (EC) decision as to whether to formally grant the conditional marketing authorization for OCA with unified labelling in the 28 countries that are Member States of the European Union, as well as European Economic Area members Iceland, Liechtenstein and Norway. As the conditions for approval, Intercept is required to provide post-approval updates on safety and efficacy analyses for OCA from the ongoing COBALT outcomes trial and a short-term trial in patients with hepatic impairment.

"We owe a tremendous debt to the many patients and physicians whose participation in the research program for OCA led to this positive outcome," said Lisa Bright, Intercept's President, International. "In addition to playing a critical role in the development of OCA, the PBC community in Europe has been the driving force in establishing the two major patient databases that have been so central to recent advances in our understanding of the disease. There is a palpable sense of excitement about the growth of PBC awareness in Europe, and the CHMP's positive opinion on OCA brings us one step closer to introducing the first new therapy for PBC in approximately two decades."

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 Obeticholic Acid (OCA)
Obeticholic acid is an 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.

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. 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

<u>Bile Acid Binding Resins</u>
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.

About Intercept

Intercept is a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat non-viral, progressive 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; 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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