May 27, 2016

FDA Grants Accelerated Approval to Ocaliva™ (Obeticholic Acid) for the Treatment of Patients with PBC

First new medicine for PBC in nearly 20 years

Investor conference call Tuesday, May 31 at 8:30 a.m. ET

NEW YORK, May 27, 2016 (GLOBE NEWSWIRE) -- Intercept Pharmaceuticals, Inc. (Nasdaq:ICPT), a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat non-viral, progressive liver diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted accelerated approval to Ocaliva (obeticholic acid) for the treatment of primary biliary cholangitis, previously known as primary biliary cirrhosis (PBC), in combination with ursodeoxycholic acid(UDCA) in adults with an inadequate response to UDCA or as monotherapy in adults unable to tolerate UDCA. Ocaliva is an agonist of the farnesoid X receptor (FXR), a nuclear receptor expressed in the liver and intestine and a key regulator of bile acid, inflammatory, fibrotic and metabolic pathways.

"Intercept was founded on the belief that targeting FXR would benefit patients with liver diseases for which there are limited or no treatment options, and Ocaliva's approval marks the culmination of more than a decade of work," said Mark Pruzanski, M.D., Chief Executive Officer and President of Intercept. "We are very pleased that the FDA has approved Ocaliva for PBC and would like to thank all the patients and investigators around the world who participated in our clinical trials to make this possible."

This indication is approved under accelerated approval based on a reduction in alkaline phosphatase (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.

"Ocaliva fills an important unmet need for the many patients with PBC who have an inadequate response to or are intolerant of UDCA, which until now has been the only approved treatment," 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 and Past President of the American Association for the Study of Liver Diseases (AASLD). "Ocaliva has demonstrated a clinically meaningful improvement in lowering ALP, a liver enzyme and biomarker that is used to track disease progression in patients with PBC. Importantly, Ocaliva maintained durable ALP reductions, which is critical for treatment of a chronic disease like PBC."

In Intercept's Phase 3 POISE trial, Ocaliva administration in combination with UDCA (or as monotherapy in UDCA-intolerant patients) met the primary composite endpoint in 46% of patients in the titration group, as compared to 10% of those receiving placebo added to UDCA ($p<0.0001$). Pruritus (itching), a common symptom of PBC that is unrelated to disease stage or outcomes, was the most common side effect observed in Ocaliva-treated patients. However, pruritus associated with Ocaliva treatment was generally less in patients who were on the dose titration regimen (5 mg once-daily increasing to 10 mg once-daily); one patient (1%) in the titration group discontinued from the study due to pruritus. Additional side effects observed during the trial included fatigue, abdominal pain and discomfort, rash, oropharyngeal pain, dizziness, constipation, arthralgia, thyroid function abnormality and eczema.

"PBC affects people in the prime of their lives and, for some, the potential need for a liver transplant is a constant concern during these important years," said Linie Moore, a PBC patient and President of the PBCers Organization, the leading PBC patient support group in the U.S. "After nearly two decades with only one approved treatment, we are thrilled to welcome this important new medicine for people living with PBC."

Ocaliva is expected to be available to PBC patients in the U.S. within 7-10 days and will be distributed through a specialty pharmacy network. Intercept is dedicated to helping ensure that people with PBC can access Ocaliva and has launched Interconnect™, a comprehensive and personalized patient support services program. Through Interconnect, dedicated Care Coordinators will guide patients through disease education, treatment support and, for eligible patients, financial assistance options, which may include reimbursement support, co-pay assistance or access to Ocaliva at no cost. For more information about Interconnect Support Services and U.S. Distribution, call 1-844-622-4278 or visit www.Interconnectsupport.com.

Investor Conference Call Information
Intercept will host an investor conference call on Tuesday, May 31 at 8:30 a.m. ET to discuss the accelerated approval of Ocaliva. The live event will be available on the investor page of the Intercept website at http://ir.interceptpharma.com or by calling (855) 232-3919 (toll-free domestic) or (315) 625-6894 (international) five minutes prior to the start time (no passcode required). A replay of the call will be available on the Intercept website approximately two hours after the completion of the call and will be archived for two weeks.

About Primary Biliary Cholangitis, Formerly Known as Primary Biliary Cirrhosis
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 the Phase 3 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 POISE data showed that Ocaliva, at both a 10 mg dose and a 5 mg dose titrated to 10 mg, met the trial's primary endpoint of achieving a reduction in serum 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. Pruritus was the most frequently reported adverse event associated with Ocaliva treatment. In a group of patients who initiated Ocaliva at a 5 mg once-daily dose and titrated up to 10 mg once daily, only one patient (1%) discontinued from the study due to pruritus as compared to seven patients (10%) in the 10 mg dose group and after 12 months of treatment, efficacy was essentially equivalent to those patients who started the study at the 10 mg dose. Based on these results, a 5 mg to 10 mg titration regimen is recommended for Ocaliva dosing in PBC. Decreases in HDL-C were observed during treatment.

About Ocaliva™ (obeticholic acid)
Ocaliva is indicated in the United States for the treatment of primary biliary cholangitis (PBC) in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA, or as monotherapy in adults unable to tolerate UDCA.

The indication is approved under accelerated approval based on a reduction in alkaline phosphatase (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.

A marketing authorization application for Ocaliva for the treatment of PBC was accepted by the European Medicines Authority (EMA) in June 2015 and is currently under review. The brand name Ocaliva has been provisionally approved by the EMA.

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.

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. The FDA has granted obeticholic acid breakthrough therapy designation for the treatment of NASH with liver fibrosis. Intercept owns worldwide rights to obeticholic acid outside of Japan, China and Korea, where it has out-licensed the product candidate to Sumitomo Dainippon Pharma. Intercept's pipeline of product candidates includes other novel bile acid analogs such as INT-767, which is in clinical development. 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 as a treatment for PBC by healthcare providers, patients and payors, 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: the initiation, cost, timing, progress and results of our development activities, preclinical studies and clinical trials; the timing of and our ability to obtain and maintain regulatory approval of OCA, INT-767 and any other product candidates we may develop, particularly the possibility that regulatory authorities may require clinical outcomes data (and not just results based on achievement of a surrogate endpoint) as a condition to any marketing approval for Ocaliva for PBC in countries outside the United States and OCA for indications beyond PBC, and any related restrictions, limitations, and/or warnings in the label of any approved product candidates; our plans to research, develop and commercialize our product candidates; our ability to obtain and maintain intellectual property protection for its product candidates; our ability to successfully commercialize our product candidates; the size and growth of the markets for our product candidates and our ability to serve those markets; the rate and degree of market acceptance of any future products, which may be affected by the reimbursement that our products receive from payors; the success of competing drugs that are or become available; regulatory developments in the United States and other countries; the performance of third-party suppliers and manufacturers; our collaborators' election to pursue research, development and commercialization activities; our ability to attract collaborators with development, regulatory and commercialization expertise; our need for and ability to obtain additional financing; our estimates regarding expenses, future revenues and capital requirements and the accuracy thereof; our use of cash and short term investments; our ability to 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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