December 14, 2016

European Commission Grants Intercept's Ocaliva® (obeticholic acid) Marketing Authorization for the Treatment of Primary Biliary Cholangitis

NEW YORK, Dec. 14, 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 that the European Commission has granted conditional approval for Ocaliva (obeticholic acid) 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. Ocaliva is a potent and selective agonist of the farnesoid X receptor (FXR), which is expressed at high levels in the liver and intestine and thought to be a key regulator of bile acid, inflammatory, fibrotic and metabolic pathways.

"The approval of Ocaliva in Europe provides a new therapeutic option for a substantial group of PBC patients who are not achieving treatment goals with UDCA alone or who cannot tolerate UDCA," said Frederik Nevens, M.D., Ph.D., University Hospitals Leuven & KU Leuven, Belgium, and the lead investigator of the Phase 3 POISE clinical study. "Despite the availability of UDCA, many patients have remained at significant risk of adverse outcomes with no alternative treatment option available. Ocaliva can now help fill an important unmet need for these patients."

"We are delighted to be introducing the first new therapeutic option for PBC in nearly 20 years in Europe where this disease is a major reason for liver failure and a leading cause of liver transplant in women," said Lisa Bright, Intercept's President, International. "Following approval in the U.S. earlier this year, Ocaliva's marketing authorization in Europe represents another big step in Intercept's mission to provide patients with worldwide access to our innovative therapy. This great achievement will motivate us further to continue developing solutions that improve the lives of people with progressive non-viral liver diseases."

The marketing authorization allows Intercept to market Ocaliva in 28 countries that are member states of the European Union, as well as 3 additional European Economic Area member states. As conditions of the approval, Intercept is required to provide post-approval updates on safety and efficacy analyses for Ocaliva from the ongoing Phase 4 COBALT outcomes study and a short-term study in patients with hepatic impairment.

"As a community, our priority is to advocate for changes which ensure that people diagnosed with PBC have the best possible prognosis," said Tatjana Reic, President of the European Liver Patients Association (ELPA). "With this in mind, we are excited about this advance for patients with an inadequate response or intolerability to the current available treatment. Such patients will soon have access to a new treatment option to manage their PBC."

The marketing authorization was based on efficacy and safety data derived from three randomized double-blind, placebo-controlled clinical trials evaluating the effect of Ocaliva on alkaline phosphatase (ALP) and bilirubin in patients with PBC. It 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.

In the Phase 3 POISE study, nearly half of patients (46%) in the titration group treated with Ocaliva in combination with UDCA achieved the primary endpoint compared to 10% in the control group (placebo added to UDCA) ($p<0.0001$). Additionally, 77% of patients taking Ocaliva in combination with UDCA achieved a reduction of more than 15% in ALP at 12 months, compared to 29% taking UDCA alone.

The most commonly reported adverse reactions were pruritus (63%) and fatigue (22%). Adverse reactions leading to discontinuation were 1% in the Ocaliva titration arm and 11% in the Ocaliva 10 mg arm. The most common adverse reaction leading to discontinuation was pruritus. The majority of pruritus occurred within the first month of treatment and tended to resolve over time with continued dosing.

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 December 2016, Ocaliva received conditional marketing authorization in Europe for the treatment of PBC in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA or as monotherapy in adults unable to tolerate UDCA, conditional to the company providing further data post-approval to confirm benefit. In May 2016, the U.S. Food and Drug Administration granted accelerated approval to Ocaliva for the treatment of PBC. For full prescribing information in the U.S., visit Ocaliva.com.

EU IMPORTANT SAFETY INFORMATION
Contraindications
Hypersensitivity to the active substance or to any of the excipients and complete biliary obstruction.

Warnings and Precautions
Elevations in alanine amino transferase (ALT) and aspartate aminotransferase (AST) have been observed in patients taking obeticholic acid. Clinical signs and symptoms of hepatic decompensation have also been observed. These events have occurred as early as within the first month of treatment. Liver-related adverse events have primarily been observed at doses higher than the maximum recommended dose of 10 mg once daily. Patients should be monitored during treatment with Ocaliva for elevations in liver biochemical tests and for the development of liver-related adverse events. Dosage adjustments are needed for patients with moderate (Child-Pugh Class B) or severe (Child-Pugh Class C) hepatic impairment.

Severe pruritus was reported in 23% of patients treated with Ocaliva 10 mg arm, 19% of patients in the Ocaliva titration arm and 7% of patients in the placebo arms. 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 binding resins or antihistamines, dose reduction, reduced dosing frequency and/or temporary dose interruption.

Adverse Reactions
The most commonly reported adverse reactions were pruritus (63%) and fatigue (22%). Other common adverse reactions observed in clinical trials (> 5%) were abdominal pain and discomfort, rash, oropharyngeal pain, dizziness, constipation, arthralgia, thyroid function abnormality and eczema.

Drug Interaction
Bile acid binding resins such as cholestyramine, colestipol or colesevelam adsorb and reduce bile acid absorption and may reduce efficacy of obeticholic acid. When concomitant bile acid binding resins are administered, obeticholic acid should be taken at least 4-6 hours before or 4-6 hours after taking a bile acid binding resin, or at as great an interval as possible.

For detailed safety information for Ocaliva (obeticholic acid) 5 mg and 10 mg tablets including posology and method of administration, special warnings, drug interactions and adverse drug reactions, please see the European Summary of Product Characteristics that can be found on www.ema.europa.eu once posted.

About the POISE Study
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 study'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 regulatory bodies outside the United States and Europe and the timelines related thereto, the availability of OCA for the treatment of PBC other jurisdictions outside the United States and Europe 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.

Contact
For more information about Intercept Pharmaceuticals, please contact:

Mark Vignola
+1-646-747-1000
investors@interceptpharma.com

Christopher Frates
+1-646-757-2371
media@interceptpharma.com