November 1, 2016

Intercept Announces Ocaliva® (Obeticholic Acid) Data in PBC to be Presented at the 2016 AASLD Annual Meeting

Intercept will collaborate with academic leaders on a new NASH patient registry

NEW YORK, Nov. 01, 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 multiple Ocaliva® (obeticholic acid) for PBC and INT-767 data presentations at the upcoming American Academy for the Study of Liver Diseases (AASLD) Annual Meeting (The Liver Meeting®), taking place November 11 — 15 in Boston, MA.

"We have numerous presentations at this year's Liver Meeting, among them an oral presentation examining Ocaliva's effects on non-invasive fibrosis measurements in patients with PBC," said David Shapiro, M.D., Intercept's Chief Medical Officer & Executive Vice President, Development. "Other PBC presentations include an evaluation of Ocaliva data using a long-term prognostic model developed by the UK-PBC Study Group and analyses of Ocaliva's safety and efficacy in PBC patient populations with end-stage liver disease and renal disease. In addition, we look forward to sharing an analysis of fibrosis data from the FLINT trial in NASH and new preclinical research examining INT-767 — our FXR/TGR5 dual agonist — in animal models of NASH and metabolic disease."

Intercept also announced its sponsorship of the new TARGET-NASH patient registry, which will advance the understanding of NASH diagnosis and management across multiple populations in a real world setting. As the NASH treatment landscape evolves, the registry will evaluate the safety and effectiveness of new agents across populations not included or underrepresented in Phase 3 clinical trials. TARGET-NASH is led by an academic steering committee chaired by Drs. Arun Sanyal of Virginia Commonwealth University, Ken Cusi of the University of Florida and Brent Tetri of St. Louis University. The registry is currently enrolling and additional details about TARGET-NASH are available at ClinicalTrials.gov.

In the United States, Ocaliva was recently approved by the FDA for the treatment 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. Obeticholic acid is also being investigated for patients with NASH and liver fibrosis, as well as primary sclerosing cholangitis and biliary atresia.

Intercept will be exhibiting at booth 524 throughout the Liver Meeting. Select presentations include:

Oral Presentation:

Monday November 14, 3:00 pm - 4:30 pm ET
"Long-Term Effect of Obeticholic Acid on Transient Elastography and AST to Platelet Ratio Index in Patients with PBC" (Abstract #209)
Gideon M. Hirschfield, Annarosa Floreani, Palak J. Trivedi, Richard Pencek, Alexander Liberman, Tonya Marmon, Leigh MacConell

Posters Presentations in PBC and NASH:

"Efficacy of Obeticholic Acid Treatment in Patients with Primary Biliary Cholangitis with Cirrhosis" (Abstract #366)
John M. Vierling, Gideon M. Hirschfield, David Jones, Roberto J. Groszmann, Kris V. Kowdley, Richard Pencek, Tonya Marmon, Leigh MacConell

"Predicted Risk of End Stage Liver Disease with Continued Standard of Care and Subsequent Addition of Obeticholic Acid in Patients with PBC" (Abstract #361)
Kris V. Kowdley, Hemant Shah, Andrew Mason, Velimir A. Luketic, Richard Pencek, Tonya Marmon, David Shapiro, Roya Hooshmand-Rad

"Efficacy of Obeticholic Acid in Patients with Primary Biliary Cholangitis and Renal Impairment" (Abstract #401)

Paul J. Pockros, K. Gautham Reddy, Janet Owens-Grillo, Tonya Marmon, Leigh MacConell

"Subgroup Analysis Comparing Obeticholic Acid versus Placebo for Fibrosis Improvement: a Post-hoc Analysis of the FLINT Trial" (Abstract #1074)
Bilal Hameed, Norah Terrault, Rohit Loomba, Arthur J. McCullough, Manal F. Abdelmalek, Kris V. Kowdley, Brent A. Tetri, Arun J. Sanyal, Lois Lee, Beatrice Ferguson, Reshma Shringarpure, David Shapiro, Naga P. Chalasani

INT-767 Preclinical Poster Presentations:

"The FXR/TGR5 Dual Agonist INT-767 Reduces NAFLD Activity Score and Fibrosis Stage and Improves Plasma and Hepatic Lipid Profiles in the GUBRA-AMLN Mouse Model of Diet-induced and Biopsy-confirmed Nonalcoholic Steatohepatitis" (Abstract #1508)
Jonathan Roth, Michael Feigh, Sanne Skovgård Veidal, Kristoffer Rigbolt, Jacob Jelsing, Niels Vrang, Mark Young

"The dual FXR/TGR5 agonist INT-767 inhibits nonalcoholic steatohepatitis development in a rabbit model of metabolic syndrome" (Abstract #1521)
Paolo Comeglio, Sandra Filippi, Ilaria Cellai, Elena Maneschi, Francesca Corcetto, Chiara Corno, Annamaria Morelli, Luciano Adorini, Mario Maggi, Linda Vignozzi

"The FXR/TGR5 dual agonist INT-767 prevents and reverses Western diet-induced NASH and modulates major lipid metabolic pathways in mice" (Abstract #1528)
Xiaoxin Wang, Andrew Libby, Suman Ranjit, Dong Wang, Yuhuan Luo, David J. Orlicky, James McManaman, Evgenia Dobrinskikh, Enrico Gratton, Mark Young, Luciano Adorini, Moshe Levi

Obeticholic Acid Preclinical Poster Presentations:

"Obeticholic Acid Does Not Affect the Hepatic Metabolism of Hormonal Birth Control in Human Sandwich Cultured Hepatocytes" (Abstract #394)
Jeffrey Edwards, Yuanyuan Zhang, Jonathan Jackson, Kenneth Brouwer

"Obeticholic acid reduces plasma HDL-cholesterol levels and promotes transhepatic cholesterol efflux in hyperlipidemic hamsters via a mechanism involving upregulation of hepatic SR-BI" (Abstract #1533)
Bin Dong, Mark Young, Xueqing Liu, Amar Singh, Jingwen Liu

"Obeticholic acid, a synthetic FXR agonist, prevents hepatic inflammation and fibrosis in a novel mouse model of non-alcoholic steatohepatitis" (Abstract #1609)
Toshihiro Goto, Michiko Itoh, Sayaka Kanai, Takayoshi Suganami,Yoshihiro Ogawa

A full list of sessions at AASLD, including symposia, relating to obeticholic acid is available on the AASLD website.

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®
Ocaliva (obeticholic acid) for the treatment of PBC 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.

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, 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