October 11, 2017

Intercept Announces Data to be Presented at The Liver Meeting® 2017

Results from the Phase 2 AESOP Trial in Primary Sclerosing Cholangitis to be Presented in Late Breaking Oral Session

NEW YORK, Oct. 11, 2017 (GLOBE NEWSWIRE) -- Intercept Pharmaceuticals, Inc. (Nasdaq:ICPT), a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat progressive non-viral liver diseases, today announced that multiple obeticholic acid (OCA) abstracts will be presented at The Liver Meeting® 2017, the Annual Meeting of the American Association for the Study of Liver Diseases (AASLD), taking place October 20-24 in Washington, D.C. Among the highlights is a late breaking oral presentation of results from the Phase 2 AESOP trial evaluating OCA for the treatment of patients with primary sclerosing cholangitis (PSC).

"Our data at this year's Liver Meeting add to the large body of evidence supporting the use of Ocaliva to treat PBC, and underscore Intercept's ongoing commitment to advancing the clinical development of OCA in other progressive, non-viral liver diseases with high unmet need," said David Shapiro, M.D., Chief Medical Officer of Intercept. "In addition to new research in PBC and NASH, we are looking forward to the first presentation of results from our Phase 2 trial of OCA in PSC, a devastating cholestatic liver disease for which there are currently no approved treatment options."

Intercept will be exhibiting at booths 533 and 242 throughout the meeting. Select presentations at The Liver Meeting include:

Late-Breaking Oral Presentation:

Monday, October 23, 2017 — 2:30-4:30 p.m. ET

"The AESOP Trial: A Randomized, Double-Blind, Placebo-Controlled, Phase 2 Study of Obeticholic Acid in Patients with Primary Sclerosing Cholangitis"
Kris V. Kowdley, Christopher L. Bowlus, Cynthia Levy, Raj Vuppalanchi, Annarosa Floreani, Pietro Andreone, Nicholas F. LaRusso, Roshan Shrestha, James Trotter, David S. Goldberg, Simon Rushbrook, Gideon M. Hirschfield, Courtney Van Biene, Richard Pencek, Leigh MacConell, David Shapiro

Poster Presentations:

Friday, October 20, 2017 — 8 a.m. — 5:30 p.m. ET

"Unmet Need of Patients with Primary Biliary Cholangitis (PBC) Based on Alkaline Phosphatase (ALP) Threshold Using Large Database with Electronic Medical Records (EMRs) and Claims Data: A Cross-sectional Analysis" (Abstract #277)
Zobair M. Younossi, Robert S. Epstein, Marcie Strauss, Shailja Dixit

"Development of a Dose Regiment for Obeticholic Acid in Patients with Primary Biliary Cholangitis and Hepatic Impairment" (Abstract #296)
Jeffrey Edwards, Carl LaCerte, Thomas Peyret, Nathalie H. Gosselin, J.F. Marier

"Efficacy of Obeticholic Acid Treatment Through 24 Months of Open-Label Extension in Patients with Primary Biliary Cholangitis and Cirrhosis: Data From POISE" (Abstract #306)
John M. Vierling, Gideon M. Hirschfield, David Jones, Roberto J. Groszmann, Kris V. Kowdley, Richard Pencek, Elizabeth Smoot Malecha, Leigh MacConell

"Reductions in Abnormal Direct Bilirubin with Obeticholic Acid in Patients with Primary Biliary Cholangitis" (Abstract #307)
Albert Pares, Victor Vargas, Mitchell L. Shiffman, Gideon M. Hirschfield, Pietro Invernizzi, Alexander Liberman, Elizabeth Smoot Malecha, Janet Owens-Grillo, Juan C. Lopez-Talavera

A full list of sessions at The Liver Meeting, including symposia, 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 Nonalcoholic Steatohepatitis

Nonalcoholic steatohepatitis (NASH) is a serious progressive liver disease caused by excessive fat accumulation in the liver that induces chronic inflammation, resulting in progressive fibrosis (scarring) that can lead to cirrhosis, eventual liver failure, cancer and death. There are currently no medications approved for the treatment of NASH. The proportion of liver transplants attributable to NASH has increased rapidly in past years and by 2020 the disease is projected to become the leading indication for liver transplant.

About Primary Sclerosing Cholangitis

Primary sclerosing cholangitis (PSC) is a rare, life-threatening, chronic cholestatic liver disease characterized by progressive destruction of bile ducts that leads to the development of cirrhosis and end-stage liver disease or cancer in a majority of patients. There are no approved therapies for PSC, and estimated survival time from PSC diagnosis to death or liver transplant is 14.5 years. Approximately 65% of PSC patients are male, and 60%-80% of patients have concomitant inflammatory bowel disease (IBD), most often ulcerative colitis. Although it is a rare disease, PSC is the seventh leading indication for liver transplant in adults in the United States.

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.

This indication is approved under accelerated approval based on a reduction in alkaline phosphatase (ALP), as a surrogate endpoint which is reasonably likely to predict clinical benefit, including an improvement in liver transplant free-survival. 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. Intercept is currently enrolling COBALT, a Phase 4 clinical outcomes trial of Ocaliva in patients with PBC with the goal of confirming clinical benefit on a post-marketing basis.

In December 2016, Ocaliva received conditional marketing authorization in Europe for the treatment of PBC in combination with 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. Ocaliva received conditional approval from Health Canada in May 2017.

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 (HDLC). 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 the U.S. 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 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, please visit www.interceptpharma.com or connect with the company on Twitter and LinkedIn.

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, regarding the potential of OCA to treat patients with PSC 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 potential benefit and commercial potential of Ocaliva in PBC, and Intercept's ability to maintain its regulatory approval in jurisdictions in which Ocaliva is approved for use 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 ones in which it is approved 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 products and 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 products and 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 products and product candidates; Intercept's ability to successfully commercialize its products and product candidates; the size and growth of the markets for Intercept's products and 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 received 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; 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; Intercept's need for and ability to obtain additional financing; Intercept's estimates regarding expenses, revenues and capital requirements and the accuracy thereof; Intercept's use of cash and short-term investments; 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, 2016 filed on March 1, 2017 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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