Intercept to Present New NASH and PBC Data at the Digital International Liver Congress™ 2020

August 21, 2020

New analyses of the interim analysis data from the Phase 3 REGENERATE study describe the benefit of obeticholic acid (OCA) on noninvasive measures of liver fibrosis in NASH patients on treatment for at least 24 months

Additional long-term data in PBC highlight the safety and durable efficacy of OCA through six years of open label treatment

NEW YORK, Aug. 21, 2020 (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 abstracts regarding the treatment of primary biliary cholangitis (PBC) and nonalcoholic steatohepatitis (NASH) with OCA will be presented at the Digital International Liver Congress™ 2020, the 55th Annual Meeting of the European Association for the Study of the Liver (EASL), to be held virtually from August 27, 2020 to August 29, 2020.

"The new data to be presented at this year's International Liver Congress add to the already substantial body of evidence supporting the anti-fibrotic efficacy of OCA in patients with advanced fibrosis due to NASH," said Mark Pruzanski, M.D., President and CEO of Intercept. "We are also excited to see that several Ocaliva® PBC abstracts that deepen the understanding of our drug's long-term safety and efficacy profile will be presented, including one presenting the six-year data from the open label extension of our Phase 3 POISE trial. On behalf of all my colleagues at Intercept, I'd like to thank EASL for providing this virtual forum for important new hepatology research and scientific exchange during the COVID-19 pandemic."

Presentations at the Digital International Liver Congress include:

Late-Breaker Poster Presentation

"Obeticholic acid demonstrates sustained improvements at month 24 in transaminases and non-invasive markers of fibrosis: results of a post hoc analysis from the interim analysis of the REGENERATE study" (LBP19)
Rohit Loomba, Vlad Ratziu, Quentin M. Anstee, Stephen Harrison, Arun Sanyal, Mary Rinella, Zobair Younossi, Zachary Goodman, Pierre Bedossa, Reshma Shringarpure, Huafeng Zhou, Aditya Venugopal, Mazen Noureddin

Oral Presentation

"Obeticholic acid (OCA) improves experimental non-invasive markers of NASH and advanced fibrosis: results of a secondary analysis from the month-18 interim analysis of the REGENERATE study" (AS075)
Jerome Boursier, Rohit Loomba, Quentin M. Anstee, Stephen Harrison, Arun Sanyal, Mary Rinella, Zobair Younossi, Zachary Goodman, Pierre Bedossa, Céline Fournier, Michael Stenkilsson, Reshma Shringarpure, Luna Zaru, Aditya Venugopal, Leigh MacConell, Vlad Ratziu

General Poster Presentations

"The burden of disease associated with non-alcoholic steatohepatitis patients under standard of care" (THU048)
Raluca Pais, William Green, Stuart Mealing, Aldo Trylesinski, Sandrine Cure, Heather Davies

"Predicted risk of end stage liver disease utilizing the UK-PBC risk score with continued standard of care and subsequent addition of obeticholic acid for 60 Months in patients with primary biliary cholangitis" (THU114)
David E. Jones, Marco Carbone, George Mells, Alexander Liberman, Elizabeth Smoot Malecha, Leigh MacConell

"Noninvasive tests for assessing fibrosis in patients with non-alcoholic fatty liver disease: an evaluation of combining test results" (FRI009)
Catherine Vick, Andrew Joyce, Amy Law, Molly Sherwood, Essy Mozaffari, Bruce Wong

"Obeticholic acid improves hepatic fibroinflammation as assessed by multiparametric magnetic resonance imaging: interim results of the REGENERATE trial" (FRI066)
Rohit Loomba, Quentin M. Anstee, Stephen Harrison, Arun Sanyal, Vlad Ratziu, Zobair Younossi, Zachary Goodman, Pierre Bedossa, Rajarshi Banerjee, Michael Stenkilsson, Reshma Shringarpure, Luna Zaru, Aditya Venugopal, Leigh MacConell, Mary Rinella

"Obesity-specific health-related quality of life in patients with non-alcoholic steatohepatitis: results from the REGENERATE study" (FRI080)
Zobair Younossi, Maria Stepanova, Fatema Nader, Rohit Loomba, Quentin M. Anstee, Vlad Ratziu, Stephen Harrison, Arun Sanyal, Jacob George, Susanne Beckebaum, David Orr, Giuseppe Mazzella, Victor Vargas, Lise Lotte Gluud, Rifaat Safadi, James Trotter, Jaideep Behari, David Sheridan, Muhammad Y. Sheikh, Martin Bonacci, Gail Cawkwell, Bruce Wong, Pierre Bedossa, Zachary Goodman, Mary Rinella, on behalf of the REGENERATE Study Investigators

"Durability of biochemical improvements through six years of open label treatment with obeticholic acid in patients with primary biliary cholangitis who did not achieve the POISE criteria" (FRI146)
Gideon M. Hirschfield, Marco Carbone, David E. Jones, Bettina E. Hansen, Andreas E. Kremer, Michael Trauner, Alexander Liberman, Elizabeth Smoot Malecha, Leigh MacConell

"Efficacy and tolerance of obeticholic acid in patients with primary biliary cholangitis and inadequate response to ursodeoxycholic acid in real life: interim analysis of the OCARELIFE study" (FRI180)
Vincent Leroy, Christophe Corpechot, Jérôme Dumortier, Laurent Alric, Dominique Larrey, Sébastien Dharancy, Olivier Chazouilleres, Alexandra

Heurgue, François Boer, Aldo Trylesinski

A full list of sessions at the Digital International Liver Congress™ 2020 is available at https://ilc-congress.eu.

About Liver Fibrosis due to NASH

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. Advanced fibrosis is associated with a substantially higher risk of liver-related morbidity and mortality in patients with NASH. In the United States, NASH is currently the second leading cause for liver transplantation overall, and in females, the leading cause. NASH is anticipated to become the leading indication for liver transplantation in Europe within the next decade. There are currently no medications approved for the treatment of NASH.

About the REGENERATE Study

REGENERATE is a Phase 3, randomized, double-blind, placebo-controlled, multicenter study assessing the safety and efficacy of obeticholic acid (OCA) on clinical outcomes in patients with liver fibrosis due to NASH. A pre-specified 18-month analysis was conducted to assess the effect of OCA on liver histology comparing month 18 biopsies with baseline. REGENERATE has completed target enrollment for the clinical outcomes cohort, with 2,480 adult NASH patients randomized over 300 qualified centers worldwide, and is expected to continue through clinical outcomes for verification and description of clinical benefit. The end-of-study analysis is designed to evaluate the effect of OCA on all-cause mortality and liver-related clinical outcomes, as well as its long-term safety.

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) and nonalcoholic steatohepatitis (NASH). Founded in 2002 in New York, Intercept 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.

About Primary Biliary Cholangitis

Primary biliary cholangitis (PBC) is a chronic, progressive liver disorder that mostly affects 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 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 the accelerated approval pathway 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. We are conducting a Phase 4 clinical outcomes trial, which we refer to as our COBALT trial, of OCA 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, conditioned upon us providing further data post-approval to confirm benefit. For detailed safety information for Ocaliva 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.

U.S. IMPORTANT SAFETY INFORMATION FOR OCALIVA IN PBC

WARNING: HEPATIC DECOMPENSATION AND FAILURE IN INCORRECTLY DOSED PBC PATIENTS WITH CHILD-PUGH CLASS B OR C OR DECOMPENSATED CIRRHOSIS

- **In postmarketing reports, hepatic decompensation and failure, in some cases fatal, have been reported in patients with Primary Biliary Cholangitis (PBC) with decompensated cirrhosis or Child-Pugh Class B or C hepatic impairment when OCALIVA was dosed more frequently than recommended.**
- **The recommended starting dosage of OCALIVA is 5 mg once weekly for patients with Child-Pugh Class B or C hepatic impairment or a prior decompensation event.**

Contraindications

OCALIVA is contraindicated in PBC patients with complete biliary obstruction.

Warnings and Precautions

Hepatic Decompensation and Failure in Incorrectly-Dosed PBC Patients with Child-Pugh Class B or C or Decompensated Cirrhosis

In postmarketing reports, hepatic decompensation and failure, in some cases fatal, have been reported in PBC patients with decompensated cirrhosis or Child-Pugh B or C hepatic impairment when OCALIVA was dosed more frequently than the recommended starting dosage of 5 mg once weekly. Reported cases typically occurred within 2 to 5 weeks after starting OCALIVA and were characterized by an acute increase in total bilirubin and/or ALP concentrations in association with clinical signs and symptoms of hepatic decompensation (e.g., ascites, jaundice, gastrointestinal bleeding, worsening of hepatic encephalopathy).

Routinely monitor patients for progression of PBC disease, including liver-related complications, with laboratory and clinical assessments. Dosage

adjustment, interruption or discontinuation may be required. Close monitoring is recommended for patients at an increased risk of hepatic decompensation. Severe intercurrent illnesses that may worsen renal function or cause dehydration (e.g., gastroenteritis), may exacerbate the risk of hepatic decompensation. Interrupt treatment with OCALIVA in patients with laboratory or clinical evidence of worsening liver function indicating risk of decompensation, and monitor the patient's liver function. Consider discontinuing OCALIVA in patients who have experienced clinically significant liver-related adverse reactions. Discontinue OCALIVA in patients who develop complete biliary obstruction.

Liver-Related Adverse Reactions

Dose-related, liver-related adverse reactions including jaundice, worsening ascites and primary biliary cholangitis flare have been observed in clinical trials, as early as one month after starting treatment with OCALIVA 10 mg once daily up to 50 mg once daily (up to 5-times the highest recommended dosage). Monitor PBC patients during treatment with OCALIVA for elevations in liver biochemical tests and for the development of liver-related adverse reactions.

Severe Pruritus

Severe pruritus was reported in 23% of PBC patients in the OCALIVA 10 mg arm, 19% of PBC patients in the OCALIVA titration arm, and 7% of PBC patients in the placebo arm in a 12-month double-blind randomized controlled trial of 216 PBC 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. Consider clinical evaluation of PBC patients with new onset or worsening severe pruritus. 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). Dose-dependent reductions from baseline in mean HDL-C levels were observed at 2 weeks in OCALIVA-treated PBC patients, 20% and 9% in the 10 mg and titration arms, respectively, compared to 2% in the placebo arm. Monitor PBC patients for changes in serum lipid levels during treatment. For PBC patients who do not respond to OCALIVA after 1 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 for PBC were pruritus, fatigue, abdominal pain and discomfort, rash, oropharyngeal pain, dizziness, constipation, arthralgia, thyroid function abnormality, and eczema.

Drug Interactions

Bile Acid Binding Resins

Bile acid binding resins such as cholestyramine, colestipol, or colesevelam adsorb and reduce bile acid absorption and may reduce the absorption, systemic exposure, and efficacy of OCALIVA. If taking a bile acid binding resin, take OCALIVA at least 4 hours before or 4 hours after taking the bile acid binding resin, or at as great an interval as possible.

Warfarin

The International Normalized Ratio (INR) decreased following coadministration of warfarin and OCALIVA. Monitor INR and adjust the dose of warfarin, as needed, to maintain the target INR range when coadministering OCALIVA and warfarin.

CYP1A2 Substrates with Narrow Therapeutic Index

Obeticholic acid, the active ingredient in OCALIVA, may increase the exposure to concomitant drugs that are CYP1A2 substrates. Therapeutic monitoring of CYP1A2 substrates with a narrow therapeutic index (e.g. theophylline and tizanidine) is recommended when coadministered with OCALIVA.

Inhibitors of Bile Salt Efflux Pump

Avoid concomitant use of inhibitors of the bile salt efflux pump (BSEP) such as cyclosporine. Concomitant medications that inhibit canalicular membrane bile acid transporters such as the BSEP may exacerbate accumulation of conjugated bile salts including taurine conjugate of obeticholic acid in the liver and result in clinical symptoms. **If concomitant use is deemed necessary, monitor serum transaminases and bilirubin.**

Please see Full Prescribing Information, including Boxed WARNING and Medication Guide for OCALIVA.

To report SUSPECTED ADVERSE REACTIONS, contact Intercept Pharmaceuticals, Inc. at 1-844-782-ICPT or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements, including, but not limited to, statements regarding the progress, timing and results of our clinical trials, including our clinical trials for the treatment of nonalcoholic steatohepatitis ("NASH"), the safety and efficacy of our approved product, Ocaliva (obeticholic acid or "OCA") for primary biliary cholangitis ("PBC"), and our product candidates, including OCA for liver fibrosis due to NASH, the timing and acceptance of our regulatory filings and the potential approval of OCA for liver fibrosis due to NASH, the review of our New Drug Application for OCA for the treatment of liver fibrosis due to NASH by the U.S. Food and Drug Administration (FDA), our intent to work with the FDA to address the issues raised in the complete response letter (CRL), the potential commercial success of OCA, as well as our strategy, future operations, future financial position, future revenue, projected costs, financial guidance, prospects, plans and objectives.

These statements constitute forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict,"

“project,” “target,” “potential,” “will,” “would,” “could,” “should,” “possible,” “continue” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this release, and we undertake no obligation to update any forward-looking statement except as required by law. These forward-looking statements are based on estimates and assumptions by our management that, although believed to be reasonable, are inherently uncertain and subject to a number of risks. The following represent some, but not necessarily all, of the factors that could cause actual results to differ materially from historical results or those anticipated or predicted by our forward-looking statements: our ability to successfully commercialize Ocaliva for PBC; our ability to maintain our regulatory approval of Ocaliva for PBC in the United States, Europe, Canada, Israel, Australia and other jurisdictions in which we have or may receive marketing authorization; our ability to timely and cost-effectively file for and obtain regulatory approval of our product candidates on an accelerated basis or at all, including OCA for liver fibrosis due to NASH following the issuance of the CRL by the FDA; any advisory committee recommendation or dispute resolution determination that our product candidates, including OCA for liver fibrosis due to NASH, should not be approved or approved only under certain conditions; any future determination that the regulatory applications and subsequent information we submit for our product candidates, including OCA for liver fibrosis due to NASH, do not contain adequate clinical or other data or meet applicable regulatory requirements for approval; conditions that may be imposed by regulatory authorities on our marketing approvals for our products and product candidates, including OCA for liver fibrosis due to NASH, such as the need for clinical outcomes data (and not just results based on achievement of a surrogate endpoint), any risk mitigation programs such as a REMS, and any related restrictions, limitations and/or warnings contained in the label of any of our products or product candidates; any potential side effects associated with Ocaliva for PBC, OCA for liver fibrosis due to NASH or our other product candidates that could delay or prevent approval, require that an approved product be taken off the market, require the inclusion of safety warnings or precautions, or otherwise limit the sale of such product or product candidate; the initiation, timing, cost, conduct, progress and results of our research and development activities, preclinical studies and clinical trials, including any issues, delays or failures in identifying patients, enrolling patients, treating patients, retaining patients, meeting specific endpoints in the jurisdictions in which we intend to seek approval or completing and timely reporting the results of our NASH or PBC clinical trials; our ability to establish and maintain relationships with, and the performance of, third-party manufacturers, contract research organizations and other vendors upon whom we are substantially dependent for, among other things, the manufacture and supply of our products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and our clinical trial activities; our ability to identify, develop and successfully commercialize our products and product candidates, including our ability to successfully launch OCA for liver fibrosis due to NASH, if approved; our ability to obtain and maintain intellectual property protection for our products and product candidates, including our ability to cost-effectively file, prosecute, defend and enforce any patent claims or other intellectual property rights; the size and growth of the markets for our products and product candidates and our ability to serve those markets; the degree of market acceptance of Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH or our other product candidates among physicians, patients and healthcare payors; the availability of adequate coverage and reimbursement from governmental and private healthcare payors for our products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and our ability to obtain adequate pricing for such products; our ability to establish and maintain effective sales, marketing and distribution capabilities, either directly or through collaborations with third parties; competition from existing drugs or new drugs that become available; our ability to prevent system failures, data breaches or violations of data protection laws; costs and outcomes relating to any disputes, governmental inquiries or investigations, regulatory proceedings, legal proceedings or litigation, including any securities, intellectual property, employment, product liability or other litigation; our collaborators’ election to pursue research, development and commercialization activities; our ability to establish and maintain relationships with collaborators with development, regulatory and commercialization expertise; our need for and ability to generate or obtain additional financing; our estimates regarding future expenses, revenues and capital requirements and the accuracy thereof; our use of cash and short-term investments; our ability to acquire, license and invest in businesses, technologies, product candidates and products; our ability to attract and retain key personnel to manage our business effectively; our ability to manage the growth of our operations, infrastructure, personnel, systems and controls; our ability to obtain and maintain adequate insurance coverage; the impact of COVID-19, including any impact on our results of operations or financial position, related quarantines and government actions, delays relating to our regulatory applications, disruptions relating to our ongoing clinical trials or involving our contract research organizations, study sites or other clinical partners, disruptions relating to our supply chain or involving our third-party manufacturers, distributors or other distribution partners, facility closures or other restrictions, and the extent and duration thereof; the impact of general U.S. and foreign economic, industry, market, regulatory or political conditions, including the potential impact of Brexit; and the other risks and uncertainties identified in our periodic filings filed with the U.S. Securities and Exchange Commission, including our Annual Report on Form 10-K for the year ended December 31, 2019 and our Quarterly Report on Form 10-Q for the quarter ended June 30, 2020.

Contact

For more information about Intercept, please contact:

Lisa DeFrancesco
+1-646-565-4833
investors@interceptpharma.com

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

Source: Intercept Pharmaceuticals, Inc.