



Intercept to Present Results from the First Successful Phase 3 Trial in Patients with Liver Fibrosis Due to NASH at the International Liver Congress™ 2019

April 4, 2019

Multiple additional abstracts evaluating obeticholic acid (OCA) for the treatment of patients with progressive non-viral liver diseases to be presented at the meeting

NEW YORK, April 04, 2019 (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 OCA abstracts will be presented at the International Liver Congress™ 2019, the 54th Annual Meeting of the European Association for the Study of the Liver (EASL), in Vienna, Austria from April 10, 2019 through April 14, 2019.

"We are thrilled to be presenting our groundbreaking Phase 3 REGENERATE results during the Opening Ceremony and General Session of the 2019 International Liver Congress, which promises to be a watershed event for the hepatology community," said Mark Pruzanski, M.D., President and Chief Executive Officer of Intercept. "In addition to REGENERATE, we will be presenting a late-breaking poster on a placebo-controlled study of OCA showing physiologic improvement of liver function in NASH patients with fibrosis, as assessed by the HepQuant quantitative liver function test. In PBC, we are looking forward to a late-breaking oral presentation of an investigator-initiated study which showed that the addition of bezafibrate to OCA improved both cholestasis and pruritus, underscoring the rationale for our plan to develop a fixed dose combination of these two agents."

Presentations at the International Liver Congress include:

Oral Presentations

General Session and Opening Ceremony Oral Presentation

Thursday, April 11, 2019 from 3:15 – 3:30 p.m. CEST

"Positive Results from REGENERATE: A Phase 3 International, Randomized, Placebo-Controlled Study Evaluating Obeticholic Acid Treatment for NASH" (Abstract GS-06)

Zobair Younossi, Vlad Ratziu, Rohit Loomba, Mary Rinella, Quentin M. Anstee, Zachary Goodman, Pierre Bedossa, Andreas Geier, Susanne Beckebaum, Philip Newsome, David Sheridan, James Trotter, Whitfield Knapple, Eric Lawitz, Kris Kowdley, Aldo Montano-Loza, Jerome Boursier, Philippe Mathurin, Elisabetta Bugianesi, Guiseppe Mazzella, Antonio Oliveira, Helena Cortez-Pinto, Isabel Graupera, David Orr, Lise Lotte Gluud, Jean-Francois Dufour, David Shapiro, Jason Campagna, Luna Zaru, Leigh MacConell, Reshma Shringarpure, Stephen Harrison, Arun Sanyal

Late-Breaker Oral Presentation

Saturday, April 13, 2019 from 5:00 – 5:15 p.m. CEST

"Bezafibrate improves the effect of obeticholic acid on cholestasis in patients with primary biliary cholangitis" (Abstract LB-05)

Lena Smets, Liliane Schrijvers, Hannelie Korf, Schalk Van der Merwe, Frederik Nevens

Poster Presentations

Late-Breaker Poster Presentation

Thursday, April 11, 2019 from 9:00 a.m. – 5:00 p.m. CEST

"Effect of Obeticholic Acid on Liver Function in Patients with Fibrosis due to NASH" (Abstract LBP-18)

Naim Alkhouri, Gregory Everson, Steve Helmke, Jianfen Chen, Carl LaCerte, Michael Stenkilsson, Jeffrey Edwards

General Poster Presentations

Friday, April 12, 2019 from 9:00 a.m. – 5:00 p.m. CEST

"Inadequate response to UDCA among PBC patients under routine care in the US: Rising serum bilirubin even in the normal range is a risk factor and subsequent clinical follow-up differs based on treatment response" (Abstract FRI-023)

Stuart C. Gordon, Talan Zhang, Christopher L. Bowlus, Keith Lindor, Carla V. Rodriguez, Robert J. Romanelli, Irina V. Haller, Heather Anderson, Jeffrey J. VanWormer, Joseph A. Boscarino, Mark A. Schmidt, Yihe G. Daida, Amandeep Sahota, Jennifer Vincent, Kuan-Han Hank Wu, Sheri Trudeau, Jia Li, Christina Melkonian, Lorelee B. Rupp, Mei Lu

"Long-term assessment of the effects of obeticholic acid in patients with primary biliary cholangitis on immune and inflammatory markers" (Abstract FRI-026)

Gideon M. Hirschfield, Frederik Nevens, Mitchell L. Shiffman, Joost P.H. Drenth, Christopher L. Bowlus, Victor Vargas, Pietro Andreone, Alexander Liberman, Elizabeth Smoot Malecha, Leigh MacConell, Michael Traunder

"Long-term obeticholic acid treatment is associated with improvements in collagen morphometry in patients with primary biliary cholangitis" (Abstract FRI-033)

Andreas E. Kremer, Christopher L. Bowlus, Pierre Bedossa, Albert Parés, Lisa M. Forman, Joost P.H. Drenth, Stephen Ryder, Luigi Terracciano, Yuying Jin, Alexander Liberman, Richard Pencek, Leigh MacConell, Paul J. Pockros

“Change in lipids: Characteristics and response to obeticholic acid in TARGET-PBC, a diverse, large United States real-world cohort” (Abstract FRI-037)

Cynthia Levy, Marlyn J. Mayo, Elizabeth J. Carey, Ester C. Little, W. Ray Kim, Karen Deane, Richard Zink, Robert Sandefur, Christopher L. Bowlus

“Primary biliary cholangitis – Autoimmune hepatitis overlap syndrome: Characteristics and response to obeticholic acid in TARGET-PBC, a diverse, large United States real-world cohort” (Abstract FRI-043)

Marlyn J. Mayo, Christopher L. Bowlus, Elizabeth J. Carey, Ester C. Little, Karen Deane, Richard Zink, Robert Sandefur, W. Ray Kim, Cynthia Levy

Saturday, April 13, 2019 from 9:00 a.m. – 5:00 p.m. CEST

“Evaluation of anti-fibrotic properties of OCA and INT-767 in an in vitro system of NAFLD” (Abstract SAT-337)

Beatrice Anfuso, Natalia Rosso, Luciano Adorini, Claudio Tiribelli

“Morphometric collagen analysis discerns anti-fibrotic effects of INT-767 and OCA in NASH mouse models using second harmonic generation imaging” (Abstract SAT-342)

Li Chen, Mathieu Petitjean, Luciano Adorini, Jonathan Roth

“Obeticholic acid reduces matrix metalloproteinases activity via iNOS modulation in hepatic ischemia/reperfusion injury” (Abstract SAT-389)

Laura G. Di Pasqua, Guisepina Palladini, Clarissa Berardo, Veronica Siciliano, Luciano Adorini, Plinio Richelmi, Massimiliano Cadamuro, Luca Fabris, Stefano Perlini, Mariapia Vairetti, Andrea Ferrigno

A full list of sessions at the International Liver Congress™ 2019 relating to OCA is available on the [International Liver Congress website](#).

Other Presentations

Intercept will host an investor event on Thursday, April 11, 2019 starting at 5:45 p.m. CET. During this event, management will review the REGENERATE data presented at the International Liver Congress™ 2019. Webcast information for this event will be available on the investor page of Intercept's website at <http://ir.interceptpharma.com>. Archived webcasts will be available on Intercept's website for approximately two weeks.

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, and as early as 2020, the disease is projected to become the leading cause of liver transplants in the United States. There are currently no medications approved for the treatment of NASH.

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 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. 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.

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](#).

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 development candidates, including OCA for NASH, the timing and acceptance of our potential regulatory filings and potential approval of OCA for NASH or any other indications in addition to PBC, the timing and potential commercial success of OCA and any other product candidates we may develop and our strategy, future operations, future financial position, future revenue, projected costs, financial guidance, prospects, plans, objectives of management and expected market growth.

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; 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, 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 timely and cost-effectively file for and obtain regulatory approval of our product candidates, including OCA for NASH, in the United States, Europe and our other target markets; conditions that may be imposed by regulatory authorities on our marketing approvals for our 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 contained in the label of any of our products or product candidates; any potential side effects associated with Ocaliva for PBC, OCA for 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; 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 NASH, and our clinical trial activities; our ability to identify, develop and successfully commercialize our products and product candidates, including our ability to timely and successfully launch OCA for 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 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 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, 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 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, 2018.

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