



## Intercept Announces New NASH and PBC Data to be Presented at The Liver Meeting® 2019

October 28, 2019

*New analyses from Phase 3 REGENERATE study evaluating the effects of OCA on non-invasive liver fibrosis tests and NASH patient-reported outcomes*

*Five-year analysis of long-term Ocaliva® treatment in patients with PBC selected for inclusion in 'Best of the Liver Meeting' abstracts*

NEW YORK, Oct. 28, 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 abstracts on obeticholic acid (OCA) will be presented at The Liver Meeting® 2019, the Annual Meeting of the American Association for the Study of Liver Diseases (AASLD), taking place from November 8, 2019 through November 12, 2019 in Boston, Massachusetts. Highlights include new data from the interim analysis of the ongoing pivotal Phase 3 REGENERATE study in patients with liver fibrosis due to nonalcoholic steatohepatitis (NASH) and a late-breaking oral presentation evaluating the long-term efficacy of Ocaliva in patients with primary biliary cholangitis (PBC).

"With two oral presentations in NASH, a late-breaking PBC presentation, numerous posters and an increasing body of research on OCA being presented by independent investigators, this is certainly an eventful Liver Meeting for Intercept," said Christian Weyer, M.D., M.A.S., Intercept's Executive Vice President, Research & Development. "As the first Phase 3 study to demonstrate fibrosis improvement in patients with NASH, REGENERATE continues to provide the medical community with important insights that we believe will help shape the care pathway for these patients. New analyses examining the effects of OCA on noninvasive measures of liver fibrosis and patient-reported outcomes are of particular clinical relevance because they provide a window into how OCA's efficacy and tolerability will be viewed in a real-world setting."

Select Intercept and independent presentations at The Liver Meeting include:

### Oral Presentations:

Sunday, November 10, 2019 – 8:30 a.m. ET

#### **"Obeticholic Acid Treatment in Patients with Nonalcoholic Steatohepatitis: A Secondary Analysis of the REGENERATE Study Across Fibrosis Stages" (Abstract # 0034)**

*Arun J. Sanyal, Vlad Ratziu, Rohit Loomba, Mary E. Rinella, Quentin M. Anstee, Zachary D. Goodman, Pierre Bedossa, Mandana Khalili, Jerome Boursier, Laura Stinton, Giulio Marchesini, Michael E. Allison, Jacob George, Perttu Arkkila, Luna Zaru, Leigh MacConell, Reshma Shringarpure, Zobair M. Younossi*

Sunday, November 10, 2019 – 10:30 a.m. ET

#### **"The Impact of Pruritus on Patient-Reported Outcomes (PROs) in Patients with Non-alcoholic Steatohepatitis (NASH) Treated with Obeticholic Acid (OCA)" (Abstract # 0056)**

*Zobair M. Younossi, Maria Stepanova, Fatema Nader, Rohit Loomba, Quentin M. Anstee, Vlad Ratziu, Stephen A. Harrison, Arun J. Sanyal, Jacob George, Susanne Beckebaum, David W. Orr, Giuseppe Mazzella, Victor Vargas, Lise L. Gluud, Rifaat Safadi, James F. Trotter, Jaideep Behari, David A. Sheridan, Muhammad Y. Sheikh, Gail Cawkwell, Bruce Wong, Pierre Bedossa, Zachary D. Goodman, Mary E. Rinella and on behalf of the REGENERATE Study Investigators*

### Late-Breaking Oral Presentation:

Monday, November 11, 2019 – 3:45 p.m. ET

#### **"Durable Response in the Markers of Cholestasis Through 5 Years of Open-Label Extension Study of Obeticholic Acid in Primary Biliary Cholangitis" (Abstract # LO6)**

*Frederik Nevens, Mitchell L. Shiffman, Joost PH Drenth, Christopher L. Bowlus, Victor Vargas, Pietro Andreone, Karel J. Van Erpecum, Alexander Liberman, Richard Pencek, Elizabeth Smoot Malecha, Leigh MacConell, Michael H. Trauner*

### Poster Presentations:

Friday, November 8, 2019 – 8:00 a.m. ET

#### **"The Economic Cost and Health Burden of Nonalcoholic Steatohepatitis in the EU5 Countries" (Abstract # 0395)**

*Philip N. Newsome, Jörn Schattenberg, Lawrence Serfaty, Alessio M. Aghemo, Salvador Augustin, Emmanuel A. Tsochatzis, Ali E. Canbay, Victor de Ledingham, Elisabetta Bugianesi, Manuel Romero-Gomez, Stephen D. Ryder, Heike Bantel, Jerome Boursier, Salvatore Petta, Javier Crespo, Laurent Castera, Vincent Leroy, Claude Le Pen, Frank-Ulrich Fricke, Rachel A. Elliott, Vincenzo Atella, Jorge Mestre-Ferrandiz, Lefteris Floros, Aleksandra Torbica, Alice Morgan, Sally Hartmanis, Aldo Trylesinski, Emily Stirzaker, Sharad Vasudevan, Lynne Pezzulo, Vlad Ratziu*

Saturday, November 9, 2019 – 2:00 p.m. ET

#### **"Effects of Obeticholic Acid on APRI and GLOBE Score in Patients with Primary Biliary Cholangitis" (Abstract # 1261)**

*Maren H. Harms, Gideon Hirschfield, Annarosa Floreani, Marlyn J. Mayo, Albert Parés, Alexander Liberman, Elizabeth Smoot Malecha, Richard Pencek, Leigh MacConell, Bettina E. Hansen*

#### **"Long-Term Outcomes of Patients with Advanced Fibrosis Due to Non-alcoholic Steatohepatitis (NASH) at Risk of Progressing to Cirrhosis**

**Under Standard of Care” (Abstract # 1217)**

Raluca Pais, William Green, Stuart Mealing, Aldo Trylesinski Sr., Sandrine Cure, Heather Davies

**“Obeticholic Acid Improves Hepatobiliary Secretion of Bile Acids in Patients with PBC” (Abstract # 1269)**

Kristoffer Kjærgaard, Kim Frisch, Ole L. Munk, Alan F. Hofmann, Michael Soerensen, Jacob Horsager, Anna C. Christina Schacht, Mary Erickson, David A. Shapiro, Susanne Keiding

**“Mortality Risk of Patients with Hepatic Decompensation from Primary Biliary Cholangitis in the Obeticholic Acid Era” (Abstract # 1290)**

Ajitha Mannalithara, W. Ray Kim, Pimsiri Sripongpan, Allison J. Kwong, Aparna Goel

Sunday, November 10, 2019 – 8:00 a.m. ET

**“Obeticholic Acid (OCA) Improves Non-Invasive Markers of Fibrosis in Patients with Non-alcoholic Steatohepatitis (NASH): A Secondary Analysis of the Phase 3 REGENERATE Study” (Abstract # 1715)**

Quentin M. Anstee, Stephen A. Harrison, Arun J. Sanyal, Vlad Ratziu, Mary E. Rinella, Zobair M. Younossi, Jerome Boursier, Sven M. Francque, Anja Geerts, Salvatore Petta, Elisabetta Bugianesi, Manuel Romero-Gomez, Jörn Schattenberg, Souvik Sarkar, Maurizio Bonacini, Maria Luisa Yataco, Michael K. Porayko, Asma Siddique, Jean-Francois Dufour, Tom Ferro, Aditya Venugopal, Luna Zaru, Reshma Shringarpure, Leigh MacConell, Zachary D. Goodman, Rohit Loomba

Monday, November 11, 2019 – 8:00 a.m. ET

**“Biliary and Plasma Bile Acid Profiling During Obeticholic Acid Treatment in Patients with Primary Biliary Cholangitis and Non-alcoholic Steatohepatitis and in Healthy Volunteers” (Abstract # LP16)**

Elsemieke De Vries, Jeffrey Edwards, Carl LaCerte, Mary Erickson, David A. Shapiro, Ulrich Beuers

**“Assessment of Patient-Reported Outcomes (PROs) in Patients with Non-alcoholic Steatohepatitis (NASH) Treated with Obeticholic Acid (OCA): Results from REGENERATE Phase 3 Clinical Trial” (Abstract # 2324)**

Zobair M. Younossi, Maria Stepanova, Fatema Nader, Rohit Loomba, Quentin M. Anstee, Vlad Ratziu, Stephen A. Harrison, Arun J. Sanyal, James F. Trotter, Muhammad Y. Sheikh, Aldo J. Montano-Loza, Antonio Olveria, Laura M. Stinton, David A. Sheridan, Victor Vargas, Lise L. Gluud, Rifaat Safadi, Jörn Schattenberg, Mandana Khalili, David W. Orr, Markus Peck-Radosavljevic, Michael E. Allison, Gail Cawkwell, Bruce Wong, Pierre Bedossa, Zachary D. Goodman, Mary E. Rinella and on behalf of the REGENERATE Study Investigators

**“Safety, Pharmacokinetics and Pharmacodynamics of Obeticholic Acid in Patients with Nonalcoholic Steatohepatitis and Fibrosis or Cirrhosis” (Abstract # 2294)**

Naim Alkhouri, Fred Poordad, Eric J. Lawitz, Jason Boyd, Jeffrey Edwards

A full list of sessions at The Liver Meeting is available on the AASLD website at: <https://www.aasld.org/event/liver-meeting>.

**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](http://www.interceptpharma.com) or connect with the company on [Twitter](#) and [LinkedIn](#).

**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 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 more than 2,400 adult NASH patients randomized across 339 qualified centers worldwide, and will continue through clinical outcomes for verification and description of clinical benefit. The end-of-study analysis will evaluate the effect of OCA on all-cause mortality and liver-related clinical outcomes, as well as its long-term safety.

**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](http://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](http://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 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, 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 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.

## **Contact**

For more information about Intercept, please contact:

Lisa DeFrancesco  
+1-646-565-4833  
[investors@interceptpharma.com](mailto:investors@interceptpharma.com)

Christopher Frates  
+1-646-757-2371  
[media@interceptpharma.com](mailto:media@interceptpharma.com)



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